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More than mobility: applying health economics to wheelchair interventions for disabled children

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More than mobility: Applying health economics to wheelchair interventions for disabled children

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Dedication

I would like to dedicate this thesis to Grandpa John, Teddy and Raphael.

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Thesis Summary (300 words)

Wheelchairs provide a wide range of benefits to disabled children, including positive health, developmental and social outcomes. National and international reports have highlighted the need for improved access to wheelchairs for disabled children. Health economics could have an important role in ensuring that the most cost-effective equipment is provided to children. Due to a lack of economic evidence this is not currently possible. NICE cannot provide clear and reliable guidance to the NHS without appropriate evidence of effectiveness and cost-effectiveness. NICE recommend the QALY as their primary measure of choice. It is therefore paramount to understand how to apply health economics methods of evaluation to wheelchair interventions.

The overall aim of this thesis was to explore the application of health economics to wheelchair interventions for disabled children, and to understand how best to evaluate the cost-effectiveness of wheelchair interventions for children. A number of techniques were used, including: a mixed-method systematic review (chapter three); a wheelchair costing case study (chapter four); a pilot discrete choice experiment to understand wheelchair service user priorities (chapter five); a quantitative statistical analysis of health-related quality of life outcome measures (chapter six); and a qualitative explorative study of how disabled children define quality of life (chapter seven).

This thesis represents the first academic application of health economics to wheelchair interventions for disabled children. The use of generic preference-based HRQoL outcome measures may not be suitable in disabled children, particularly younger children. Future research should ensure use of mixed methods, including qualitative methods. If the use of QALYs is continued in this setting, more robust and sensitive methods of utility data collection are needed. Alternatively the capability approach could be used in place of QALYs. Evidence of cost-effectiveness has the potential to promote reform of NHS wheelchair services and subsequently improve outcomes for disabled children.

List of abbreviations

ASBI-	Adaptive Social Behaviour Inventory
ASSIA-	Applied Social Sciences Index and Abstracts
BDI-	Battelle Developmental Inventory
BIME-	Bath Institute of Medical Engineering
CASP-	Critical Appraisal Skills Programme
CBA-	Cost-Benefit Analysis
CCA-	Cost-Consequence Analysis
CEBMa-	Center for Evidence-Based Management
CEA-	Cost-Effectiveness Analysis
CHU-9D-	Child Health Utility 9 Dimension
CI-	Confidence Interval
CL-	Confidence Limit
CINAHL-	Cumulative Index to Nursing and Allied Health Literature
CMA-	Cost-Minimisation Analysis
COPM-	Canadian Occupational Performance Measure
COREQ-	Consolidated Criteria for Reporting Qualitative Studies
CSIP-	Care Services Improvement Partnership
CUA-	Cost-Utility Analysis
DARE-	Database of Abstracts of Reviews of Effects
DCE-	Discrete Choice Experiment
DES-	Department for Education and Skills
ECI-	Early Coping Inventory

EPIOC-	Electric Powered Indoor/Outdoor Chair
EPPI-centre-	Evidence for Policy and Practice Information and Co-ordinating Centre
EQ-5D-	EuroQol 5 Dimension
GMFM-	Gross Motor Functional Measure
HRQoL-	Health-related quality of life
HTA-	Health Technology Assessment
HUI-	Health Utilities Index
ICECAP-	Icepop Capability measures
ICIS-	Impact of Childhood Illness Scale
MATCH-	Matching Assistive Technology & Child
MAUF-	Multi-Attribute Utility Function
MEDLINE-	Medical Literature Analysis and Retrieval System Online
MeSH-	Medical Subject Headings
MRS-	Marginal Rate of Substitution
MWC-	Manual Wheelchair
NFPO-	Not for profit organisation
NHS-	National Health Service
NHS EED-	National Health Service Economic Evaluation Database
NICE-	National Institute for Health and Care Excellence
OT-	Occupational Therapist
PBMA-	Programme Budget Marginal Analysis
PEDI-	Pediatric Evaluation of Disability Inventory
PedsQL-	Pediatric Quality of Life Inventory

PSSC-	Parental Stress and Support Checklist
PSSRU-	Personal Social Services Research Unit
PIQ-	Performance Intelligence Quotient
PKBS-	Preschool and Kindergarten Behaviour Scales
PLS-3-	Preschool Language Scale-3
PPVT-	Peabody Picture Vocabulary Tests
PWC-	Powered wheelchair
QALY-	Quality adjusted life year
QoL-	Quality of life
QUEST-	Quebec User Evaluation of Satisfaction with assistive Technology
RCT-	Randomised controlled trial
SD-	Standard Deviation
SF36-	Short Form 36
SMD-	Social Model of Disability
SS-	Sample size
TTO-	Time-Trade Off
UK-	United Kingdom
VAS-	Visual Analogue Scale
VIQ-	Verbal Intelligence Quotient
WHO-	World Health Organisation
WK-	Whizz-Kidz (wheelchair charity for children)
York CRD-	University of York Centre for Research and Dissemination

Terminology

To maintain clarity, I define a number of key concepts and terminology used throughout this thesis as follows:

- The term "disabled child" is used to refer specifically to any physically disabled person aged 18 or under who uses a wheelchair to aid mobility. The term "child" is also used to refer to a "disabled child" where the definition is obvious.
- The term "wheelchair service" is used to define any private, state or not-for-profit organisation (NFPO) run service supplying wheelchairs to disabled people based upon assessment of mobility needs by a qualified professional.
- The term "wheelchair provision" is used to define the supply of a wheelchair intervention to a disabled person by a wheelchair service (as defined above).
- The term "wheelchair intervention" is used to define any wheelchair supplied to a disabled person by a wheelchair service (as defined above).
- The term "effectiveness" refers to all relevant clinical and non-clinical outcomes related to wheelchair use, such as (but not restricted to): cognitive, physical and behavioural development; functional mobility and motor skills; independence; educational achievement; social interaction; initiative development; physical and/or emotional wellbeing; and health-related quality of life. "Effectiveness" is not used to refer to biomechanical outcomes, such as propulsion patterns.

Chapter One: Introduction

1.1. Chapter summary

This chapter outlines the aims and objectives of the thesis, followed by a detailed introduction to the topic area. This includes: an exploration of the context of wheelchair provision for disabled children in the UK; the principles and theoretical background of health economics and economic evaluation in healthcare; the role of the social model of disability in health economics; the need for economic analysis in wheelchair provision; and how health economics methods can be applied in this context. I will also outline the structure of the thesis and give a brief overview of the content of each chapter.

1.2. Rationale for thesis

In 2010 the National Assembly for Wales Health, Wellbeing and Local Government Committee published findings from their inquiry into National Health Service (NHS) wheelchair service in Wales (National Assembly for Wales, 2010). This inquiry was in response to a large number of service user complaints and charity reports regarding the state of wheelchair services in Wales. One of the key messages from this report was that wheelchair services were not currently meeting the needs of children due to a range of issues including long waiting times, focus on clinical needs and strict eligibility criteria. This was not the first government report to make such recommendations; in fact the issues surrounding wheelchair services in England and Wales have been ongoing for many years (Audit Commission, 2002; NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2005). What was apparent from these reports was that services were limited by tight budgets, and that there was a need for an evidence-based approach to eligibility criteria and provision. Having previously worked with disabled children in education and clinical research settings, I found it fascinating and alarming that the provision of essential assistive technologies could be fraught with so many issues.

At the time the National Assembly for Wales report was published I was working as a Research Officer for a regional research network and completing my Masters in Public Health and Health Promotion at Bangor University. I had recently completed modules in health economics, which I found to be a captivating topic area. Studying health economics gave me an entirely different perspective on the value and cost of health and the need for appropriate systems of healthcare financing. I had therefore decided that health economics was an area of research that I wanted to pursue. I was invited by my current supervisors to carry out a health economics PhD research programme inspired by the issues raised in the National Assembly for Wales report. I was named as the student applicant on a successful NISCHR PhD studentship bid, which I subsequently started in early 2011. Considering my past experience in clinical research and working with disabled children,

this was an excellent opportunity for me to explore an interesting, topical area of research and to develop my skills personally and professionally. The application of health economics to wheelchair provision was particularly relevant for me because of my growing interest in health economics and my experience in paediatric research.

The lack of National Institute for Health and Care Excellence (NICE) Health Technology Assessment (HTA) guidance on wheelchair interventions for children highlighted both the need for evidence and the lack of attention given to this area of research. Due to the influence of NICE and quality-adjusted life year (QALY) estimates, there was a need to understand how to apply health economics in this setting in order to bridge the gap in knowledge between the needs of disabled children and the finite budgets of wheelchair services. I therefore pursued this PhD research programme with the intention of examining how health economics could be applied in this unique setting, and with a desire to develop the evidence base to guide service development to meet the needs and enhance the outcomes of disabled children.

The PhD programme of research was designed around understanding how to conduct cost-effectiveness analysis in this setting (both in terms of appropriate costing and outcome measurement) and understanding service user needs and preferences. This influenced the methods used in the separate studies; firstly a mixed-method systematic review was undertaken to understand the topic area from a number of complementary view points; secondly, a costing case-study was conducted to understand how best to cost wheelchair interventions for children for the purpose of economic evaluation; thirdly a discrete choice experiment (DCE) was conducted to understand service user needs and preferences for service attributes; and finally analysis of the applicability and reliability of standard health-related quality of life (HRQoL) measures was examined, both quantitatively and qualitatively, to understand if standard approaches to outcome measurement for the purpose of cost-effectiveness analysis could be applied in this setting. The culmination of these different methods was a multi-faceted examination of the application of health economics in this specific setting.

1.3. Aims of the thesis

The overall aim of this thesis is to explore the application of health economics to wheelchair interventions for disabled children, and specifically to understand how best to apply methods of economic evaluation to the assessment of cost-effectiveness of wheelchairs and wheelchair services for disabled children. A number of thesis objectives were used to fulfil this aim:

- Through a mixed-method systematic review explore effectiveness evidence, service user perspectives, current policy and economic evidence relating to wheelchairs for disabled children, in order to develop a conceptual framework to inform future research and wheelchair service development (chapter three).
- Examine the costs associated with the supply of a wheelchair to a disabled child (chapter four).
- Undertake a pilot DCE to explore families' views on the most important attributes of wheelchair services (chapter five).
- Assess the appropriateness of the EQ-5D-Y and HUI HRQoL outcome measures for eliciting accurate HRQoL estimates from disabled children (and their parents by proxy) (chapter six).
- Examine how HRQoL is defined by disabled children and their parents, and how it can best be measured for the purpose of economic evaluation (chapter seven).

This PhD thesis was funded by the NISCHR social care PhD studentship award. NISCHR is a Welsh government body that guides NHS and social care research in Wales, aiming to improve health and healthcare today and in the future. In order to address the aims of this thesis, a programme of research was developed called the Wheels Project. Complementary data collection methods were used, including a DCE, measurement of HRQoL, and qualitative semi-structured interviews.

1.4. Research questions and objectives

Chapter three: What evidence, relating specifically to disabled children, currently exists regarding wheelchair effectiveness, wheelchair service user perspectives, current policy guidance and cost-effectiveness? Objectives:

- To establish what evidence currently exists regarding the effectiveness of wheelchairs in terms of clinical, social, educational and developmental benefits for disabled children.
- To establish what evidence currently exists regarding the perceived barriers and facilitators of providing and using wheelchairs for disabled children, taking into account the different perspectives of disabled children, parents/carers, and healthcare professionals.
- To gather current policy, not-for-profit organisation publications and clinical guidelines regarding wheelchair provision for disabled children.
- To establish what evidence currently exists regarding the costs, economic implications and incremental benefits of wheelchair interventions for disabled children.

- To understand the extent to which intervention study outcomes and policy recommendations reflect the barriers and facilitators of wheelchair use (expressed in opinion evidence).
- To build a conceptual framework mapping areas for future research and service development to facilitate cost-effective wheelchair services for disabled children.

Chapter four: What are the costs associated with the provision of a wheelchair to a disabled child, taking into account base wheelchair costs, customisation, maintenance, staff time and overheads? Objectives:

- To compare the relative wheelchair and customisation costs for different types of wheelchairs.
- To estimate staff time and costs associated with the provision of a wheelchair.
- To examine theoretical cost savings associated with recycling wheelchairs.

Chapter five: How do disabled children and their parents prioritise different attributes of wheelchair services? Objectives:

- To compare the preferences of disabled children and their parents for different attributes of wheelchair services.
- To calculate hypothetical marginal rate of substitution values for different configurations of wheelchair services using cost-contribution as the denominator.
- To evaluate the use of DCE methods in disabled children in relation to wheelchair services.

Chapter six: Are generic preference-based HRQoL outcome measures, such as the EQ-5D-Y and HUI2/3, appropriate for eliciting accurate utility estimates from disabled children (and their parents by proxy)? Objectives:

- To compare the HRQoL results of disabled children and their parents by proxy.
- To assess correlation between the EQ-5D-Y and HUI measures, and respondent type (child and parent proxies).
- To assess the construct validity of the EQ-5D-Y and HUI measures, with consideration of validity between measures and respondent type (child and parent proxies).
- To assess the agreement between the EQ-5D-Y and HUI measures, and respondent type (child or parent proxies).

Chapter seven: How do disabled children and their parents define quality of life (QoL) in relation to wheelchair use, and to what extent do the EQ-5D-Y and HUI measures reflect their opinions?
Objectives:

- To understand the key domains of QoL defined by disabled children and their parents in relation to wheelchair use and mobility impairment.
- To examine differences in how disabled children and parents define QoL in relation to wheelchair use and mobility impairment.
- To explore the extent to which generic preference-based HRQoL measures, such as the EQ-5D-Y and HUI2/3, reflect how disabled children and their parents define HRQoL in relation to wheelchair use.

1.5. Thesis structure

I will address the thesis questions and objectives across seven additional chapters, five of which present empirical research conducted and written during the process of this PhD studentship.

Chapter two: A summary of common methods for chapters 4-7, including a breakdown of recruitment strategy, study setting, ethical considerations and data collection methods.

Chapter three: A mixed-method systematic review of effectiveness evidence, service user perspectives, policy guidance and cost-effectiveness evidence. The findings were synthesised across four streams of evidence in order to develop a conceptual framework to inform future research and wheelchair service development.

Chapter four: A costing case study to assess the costs associated with the supply of a wheelchair to a disabled child, taking into account capital and operational costs. The costs were annuitised over the expected length of life of the wheelchair and sensitivity analysis performed to account for difference in length of viable wheelchair use and number of recycles.

Chapter five: A pilot DCE to determine how wheelchair service users view the relative importance of different attributes of wheelchair services. The preferences of disabled children and their parents regarding wheelchair services were obtained through a pilot DCE and analysed in relation to current policy intentions and government guidance.

Chapter six: A range of statistical analyses were undertaken to assess the appropriateness of the EQ-5D-Y and HUI2/3 instruments for eliciting accurate utility estimates from disabled children and their parents by proxy. Spearman's rank was used to assess correlation between

respondent types (child and parent) and measures, while Bland-Altman plots were used to assess agreement.

Chapter seven: A qualitative explorative study of how disabled children and their parents (by proxy) define QoL in relation to wheelchair use. The appropriateness of existing HRQoL measures (EQ-5D-Y and HUI2/3) is explored within this context, and the domains of health and QoL identified by participants are used to understand how best to measure HRQoL in this population and mapped on to existing capability approaches as an alternative to utility measurement.

Chapter eight: A synthesis of the results from the previous five empirical chapters, including a summary of all findings which specifically address the research objectives. I make methodological recommendations for the design of future health economics studies in wheelchair provision for disabled children, and recommendations for future wheelchair commissioning.

1.6. Understanding the context of wheelchair provision for disabled children

1.6.1. Disability prevalence

It is estimated that between 10% and 15% of the world's population live with some form of disability (World Health Organization, 2008a; 2011). One in ten disabled people require a wheelchair to provide essential mobility assistance (Sheldon and Jacobs, 2007); thus an estimated 1% of the global population require a wheelchair to maintain mobility. Approximately 5% of children worldwide (around 95 million children aged 14 or under) have a disability (World Health Organization, 2008b).

1.6.2. Need for assistive technologies for mobility

Access to appropriate mobility equipment is a worldwide issue, particularly in low-income countries (World Health Organization, 2008c). It is estimated that 20 million people worldwide do not have access to appropriate wheelchair equipment to maintain mobility and independence (World Health Organization, 2008c). Disabling barriers include lack of adequate policy, services and funding (World Health Organization, 2011), which limit appropriate supply of essential wheelchairs. Currently there is inadequate evidence to facilitate appropriate service provision and support for disabled people (World Health Organization, 2011). This relates to both understanding of intervention effectiveness/cost-effectiveness and estimates of disability prevalence. Health economics has the potential to lead dynamic and widespread positive change in wheelchair services, both in the UK and

internationally, however there is a current lack of evidence on how to apply health economics in this field.

Independent mobility for disabled people and provision of equipment to facilitate this is considered a human right, with calls for all countries to ensure that disabled people are able to access essential equipment to promote mobility and independence (United Nations, 1993). Without adequate wheelchair provision many disabled people are caught in a cycle of poverty and deprivation, lacking the ability to access education, work and social facilities (World Health Organization, 2008c). Disabled people are more likely to be unemployed than non-disabled people, and when employed tend to earn less (World Health Organization, 2011). These issues also have national economic impacts due to loss of productivity and health service resource use (World Health Organization, 2011).

Children require wheelchairs and other assistive mobility technologies for a variety of different disabilities and conditions, including cerebral palsy, muscular dystrophy, spinal muscular atrophy, traumatic brain injury and spina bifida. Each disabled child will have different clinical needs to consider, such as postural, pelvic and head/neck support. For instance, children with cerebral palsy have the greatest need for specialised seating systems, while children with spina bifida have the highest demand for pressure management systems (Lau et al, 2008). All of these different clinical issues must be considered alongside the mobility needs of each child. Wheelchairs offer essential mobility to children who are unable to walk independently or have limited mobility; therefore they must be suitable for use in all places the child desires to use them, such as at school, home and leisure facilities (Welsh Assembly Government, 2005). In some cases children require more than one type of assistive technology or wheelchair to fully realise their mobility potential, for instance children with cerebral palsy can require both a manual and powered wheelchair as their needs may be different in different situations (e.g. at school and at home) (Rodby-Bousquet & Hägglund, 2010).

Other assistive mobility interventions, such as walkers and leg braces, offer a range of clinical and holistic benefits for disabled children, however wheelchairs offer a unique perspective for health economists due to the level of customisation/adaptation available; the economic burden of these relatively expensive NHS interventions; and the vast range of outcomes. Furthermore, issues with wheelchair services in the UK have been well documented over the course of many years without significant change to standards, highlighting a real need for evidence to guide service development.

1.6.3. Wheelchair provision for disabled children in the UK

In the UK there are an estimated 770,000 disabled people under the age of 16 (Contact a Family, 2011), approximately 70,000 of whom have unmet mobility needs (Whizz-Kidz[WK], 2011). Providing the right wheelchair at the right time can offer a range of holistic benefits for disabled children and young people (Muscular Dystrophy Campaign, 2010), for instance functional mobility improvement (Jones et al, 2003); psychosocial development (Furumasu et al, 2008); development of communication skills (Butler, 1986; Jones et al, 2003; Jones et al, 2012); increased independence (Wiart et al, 2004; Jones et al, 2003); reduction in challenging behaviours (Furumasu et al, 2008) and better quality QoL through reduced pain and deformity (Tefft et al, 2011). In some circumstances powered mobility devices offer the only opportunity for independent movement to severely disabled children, which in turn allows participation in activities and sports which promote emotional and physical development (Department of Health [DoH], 2004).

Wheelchairs are more than a clinical intervention or a mode of transport; they give disabled children new opportunities and a new lifestyle. These in turn can feedback into the health of the child due to the wide variety of developmental benefits facilitated by independent movement and social interaction (Durkin, 2009). Due to the wide variety of beneficial outcomes elicited by correct wheelchair provision, it is important that wheelchair services adopt a holistic approach to wheelchair provision and assessment in order to maximise the potential of each disabled child (DoH Commissioning Team, 2010).

In response to a large number of service user complaints and reports from leading disabled children's charities, a national inquiry into NHS wheelchair services in Wales was launched in 2010 (National Assembly for Wales, 2010). Welsh NHS wheelchair services were not providing adequate mobility equipment to enable children to lead fulfilled lives. A number of recommendations were subsequently reported, including reduced waiting times (particularly for children with complex needs); adopting a holistic approach to assessment and provision (e.g. consider social, educational and developmental needs); and development of review procedures and information provision. This was not the first inquiry into NHS wheelchair service, in fact a number of previous government reports have published similar recommendations (Audit Commission, 2002; NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2005). The rise in charities such as WK, who provide wheelchairs to disabled children both in and outside of the NHS, demonstrates that NHS services are still not meeting the needs of all disabled children. This is an issue which has been ongoing for many years, and yet there is still a distinct lack of evidence in this field.

Limited budgets and strict eligibility criteria place restrictions on what equipment can be provided to disabled children by NHS wheelchair services. This restrictive approach to provision highlights the issues faced by NHS services, as they attempt to balance the needs of each individual whilst maintaining a minimum standard of care for all. The reality is that NHS services are often unable to provide the most appropriate equipment to children with complex needs. Disabled children and their families are then forced to pay for equipment privately or to approach charities to help raise funding. Funding issues and strict eligibility criteria, particularly for powered wheelchairs (PWCs), are therefore limiting disabled children's access to essential equipment, regardless of their individual needs (Sanderson et al, 2000).

1.6.4. The case for powered mobility for young children

In 1996 the UK government initiated the wheelchair voucher scheme to improve service choice and to provide financial aid to disabled people choosing to buy a wheelchair privately (Sanderson et al, 2000). The voucher scheme allowed wheelchair users to receive a voucher to the value of the NHS wheelchair they had been offered and use that to help pay for a wheelchair privately. Additionally, the UK government funded a specific Electrically Powered Indoor/Outdoor Chair (EPIOC) initiative in order to increase provision of essential powered mobility equipment to people with severe disabilities. However, due to strict eligibility criteria regarding safe use of EPIOCs, children still face restrictions in receiving powered mobility equipment (Sanderson et al, 2000; WK, 2011).

It is still common practice for NHS services to restrict provision of powered mobility equipment to children under the age of five due to safety concerns (WK, 2011). This is contrary to evidence which demonstrates the wide ranging benefits of early independent movement (Furumasu et al, 2008; Bottos et al, 2001; Jones et al, 2003). In circumstances where a PWC is not clinically suitable for a young child transitional powered riding toys offer an appropriate alternative to encourage independent movement (Tefft et al, 1999), however due to budget limitations few NHS services are able to provide such equipment. The use of strict eligibility criteria has the potential to limit beneficial supply of equipment, particularly when attributes such as age are used to arbitrarily restrict access (Barnardos & WK, 2006).

By exploring how best to measure the costs and effectiveness of wheelchairs and other assistive technologies, health economics can help to determine which interventions are a cost-effective method of improving the QoL and health of disabled children. Furthermore, by assessing a range of different types of wheelchairs and other assistive technologies, services could focus on those interventions with the most favourable cost-effectiveness evidence. If certain interventions are not

found to be cost-effective then other means of promoting independent mobility and postural support could be prioritised if favourable evidence was available. Appropriate application of health economics techniques and principles has the potential to encourage provision of the most cost-effective equipment to all disabled children who need it. This evidence could be used to further develop the economic toolbox and promote evaluation of technologies to support children (and adults) living with disability, a topic area which is currently lacking evidence.

1.6.5. The need for change in wheelchair services for children in the UK

Wheelchairs can be a relatively expensive intervention for the NHS, with costs for equipment at their highest in the 0-15 age group (Bamer et al, 2010). NHS wheelchair services often struggle to supply the most appropriate equipment to each disabled child due to issues with inefficiency, funding, waiting lists and eligibility criteria (WK, 2011). Inefficient services can lead to additional (but essentially avoidable) expenditure due to time-consuming processes and complex procurement strategies (Sanderson et al, 2000). Previous reports have recommended the integration of services between health, social care, education, voluntary and charitable organisations to promote better services through joint funding and provision (Prime Minister's Strategy Unit, 2005; Audit Commission, 2002).

Many disabled children and their families approach charities to help fund wheelchairs because they haven't been able to obtain the equipment they need through NHS services, often due to funding or eligibility issues. This raises some contentious issues regarding the remit of wheelchair services and the level of care they provide. The WK partnership with NHS wheelchair services demonstrated that improvements can be made through partnership; through joint working these services were able to improve provision, outcomes and waiting times whilst reducing costs (Frontier Economics, 2011). However, the applicability of this approach to all NHS wheelchair services is likely to be limited as organisations such as WK do not have the scope to partner with all services in the UK. Therefore additional evidence is needed to improve the provision of wheelchairs to disabled children.

In order to improve services evidence-based decision-making is needed. This in turn requires the development of robust estimates of effectiveness via validated clinical outcome measures (DoH, 2008). If appropriate preference-based measures were available cost per QALY estimates for wheelchairs and other forms of assistive technology could be generated and used to help prioritise different types of interventions and guide provision. At present specific outcome measures for children are currently limited and even more so for disabled children. NICE recommends the QALY as a primary outcome measure (NICE, 2013); the QALY represents an aggregate of quantity and

quality of life (Stevens and Palfreyman, 2012). The current accepted threshold for a QALY is between £20,000 to £30,000. To date NICE have not published any economic evidence or guidance relating specifically to wheelchairs for children, reflecting the difficulty of applying traditional health economics methods of economic evaluation to disabled children.

1.6.6. Personal and economic impact of disability

Disabled people are half as likely to have a degree compared to non-disabled people (Evans, 2007). Although the UK Government currently provides extra financial help for disabled people in education, disabled people face additional barriers when attempting to enter the workforce (DirectGov, 2014).

Disabled people represent a fifth of the working age population (Disability Rights Commission, 2006). Less than 50% of registered disabled people of working age are employed (Evans, 2007). It has been estimated that raising the employment rate of disabled people to that of non-disabled people would boost the economy by £13 billion (Evans, 2007). Schemes such as the government funded Access to Work scheme meet any additional employment costs related to disability, but crucially, only once a disabled person has secured a job. It is estimated that family carers save the NHS £119 billion every year (Carers UK, 2011). A 1% change in the number of carers or time spent caring would cost the UK £1billion in extra care costs (Carers UK, 2011).

There are considerable extra costs associated with raising a disabled child, and parents frequently find it difficult to manage their child's care whilst one or both continue in paid employment (Yeandle et al, 2007). There is a 'new' generation of sandwich carers who are increasingly caring for children and parents, and women in particular are sacrificing employment opportunities and pension contributions to provide long-term care to family members (Yeandle et al, 2007). Disability increases the likelihood of poverty through increased expenditure, reduced income, lifelong caring and inequitable opportunities to contribute to pensions.

Around 30% of disabled people live in relative poverty, compared to around 16% of non-disabled people (Leonard Cheshire Disability, 2008). 17% of families with a disabled child are going without food, 21% are going without heating and 26% are going without specialist equipment or adaptations (Contact a Family, 2012). It has been suggested that disability poverty is the missing link in efforts to tackle relative poverty in the UK (Leonard Cheshire Disability, 2008). Poverty here takes a wider definition including poverty of opportunity and lifetime aspiration (Leonard Cheshire Disability, 2008).

1.7. The principles of health economics

1.7.1. Healthcare as an economic good

Health economics has developed as a discipline related to but distinctly different from economics. The general principles of economics apply to health economics but are differentiated by the unique nature of healthcare as a good or service. Health economics is not simply the application of economic theory to healthcare; it is defined by separate theoretical and analytical backgrounds and methods specific to health and healthcare (Morris et al, 2007). It embraces the uncertainty of our lifetime demand for healthcare in order to better understand the economic implications of healthcare (Fuchs, 1996). The general public perception of health economists can often be negative (seen as 'cost-cutters'), but in reality the role of health economists (and economists more generally) is to optimise outcomes from finite resources (McCrone, 1998), and, as dispassionate analysts, present a range of options for resource use and related opportunity costs.

Economics is about understanding what society produces, how it is produced and for whom it is produced. There are two broad economic perspectives: positive and normative economics. Positive economics is concerned with facts, relationships and the description of economic phenomena. Normative economics is concerned with value judgments about economic fairness, equity and what should be the focus of economic policy and outcomes. Health economics is concerned with applying economic theory and techniques to health and healthcare, with the aim of informing policy and aiding health related decision-making for individuals, healthcare providers, governments and insurers (Morris et al, 2007). Both positive and normative economic perspectives are employed in health economics.

The basics of economic theory lie in the principle of competitive markets. Competitive markets contain free sellers providing goods to free buyers. Goods require resources, such as raw materials, labour, skills and equipment. Market equilibrium is achieved when the quantity of goods produced meets the total quantity of goods required by the consumer, and an exchange price for the goods is mutually accepted by the seller and the consumer (Brazier et al, 2007). Economists theorise that in a free market this equilibrium will be achieved naturally without outside influence from regulators (Brazier et al, 2007). Pareto efficiency is reached when there is no wastage and all parties are equally satisfied with the market, likewise no change can be made to improve the situation for one party without worsening the situation for another. A related concept is Pareto improvement, which is defined as a reallocation of resources that either increases the utility of all members of an economy, or at the least benefits some without detrimentally affecting others (Drummond & McGuire, 2001).

In an ideal market there is no need for economic evaluation as all parties are equally satisfied, but in reality achieving such equilibrium is impossible in a healthcare setting. Healthcare fails to be a perfect market due to market failure, thus government intervention is necessary. Most countries fund their healthcare system through tax and/or insurance. There are three main reasons market failure occurs in healthcare:

1. Lack of certainty- In general people do not know when they will become ill, and thus the healthcare services they require cannot be predicted. Furthermore, the benefits gained from a health service are not certain or equal between individuals (Arrow, 1963). This gives rise to healthcare insurance, which can lead to issues of consumer moral hazard such as reduced risk avoidance and relative overconsumption of services and resources (Brazier et al, 2007).
2. Lack of information symmetry- Within a healthcare market the consumer (the patient) is not independent from the supplier (the doctor, for instance). The doctor acts simultaneously as an agent and supplier of healthcare, and furthermore has more information about the illness and possible healthcare interventions available to the patient. Traditionally, the patient must delegate choice to the doctor as they lack the knowledge to make their own treatment choices, thus knowledge is a commodity when there is uncertainty (Arrow, 1963). This can lead to supplier moral hazard in the form of supplier-induced demand. There is also a lack of symmetry of information between insurers and patients, as insurers examine illness risk from a whole distribution perspective, while individuals only observe their own risk (Brazier et al, 2007).
3. Externalities- Positive externalities refer to impacts on others due to consumption by an individual. A clear example being the case of vaccinations reducing the spread of communicable illness to others (Culyer, 1971).

Within the discipline of health economics, defining market forces and equilibrium has practical and philosophical issues. Health and healthcare can be defined as a good or a service. Resources (or inputs) are scarce but are needed to produce a good or service (output). These scarce resources may be personnel, equipment, facilities, knowledge and so on (Brazier et al, 2007). Patients should be viewed as consumers seeking "good health" rather than the healthcare service itself, which is an important distinction to make (Grossman, 1972). The link between inputs and outputs can be complex and mitigated by other factors, such as environment and setting. For instance, healthcare is only one of many factors which influence health; other factors such as sanitation, nutrition and shelter also affect overall health (Arrow, 1963).

1.7.2. Rationing in healthcare

The key issue recognised by health economists, and facing policymakers/service commissioners, is that the need for outputs (health) is infinite, while the resources (healthcare services) are always finite. This scarcity of resources to meet demand thus necessitates resource optimisation and subsequently rationing. Rationing can be in the form of deciding which services to provide, whom to provide them for or how they are produced. Allocation of resources requires a centralised, regulated system; the antithesis of an unregulated free market. In a free market system market price is used as a form of rationing, while in a public healthcare system non-price rationing is achieved through techniques such as waiting lists and eligibility criteria.

In order to achieve equity within a market, government intervention may be required to regulate consumption and distribution. Without such intervention an imperfect market such as healthcare is likely to be inefficient and inequitable (Brazier et al, 2007). This raises the issue of how equity in healthcare should be defined, for instance should resources be distributed according to equality of utilisation, access or health? Culyer and Wagstaff (1993) argue that the fairest way to define equity in healthcare is to ground it in an egalitarian approach to health, whereby equality of health or ability to flourish is prioritised above other notions of equality. The "demand for health" approach states that there is a link between health status, health-related behaviour, socioeconomic factors and health inequalities (Wagstaff, 1986), although how these are linked and the direction of causality is complex.

1.7.3. Allocation of healthcare resources

In viewing health as an economic good great care must be taken in deciding who is responsible for paying for it and how it is distributed. Within a publicly funded healthcare system individuals and society as a whole correct market imperfections through taxes. Taxes allow governments to rectify imperfections within the healthcare market by taxing activities where provision is greater than the Pareto efficient level (Morris et al, 2007). In theory each individual has fair and equitable access to healthcare, although their individual needs and outcomes mitigate actual resource use. In private insurance systems individuals pay for healthcare at different levels. Private insurance systems will exist as long as insurers are able to provide healthcare at the price individuals are willing to pay (Morris et al, 2007). This is based on the assumption that incidence of disease is uncertain, that individuals are naturally risk-averse and that utility maximisation is always a priority (Pauly, 1968). Insurance based systems are most efficient when all parties are aware of the relevant risks, but due

to the uncertainty of onset and duration of ill health, purchasing of health and social care insurance can cause issues (Normand, 1991).

In healthcare markets, due to finite resources, trade-offs must be made between services and sometimes people. By allocating resources to one service or group, this takes resources from another; this is described as opportunity cost. The benefits of an alternative service are foregone due to resources being allocated elsewhere. In an economic system where resources must be allocated sparingly opportunity cost is a key concept that accounts for the value in the next best use of resources.

One of the aims of health economics is to provide evidence that allows decision-makers to make informed decisions about the allocation of resources, whether this is in response to patient demand, efficiency goals or service equity (Brazier et al, 2007).

1.7.4. The cost of illness

Illness exerts an economic burden on society and the individual. Treating illness and maintaining optimum health is expensive, both to the health service and to the individual. The opportunity cost to the individual lies in the value of activities that they have foregone due to illness, likewise society feels this burden due to loss of productivity (Tarricone, 2005). The cost of illness is multifaceted and economic burden is not just related to the cost of providing a health service. Measuring all of the costs of illness is extremely difficult, and basing decision-making on the economic burden of an illness is risky as this can marginalise groups in society who are less able to contribute financially, such as disabled people.

The cost of producing one more unit of a defined output (e.g. health), is the marginal cost. The cost of adding or subtracting one additional unit of output is the incremental cost. It is arguably the marginal cost which is of most importance for health economists when comparing similar services, as the marginal difference is the key indicator of change, and under certain conditions we should expand production until marginal cost is equal to marginal difference.

1.7.5. Welfarism and extra-welfarism

There are two dominant approaches to normative analysis of health and healthcare: welfarism and extra-welfarism. Welfarism is defined as the systematic analysis of the social desirability of a defined activity or arrangement (Morris et al, 2007), for instance allocation of resources within a national health service. Welfarism uses value judgements to produce a ranking of alternative social states that could be chosen by society, thus welfarism is based on normative principles of economics and

the relative desirability of alternative economic outcomes (Morris et al, 2007). Accepted assumptions in welfarism are that social welfare is a function of individual welfare; outcomes should be valued by affected individuals; and individual utilities are a result of consumed commodities (goods and services used to achieve valuable life [Bleichrodt and Quiggin, 2013]) (Birch and Donaldson, 2003). Welfarism therefore takes an individualistic standpoint, whereby relevant social choices can only be made by the individuals who will be impacted by those choices (Culyer, 1971). Individual utility (or welfare) is identified in all relevant outcomes and social welfare exists as a function of individual utility (Brouwer et al, 2008). Welfarism therefore assumes that individuals (consumers) are the best judge of their own utility and welfare (Morris et al, 2007), and takes a consequentialist stance by only taking into account outcomes generated by the consumption of particular types and amounts of goods/services and the subsequent impact on utility (Morris et al, 2007). Welfarism also assumes that choice reveals preference (Culyer, 1971), as illustrated in the DCE method.

The objective of welfare economics is to create a decision rule that allows ranking of states of a wide range of arrangements and activities based on specific outcomes, such as individual utility. Issues arise when trying to aggregate individual preferences, as the desirability of certain activities and arrangements relies on trade-offs between the utility of individuals (Morris et al, 2007). Therefore, social choice must represent a broader view of society, such as that defined by the Pareto principle. The Pareto principle states that social welfare can only increase if the welfare of at least one individual increases without diminishing the welfare of anyone else (Brouwer et al, 2008). In welfare economics the Pareto principle assumes that individuals can consistently and effectively rank states of the world, which in turn can be used to aggregate individual preferences into a social welfare ordering of the social desirability of all possible states (Morris et al, 2007).

A weak Pareto improvement would be a change that increases the utility of all affected people, whilst a strong Pareto improvement would improve the utility of at least one person without diminishing the utility of anyone else. The Pareto principle can therefore be used to identify states that may be considered socially more desirable or less desirable based on the utility trade-offs between different individuals. Taking the QALY as an example, Welfarism assumes that the QALY represents an individual's utility concerning their own health (Brazier et al, 2008).

Welfare economics faces several major criticisms, for instance it does not consider the distribution of utility across individuals, and thus supposes optimality only on one dimension (Drummond & McGuire, 2001). Initial distribution of income and welfare within society is also taken as a given, thus Paretian criteria need only be fulfilled by subsequent changes and not address underlying

distributive issues (Brouwer et al, 2008). Furthermore, ranking of all possible states would be implausible, therefore any conclusions based on ranking of states would be incomplete, and would require additional inter-personal comparisons (Drummond & McGuire, 2001). Issues arise from individual preference and choice under uncertainty, as actions may violate the conditions of welfare economics in a non-systematic way, and such violations cannot be systematically corrected at the individual level (Brazier et al, 2007).

Extra-welfarism takes a different approach to welfare economics and differs from welfarism in a number of ways: firstly it considers a variety of outcomes beyond utility; secondly sources of valuation are extended beyond just the affected individual; thirdly the weighting of outcomes is not necessarily preference-based; and finally interpersonal comparisons of wellbeing are permitted on a range of dimensions (Brouwer et al, 2008). Extra-welfarism rejects exclusive focus on individual utility and takes a wider view that includes outcomes such as happiness, social interaction and pain. This approach is relevant to health as the development of public healthcare systems reflects the need to allocate resources fairly and efficiently within financial constraints. Accordingly, health is viewed as a positive influence on social welfare function in itself.

Extra-welfarism led to the development of the QALY, which has been an important part of measuring health outputs in health economics for three decades. The QALY has become particularly popular because it offers a generic, universal approach to outcome measurement which can be used to compare the effectiveness of disparate and unrelated interventions using a single measure (Whitehead and Ali, 2010). This is particularly useful when having to make allocative decisions between services and interventions that are essentially incomparable in their native outcomes. As healthcare resources are finite, QALYs can provide a means to make optimal decisions about resource allocation. However, the QALY has received criticism, for instance it can be too narrow in focus and thus neglects important aspects of health and functioning (Mooney, 1989). Furthermore in health states where QoL takes precedent over quantity of life (e.g. chronic illness, life-limiting conditions and disability) the QALY can devalue interventions outcomes (Phillips, 2009).

The capability approach (Sen, 1993) offers an alternative form of extra-welfarism. The principles of the capability approach have been used to develop tools to evaluate healthcare and interventions based on functioning and capability rather than just utility. The relationship between actual functionings and capability to function (or to achieve functionings) is key to evaluation based on the capability approach. This considers both what an individual is actually able to achieve and what they choose to achieve, and thus personal choice is considered in evaluations. Focusing on achievement alone neglects to consider the role of the individual and their freedom of choice (Sen, 1993).

Therefore, under these assumptions evaluation of outcomes should consider actual ability to achieve valuable functionings (Robeyns, 2003).

Under the Pareto principle interpersonal comparisons of utility are of little benefit and difficult to analyse; we are unable to judge between states of the world that are equally efficient but offer different distributions between individuals. However by examining outcomes beyond utility, such as health or capability, it is possible to make meaningful interpersonal comparisons (Brouwer et al, 2008). This violates traditional von Nuemann-Morgenstern utility theory, which states that when an individual is presented with uncertainty their choice will reflect a need to maximise expected utility (Binmore, 2009). It is difficult to know for certain that a healthy person has higher utility than an unhealthy person, however using health as a quantitative measure we do know that a healthy person has more health (Brouwer et al, 2008). This principle allows health economists to evaluate the effectiveness of interventions and services based on a number of key factors such as health, HRQoL, health equality and capability outcomes, rather than just utility.

The application of the capability approach in health economics and economic evaluation is still relatively limited. A number of key methodological issues are still widely debated, for instance, deciding which capabilities and functionings to measure and how to measure/value them (Coast et al, 2008). However, the development of the ICECAP measures of capability (Al-Janabi et al, 2012) has demonstrated that the capability approach can be applied practically in adult healthcare evaluations, providing a strong basis for future capability based evaluations.

1.7.6. The principles of economic evaluation

The principles of welfare and extra-welfare economics form the basis of economic evaluation in healthcare. To date the QALY continues to be the outcome of choice for most economic analyses in the UK. The choice and focus of outcomes/analyses highlight the underlying conceptualisation of welfare adopted within an economic evaluation, for instance cost-benefit analysis reflects a welfarist approach to analysis, as all outcomes are valued in monetary units (Brazier et al, 2008), while cost-utility and the capability approach reflect extra-welfarist principles.

Economic evaluation is the way in which health economists aid the decision-making process within healthcare resource allocation. Economic evaluation allows these decisions to be made in an efficient manner by comparing the relative costs and benefits of alternative healthcare interventions (Brazier, et al 2007), although a trade-off between efficiency and equity is always needed. There are five types of traditional economic evaluation:

1. Cost-benefit analysis (CBA): In its simplest form, CBA is an inventory of all costs and benefits (measured in a single unit) of an intervention or service (Morris et al, 2007). CBA is differentiated from the other forms of economic analysis in healthcare as it values all outcomes in monetary units (Brazier et al, 2007). Outcomes are given a monetary value and analysed accordingly, including health outcomes. The net social benefit of an intervention is calculated by subtracting the incremental cost from the incremental benefit (Neumann et al, 2000). Willingness to pay and the human capital approaches are commonly used to place monetary value on outcomes (McCrone, 1998). CBA allows all interventions to be assessed solely in terms of expenditure and gain, which is beneficial for making comparisons between interventions and services that may not be otherwise comparable. For instance, CBA could be used by government agencies to compare public expenditure across different publicly-funded services (McCrone, 1998).
2. Cost-minimisation analysis (CMA): this form of economic evaluation examines which competing alternatives are the least costly. This form of analysis requires that outcomes are identical in both alternatives, which limits the use of CMA in many situations, as outcomes are rarely identical between different interventions or service provisions (Brazier et al, 2007). CMA does have use in some circumstances, for instance it could be used effectively when outcomes are relatively simple and clearly defined, such as antenatal care where the desired outcome is almost always the birth of healthy children (McCrone 1998). In this circumstance alternative processes of care could indeed be compared based solely on cost.
3. Cost-effectiveness analysis (CEA): this form of evaluation compares alternative methods of achieving a predetermined outcome, for example additional life years. CEA directly shows the relationship between costs and effects for a specified intervention and comparator (Neumann et al, 2000). The intervention which incurs the least cost and highest benefit (or equal benefit) is determined the most cost-effective intervention (Brazier et al, 2007). An incremental cost-effectiveness ratio (ICER) may be calculated to compare relative costs and incremental benefits of differing but comparative interventions. CEA relies on a single outcome measure, as cost cannot be attributed to two separate outcomes or when outcomes may be mutually exclusive (i.e. extended life but decreased QoL) (Brazier et al, 2007). Direct comparison between the value of costs and effects is not possible (Morris et al, 2007).
4. Cost-utility analysis (CUA): for the purpose of economic evaluation, utility is defined as the preference for a particular health state or outcome (Brazier et al, 2007). CUA is an extension of expected utility theory, which assumes that rational decision-making can be made in

uncertain circumstances (Neumann et al, 2000). CUA compares the cost of alternative interventions with utility outcomes. The outcome most often used in the UK is the QALY, which is a calculation of the time spent in a defined health state multiplied by the preference weight (utility) associated with that health state (Stevens and Palfreyman, 2012). Much like the ICER, the QALY can be compared in terms of incremental cost per QALY. The benefit of the QALY is that it creates a universal measure that can be used to compare disparate interventions across disease states, thus allowing comparison of interventions that could not be compared using condition or disease-specific outcomes. There are many critics of the QALY, but at present it still represents the most commonly used universal tool to compare outcomes between unrelated interventions.

5. Cost-consequence analysis (CCA): CCA is somewhat different to the other forms of economic analysis as the aim is to consider a wide range of consequences occurring from an intervention and to present them in a disaggregated fashion. For example, CCA may consider the age, life-stage or socio-economic status of participants (Brazier et al, 2007). Results are presented separately and decision-makers are able to review findings from a range of perspectives.

1.7.7. Prioritisation and disinvestment in healthcare

Prioritisation is concerned with the allocation and re-allocation of resources within health services that produces the greatest benefit from finite resources. Public healthcare systems, such as the NHS, reflect dissatisfaction with unregulated healthcare markets. The development and maintenance of public healthcare systems requires choices to be made about the allocation of resources, which in turn necessitates the prioritisation of services and treatments. In times of economic recession resources available for healthcare may be capped or reduced. Processes of disinvestment may be used to enable re-allocation of resources or to address budgetary shortfalls (Daniels et al, 2013). Disinvestment can take a number of forms, including full withdrawal of services, restrictions to access or substitution for more efficient services (Daniels et al, 2013). Opportunity cost, marginal benefits/costs (Donaldson et al, 2010) and allocative efficiency (Peacock, 1998) are key to the practice of disinvestment, as potential foregone benefits and relative marginal benefits of services guide the allocation and re-allocation of resources. Rational disinvestment is a similar principle, but focuses on minimising harm from unavoidable budget cuts in order to maximise benefits for the wider population (Donaldson et al, 2010).

Efficiency can be achieved in two ways; minimising the cost of producing a particular output, or maximising the output within a defined budget. Programme budget marginal analysis (PBMA) is built

on the principle of allocative efficiency, with health related benefits the output of interest (Peacock, 1998). Programme budgeting considers both past and future resource allocation in a specific programme, while marginal analysis appraises added or lost benefits and costs from investment and disinvestment (Edwards et al, 2014). Underlying the PBMA technique of disinvestment is the aim to maximise health outcomes from limited health service resources. In order for allocative efficiency to be achieved, costs and outcomes of a range of services must be considered as part of health service planning (Peacock, 1998).

To achieve appropriate re-allocation of resources it is also important to consider resources from a range of perspectives: resource availability; current use of resources; which services could benefit most from additional resources; which services could maintain output with reduced resources; and finally which services could receive less resources (despite effectiveness) in order for a more effective service to receive more (Donaldson et al, 2010).

In times of recession, approaches such as PBMA are accepted ways of disinvestment (Donaldson et al, 2010). These techniques to support decision-making require data and evidence (Brambleby & Fordham, 2003) and thus cannot be carried out without existing robust and reliable health economics evidence.

Health economics has an important role to play in evaluating interventions and the benefits of integrated health and social care systems. Integrated care is particularly important for situations where care may be detrimentally affected by poor coordination, for instance in the care of children and adults with disabilities (Goodwin, et al 2012). There are, however, barriers to effective rational disinvestment, such as the time it takes to develop appropriate economic frameworks within health service management processes, and ensuring that adequate outcome measures are available within and between services (Donaldson et al, 2010). Generic, preference-based HRQoL outcome measures allow comparison of unrelated interventions and patient groups, and thus are an effective tool for aiding rational disinvestment decisions. Their applicability and validity is however uncertain in some patient groups, for instance disabled people and young children, calling into question the appropriateness of comparing HRQoL outcomes across patient groups.

1.7.8. The role of the National Institute for Health and Care Excellence

In recent years the NICE has promoted the use of concepts such as choice and opportunity cost to guide NHS expenditure and commissioning. The increase in cost per QALY estimates and subsequent guidance based on this data indicates the influence that health economics can have on healthcare.

NICE now also covers social care and public health alongside healthcare, thus the application of health economics in all areas of care must be robust and reliable.

Evidence-based decision-making requires evidence of cost-effectiveness to be robust and favourable, thus for guidance to be appropriate there must be sufficient evidence (NICE, 2008). Without this evidence NICE cannot make clear judgements and guidance on particular interventions or services. Implicitly, this could lead to certain areas of healthcare with existing evidence bases being prioritised unduly. If current methods are inappropriate then lack of evidence does not necessarily equate to lack of effectiveness. The solution is to find alternative ways of measuring benefit, while still adhering to the common procedures for evaluation in order to maintain comparability.

NICE provides independent guidance on public health, health promotion, medical treatment and illness prevention. The NHS uses this guidance to prioritise services and interventions and inform healthcare provision. The remit of NICE guidance falls under four programmes, including technology appraisal and clinical guidelines. Guidance is developed using best available effectiveness and cost-effectiveness evidence at the time. As evidence is often incomplete or of relatively low quality, judgements of scientific value must be made. Issues of equity must be considered alongside evidence of efficiency, therefore social value judgements which reflect society rather than science are necessary (NICE, 2008).

1.7.9. Defining efficiency and equity in healthcare

Efficiency in health economics can be defined as the use of finite resources to meet the health and healthcare needs of society; inefficiency refers to circumstances where additional health outcomes could be achieved by reallocating current resources elsewhere (Palmer and Torgerson, 1999). The measurement of efficiency is concerned with the relationship between inputs and outputs of a health system. Outputs may be intermediary (waiting times, patients screened) or final health outcomes (QALYs, lives saved, diseases cured), although effectiveness is best measured using health outcomes (Palmer and Torgerson, 1999). Efficiency can be measured as the value for money for a given use of resources (Palmer and Torgerson, 1999).

Two distinct concepts of efficiency are prominent in discussions about healthcare: ability to achieve an outcome using the minimum input and resources required (technical efficiency); and the maximisation of outcomes using a specified level of resources (allocative efficiency). Technical efficiency is concerned with how best to achieve an objective, for instance how to maintain a given output whilst reducing resource use (Shiell et al, 2002). Allocative efficiency is concerned with the

extent to which an outcome is worth pursuing, for instance deciding which services to invest in to get the best overall health outcomes for a given sample of people (Shiell et al, 2002). What measurement of efficiency doesn't always account for is the concept of equity; whether the distribution of resources and outcomes meets social judgements of fairness. When resources are finite judgements need to be made regarding the fair allocation of resources (Shiell et al, 2002).

Equity in healthcare is defined by the fair distribution of healthcare and health outcomes within society (Soares, 2012), and can therefore be described as an ethical concept related to distributive justice (Braveman and Gruskin, 2003). Equity can be broken down into a number of concepts, including equity of utilisation, equity of access and equity of health outcomes (Oliver and Mossialos 2004). Horizontal equity refers to equity between individuals of the same group or with similar circumstances, for instance socioeconomic status or geographical location (Morris et al, 2007). Vertical equity considers unequal treatment of unequal individuals, such as individuals with different circumstances or needs being treated differently (Morris et al, 2007).

In order to achieve equity according to some social concept of distributive justice, redistribution of benefits, resources and services may be required. Definitions of equity may vary greatly between individuals, societies and contexts, thus the subjectivity of equity can hinder a universal operational definition (Braveman and Gruskin, 2003).

Societal attempts to realise equity through redistribution of resources and health outcomes is exemplified by progressive tax funded healthcare systems, where highest earners contribute the most in terms of taxation. In theory, wealth is consequently redistributed throughout society and ability to pay does not affect access to healthcare. In practice, health inequalities continue to exist in countries such as the UK due to ongoing profound social and economic inequalities (Lakasing, 2009).

With the redistribution of wealth come impacts to efficiency, for example income tax essentially takes money out of society to redistribute it more equally. This epitomises the basis of equity-efficiency trade-off; as equity increases, efficiency decreases and vice versa. As an example, specifically screening a known high-risk group for a particular condition lacks equity, however if screening was rolled out universally costs and ineffectiveness and therefore inefficiency would increase (Sassi et al, 2001). Inefficiency leads to situations where health outcomes could be increased by reallocating current resources elsewhere (Palmer and Torgerson, 1999), thus a balance is needed between equity and efficiency.

There is debate in both traditional economics and health economics about the trade-off between equity and efficiency and how best to achieve balance. A common issue with this debate is that

equity is an outcome of a healthcare system, while efficiency is the relationship between input and output (Reidpath et al, 2012), therefore trading one for the other is problematic. However, when considering a healthcare system it is plausible to define health equity as a potential output and to explore means to achieve that output in an efficient manner (Reidpath et al, 2012). Reidpath et al (2012) argue that the question needs to be rephrased to look at how a society prioritises equitable distribution of health outcomes and maximisation of health gains.

The QALY is used as a proxy for health maximisation in healthcare systems (Wagstaff, 1990), and thus a basis for measuring efficiency. The QALY framework underpins NICE's approach to evaluating the efficiency of health services and treatments. Use of the QALY assumes that maximising health output is the key objective of a healthcare system, and that use of a single measure can appropriately capture all of the relevant outcomes needed to assess efficiency. The relative simplicity of the QALY framework allows widespread use and comparability, however, the use of a single metric reduces sensitivity and applicability, thus other important effects and outcomes may be neglected in subsequent decision making (Coast, 2004).

There is an argument, albeit a contentious one, that resource allocation based on QALY data is equitable if QALYs are valued the same for everybody, as intrinsically this implies allocative decisions value health gains equally for all members of society (Wagstaff, 1990). However, in decision-making scenarios QALYs are aggregated across a sample, thus QALY gains are assumed to be equal between individuals. Under the QALY framework equity cannot be easily examined because interpersonal comparison of QALY gains cannot be made, therefore individual need cannot be accounted for (Soares, 2012).

Concerns about equity can, however, be included in decision-making about resources. For instance multi-criteria decision analysis and deliberative processes can be used to consider equity alongside evidence of cost-effectiveness (Soares, 2012). Furthermore, the application of explicit equity weights for health outcomes has been debated, although a consensus on how to apply this approach has not been reached (Wailoo et al, 2009). At present NICE uses deliberative processes to implicitly include considerations of equity in decision-making alongside QALY evidence (Soares, 2012).

As a result of incorporating social value judgements into decision-making, NICE have approved several medicines with ICER estimates above the current cost-effectiveness threshold of £20,000 to £30,000 per QALY. As an example, medicines which prolong life in end-of-life care have often been afforded more lenience in cost-effectiveness decision making. This is to reflect the assumed societal perspective that special value should be given to treatments which prolong life (Linley and Hughes,

2013). This is based on the assumption that QALYs obtained by patients at the end of life have more social value than QALYs obtained in other circumstances (Colins and Latimer, 2013). However, the legitimacy of this assumption has been called into question, as evidence indicates that societal preferences do not support the prioritisation of end-of-life treatments (Linley and Hughes, 2013). Although the incorporation of social value judgements enables social values of equity to be considered in NICE decision making, assumptions about societal priorities and preferences must be evidence based.

1.7.10. NICE social value judgements and ethical considerations

Underpinning all guidance produced by NICE are a number of key principles regarding ethics, legislation and procedure. All guidance relating to clinical and health practice adheres to principles of autonomy (respecting the rights of individuals to make informed choices about their healthcare) and distributive justice (provision of services in a fair and balanced manner) (NICE, 2008). Although these principles cannot always be upheld at the individual level, for instance children who are not deemed to have autonomy or adults who lack mental capacity, they form a basis for guidance. NICE also demonstrates procedural justice as decisions on healthcare, interventions and guidance are transparent and reasoning behind decisions explicit (NICE, 2008). It is natural within society for there to be disagreement about principles of prioritisation, resource allocation and rationing (Daniels, 2000), therefore publicly funded bodies such as NICE must be accountable for reasonableness (NICE, 2008); in other words guidance and decisions advocated by NICE must be relevant, reflective of the views of key stakeholders, regulated, transparent and publicised.

The basis for evaluation in NICE is through comparison of intervention cost and health state preference; cost-utility. Utilities in economic evaluation are cardinal values used as preference weights for particular health outcomes or defined health states. In order to measure utility health states must be defined and then their value weighted. A number of methods exist to do this directly, including health state rating scales, time trade-off (sacrificing time to avoid reduced health) and standard gamble (risking being in full health or death). Preference-based measures, such as HRQoL questionnaires, are indirect methods of measurement as health states are pre-scored by, for example, a large sample of the general public. Each combination of different domain levels within preference-based measures, such as the EQ-5D, represents a different health state and thus each state has a different utility weight (Brazier et al, 1999).

The health outcome measure used by NICE is the QALY. The QALY reflects a social value judgement that both quality and quantity of life are important, and also demonstrates that many interventions

aim to improve QoL rather than length of life (NICE, 2008). An underlying social value judgement of NICE technology appraisals is that QALYs are equal; specifically a QALY gained or lost by one individual is equivalent to any other individual (Rawlins and Culyer, 2004). Therefore all QALYs are weighted the same and a QALY is of equal importance to each person. However, the true social value of a QALY is not so simple, particularly when determining if QALYs should be weighted differently to address healthcare equity issues. The Secretary of State's directions to NICE state that the degree of clinical need of patients should be taken into account in evaluations (Shah et al, 2013). Likewise, NICE advisory bodies have given special weighting based on severity of illness. The health gains of the severely ill are often valued higher than equal gains in healthier populations (Shah et al, 2013). The notion that 'a QALY is a QALY is a QALY' is not necessarily upheld when QALYs are valued differentially to address issues of equity, whether explicitly or implicitly. For instance, implicit additional QALY gain weight is observed in the prioritisation of interventions for disadvantaged populations in favour of more cost-effective ways of improving wider population health (Shah et al, 2013).

This raises some interesting ethical considerations, for instance should QALY gains be considered less important in socially advantaged groups and are the QALY gains of healthy individuals less important than those of less healthy individuals? It is important to establish how society prioritises equitable distribution of health outcomes and maximisation of health gains (Reidpath et al, 2012), and how best to maximise resources to create a fair balance between equity and efficiency.

Legislation on human rights, discrimination and equality states that patients should not be denied or restricted access to healthcare due to disability or age (and other factors such as ethnicity and gender) (NICE, 2008). McMillan et al (2006) state that the principles of biomedical ethics are not equal in healthcare priorities; justice overrides the other ethical principles. Therefore, when setting priorities the opportunity cost of how services should be weighted against one another is of greatest importance.

NICE states that it aims to take special account of the needs of disabled people, however this is not currently reflected in the prioritisation of particular types of guidance. The principles of economic evaluation are explicitly described by NICE, however the approach to realising equity is less specific, particularly in terms of balancing efficiency and equity (Shah et al, 2013).

1.7.11. Defining and measuring quality of life

Patient reported outcomes, such as QoL and HRQoL measures, have become an important tool in the NHS as relevant data about intervention outcomes can be sourced directly from patients (or

carers as proxies (Stevens and Palfreyman, 2012). The World Health Organization (1997) defines QoL as a broad concept affected by physical health, psychological state, level of independence, social relationships, personal beliefs, environment, culture, value system, personal goals, expectations, and concerns. HRQoL is specifically concerned with the impact of illness and treatment in relation to QoL. Most definitions of HRQoL define it as a subjective and multi-dimensional construct (Matza et al, 2004). For instance, HRQoL can be defined as an individual's perception of the impact of health status on QoL, including physical, psychological and social functioning (Leidy et al, 1999). When applied to children, definitions of HRQoL must take into account the specific contextual factors of childhood, as children's HRQoL is likely to be affected differently to that of an adult due to the unique social contexts they inhabit (such as the family, friends and school) (Matza et al, 2004). This can make comparisons between child and adult HRQoL difficult. For the purpose of this thesis, I define QoL as a broad concept encompassing physical, social and emotional wellbeing and an individual's satisfaction with their life conditions (Felce and Perry, 1995). I define HRQoL as the specific impact of illness, disability, physical health and mental health on subjective QoL.

In order for a generic HRQoL measure to be suitable for calculating QALYs as part of cost-utility analysis it must: be preference-based (the relative importance of different domains must be accounted for); contain a health state classification system (with preference weighted health states); and be based on an equivalent scale of 1 as perfect health and 0 as death (Stevens and Palfreyman, 2012).

There are, however, issues with using generic HRQoL measures. It is recommended that health state preferences and subsequent weights are derived from general public perceptions of health state valuation (Griebsch et al, 2005), and thus may not reflect the specific needs of subset populations (e.g. children, wheelchair users). Secondly, the domains of HRQoL may be too broad and therefore insensitive to changes experienced by people with specific diseases or conditions (Harding, 2001). Based on the NICE reference case (NICE, 2013), the use of the QALY as a measure of effectiveness makes a value judgement that HRQoL can be fully measured in terms of mobility, self-care, ability to complete usual activities, absence of pain and discomfort and absence of anxiety and depression; the domains of the EQ-5D generic HRQoL measure (Rawlins and Culyer, 2004). The relevance of these domains to people with disabilities, and the subsequent preference weights, is not clear.

The alternative to generic measures are disease and condition specific HRQoL measures (or bolt-on versions). Such measures are often more sensitive and elicit data that could not be measured with generic tools (Stevens and Palfreyman, 2012), but they can also be too narrow and incomparable to generic measures. This means that it is difficult or impossible to make comparisons with different

disease groups or healthy populations (Eiser, 1997). Furthermore, they cannot easily be mapped on to QALY calculations, making them incompatible with current NICE methods of economic evaluation. If specific measures were used for each condition it would mean that disparate interventions and services could not be directly compared in terms of utility gains and health outcomes. Therefore, appropriate generic measures are essential.

1.7.12. Applying health economics to disability and equality

The United Nations (1993) produced rules for the equalisation of opportunities of disabled people, which refers to the process through which society and the environment should be made accessible to all, with specific relevance to disabled people. These rules state that resources should be used in a way that facilitates the full participation of all members of society, including awareness-raising, effective medical care, rehabilitation to sustain independence, and support services (including assistive devices) to increase independence.

The United Nations convention on the rights of persons with disabilities (Schulze, 2010) deals specifically with the rights of children with disabilities, and states that all necessary measures should be taken to ensure disabled children experience the same human rights and fundamental freedoms as other children. Furthermore, disabled children should have the right to express their views on all matters affecting them, and they should have equal access to play, recreation, education and community life. Disabled children should have access to all appropriate specialist assistance, including support for caregivers, to promote dignity and self-reliance (Schulze, 2010).

In the UK the Equality Act 2010 prohibits discrimination against disabled people, both on a personal and institutional level (Office for Disability Issues, 2010). Under this act public authorities have an obligation to eliminate discrimination, harassment and victimisation, and to promote equality of opportunity (Davis, 2012). By 2025 the government aims for disabled people to have full opportunities and choice to improve their QoL and to be respected as equal members of society (Office for Disability Issues, 2006). Public services, such as health and social care, must be proactively developed to ensure that inequality is tackled and equal opportunities are promoted (Office of Disability Issues, 2006). Evidence of cost-effectiveness is needed to guide resource allocation based on efficiency goals. NICE HTA guidance is utilised in mainstream healthcare decision-making, but to date there has been no guidance on wheelchairs for disabled children.

With regards to healthcare reform, it is now commonplace to consider the views and experiences of service users, therefore it is imperative that the voices of disabled children and their carers are heard. Services should be flexible, individualised and embedded in a multi-agency approach that

focuses on healthcare outcomes (Every Disabled Child Matter, 2011). The DoH is committed to improving the outcomes of disabled children by ensuring relevant and reflective outcomes are used in healthcare, and that the choices of children and their families are taken into account (DoH Commissioning Team, 2010). A government review highlighted the need for evidence of effectiveness and validated clinical outcome measures to promote successful health services (DoH, 2008), which is particularly relevant in the case of children and disability.

1.7.13. Social Model of Disability

The Social Model of Disability (SMD) is directly oppositional to traditional medical models of disability and health, which treat disability as an individual problem; a deficit in ability which should be treated solely through medical intervention. The medical model assumes that the causes of disability are therefore a direct result of individual abnormal physical functioning, thus defining disability as a disadvantage and human diversity as a scale from normal to abnormal (Terzi, 2004). The SMD is focussed on how social oppression and discrimination disables those with impairments. Disability is therefore defined as being a direct result of societal barriers to participation and independence (Oliver, 1998). The negative attitudes of other members of society give rise to conflict between dominant and subordinate groups (e.g. disabled people) in society, which in turn causes institutional discrimination and an internalised perception of reduced capability and self-efficacy amongst disabled people (Lang, 2007).

In society, disabled people are perceived as dependent, which is reflected in the focus on functional limitations and the need for state provision of education, healthcare and financial support (Lang, 2007). For disabled children this idea of dependency is less relevant as all children are dependent, but limiting independence creates institutionalised dependency and a perception that disabled people are unable to care for themselves; an issue which extends from childhood to adulthood. Materialist understanding of disability defines impairment and disability as two separate concepts; impairment is a bodily state characterised by physical or cognitive malfunction, while disability is the disadvantage or restriction faced by people with impairments due to societal, organisational and/or institutional barriers (Lang, 2007). Therefore, disability is culturally defined and arguably different in different societal structures. The rise of individualism is reflected in the medical model of disability and the definition of disability as an individual pathology (Lang, 2007).

There is disagreement within the field of disability research as to whether individual experiences of disabled people are significant, or whether individual focus dilutes the political strength of the SMD. Where traditional SMD designates a separation between impairment and disability, modern SMD

acknowledges the relationship between impairment, society and disability. For instance, the physical and emotional pain of impairment impacts the experience of disability and vice versa (Shakespeare and Watson, 2002). By compartmentalising the experience of disability into distinct social and physical experiences the subjective experience of impairments may be ignored (Lang, 2007).

Impairments have varying degrees of impact on health and capability, and generate different responses from society. For instance there are differences in the way people with visible and invisible impairments are treated by society; those with invisible impairments may not face disablement from society but may still experience impacts to function, personal identity and wellbeing (Shakespeare and Watson, 2002). Likewise, in childhood disability barriers of 'being' (hurtful, hostile, discriminatory and inappropriate behaviour from others in society leading to negative sense of self) appear to have more impact than 'doing' barriers (physical, economic, material, and environmental barriers to participation), which lay down the foundations for self-confidence and self-worth in adulthood (Connors and Stalker, 2007). Different types of impairments have different impacts on disablement. Therefore, broadly grouping types of impairments is necessary to acknowledge their differences in functional, presentational, individual and social implications (Shakespeare and Watson, 2002).

In order to create a "non-disablist" society a number of developments would be needed. Firstly anti-discrimination legislation must protect the rights of disabled people, something which has happened widely in the UK; secondly, all appropriate services should be made accessible to disabled people through proper infrastructure, something which has happened in theory but not in practice in the UK; thirdly, adequate state funding must be in place to bring about widespread change to infrastructure and society; and finally, social awareness of disability must be raised to combat discrimination and oppression.

In practice the integration of both medical and social models of disability may prove to be the most appropriate way forwards. A medical approach allows the treatment and management of pain, nutrition and treatable illness whilst consideration of social needs and how medical intervention can impact these allows maximisation of social participation. Services and technologies should aim to assist disabled people to be active and fulfilled members of society through better health and enhanced participation.

The government has committed to tackling the 'disabling environment' by improving the inclusion of disabled children in education, healthcare, housing and leisure services (DoH, 2004). There is still a

need to understand the factors which limit NHS services for disabled people and which promote social/political barriers to inclusion.

1.7.14. Qualitative methods in health economics

Translating health economics research and prioritisation outcomes into real world healthcare practice is paramount to ensure that health economics has a positive impact on society. The practical influence of health economics research on health service management and priority setting has thus far been difficult to guarantee (Smith et al, 2009). The application of qualitative methods (such as focus groups, interviews and thematic analysis), could be used to understand and address the barriers to the practical application of health economics research (Smith et al, 2009). The NHS National Institute for Health Research (NIHR) HTA programme has embraced the use of qualitative methods and methodologies, stating that although such techniques have limitations, they can provide valuable data on the implementation and impact of health technologies when used and conducted appropriately (Murphy et al, 1998).

Qualitative methods allow researchers to understand a topic within its own context (Coast et al, 2004). However, there are still relatively few published health economics papers incorporating qualitative methods. By contrasting and synthesising qualitative findings with quantitative findings a broader understanding of a topic may be developed. Health economics is often a positivist discipline that relies heavily on quantitative data, aiming to systematically analyse the cost and effect of healthcare interventions and extrapolate these results from a sample to a population. Qualitative research uses much smaller sample sizes, which has brought criticism about the generalisability of findings (Coast et al, 2004). However, inductive use of qualitative data may help health economists to build models, or could be used to analyse data in terms of typicality to the wider population and applied accordingly (Coast et al, 2004).

It may be argued that use of qualitative data in health economics goes against the very basics of the discipline by eschewing statistical data and objective systematic processes, but qualitative data is of great value for increasing the relevance of economic theory in real world healthcare (Coast et al, 2004). Traditional welfare economics assumes that the behaviour of individuals is rational and predictable, but this is not always the case. Qualitative evidence can be used to understand behaviour and focus on the actions of individuals from their perspective rather than assuming theoretical rationality. In the context of disability and wheelchair interventions, qualitative methods could be used to understand how disabled people define QoL and how that definition differs from

the general population. Such data could have major implications on how standard measures of HRQoL are applied in marginal groups such as disabled children.

1.7.15. The potential usefulness of existing generic HRQoL measures in the economic evaluation of interventions for disabled children

Disabled children require wheelchairs for a wide range of conditions and injuries, thus generic outcome measures of effectiveness may be beneficial to allow large samples of comparable data to be collected and to facilitate comparisons within this diverse group. Provision of health economics evidence can be used to develop NHS services, such as wheelchair services, as it can provide the tools to evaluate the clinical and economic impacts of interventions. These findings are important in prioritising and commissioning services within the NHS and guiding funding allocation.

Economic evaluation using generic, preference-based HRQoL measures and cost-utility analysis should in theory allow benefits of wheelchair interventions to be valued in terms of a universal measure of utility (such as health state preference). In theory, the application of cost per QALY calculations to wheelchairs would allow direct comparison with spending on other unrelated healthcare interventions, such as medication and surgical procedures. However, outcomes from wheelchair interventions are numerous and varied, thus utility preference alone may not be sensitive to capture the impact of wheelchair interventions, particularly when utility weights are based on general population preferences (e.g. Kind et al, 1999).

To date NICE has produced no health technology guidance relating to wheelchair interventions for children. This may in part be due to the inherent difficulty of assessing diverse interventions in a diverse population, but it could also reflect an underlying medical model of health and health economics, which is often at odds with the needs of disabled people. It is imperative that guidance is developed to create uniform, evidence-based and high quality wheelchair services across the UK. In order to do so there first needs to be an understanding of how to measure effectiveness and cost-effectiveness of wheelchair interventions for children. Furthermore, there is a growing focus on return on investment analysis (Cabinet Office, 2009), which could be used to generate economic evidence relating to supporting disabled people. For instance, if a young person with a disability can be supported into a career, then they will work and pay tax.

It may be beneficial to consider addressing the concept of capability poverty. The term capability defines wellbeing as coming from an individual's ability to 'do' and 'be' the things that are important to them. The capability approach aims to address the wellbeing of individuals with shortfalls in capability (Mitchel et al, 2013), for instance those with disabilities. Understanding individual's

capabilities, or potential to function, reflects the principles of the SMD (Francis & Byford, 2011). However, the use of capability outcome measures (such as ICECAP [Al-Janabi et al, 2012]) is still limited, particularly in children, and there are theoretical and methodological issues with using capability as a proxy for health and QoL.

Wheelchairs are rarely provided to increase length of life, and although they do facilitate clinical and functional benefits, they also provide essential mobility to enhance participation and social interaction. Incorporating the capability approach could potentially allow evaluation of actual ability to achieve valuable functionings, such as independence, participation and social interaction. This would facilitate a move away from a medical model of economic evaluation, and allow a combination of both functional and social outcomes to inform economic evaluations. At present there are no validated child-specific capability measures for use in economic evaluation, which exemplifies the important philosophical and methodological questions relating to what capability means for a child.

1.8. Conclusion

It is apparent that determining the best way to examine effectiveness and cost-effectiveness of wheelchair interventions is difficult but essential. In order to do so it is important to first examine what evidence exists regarding the clinical and cost-effectiveness of wheelchair interventions for children, followed by an exploration of how disabled children and their parents define HRQoL, and the best ways of measuring HRQoL in this population. Furthermore, understanding service preferences of wheelchair service users through the use of DCE methods could help to guide the development of services that provide better outcomes for disabled children.

Chapter Two: Methods: An overview of recruitment methods, data collection and ethical considerations from the Wheels Project.

2.1. Chapter summary

In order to limit repetition across the thesis this chapter gives an overview of the methods and data protection processes relevant to all of the four empirical primary data chapters (4-7). In this chapter I give an overview of the study setting, recruitment processes, data collection methods and relevant ethical/data protection considerations. Specific methodological details are presented separately in each relevant chapter.

2.2. Introduction

To address the aims and objectives of this thesis a PhD programme of research called the Wheels Project was developed, funded by the NISCHR Social Care PhD Studentship Award. Data for chapters four to seven were collected as part of this programme of research. As part of the Wheels Project a range of data collection methods were used: collection of wheelchair service cost data; a pilot DCE; measurement of HRQoL; and qualitative interviews exploring definitions of QoL. Where possible all of this data was collected from each participant, although in some circumstances participants chose to decline certain aspects of the data collection. Sample sizes are reported for each separate method.

2.3. Partner wheelchair suppliers

In order to carry out the research I partnered with three wheelchair services/providers:

- Wrexham NHS Posture & Mobility Service: an NHS wheelchair service.
- WK: a charity funded wheelchair service for children.
- DesignAbility at the Bath Institute of Medical Engineering (BIME): a charity funded wheelchair manufacturer and provider.

WK is the leading children's wheelchair charity in the UK, supporting over 1700 children with mobility impairments in 2013 alone (WK, 2013a). BIME manufacture the Wizzybug, one of the only PWCs designed specifically for children under the age of 5.

2.4. Research setting and participants

The research was conducted from a public and voluntary sector perspective. The sampling frame for the Wheels Project was disabled children aged 18 or under who use a wheelchair (provided by one of the partner wheelchair suppliers), and their parents. Where children were unable to participate due to age or capacity issues their parent was recruited as a proxy.

2.5. Recruitment

Participants were recruited between June and October 2013 from the three recruiting partner organisations. Sample size was not pre-determined and convenience sampling was used. The partner organisations were made aware of the inclusion and exclusion criteria prior to commencing recruitment (table 2.1). As I was interested in all children and young people who require manual and/or powered wheelchairs to enable mobility due to a long term (>6months) mobility impairment, I was not explicit about the types of conditions and disabilities of interest. I took this decision to focus on mobility impairment generally, rather than specific disabilities and conditions.

Table 2.1: Inclusion/Exclusion Criteria

Inclusion Criteria	Exclusion Criteria
• Children and young people with long term (>6 months*) mobility impairments • Child aged ≤ 18 years • Child requires a manual and/or powered wheelchair/pushchair/buggy for the purposes of mobility • Parent(s) or guardian(s) able to give informed consent to take part in the study, and parent/guardian able to give proxy consent (where required) • Parent(s), guardian(s) or partner of a child or young person with a long term mobility impairment who require a wheelchair *long term mobility impairment defined as having existed for 6 months or more, or expected to last for 6months or more	• Any significant social or emotional problems or challenging behaviours where such problems in the opinion of the family or clinical team are likely to impair a child's ability to take part in the study or pose a risk to the researcher or the child. • Parental and/or child inability to communicate in English or Welsh

Participants were initially sent a study invitation pack containing child and parent versions of the participant information sheet and the study questionnaire (containing demographic questions and the HRQoL measures) (see appendices A.1 and A.2 for relevant recruitment materials). The study invitation pack was addressed to parents for children under the age of 16. Welsh versions of the participant information sheets and covering letters were produced to aid recruitment in Wales.

Once a completed initial questionnaire was returned, participants were invited to take part in an interview and to complete a DCE questionnaire. Disabled children over the age of 16 and their parents were given the option to take part in the study on their own or as a child/parent dyad. Participants were also given the option to complete the DCE, take part in the interview or both.

Before commencing the interview the study was explained in full to participants and they were informed of the interview process and the aims of the research. Participants were then asked to complete an additional consent/assent form to indicate that they understood and agreed to take part. Children under the age of 16 completed an assent form and their parents completed a proxy consent form. Interviews were conducted in the home of participants and were recorded using a digital voice recorder. Interviews were not repeated due to time constraints

2.6. Ethical considerations and data protection

The Wheels Project was sponsored by Bangor University and ethically approved by an NHS research ethics committee and a Bangor University ethics committee (see appendices B.1-B.3 for approval letters). As some participants were considered vulnerable (due to age and disability), I followed guidance published by the General Medical Council (2007, 2010), Royal College of General Practitioners (2010) and National Research Ethics Service (2007) to ensure recruitment, consent and assent procedures were appropriate and ethical (see appendices C.1-C.3 for consent/assent forms). Furthermore I completed training courses in level 2 Child Safeguarding and Good Clinical Practice in paediatric research prior to commencing the study. Strict procedures were in place to assess mental capacity of participants and to ensure the safety of participants and the researcher.

All identifiable participant data was stored electronically in a password protected file on an encrypted computer. All interview transcripts were anonymised and stored electronically on an encrypted computer. Paper copies of consent forms were stored in a locked filing cabinet in a lockable room.

2.6.1. Child safeguarding

The supervisory team members were all familiar with child safeguarding procedures. It was agreed that if a child disclosed any information that raised serious concerns about their safety I would initiate local child safeguarding procedures. The process was as follows: I would follow the child's wheelchair service child safeguarding procedures if concerns were raised regarding the safety of a child; if serious concerns were raised I would discuss with the child that I would contact their

wheelchair service and seek the child's permission if safe to do so; I would contact my supervisors straight away.

Confidentiality would only be broken if permission was not given by the child. It was decided that the best protocol would be to refer the issue to the child's wheelchair service, who would have procedures in place to deal with child safeguarding issues. All participants were made aware that maintaining confidentiality would be an issue if serious concerns about their safety or that of another child or vulnerable adult were raised. This was stated on the information sheet and was further explained during the consent/assent procedure.

2.6.2. Risks to participants

I took all precautions to protect potential participants from any harm or risk. The Disclosure and Barring Service (DBS) clearance for access to children is mandatory, and thus was completed prior to data collection. All members of the supervisory team involved in the study had up-to-date good clinical practice certificates, copies of which were kept in the study master file located at Ardudwy Building, Bangor University.

As there was no planned intervention and standard validated HRQoL measures were used, the studies posed very little risk to participants. The use of qualitative interviews has the potential to cause distress if there is discussion of sensitive subjects. To limit any potential issues I informed participants that they could stop at any time, likewise I decided beforehand that if I felt that a participant was becoming distressed I would draw the interview to a close. Participants were informed that they could stop or take a break at any time. It was also decided beforehand that if a child or young person was being interviewed on their own and became distressed or upset, with the agreement of the child or young person, I would inform their parent/guardian and explain the situation to them.

All participants were given the opportunity to debrief after the interview and were given my work contact details so they could discuss any aspect of the research once I had left. The research carried minimal risk and therefore was ethical according to the Medical Research Council (2004) ethics guide for medical research involving children, likewise it was granted ethical approval by both university and NHS ethics boards.

2.6.3. Consent and assent

Participants were expected to have a range of physical impairments; furthermore I anticipated that some participants would have additional cognitive impairments, learning disabilities, communicative

impairments and complex needs. The aim was for this to be a low risk, non-intervention and inclusive programme of research, thus I did not automatically exclude any child or young person based solely on cognitive, communicative or physical impairments as the decision to participate is a simple one to make. All children should be involved in the development of services and processes designed to support and care for them (Children's Act, 2004). Therefore, the views of all children, regardless of ability or disability, should be considered equally important.

Due to the low risk nature of this research there were no specific ethical issues around consent and assent not covered by existing best practice guidance. Before the age of 16 children are not deemed to have mental capacity (General Medical Council, 2007), and usually give their assent to take part in research. They must have proxy-consent from their parent or legal guardian (unless there are specific circumstances and they are determined to be competent to make the decision without parental input). Young people over the age of 16 can provide their own consent if they have mental capacity to do so (General Medical Council, 2007), and consent cannot be provided by proxy. All children under 16 were asked to complete an assent form. Consent by proxy (from a parent or guardian) was obtained for all children under the age of 16. I have clinical research experience in determining capacity to consent in young people over 16 years, and gaining assent in under 16s. The supervisory team also have a great deal of experience in paediatric research and research with people with learning disabilities and cognitive impairment, which I was able to draw on.

Capacity was established through discussion with the participant and also with assistance from a family member when needed, in all cases this was a parent. There were also procedures in place to consult with an appointed consultee or healthcare professional who knows the young person in situations where capacity was not directly apparent but a family member could not be contacted. Young people were given the opportunity to communicate by their preferred means and were facilitated to communicate effectively, for instance with a parent helping to interpret their language if they had a speech impediment.

I followed the Royal College of General Practitioners (2010) guidance on assessing capacity, and tested capacity in four ways:

1. Can the young person communicate their decision?
2. Does the young person demonstrate understanding of the information given to them?
3. Can the young person retain the information long enough to make a decision?
4. Can the young person balance and weigh up the information given to them in order to make a decision?

In order to establish capacity I determined whether the young person had understood what was expected of them if they participate in the study and what will happen to the information they give. The project aims, confidentiality procedures, discontinuation procedures and use of their information were explained to each participant and they were asked if they understand and asked to briefly recall what had been explained to them. I ensured that each participant explicitly communicated that they had understood and that they would like to take part in the research (using their chosen method of communication; in all cases speech).

If there was any ambiguity in a young person's understanding during any aspect of the consenting procedure, I used individually-tailored approaches to explain further to see if shared understanding could be achieved. Where appropriate, I consulted with the parent to determine if the young person had understood what had been explained to them.

If a young person was not deemed to have capacity to consent to take part in the research, their legal guardian was to be consulted (Mental Capacity Act, 2005). Under the General Medical Council legal framework (2010) an adult without capacity can take part in research if:

- the potential benefits of the research outweigh the potential risks
- the research cannot be completed with only those who have the capacity to consent
- the individual's appointed consultee or legal guardian believes that participation in research would be the person's wishes if they had capacity

In this low risk programme of research, I decided that I would still involve participants if their legal representative believed that they would want to take part in appropriate parts of the study (such as conveying their feelings about their wheelchair). In this case the young person's appointed guardian or welfare attorney would be able to consent to the young person's participation in aspects of the study that were appropriate for them. The research was not considered to pose any risk to participants due the low-risk nature of the methodology and the lack of an intervention.

Although the research could have (in theory) been completed without involving young people who lack capacity, the aim of this research was to have a realistic variety of children and young people with disabilities and to value their contribution equally. If a parent or guardian did not believe that their child would like to take part in the study, or that participation would not be suitable for the child, the child was not included in the study and any subsequent discussion with them was not recorded as findings. Likewise, I decided that if a child or young person stated that they would not like to take part and would not provide assent or consent (depending on age), they would not be

included in the study. In either of these cases the parents were still given the opportunity to take part in the research on their own and would thus be consented separately.

For young people aged 16 and 17 parents can, within the legal framework set out by the General Medical Council (2007), provide consent on behalf of their child when they consider doing so to be in the child's best interest. I therefore planned to consult with the parents or an appointed legal guardian of a child aged 16 or 17 who lacked capacity, although his was not necessary due to no issues with capacity in this age range. For children aged 6 to 16, assent was determined for each individual. In line with General Medical Council guidance (2007), I ensured that each individual understood the nature, purpose and possible consequences of the research, and I ascertained this through thorough discussion with each child and pragmatic evaluation of their level of understanding. I determined whether the child had understood and could recall what the study was about, what would be asked of them and the confidentiality/discontinuation procedures. I consulted with the child's parent or guardian if there was any ambiguity about the child's understanding of any aspect during the assenting procedure.

Age appropriate and language sensitive documentation was developed. Each child participant was provided with an age appropriate information sheet, parents also received a specifically made parent information sheet. Information sheets and consent procedures were informed by the National Research Ethics Service (2007) participant information guidance.

2.6.4. Children aged 5 and under

For children aged 5 and under it was unlikely that they would be able to provide informed assent, thus parents were asked to provide proxy consent, and through discussion it was established whether each child was happy to participate. If they were not then I did not pursue their involvement in the study. It was also unlikely that they would be able to complete outcome measures, thus I only collected proxy outcome measure data in this age group. For this age group I still involved children in the interviews in an age-appropriate way, and in a way that facilitated their involvement in the study. Young children were able to participate in the study in a number of ways, and through a number of data collection methods. For instance, some young children participated fully in the interviews, while others discussed what they liked and disliked about their wheelchair or drew pictures of their perfect wheelchair. Their involvement was determined on a case-by-case basis.

2.7. Supervision

Throughout all stages of study development, data collection and writing-up of this thesis, supervisory and technical research support was provided by the PhD supervisory team. This included in-depth discussions of methods, data collection, analyses, interpretation of findings and internal quality assurance in accordance with the role of the PhD supervisory team.

2.8. Conceptual framework

In chapter three I present a novel conceptual framework which was developed from the mixed-method systematic review and maps how research and service development can lead to cost-effective wheelchair services and interventions for children. It details areas where specific developments are needed to facilitate a move towards cost-effective wheelchair services for children. This is explained in greater detail in chapter three and revisited in chapter eight. The conceptual framework helped to guide the subsequent studies reported in this thesis.

A conceptual framework is a network of related concepts, in which the interplay and relationship between concepts builds a comprehensive understanding of a given topic or phenomena (Jabareen, 2009). Conceptual frameworks are often used as a means to develop theory by grouping data under conceptual labels and examining the relationship between conceptual groups. This differs from descriptive reporting, as interpretation of data has to be undertaken to build a conceptual understanding of a phenomena (Strauss and Corbin, 1990). Conceptual frameworks can be effective tools for monitoring, measuring, and managing the performance of health systems in order to promote effective, equitable, efficient and high quality services (Arah et al, 2003). A conceptual framework can be used to map processes and mechanisms to better understand and interpret evidence within a health system perspective. For instance, the interconnected relationships between concepts can be utilised to define activities and processes and to better understand outcomes.

The conceptual framework presented in this thesis takes a health systems approach by focusing on the development of wheelchair services in the context of clinical, qualitative, economic and policy evidence. The purpose was to build a contextualised conceptual understanding of UK wheelchair services for disabled children, in order to guide the development of cost-effective services and better outcomes for disabled children. The starting point of this thesis was the systematic review reported in chapter three, which was used to develop an initial conceptual framework to guide the subsequent studies.

As this work was completed from the perspective of a health economist, I focused on the apparent lack of economic evidence in this field and what was needed in order to apply health economics in a robust manner in this setting. I found in early literature searches that there was a distinct lack of economic evidence in this field. The conceptual framework helped to place this thesis in the wider context of wheelchair service development, and the information needed to facilitate this. The conceptual framework is therefore influential in framing the context of wheelchair service development more generally. Economic evidence is needed in parallel to other service developments, such as streamlining of management and procurement strategies, application of appropriate clinical outcome measures and improving the rapidity of services.

2.9. Sequence of studies and chapters

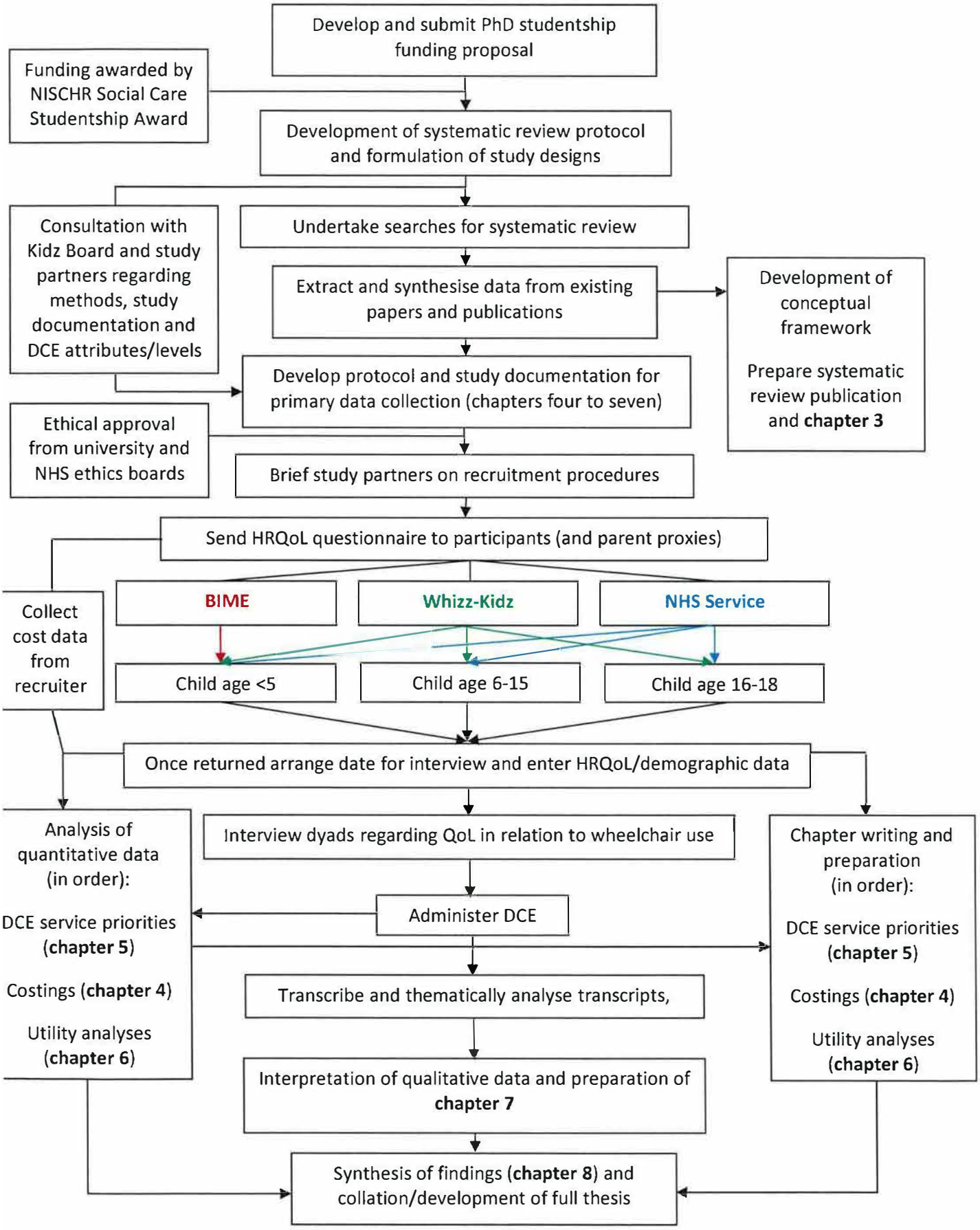


Figure 2.1: Sequence of studies and chapters

Chapter Three: Wheelchair interventions, services and provision for disabled children: A mixed-method systematic review and conceptual framework.

3.1. Chapter summary

In this chapter I present the first systematic review in this field to incorporate evidence of effectiveness, service user perspectives, policy intentions and economic evidence in order to develop a conceptual framework to inform future research and service development. 11 databases were searched. Studies were appraised for quality using one of seven appropriate tools. In total 22 studies and 14 policies/guidelines were included in this review. The results indicate that wheelchairs for disabled children (≤18 years) can provide health, developmental and social benefits. WHO and UK Government reports demonstrate the need for improved access to wheelchairs both in the UK and internationally. The use of health economics within this field is lacking. Provision of wheelchairs based on cost-effectiveness evidence is not currently possible due to a distinct lack of high quality effectiveness and economic evidence in this field. The conceptual framework developed as part of this chapter provides a novel contribution to this area of research, and builds a context for the subsequent chapters in this thesis. The results from this chapter were published in a peer reviewed journal (Bray et al, 2014).

3.2. Introduction

3.2.1. NHS wheelchair services for disabled children in the UK

Several UK government and NFPO reports have found that wheelchair services for children and young people in the UK need improvement in order to meet service user needs (National Assembly for Wales, 2010; Audit Commission, 2002; NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2005; DoH, 2004). These reports reflect the need for a better understanding of the relationship between NHS wheelchair services, effectiveness evidence, service user perspectives and policy intentions.

3.2.2. Why is a systematic review needed?

Wheelchair interventions can have a range of positive impacts on the lives and health of disabled children and young people. In order to promote effective and equitable wheelchair services both in the UK and globally, better understanding of the effectiveness and cost-effectiveness of wheelchair interventions is needed. Likewise, the opinions of young wheelchair users and their families need to be taken into account to shape services. Social theories of disability state that disability exists as both a physical and social issue. Discrimination and positivist based disability management can greatly impact equality (Oliver, 1998).

Health economics can play a specific role in the development of wheelchair services by providing essential data on the cost-effectiveness of different wheelchair interventions. This could in theory facilitate better use of resources and greater coverage of services. At present the health economics toolbox is particularly poor when applied to disabilities and children. Development of health economics methodologies based on a social model of health would promote holistic evaluation of effectiveness and cost-effectiveness.

In order to develop an appropriate set of economic tools it is important to explore existing effectiveness, service user opinion and economic evidence. The development of a conceptual framework from synthesised evidence could then be used to guide wheelchair service development in an evidence-based manner. No existing systematic reviews which address these important issues were found prior to conducting this review.

3.3. Aims and Objectives

The overarching aim was to explore current effectiveness evidence, service user perspectives, policy and economic evidence in order to develop a conceptual framework to inform future research and wheelchair service development in the UK, with international implications. Six objectives were developed to inform searching, management and interpretation of evidence:

- To establish what evidence currently exists regarding the effectiveness of wheelchairs in terms of clinical, social, educational and developmental benefits for disabled children.
- To establish what evidence currently exists regarding the perceived barriers and facilitators of providing and using wheelchairs for disabled children, taking into account the different perspectives of disabled children, parents/carers, and healthcare professionals.
- To gather current policy, not-for-profit organisation publications and clinical guidelines regarding wheelchair provision for disabled children.
- To establish what evidence currently exists regarding the costs, economic implications and incremental benefits of wheelchair interventions for disabled children.
- To understand the extent to which intervention study outcomes and policy recommendations reflect the barriers and facilitators of wheelchair use (expressed in opinion evidence).
- To build a conceptual framework mapping areas for future research and service development to facilitate cost-effective wheelchair services for disabled children.

3.3.1. Review and synthesis questions

Review questions were formulated for each of the different aspects of this review, including the overarching synthesis of evidence. See figure 3.1 for a full list of review questions.

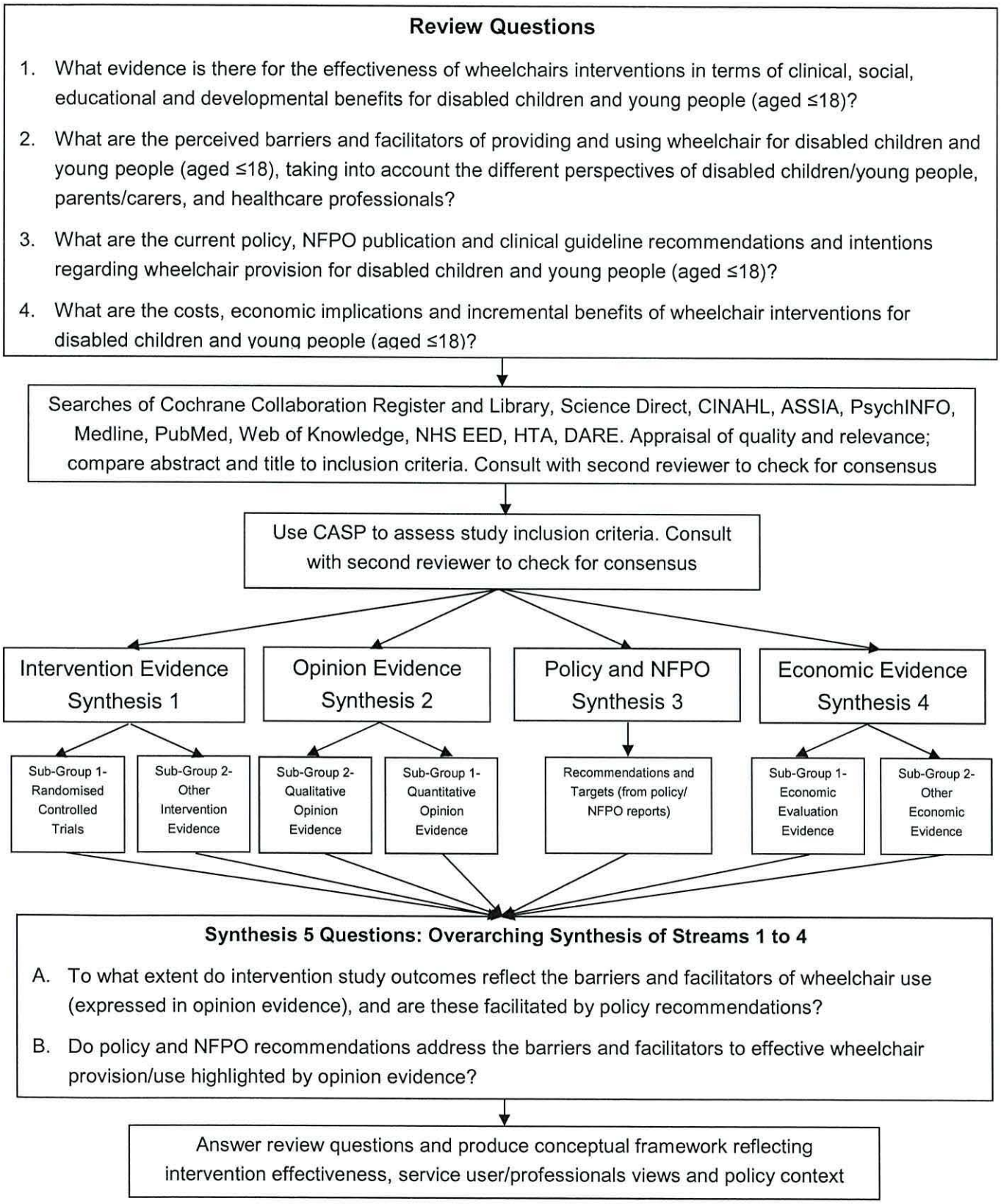


Figure 3.1: Systematic review design flowchart and review questions

3.4. Methods

3.4.1. Systematic review design

An initial scoping search of the literature was conducted to refine the review scope, processes and keywords. A variety of quantitative, qualitative and policy literature was found, demonstrating the multi-faceted nature of wheelchair interventions. It was therefore decided that a mixed-method systematic review would be the most appropriate way to address the issues of interest. The review objectives, questions and a protocol were then developed to guide the review. Searches were conducted between January and April 2012.

The review followed the University of York Centre for Research and Dissemination principles for conducting searches and extracting data (Centre for Reviews and Dissemination, 2009). A thematic synthesis approach was used to synthesise qualitative data, informed by the work of Thomas and Harden (2008), while narrative summary was used to synthesise evidence within the intervention, policy and economic streams of evidence (Centre for Reviews and Dissemination, 2009). Narrative synthesis was used for the overarching synthesis of different types of evidence (Centre for Reviews and Dissemination, 2009; Oliver et al, 2005).

An adapted Evidence for Policy and Practice Information and Co-ordinating Centre (EPPI-centre) design and methodology for mixed-method evidence was used to synthesise diverse evidence (EPPI-Centre, 2010). Evidence was streamed by evidence and methodology type and results were then synthesised across the streams in a final overarching synthesis (see figure 3.1).

A full audit trail was recorded during each stage of the review to enable replicable methods and outcomes. During the screening process each study was screened independently by myself and a second reviewer, as per systematic review good practices. Where reviewer opinions differed on the inclusion of a specific study, discussion was used to reach a consensus on whether the study should be included. The second reviewer also extracted data and appraised the quality of a selection of intervention studies (n=5) to check for reporting bias and errors. The findings showed good consensus between the two reviewers, although full appraisal of inter-rater reliability was not conducted due to time constraints.

3.4.2. Search methods

The main strategy for identifying studies was internet reference database searching. Inclusion and exclusion criteria were used to refine searches. Searched databases included the Cochrane

Collaboration Register and Library, Science Direct, CINAHL, Medline, ASSIA, PsychINFO, PubMed, Web of Science, DARE, NHS EED and HTA.

As wheelchair interventions have developed significantly in recent times it was deemed appropriate to restrict the intervention, opinion and economic literature searches to the last 15 years (February 1997 to February 2012). Reference list and hand-searching supplemented electronic searching. Grey literature searching was also included to limit publication bias. Due to limited translation resources, only studies written or translated into English (UK and international) were considered for inclusion. Search results were managed using the online RefWorks software.

Policy and NFPO literature was not available on academic databases and thus was identified through internet search engines (Google, Google Scholar), DoH/relevant NFPO websites and through hand-searching. Only UK policy/NFPO literature from the last 10 years (March 2002 to March 2012) was considered for inclusion to limit out-dated literature being included in the review. Although international literature was included in the other streams, it was deemed too expansive to include all international policy in this review. Nonetheless, UK policy is evidence-based including international evidence.

Search terms and keywords were a mixture of Medical Subject Heading (MeSH) and non-MeSH terms. A full list of search terms/keywords can be seen in table 3.1, and an example search strategy can be seen in appendix D.1. In order to increase search sensitivity, intervention/opinion evidence search terms were divided into three groups: 'population', 'disability' and 'intervention' (see table 3.1). In the economic evidence searches an additional search term group was added: 'study type/outcome measures'. As the aim of the mixed-method search was to find all relevant evidence, it was not necessary to explicitly define the types of studies and outcomes to be included in the review. The searches were designed to be sensitive rather than specific. Testing of search terms in the initial scoping searches was used to refine search terms and to test sensitivity prior to starting the full review.

Table 3.1: Keywords for intervention, opinion and economic evidence searches

Population	Disability	Intervention	Study type / outcome measures (economic evidence searches only)
child*	disab*	wheelchair	cost benefit
adolescen*	physically impair*	buggy	cost utility
young*	physical impair*	mobility technolog*	cost effective*
teen*	handicap*	mobility aid	qaly
disab* child*	dystroph*	powered wheelchair	quality-adjusted life year
disab* adolescen*	cerebral palsy	mobility equipment	quality adjusted life year
disab* young*	spina bifida	motorised	health economic*
disab* teen*	wheelchair*	mobility training	economic analys*
	special needs	wheelchair service	cost minimisation
	amputee	electric scooter	health care cost*
	complex needs	pushchair	healthcare cost*
	brain injury	mobility	social economic*
	brain damage*		social care economic*

Searches focused on manual and powered wheelchairs specifically due to the volume of recent inquiries in the UK into wheelchair services; their relatively high cost; and the unique benefits they provide to disabled children. Economic evidence searches were carried out separately to the intervention/opinion searches in order increase specificity. A full list of inclusion/exclusion criteria can be found in appendix D.2, with outcomes of interest specified.

3.4.3. Screening

Three stages of screening were used. During the initial screening process all duplicates were removed and identified study titles were screened for relevance. A second screening process was used to assess relevance of remaining studies by their abstract. When relevance was unclear the full study was obtained and reviewed. All relevant studies after the initial and second screening were obtained in full and screened a final time. To reduce bias a second researcher reviewed each study independently, we then reconvened to reach consensus about inclusion of studies. I did not formally screen the policy literature, as documents/publications were identified using search engines and relevant NFPO websites. Searching stopped once saturation had been reached and no new policy/guideline reports were found.

3.4.4. Data abstraction

Basic information (author, publication year, title) was collected for all studies. Additionally, I developed specific data extraction tools for each of the different types of literature. Each tool was designed for a specific type of evidence, which allowed the extraction of data to be representative of each stream of evidence. See appendix D.3 for a full list of data extraction criteria by evidence type, and appendix D.4 for data extraction tools. Summary measures could not be used across the intervention evidence due to differences in sample demographics, outcome measures and interventions.

3.4.5. Critical appraisal of quality

All studies were critically appraised using an appropriate quality appraisal tool. Due to the lack of evidence in this field critical appraisal was used to order evidence rather than exclude it. Only studies deemed to have major flaws or bias were eligible for outright exclusion. No studies were excluded on this basis. Full quality appraisal outcomes are presented in appendix D.5.

3.4.6. Evidence synthesis

Evidence was divided into four streams according to methodology and topic to enable separate syntheses by evidence type (see figure 3.1):

1. Intervention Evidence: all quantitative studies determining the effectiveness and outcomes of relevant interventions.
2. Opinion Evidence: all studies exploring perspectives and views relating to relevant interventions in childhood disability.
3. Policy and NFPO Literature: all relevant policy, NFPO and clinical guideline literature.
4. Economic Evidence: all relevant economic and cost-effectiveness evidence.

Intervention and economic streams were not statistically synthesised due to vast differences in studies and lack of comparable statistical evidence within each stream, thus narrative summary was conducted. Intervention evidence outcomes were grouped by type.

For the qualitative opinion evidence, thematic synthesis (Thomas and Harden, 2008) was conducted to identify the key themes expressed by service users and professionals regarding wheelchair provision and interventions. This process included three stages:

1. Line-by-line coding of findings to order the findings into initial codes.
2. Grouping of initial codes to form broader descriptive themes.

3. An overarching synthesis of the descriptive themes to create higher-level analytical themes.

Survey data that could be coded (such as open-ended questions) was incorporated into the thematic synthesis. For survey evidence that could not be line-by-line coded, narrative summary was used to form a structured narrative of results. These data were later synthesised with the thematic synthesis findings and incorporated into the appropriate descriptive themes.

A final over-arching narrative synthesis was undertaken to draw together the results across the different streams of evidence. I used the overarching synthesis framework developed by Oliver et al (2005) to structure this synthesis and make comparisons across the streams of evidence. To facilitate this three over-arching questions were developed (see figure 3.1).

3.4.7. Conceptual framework development

A conceptual framework for developing cost-effective wheelchair services for children and young people was refined from the overarching synthesis of evidence. Findings from the different streams of evidence were discussed in detail by the wider supervisory team then integrated, mapped, charted and refined through further discussion within the research team to build a deeper understanding.

In order to address the overarching synthesis question it was necessary to synthesise all four streams of evidence. The economic data was incorporated into the intervention stream due to the lack of evidence to warrant a separate stream. By exploring the extent to which the needs and desires of service users were reflected in policy and effectiveness data, it was possible to see how these three different aspects were related, and whether services reflected what was important to service users. Using the descriptive themes generated in the opinion evidence synthesis, an overarching *a priori* framework (Oliver et al, 2005) was developed to explore how opinion evidence regarding the facilitators of wheelchair use was reflected in effectiveness and policy evidence. The findings from the qualitative and quantitative data were synthesised using qualitative analysis methods (Thomas and Harden, 2008) to allow greater development of the findings.

The purpose was to create a conceptual framework highlighting the interplay between different aspects of wheelchair provision and use, thus reflecting the relationship between the needs of service users, the evidence of effectiveness and the policy guidelines . The resultant findings were then used to generate recommendations for how services and outcome measures can better reflect the needs of service users and carers, whilst promoting the most effective interventions. The lack of economic evidence highlights the pressing need to establish which services and interventions are

cost-effective and framed the wider context of applying health economics to wheelchair provision for disabled children.

The first step was to consider the parameters of the health system and to define the parameters of the conceptual framework. This required an exploration of the relationship between the different streams of evidence and where there were potential gaps in knowledge or areas where knowledge was not being successfully translated into practice. The systematic review findings were used to develop the conceptual framework . The second phase was to develop a detailed chart of data, which were organised by *a priori* themes found in the overarching synthesis. All relevant data for each theme were mapped on to the chart and used to examine data at a higher conceptual level mapped against the developing conceptual framework. Discussion amongst the supervisory team was used to further explore emergent findings and to develop consensus about the underlying themes and concepts across the streams of evidence. Thirdly, the most important findings were selected based on respective importance across the streams and integrated into a conceptual diagram. Finally, the findings were reviewed again to identify current gaps in evidence and knowledge. This was used to highlight on the conceptual framework where stages of action and development were currently needed to address specific issues within the body of evidence.

The conceptual framework was used to illustrate areas for service development, gaps in knowledge and required actions for current services. This was based on findings developed as part of the overarching synthesis, which highlighted the barriers and facilitators to effective wheelchair provision and service development, and the relationship between service user opinions and effectiveness evidence. The conceptual framework was therefore used to draw together the salient concepts found in the systematic review and provide a coherent interpretation of the relationship between the existing data in this topic area.

3.5. Results

3.5.1. Search and screening outcomes

A full list of included studies can be found in appendix D.6, and a full list of studies excluded at the full-text screening stage can be found in appendix D.7. See figures 3.2 and 3.3 for the screening process outcomes. In total 4144 studies were found in the intervention/opinion evidence searches, of which 2393 duplicates were removed. After screening titles and abstracts, 76 full-texts were left. In total a further 56 were excluded after screening of full-texts, leaving 20 deemed eligible for inclusion: 10 in the intervention evidence stream and 14 in the opinion evidence stream (four

studies were eligible for both streams of evidence). Reasons for exclusion included focus on adults (or inability to extract child data), lack of primary data and focus on biomechanical outcomes.

In total 389 studies were found in the economic evidence searches, of which 163 duplicates were removed (see figure 3.3). After screening titles and abstracts, seven full-texts were left. In total two were deemed eligible for inclusion. Reasons for exclusion included focus on adults and lack of primary data. In total 14 policy and NFPO reports were deemed eligible for inclusion.

Evidence could not be grouped and analysed using meta-analysis due to heterogeneity of samples, methodology, interventions and outcomes (see appendix D.6 for evidence of heterogeneity). Summary outcomes and synthesis of statistical data were also inappropriate due to heterogeneity. Narrative summary was conducted to form a structured narrative of the results.

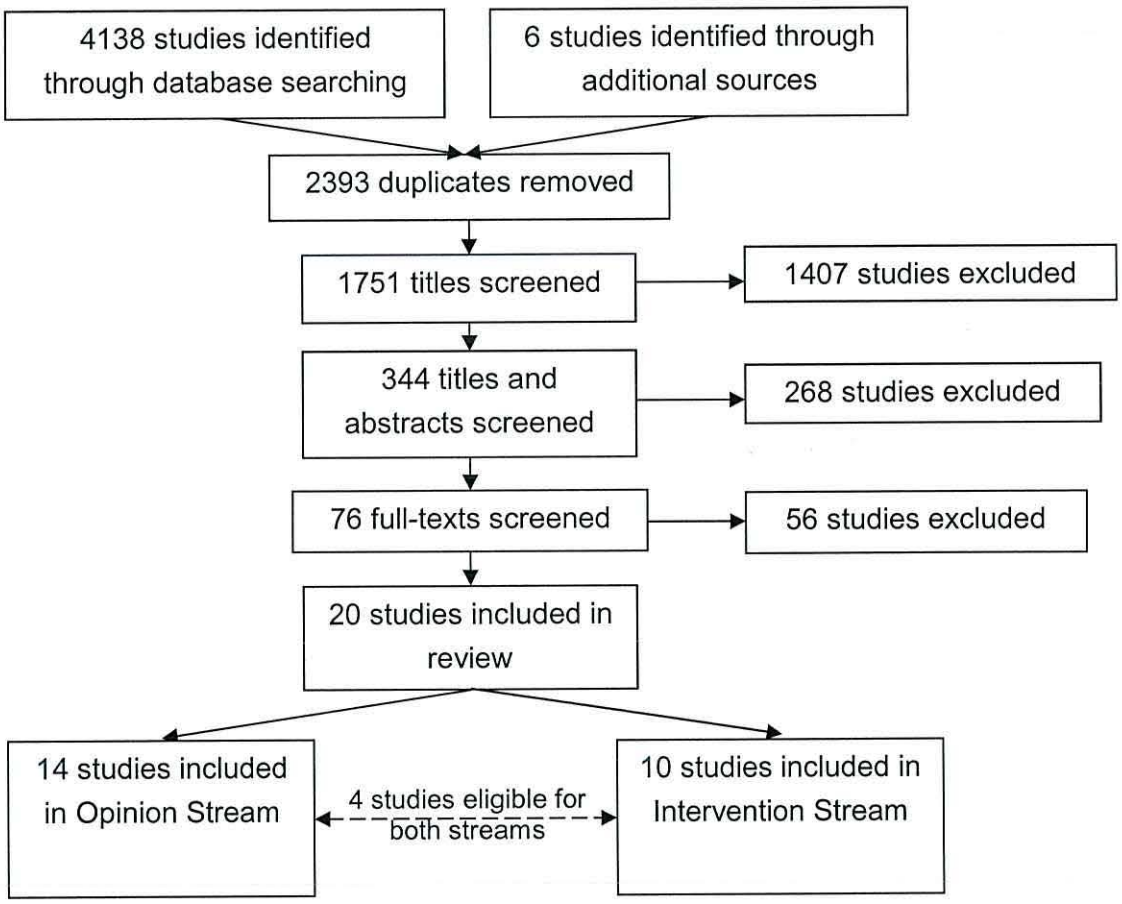


Figure 3.2: Search results for intervention and opinion evidence

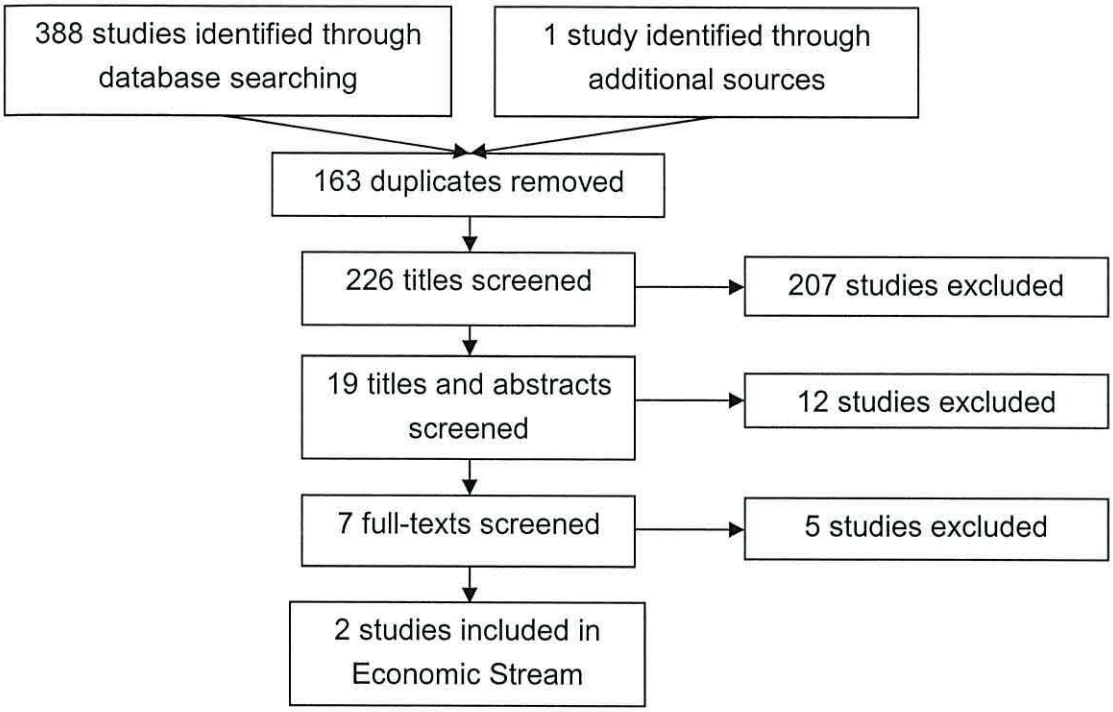


Figure 3.3: Search results for economic evidence

3.5.2. Intervention evidence

Ten studies explored the effectiveness of wheelchairs for disabled children: Seven determined the effectiveness of PWCs (Jones et al, 2003; Furumasu et al, 2008; Jones et al, 2012; Tefft et al, 2011; Bottos et al, 2001; Deitz et al, 2002; Huhn et al, 2007); one compared ultralight and lightweight manual wheelchairs (MWCs) (Meiser and McEwan, 2007); and two more broadly examined the impact of assistive devices/environmental modifications (including PWCs) (Østensjø et al, 2005; Benedict et al, 1999).

Of the 10 studies, five looked specifically at children with cerebral palsy and orthopaedic disabilities (Furumasu et al, 2008; Tefft et al, 2011; Bottos et al, 2001; Huhn et al, 2007; Østensjø et al, 2005). The remainder included children with a range of disabilities such as complex developmental delay (Deitz et al, 2002), spinal muscular atrophy (Jones et al, 2003), spina bifida (Meiser and McEwan, 2007) and motor impairment preventing functional independent mobility (including conditions such as cerebral palsy) (Jones et al, 2012; Benedict et al, 1999). Child participant ages ranged from 14 months to 12 years.

Only one randomised controlled trial (RCT) was found (Jones et al, 2012). A range of other methodologies were used, including case study, case series, quasi-experimental design, 'A-B-A' single subject design, single-subject withdrawal design and cross-sectional survey.

Only four studies employed statistical analysis and sample sizes were small; several studies used a single case-study design. The single RCT (Jones et al, 2012) was of moderate quality with a small sample size [N=28] (equally split between intervention and control groups). Outcome measures used within each study are presented in appendix D.6.

Quality appraisal of studies indicated that they were generally low quality (see appendix D.5). Risk of bias was assessed as part of the critical appraisal outcomes. The intervention evidence results are therefore presented with caution, taking into account the quality of results and potential risk of bias.

The vast majority of the evidence was in reference to PWCs, and thus understanding of MWC and adaptive buggy effectiveness is limited. There was some evidence to indicate that ultralight wheelchairs are easier to propel than lightweight wheelchairs (Meiser and McEwan, 2007).

The intervention findings were grouped by outcome/benefit and categorised using narrative summary. Statistical significance is presented where reported. The emergent categories of benefit were:

3.5.2.1. Caregiver assistance and benefits

PWCs reduce need for caregiver assistance (Jones et al, 2003; Jones et al, 2012; Østensjø et al, 2005) and reduce caregiver stress (Tefft et al, 2011). PWCs have statistically significant effects on need for caregiver assistance for mobility (p=.01, effect size [ES]=12.35 [6.5-20.5] at 90%CI) and self-care (p=.0007, ES=11.95 [7.5-16.15] at 90%CI) (Jones et al, 2012).

3.5.2.2. Social and play skills

For children with orthopaedic disabilities aged 18 to 72 months PWCs significantly positively affect: pro-social adaptive social behaviour (F=5.30, p<.05 at 95%CI); interactions with family (F=3.2, p<.05 at 95%CI); indoor play motor activities (F=4.53, p<.05 at 95%CI); quality of interactive play (F=4.24, p<.05 at 95%CI); and developmental level of symbolic play (F=4.9, p<.05 at 95%CI) (Furumasu et al, 2008).

For children with orthopaedic disabilities aged 18 to 42 months PWCs also facilitate significant improvements in: interactions with family (F[2,21]=3.3, p<.05); parental satisfaction with child's social and play skills (F[2,21]=3.27, p<.05); and parents' belief that the general public accepts their child (F[2,21]=3.65, p<.04) (Tefft et al, 2011).

3.5.2.3. *Functional movement and mobility*

PWCs improve functional mobility (Jones et al, 2003; Jones et al, 2012) and child-initiated movement (Deitz et al, 2002), with significant impacts on mobility functional skill (p=.04, ES=6.5 [2-11] at 90%CI) (Jones et al, 2012) and parental satisfaction with child's ability to go where they desire ($F[2,21]=11.69$, $p<.05$) (Tefft et al, 2011).

3.5.2.4. *Developmental benefits*

PWCs offer developmental benefits in: communication, cognition and personal-social domains (Jones et al, 2003); receptive communication skills (Jones et al, 2012); and occupational performance (Bottos et al, 2001). PWCs can significantly improve: activities of daily life (in the dimension of functional limitation) ($p<0.00001$) (Bottos et al, 2001); receptive communication (p=.03, ES=6.1 [0.95-9.2] at 90% CI) and overall development (p=.083, ES=2.0 [0.0-3.5] at 90%CI) (Jones et al, 2012).

3.5.2.5. *Driving skill and competence*

Children as young as 14 months can learn some degree of PWC driving competence (Huhn et al, 2007). PWC driving competence improves after six to eight months of use ($p<0.01$) for children with cerebral palsy aged three to eight years (Bottos et al, 2001).

3.5.3. Opinion evidence

Fourteen studies explored the opinions and perspectives of young wheelchair users, their parents/carers and related professionals (e.g. clinicians, teachers, therapists). Seven studies were related specifically to PWCs (Tefft et al, 2011; Wiart et al, 2003; Durkin, 2009; Guerette et al, 2005; Home and Ham, 2003; Staincliffe, 2003; Wiart et al, 2004) and six were related to both manual and powered wheelchairs (Østensjø et al, 2005; Benedict et al, 1999; Evans et al, 2007; Lawlor et al, 2006; Shahid, 2004; Curtin and Clarke, 2005). The majority of studies explored physical disabilities generally in children, although four of the studies looked specifically at children with cerebral palsy and orthopaedic disabilities (Tefft et al, 2011; Østensjø et al, 2005; Lawlor et al, 2006; Shahid, 2004).

Most of the participants were families of disabled children (child age range from 18 months to 18 years), although four studies also included professional participants (e.g. wheelchair suppliers, teaching staff, therapists, clinicians) (Durkin, 2009; Guerette et al, 2005; Staincliffe, 2003; Shahid, 2004) and four directly included the opinions of disabled children and young people (Wiart et al, 2003; Evans et al, 2007; Lawlor et al, 2006; Curtin and Clarke, 2005). Five studies used qualitative methodologies exclusively (including phenomenology and grounded theory) (Wiart et al, 2004;

Durkin, 2009; Evans et al, 2007; Lawlor et al, 2006; Curtin and Clarke, 2005), while the rest used either survey data (quantitative and qualitative), retrospective research or cross-sectional research.

Twelve descriptive themes were generated from line-by-line coding of the evidence (see table 3.2), which were then synthesised to make higher-order analytical themes. Analytical theme generation was focussed on PWCs due to the focus of the qualitative evidence. Making broader assumptions about other forms of assistive mobility technology (e.g. MWCs and pushchairs) would have been inappropriate due to the lack of evidence.

Table 3.2: Descriptive themes generated from opinion evidence

Descriptive Themes	Examples
Wheelchair services	Providers, repair and maintenance
Environmental factors	Home, public and school environment
Chair characteristics	Size, weight and usability
Individual ability	Health, physical and developmental readiness
Family factors	Attitude, support and finances
Safety of use	Build quality, accidents and safe use
Learning to use wheelchair	Learning mobility and wheelchair safety
Social factors	Socialisation, participation and others' attitudes
Quality of Life	Self-esteem, confidence and well-being
Physical factors	Comfort, support and positioning
Independence	Freedom and independent movement
Developmental impact	Attaining milestones

In total, five analytical themes were developed:

3.5.3.1. Wheelchair services do not consistently meet all needs of service users, and parents are resigned to this

Identified wheelchair service issues included long waiting times (Evans et al, 2007; Lawlor et al, 2006), poor maintenance procedures (Home and Ham, 2003; Evans et al, 2007; Lawlor et al, 2006), strict eligibility criteria (Home and Ham, 2003) and differing opinions of needs (Wiart et al, 2003; Wiart et al, 2004). The evidence demonstrated service-user resignation to current standards of provision, as services were perceived to be doing all that they possibly could (Evans et al, 2007).

Studies highlighted issues around lack of information provision with regards to choice of wheelchairs, potential wheelchair benefits and funding available to families (Home and Ham, 2003; Lawlor et al, 2006; Shahid, 2004). The evidence highlighted the financial burden placed on families

who purchase essential equipment and adaptations privately (Home and Ham, 2003; Wiart et al, 2003; Lawlor et al, 2006).

3.5.3.2. Parents find it difficult to accept their child's need for a wheelchair

For parents, accepting their child's wheelchair use was perceived as an admission that independent mobility would never be achieved without technological aid (Wiart et al, 2004). Parents felt that they had to come to terms with their child's use of both manual and powered wheelchairs. Many parents had negative perceptions of wheelchairs prior to their child receiving one (Wiart et al, 2004; Guerette et al, 2005). Results indicated that 84% (of n=25) of parents did not accept the idea of a PWC before provision, but 92% (of n=25) had positive feelings after PWC provision (Bottos et al, 2001). This demonstrates that a process of adjustment is required. 23% (of n=140) of wheelchair clinicians and suppliers felt that a lack of family support had a negative impact on wheelchair provision (Guerette et al, 2005).

3.5.3.3. PWCs are a tool for independence and socialisation

PWC use can help to promote independence in disabled children (Wiart et al, 2004; Bottos et al, 2001; Home and Ham, 2003; Lawlor et al, 2006), which subsequently allows greater socialisation (Evans et al, 2007). It was found that the use of a PWC had a positive effect on the attitudes of others (Wiart et al, 2004; Home and Ham, 2003) with people seeing the child as an individual in their own right (Wiart et al, 2004). This in turn allowed further socialisation and participation in age-appropriate activities due to acceptance by peers and the wider community (Wiart et al, 2004).

3.5.3.4. Wheelchairs offer a new lifestyle to disabled children and their families

Wheelchairs were perceived to offer a new lifestyle for disabled children and their families (Wiart et al, 2004; Bottos et al, 2001; Benedict et al, 1999; Home and Ham, 2003; Evans et al, 2007). PWCs were believed to provide improvements to QoL (compared to no wheelchair equipment and MWCs) (Tefft et al, 2011; Home and Ham, 2003); ability to take part in age-appropriate activities and responsibilities (Wiart et al, 2004; Evans et al, 2007); and overall freedom (Wiart et al, 2003). After PWC provision children were able to socialise more (Wiart et al, 2004; Home and Ham, 2003; Wiart et al, 2003; Evans et al, 2007; Lawlor et al, 2006); to integrate better into school and community settings (Evans et al, 2007); and were less reliant on the help of others (Wiart et al, 2004). Parents acting as 'responsive partners' facilitate children to learn how to use a PWC (Durkin, 2009).

3.5.3.5. *Structural and environmental barriers restrict wheelchair use*

Poor access to buildings (Wiart et al, 2004; Østensjø et al, 2005; Wiart et al, 2003; Lawlor et al, 2006), difficulty transporting wheelchairs (Tefft et al, 2011; Wiart et al, 2004; Østensjø et al, 2005; Guerette et al, 2005; Wiart et al, 2003; Evans et al, 2007; Shahid, 2004) and poor disabled parking facilities (Wiart et al, 2003; Lawlor et al, 2006) were identified barriers to wheelchair use. Community and social environments were reported to often be unfit for wheelchair access (Wiart et al, 2004; Wiart et al, 2003; Evans et al, 2007; Lawlor et al, 2006). The size and bulk of wheelchairs was believed to limit integration with peers as well as affecting use and transportation (Lawlor et al, 2006; Curtin and Clarke, 2005).

3.5.4. Policy and Guidelines

Fourteen policy and NFPO reports were included in the review: three were produced by NFPOs (Muscular Dystrophy Campaign, 2010; Muscular Dystrophy Campaign, 2011; Barnardos and WK, 2006); 10 were produced by UK government and DoH organisations (Welsh Assembly Government, 2005; DoH Commissioning Team, 2010; National Assembly for Wales, 2010; Audit Commission, 2002; NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2005; DoH, 2004; Care Services Improvement Partnership [CSIP], 2006; HM Treasury and Department for Education and Skills [DES], 2007; Scottish Executive, 2006); and one was a joint publication produced by the UK government and an NFPO (WK, 2011).

Findings from the policy and NFPO evidence were grouped by type of recommendation/target. Seven emergent categories were identified:

3.5.4.1. *Waiting times*

The most commonly identified recommendation was reduction of waiting times for assessment, delivery and maintenance of wheelchairs (e.g. maximum of 18 weeks from referral to delivery (Muscular Dystrophy Campaign, 2010)) (Welsh Assembly Government, 2005; DoH Commissioning Team, 2010; Audit Commission, 2002; NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2004; DoH, 2004; Muscular Dystrophy Campaign, 2010; Barnardos and WK, 2006; CSIP, 2006).

3.5.4.2. *Joint-working and multi-agency approach*

The need for joined-up working between health, social care, education and NFPOs was a recurrent theme throughout the literature, with a general aim to improve services and to extend the scope of provision (Welsh Assembly Government, 2005; DoH Commissioning Team, 2010; National Assembly for Wales, 2010; Audit Commission, 2002; Prime Minister's Strategy Unit, 2005; DoH, 2004). This

included pooling of budgets (National Assembly for Wales, 2010) and outsourcing training/tuition (DoH Commissioning Team, 2010).

3.5.4.3. Effective use and outcomes

Several publications highlighted the need for wheelchairs to be useable in all places required in order to maximise effectiveness (Welsh Assembly Government, 2005; Audit Commission, 2002; DoH, 2004). There were recommendations for assessment and provision to take into account the holistic needs of service users as part of maximising social, physical and lifestyle outcomes and promoting independence (DoH Commissioning Team, 2010; National Assembly for Wales, 2010; Audit Commission, 2002; DoH, 2004; Muscular Dystrophy Campaign, 2010).

3.5.4.4. Funding and procurement

Recommendations included: ring-fenced budgeting for PWC provision (Muscular Dystrophy Campaign, 2010); improved efficiency, productivity and innovations in the NHS wheelchair product line (DoH Commissioning Team, 2010); pooling of budgets between health, social care and education authorities (National Assembly for Wales, 2010); and efficient procurement, long-term cost control and initial investment (WK, 2011). Productivity savings should be re-invested into wheelchair and seating provision (DoH Commissioning Team, 2010).

3.5.4.5. Aftercare and information

Maintenance and review procedures need attention, with clear and defined minimum standards for reviews (Welsh Assembly Government, 2005; NHS Modernisation Agency, 2005; DoH, 2004). Service users require better information regarding grants, tuition and other relevant local services (Welsh Assembly Government, 2005; Prime Minister's Strategy Unit, 2005).

3.5.4.6. Eligibility criteria and assessment

Comprehensive access to multi-disciplinary assessments was of high priority (Prime Minister's Strategy Unit, 2005; DoH, 2004; Barnardos and WK, 2006; HM Treasury and DES, 2007). Extended equipment loan programmes (HM Treasury and DES, 2007) and national consensus of eligibility criteria/outcomes were also recommended (NHS Modernisation Agency, 2005; Scottish Executive, 2006).

3.5.4.7. *Service user involvement*

Recommendations included: designing services around the needs of service users (DoH Commissioning Team, 2010; Barnardos and WK, 2006; HM Treasury and DES, 2007); supporting service users to make informed decisions about their care (DoH, 2004; CSIP, 2006); and improving communication with users and stakeholders (National Assembly for Wales, 2010).

3.5.5. Economic evidence

Two eligible studies exploring the cost-effectiveness of wheelchairs for disabled children were found. Due to the lack of evidence and the heterogeneity of data (cost, year, outcomes, interventions etc.) it was not possible to synthesise the findings, therefore narrative summary was conducted.

Neilson et al (2000) found the cost per QALY (compared with a 'do nothing' scenario) for provision of a powered indoor/outdoor wheelchair ranged from £734 to £1378 (dependent on time horizon) based on a cost per wheelchair intervention ranging from £1500 to £2000. These results indicate that PWC interventions can be cost-effective in relation to the NICE £20,000 to £30,000 intervention cost threshold. However, estimates are based on a single subject within the study, whose age is not stated. Furthermore, costs used to generate QALYs were based on a single intervention over a 40 or 50 year time horizon, which is an unrealistic time horizon for this type of intervention.

Frontier Economics (Frontier Economics, 2011) examined the impact of NFPO (WK) involvement in the running of NHS Primary Care Trust wheelchair services for children using social return on investment analysis. Meeting unmet service demands cost an extra £108,000 and provided an additional 10.7 to 14 QALYs, resulting in a cost per QALY of between £7,700 and £9,800 for meeting unmet service demands. However, the source of utility data is not stated explicitly and the evidence has not been published by a peer reviewed journal, thus its application in this review is limited.

3.5.6. Over-arching synthesis

The majority of data were specifically related to PWC provision and use, which is reflected in the over-arching synthesis. A number of additional findings were elicited from further synthesis of the entire integrated dataset:

3.5.6.1. *Higher quality wheelchair services take into account the needs of the whole family*

Intervention and opinion evidence shows that wheelchair provision can be beneficial for both the wheelchair user and their family, for instance allowing parents to be more independent (Benedict et al, 1999; Evans et al, 2007); reduced need for caregiver assistance (Jones et al, 2003; Østensjø et al,

2005; Benedict et al, 1999); facilitation of positive parental feelings (Bottos et al, 2001); and reduction in parental stress (Tefft et al, 2011).

As use of a PWC requires family involvement it is important that access to the home and the ability to transport a wheelchair is assessed and facilitated where possible. The cost of maintenance, repairs and adaptations can be prohibitive for families (Wiart et al, 2003; Lawlor et al, 2006), thus funding arrangements at policy level should ensure that these costs are covered or available grants are signposted (Welsh Assembly Government, 2005; Muscular Dystrophy Campaign, 2010).

Each service user may benefit from having a clear point of contact for any queries they may have (Welsh Assembly Government, 2005; NHS Modernisation Agency, 2005; DoH, 2004). Services should be developed in consultation with children and families to promote patient-centred services (DoH Commissioning Team, 2010; CSIP, 2006).

3.5.6.2. Children benefit when psychosocial needs are considered alongside health needs

The psychosocial needs of children using PWCs appear to be of highest priority for service users and their parents (Wiart et al, 2004; Home and Ham, 2003; Wiart et al, 2003; Evans et al, 2007; Lawlor et al, 2006). Children benefit more when services ensure that any supplied PWC can be used in all places it is required (Welsh Assembly Government, 2005; DoH, 2004; Audit Commission, 2002). PWCs offer a range of social benefits, including increased interactions with family (Tefft et al, 2011) and pro-social adaptive social behaviour (Furumasu et al, 2008). A holistic approach to assessment, with outcomes measures which consider psychosocial, environmental, lifestyle and clinical needs are therefore important (DoH Commissioning Team, 2010; National Assembly for Wales, 2010). Additional benefits and efficiencies were also noted from joined-up working and planning between health, social services and education departments (Welsh Assembly Government, 2005; DoH Commissioning Team, 2010; National Assembly for Wales, 2010; Audit Commission, 2002; Prime Minister's Strategy Unit, 2005; DoH, 2004; HM Treasury and DES, 2007).

It is of note that the majority of opinion evidence (n=9) related to children aged under 14 years. This indicates that there may be a lack of evidence on key periods of transition, such as moving from child to adult wheelchair services.

3.5.6.3. Disabled children could benefit if policy recommendations focussed on services meeting individual needs rather than following strict eligibility criteria

Inefficiencies (such as long waiting times) need to be reduced (National Assembly for Wales, 2010; Evans et al, 2007; Lawlor et al, 2006) and loan programmes developed to allow children to try

wheelchairs before provision (Guerette et al, 2005). Strict eligibility criteria can prohibit each child receiving the correct wheelchair for them (Audit Commission, 2002; DoH, 2004; Home and Ham, 2003), thus uniform and flexible national eligibility criteria may help to address differences within and between services (NHS Modernisation Agency, 2005; Muscular Dystrophy Campaign, 2010; Scottish Executive, 2006). Joined-up working between agencies could further enhance services (Welsh Assembly Government, 2005; DoH Commissioning Team, 2010; National Assembly for Wales, 2010; Audit Commission, 2002; Prime Minister's Strategy Unit, 2005; DoH, 2004; HM Treasury and DES, 2007).

3.5.6.4. Without appropriate outcome measures the holistic benefits of PWC interventions cannot be evaluated

Evidence of effectiveness and validated clinical outcome measures are needed in all aspects of health services (DoH, 2008). The development of child-specific and reliable measures of holistic/generic benefits are needed in order to effectively measure the wider benefits of PWC interventions. With appropriate outcome measures PWC interventions cannot be assessed on their ability to provide the tangible benefits of developmental gains.

Opinion evidence highlighted the importance of independence to service users and families (Bottos et al, 2001; Wiart et al, 2004; Home and Ham, 2003; Lawlor et al, 2006). A range of developmental benefits were found in the intervention and opinion evidence (Jones et al, 2003; Jones et al, 2012; Wiart et al, 2004; Durkin, 2009; Lawlor et al, 2006). Opinion evidence highlighted the potential QoL benefits of PWCs, including reduced frustration and increased enjoyment of life, happiness, motivation and self-confidence (Home and Ham, 2003), and furthermore increased activities of daily living (Wiart et al, 2004).

3.5.6.5. Children may benefit more when physical outcomes of PWC use are seen as facilitators to wider holistic benefits, but lack of translation of evidence into practice hinders progress

The key wheelchair outcomes for service users and their families were lifestyle, social and independence effects, which was also reflected in the policy and NFPO literature. Recommendations highlight the need to set minimum standards for wheelchairs that are useable in all places required (Welsh Assembly Government, 2005; Audit Commission, 2002; DoH, 2004) and that promote independence (Audit Commission, 2002; Muscular Dystrophy Campaign, 2010) with measurable outcomes. However the translation of these recommendations into practice is apparently weak.

3.5.6.6. *Children would benefit from public buildings and spaces that promote inclusion of disabled people*

Policy and NFPO literature states that wheelchairs should be useable in all places needed (e.g. school, home and leisure) (Welsh Assembly Government, 2005; Audit Commission, 2002; DoH, 2004), however this is in reference to wheelchair provision rather than accessible public places. Poorly designed public spaces restrict children's ability to participate socially (Østensjø et al, 2005; Wiart et al, 2003; Wiart et al, 2004, Evans et al, 2007; Lawlor et al, 2006; Curtin and Clarke, 2005). Additional focus on legally enforced equality of access is therefore likely to improve wider lifestyle benefits of wheelchair users.

Home adaptation, with signposting to all funding and grant entitlements, is also important to facilitate improved QoL outcomes (Welsh Assembly Government, 2005), as some families have issues using wheelchairs due to inaccessibility of the home environment (Guerette et al, 2005; Shahid, 2004). Regular review and maintenance procedures can help to ensure that wheelchairs are fit for purpose (NHS Modernisation Agency, 2005; Muscular Dystrophy Campaign, 2010).

3.5.7. Conceptual framework

The conceptual framework (see figure 3.4) was used to map how further research and service development can lead to cost-effective wheelchair services and interventions. It was developed to highlight areas which need development and where actions for improving both the effectiveness and cost-effectiveness of wheelchair services for children are required.

Areas for future development include:

Conducting and making available high quality effectiveness, cost-effectiveness and qualitative evidence: Although a range of effectiveness evidence was collated, there was a distinct lack of high quality evidence of effect; only one RCT (Jones et al, 2012) was found and all studies had small sample sizes. Furthermore, no robust evidence of cost-effectiveness was found. This demonstrates the need for more evidence and higher quality evidence. Without appropriate evidence the development of wheelchair services cannot be conducted in an evidence-based manner. By promoting the use of more robust research methods higher quality evidence could be utilised to guide wheelchair service development. At present the literature indicates that wheelchairs can offer a range of benefits, including social (Furumasu et al, 2008; Tefft et al, 2011), functional mobility (Jones et al, 2003; Tefft et al, 2011; Jones et al 2012) and developmental benefits (Bottos et al, 2001; Jones et al 2003; Jones et al 2012), however better quality evidence is needed to fully understand the effectiveness of various types of wheelchairs for disabled children.

Developing a knowledge translation framework: In order for appropriate evidence to be utilised effectively, there needs to be a standardised approach to adopting and translating knowledge into practice. For example, The DoH Commissioning Team (2010) specifically calls for eligibility criteria to be evidence based. The evidence from both the opinion and policy streams demonstrates that issues in wheelchair services have been well known for a number of years, with reports making similar recommendations time and time again. For instance, the need for prompt services and timely provision of equipment was continuously recommended in reports ranging from 2002 to 2010 (Audit Commission, 2002; DoH, 2004; Prime Minister's Strategy Unit, 2005; Welsh Assembly Government, 2005; Barnardos and WK, 2006; HM Treasury and DES, 2007; DoH Commissioning Team, 2010; National Assembly for Wales, 2010). The fact that these issues have been ongoing for at least a decade demonstrates that a formal process for realising change is needed. Therefore, a standardised approach to using knowledge and turning policy into practice should be implemented on a national scale.

Streamlining management and procurement strategies: The WK model for wheelchair service structuring demonstrated that NHS wheelchair services could reduce costs whilst improving standards (Frontier Economics, 2011). At present many wheelchair services maintain strict eligibility criteria due to tight budgets and lack of innovation in product lines (WK, 2011). Several policy and NFPO reports discussed the need to improve funding and procurement strategies through a number of strategies, including long-term cost control procedures(WK, 2011), reinvestment of productivity savings (DoH Commissioning Team, 2010), ring-fencing PWC budgets (Muscular Dystrophy Campaign, 2010), encouraging product and procurement innovation (DoH Commissioning Team, 2010), improving maintenance procedures (NHS Modernisation Agency, 2005; Muscular Dystrophy Campaign, 2010) and maintaining recyclability of equipment (Prime Minister's Strategy Unit, 2004; Welsh Assembly Government, 2005). However, the application of these recommendations is still to be seen on a national scale.

Developing appropriate outcome measures: From both the opinion and intervention evidence, it is clear that a range of beneficial outcomes are facilitated by wheelchair interventions. However, applying appropriate measurement of such varied outcomes is difficult. For example, several qualitative studies indicated that independence is of key importance (Bottos et al, 2001; Home and Ham, 2003; Wiart et al, 2004; Lawlor et al. 2006), however measuring independence in a robust and valid way is difficult. At present appropriate outcome measures to facilitate this are lacking. Likewise, service users discussed the quality of life benefits of appropriate wheelchair provision (Home and Ham, 2003; Wiart et al, 2003; Tefft et al, 2011), and yet no quality of life data was found

in the intervention evidence. This indicates the need for validated outcome measures which reflect the outcomes that service users prioritise.

Addressing environmental barriers to wheelchair use: Environmental barriers to wheelchair use can restrict a child's ability to use their equipment in the most effective manner possible. A number of qualitative studies highlighted issues associated with poorly designed public spaces (Wiart et al 2003; Wiart et al 2004; Østensjø et al, 2005; Lawlor et al, 2006) and home environments (Shahid, 2004; Guerette et al, 2005). The lifestyle and environmental context of wheelchair provision must therefore be accounted for (DoH, 2010; National Assembly for Wales, 2010; Muscular Dystrophy Campaign, 2011), and wheelchair services must improve information provision regarding grants for home modifications (Welsh Assembly Government, 2005).

Conducting robust cost-effectiveness analyses: No robust economic evaluations or evidence of cost-effectiveness were found in the economic evidence searches. To date there has been no NICE HTA guidance on wheelchairs for disabled children. There is currently a real gap in what is known about the cost-effectiveness of wheelchairs for children, and this is potentially holding back the supply of the most appropriate equipment to disabled children, both in terms of outcomes for service users and financial costs for wheelchair services. The issue of strict eligibility criteria for wheelchair provision was frequently highlighted in the opinion and policy evidence (Wiart et al, 2003; Wiart et al, 2004; NHS Modernisation Agency, 2005; CSIP, 2006; DoH Commissioning Team, 2010; Muscular Dystrophy Campaign, 2010; Whizz-Kidz, 2011). With appropriate economic evidence wheelchair provision based on arbitrary eligibility criteria could be reduced.

Ensuring continued service development with collaboration between third party, NHS, private services and service users: The benefits of joined-up working between health, social care, education and NFPOs was a recurrent theme throughout the policy literature (Welsh Assembly Government, 2005; DoH Commissioning Team, 2010; National Assembly for Wales, 2010; Audit Commission, 2002; Prime Minister's Strategy Unit, 2005; DoH, 2004). The real-world benefits of joined-up working were demonstrated in the partnership between NHS and WK (Frontier Economics, 2011). On a smaller scale, more collaboration between NHS, social services and education authorities could improve information provision for service users (Shahid, 2004; Lawlor et al, 2006) and encourage a more holistic approach to wheelchair provision (Staincliffe, 2003).

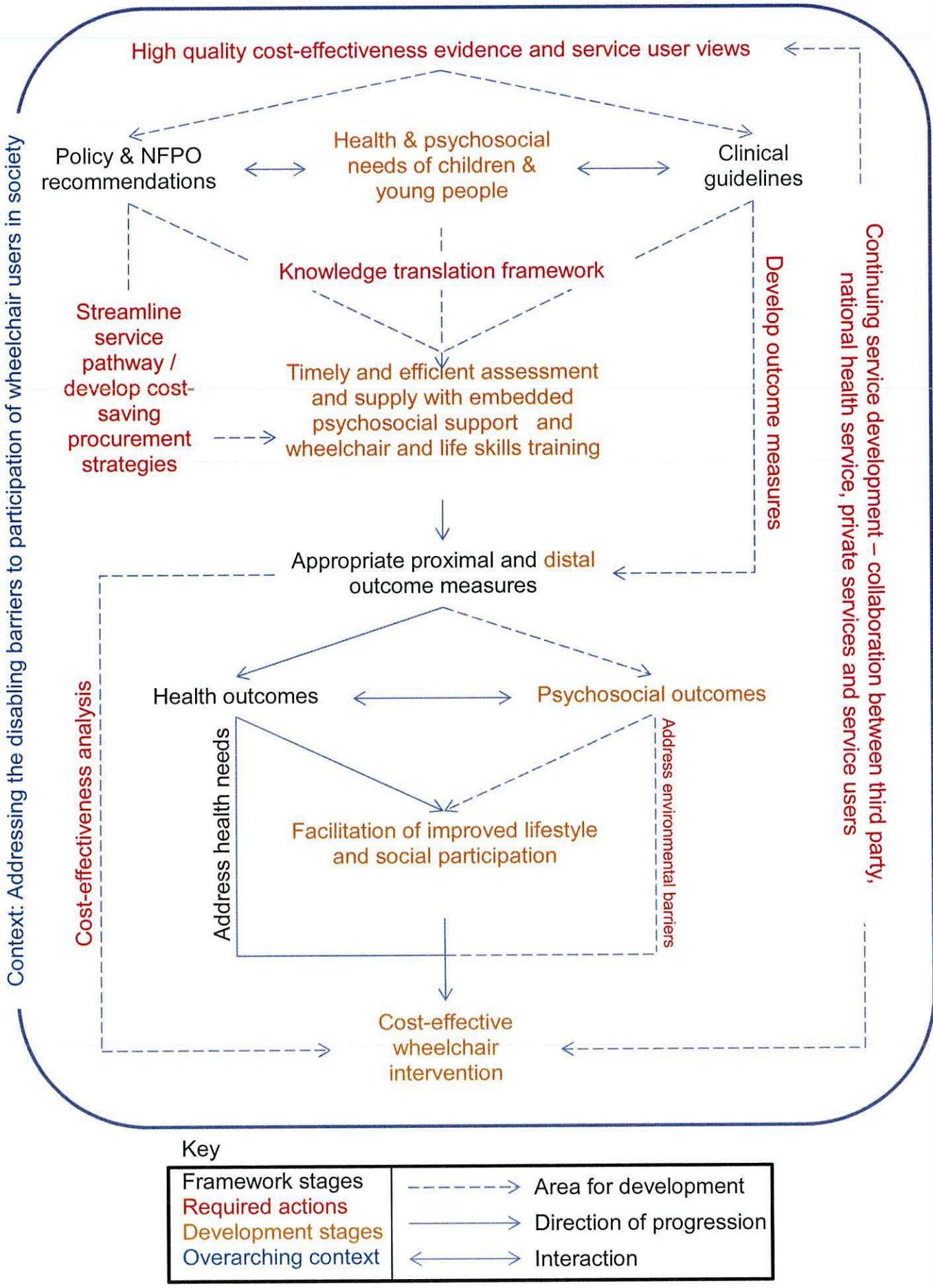


Figure 3.4: Conceptual framework for developing cost-effective wheelchair services for disabled children

3.6. Discussion

3.6.1. Main findings

The major contribution to knowledge from this novel mixed-method review comes from the synthesis of diverse evidence to form a new conceptual framework for optimal wheelchair service provision for children.

Within this overall context, the most important finding is that for disabled children wheelchairs are more than a means of mobility; they offer enhanced independence, social integration and participation in age-appropriate activities. Wheelchair interventions should be seen as facilitators to a new way of life. Nonetheless, disabled children and parents can find the transition to wheelchair use a traumatic process that is not yet sufficiently understood. Being able to individually tailor support for children and parents, and being able to measure these wider lifestyle benefits, is therefore a priority. Further research is needed to address these significant gaps in current knowledge.

To some extent UK policy and NFPO recommendations do reflect the perspectives of disabled children and their families, but at present there is a lack of effective knowledge translation to allow policy and evidence to sufficiently influence practice. Although policy recommendations do correlate with the opinion evidence, the barriers to effective provision and use of wheelchairs have continued to prevail in UK NHS services over many years (National Assembly for Wales, 2010; NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2005; Audit Commission, 2002). In order to improve outcomes for disabled children a range of service developments are needed, including consideration of how services are organised/delivered and the translation of knowledge of what works and what children want.

3.6.2. Translating knowledge into practice

The translation of evidence and knowledge into practice is not simply a case of publishing guidelines and policy. Evidence based practice requires specific action and commitment from services, for instance through the implementation of a knowledge translation framework such as the Knowledge to Action Process (Graham and Tetroe, 2009). This knowledge translation process recognises the importance of gathering and synthesising evidence in a robust and replicable manner, and emphasises use of appropriate dissemination techniques and effective exchange of knowledge between researchers and knowledge users. This process is particularly useful for areas where research may be lacking, and encourages the synthesis of evidence through systematic reviews and

meta-analyses in order to gather and build upon the current knowledge base (Graham and Tetroe, 2009).

Translation of evidence into practice is mitigated by the level of evidence, the context, the presence of facilitation and the success of implementation (assessing organisational outcomes and achievements) (Stetler et al, 2011). Services must therefore make a commitment to implementing a knowledge translation framework which promotes the translation of evidence into practice. Without specific commitment to change, services are unlikely to be developed in a way that promotes and facilitates positive change in-line with service user needs and evidence of effectiveness.

New tools have been produced to facilitate adoption of evidence into practice that could help identify the problems of evidence translation in local contexts. For example, The NHS Institute for Innovation and Improvement developed the 'Spread & Adoption' tool to aid in the assessment of innovation implementation likelihood (NHS Institute for innovation and Improvement, 2012). The purpose of this tool is to highlight small changes that can be made to promote change and ensure that an organisation is ready to implement new ideas. With the use of tools such as this, organisations can prioritise factors that require action and determine barriers to change and innovation.

3.6.3. Identifying opportunities for service development

The 'Any Qualified Provider' principle is used by the NHS to enable certain patient groups to choose from a range of approved providers for their healthcare. These approved providers may include state/private hospitals, charities, private organisations and certain retailers (e.g. private wheelchair suppliers). This allows patients to make informed decisions about their healthcare based on service attributes important to them, for instance how geographically close a service is or the quality of care provided. This principle promotes services that are developed around the holistic needs of the service user (Posture and Mobility Group, 2011) as they can seek the most appropriate provider for their needs. It has potential for wider application if more evidence of effect is available to help inform decisions. Focussing on integrating agencies to provide better care and services for disabled children is also of paramount importance. Wheelchair services need to think outside the health domain and consider the wider needs of disabled children to ensure they are not excluded from education and social settings.

There is a distinct lack of high-quality effectiveness and cost-effectiveness evidence within this field. Although many studies have used robust methods to explore bio-mechanical impacts of mobility interventions (which were not relevant for this review), these do not reflect the desired outcomes of

from a service user or carer perspective. The intervention evidence, although limited by quality, demonstrates that wheelchair interventions have a range of positive effects beyond mobility. More evidence is required to understand how effective interventions can be measured and achieved for all service users. This requires studies to use large sample sizes, robust methods and diverse outcome measures.

3.6.4. Priorities for future research

The application of health economics could enable a better understanding of the cost-effectiveness of wheelchair interventions, and thus benefit service-commissioning and funding allocation, and enable these practices to be evidence-based and equitable. The limited economic evidence in this review may be considered best evidence in the field due to the lack of other research into the cost-effectiveness of wheelchairs for disabled children.

Future research should focus on developing outcome measures, health economic methods, and exploring the use of HRQoL or capability measures to determine effectiveness from a more holistic perspective. Current wheelchair service outcome measures focus on clinical outcomes and service quality (e.g. QUEST [Demers et al, 2002)), which do not reflect all of the needs of service users. Generic preference-based measures could be used to collect utility data which in turn could be used to develop cost per QALY estimates relating to wheelchair interventions. Furthermore, this evidence would allow comparisons with other healthcare interventions and understanding of incremental cost-effectiveness. This would in turn encourage appropriate funding allocation and provision based on robust effectiveness evidence.

3.6.5. Identifying appropriate outcomes

Designing high-quality research in this field has specific challenges, particularly if looking generally at wheelchair interventions across a range of disabilities. Mobility impairment can be a result of many different conditions, and thus needs and interventions can be highly variable. This has implications for conducting large scale trials using clinical outcomes. Likewise, interventions are likely to be highly variable across different conditions. HRQoL and capability measures would allow a universal outcome that reflects the wider benefits of such interventions, and therefore would be a more appropriate approach to understanding the effectiveness of various interventions. At present there are no child-specific measures of capability available for this purpose and the applicability of HRQoL measures like the EQ-5D-Y is unknown in this setting.

Although the use of QALYs can be contentious (Nord et al, 2009; Phillips, 2009), it provides a universal measure that can compare the effectiveness of disparate interventions. For instance,

different types of wheelchairs for different types of disabilities could be compared using a single outcome (QALY gains). This data could be collected alongside clinical outcomes in order to encourage holistic interventions that fit in with the needs and desires of young wheelchair users.

At present child and parent proxy versions of validated HRQoL measures do exist, for instance the HUI measures(Horsman et al, 2003). However, their relevance for wheelchair users is still to be demonstrated. Some measures, such as the PedsQL, have additional bolt-on questions for particular conditions (such as cerebral palsy) which take into account the condition-specific aspects of QoL (Varni et al, 2005a), but cannot be used directly to calculate QALYs. There are methods of converting scores from non-preference-based measures so that they can be used for QALY calculations, but there are limitations to using such approaches (Brazier and Tsuchiya, 2010).

If wheelchair services in the UK and internationally were to adopt a single set of outcome measures a wealth of data could be generated, which could be used to evaluate the holistic effectiveness of wheelchair interventions for disabled children. This data could be used to aid the development, supply and maintenance of wheelchairs. It would promote interventions that reflect the desires of service users and would allow outcomes to be measured appropriately from the perspective of the service user and the clinician. Furthermore, services could be structured around the needs of service users.

Within a UK healthcare system context, these findings provide impetus for NICE to consider wheelchair services (both adult and child) a high priority. NICE provides national clinical guidelines on healthcare interventions, medication and new health technologies in order to ensure high quality and evidence based care for patients within the NHS. To date NICE have produced little guidance on disability interventions.

3.6.6. Review limitations

No major deviations from the protocol were noted. In the spirit of transparency, it is worth considering some potential limitations. The original aim was to understand wheelchair interventions more generally, however due to the general focus in the literature on PWC interventions, the findings have greater relevance to PWCs. Over half of the intervention studies looked specifically at children with cerebral palsy. Furthermore, the intervention evidence was of low quality and at risk of bias, thus the findings must be viewed with caution.

Although evidence included in this review may not be universally generaliable to all conditions, it still offers a better understanding of how wheelchairs can positively impact the health and wellbeing of

disabled children. More research is needed to see if these benefits are universal across all conditions and interventions.

The lack of economic evidence highlights the issues of applying health economics to wheelchair provision for disabled children and justifies further research within this field. The lack of RCTs in this field highlights the ethical and methodological issues of wheelchair intervention studies in children. However, the study by Jones et al (2012) establishes that an RCT can be a useful and ethically sound approach when conducted appropriately. For instance, it is unethical to withhold wheelchairs from those who require them, thus standard issue wheelchairs could be used in the control group and more technologically advanced equipment in the intervention group. Likewise research examining manual versus powered wheelchairs could utilise a similar RCT setup.

Only evidence written or translated into English was included in this review, which may have excluded valuable research written in other languages.

3.7. Conclusion

Wheelchairs offer varied benefits to disabled children in terms of health, developmental and social outcomes. At present NHS wheelchair services in the UK are not meeting all of children's needs and service development is required.

Findings derived from the evidence are relevant for NHS services and have some implications for wheelchair services globally. Wheelchair services have an invaluable role in promoting equality for disabled people. If these services can address disabling barriers for children at a young age, they may be able to facilitate more inclusion in education and society.

There are important gaps in current knowledge regarding health economic methods and available outcome measures, which hinders further service development and research. Health economics has an important role in developing effective, efficient and equitable wheelchair services in the UK. The lack of economic evidence in this field highlights the lack of appropriate methods to measure cost-effectiveness. Establishing the cost-effectiveness of interventions is a priority to promote efficient services, and in order to do so additional research is needed into the appropriateness of standard health economics methods for economic evaluation and service development. In the subsequent chapters I will address these issues.

Chapter Four: Costing wheelchair interventions for disabled children: A case study of state and charity wheelchair services in the UK.

4.1. Chapter summary

This chapter presents a costing case-study of three wheelchair services in the UK. Some families are forced to obtain wheelchairs through charitable and private organisations due to NHS provision restrictions. In the previous chapter I found little evidence from an appropriate public sector perspective concerning the cost-effectiveness of providing wheelchairs to children. If this evidence was available it could be used to inform commissioning of services. Carrying out a small scale case-study of costing across three wheelchair services will provide a better understanding of how to cost wheelchair interventions on a wider scale for the purpose of economic evaluation.

The capital and operating costs were collected from each service for each participant. Additional cost data was obtained from national sources. Sensitivity analysis was conducted to account for difference in the number of times each wheelchair is refurbished and recycled. Capital costs were annuitised over the expected length of life of the wheelchair. This chapter demonstrates that costing wheelchair interventions requires a number of factors to be taken into account, particularly the expected life of each wheelchair and the cost of customisation.

4.2. Introduction

4.2.1. Types of wheelchairs for disabled children

Wheelchair interventions for children can be broadly grouped into four categories: adapted buggies, standard MWCs, active user MWCs and PWCs. Child standard MWCs are generally designed for attendant control (i.e. controlled by a parent or carer) and are less adaptable, while active user MWCs are lightweight and better designed for self-propulsion and customisation. PWCs can also vary, for instance they may be designed for indoor, outdoor or multipurpose use. A child may need access to more than one type of wheelchair at any given time, for instance using an active user MWC for home/social use and a PWC at school to reduce fatigue. Furthermore, the equipment needs of each child can change over time due to changes in their health or abilities.

4.2.2. Wheelchair services for disabled children

NHS wheelchair services in the UK are the main provider of wheelchairs for disabled children. In order to obtain a publicly funded wheelchair children must have their needs assessed by a qualified NHS therapist at a wheelchair service (often referred to as 'posture and mobility' or 'artificial limb and appliance' services). Eligibility criteria and assessments vary greatly between different services (Goddard, 2008). In some circumstances children and their families may purchase wheelchair equipment privately or raise funds through charitable organisations to fund specific wheelchairs. In

1996 the government introduced the voucher scheme, which allows patients to opt out of NHS provision in favour of a voucher for equivalent value of the NHS wheelchair they were offered, which can be used to fund a private wheelchair (Sanderson et al, 2000).

Patients may choose to fund wheelchairs outside of the NHS for many reasons; often this occurs when the NHS is unable to fund specific pieces of equipment due to budgetary constraints, contract restrictions, strict eligibility criteria or lack of professional expertise when a child has very complex needs. For instance, PWCs are often difficult to obtain for younger children through the NHS due to safety concerns; a scenario which has been ongoing for many years (Sanderson et al, 2000; WK, 2011). This has raised issues regarding the equity of healthcare provision for disabled children and the exact remit of NHS wheelchair services. A number of inquiries into NHS wheelchair services have called for comprehensive multi-disciplinary assessments of mobility needs to be embedded in NHS wheelchair services (Prime Minister's Strategy Unit, 2005; NHS Modernisation Agency, 2007; National Assembly for Wales, 2010).

The systematic review results presented in chapter three illustrate that there is currently no evidence from an appropriate public sector perspective concerning the cost-effectiveness of providing wheelchairs to children (Bray et al, 2014). NICE uses evidence of clinical and cost effectiveness to advise NHS funding allocation, thus additional research is needed. A case-study of costing wheelchairs interventions for children can provide a novel template for future economic evaluations in this field.

4.2.3. Costing wheelchair interventions

In order to carry out appropriate cost-effectiveness analysis it is important to understand how to cost wheelchair interventions for children. A number of factors must be taken into account, including the capital cost of equipment, staff costs, overheads and other important factors such as repair and maintenance costs. Due to the variance in the way different services are run it can be difficult to make generalisable statements about costs, furthermore wheelchair intervention costs can be drastically different depending on the level of customisation required by each child. Therefore there are many issues to be explored with regards to costing wheelchair interventions. Carrying out a small scale case-study of costing across three suppliers will provide better understanding of how to cost wheelchair interventions on a wider scale and the potential differences between state and charity wheelchair providers.

4.3. Aims and Objectives

The overarching aim was to examine the costs associated with the supply of a wheelchair to a disabled child, taking into account differences between state and charity services. Secondary objectives were:

- To compare the relative wheelchair and customisation costs for different types of wheelchairs.
- To estimate staff time and costs associated with the provision of a wheelchair.
- To analyse the annual and total cost associated with wheelchair provision.
- To examine theoretical cost savings associated with recycling wheelchairs.

4.4. Methods

See chapter two for details on recruitment, data protection and ethical considerations.

4.4.1. Data collection

Once participants agreed to take part in the Wheels Project I contacted their wheelchair supplier to gather data regarding the wheelchair that had been supplied, any customisation/optional extras provided and all other associated costs. I collected relevant demographic information from the participant/parent proxy about the child's disability and wheelchair use, including diagnosis, length of time using a wheelchair, type of wheelchair used and frequency of wheelchair use.

4.4.2. Costing and analysis

I adopted a public and voluntary sector perspective, taking into account costs accrued by the health service and the two charitable organisations (WK and BIME). Family/parent costs associated with private purchase of equipment or maintenance were not taken into account. It is important to explicitly state the perspective and approach to costing, as these help to determine which costs are included and how analyses are performed. Perspective is particularly important in economic evaluations as the decision to include or exclude certain categories of cost can have a direct impact on subsequent resource allocation decisions (Byford and Raftery, 1998). For instance, adopting a non-societal perspective may inhibit measurement of wider societal welfare benefits. Conversely, it can help to focus on services and individuals directly impacted by a given intervention. Depending on the intervention of interest, perspective can therefore have a distinct effect on the outcomes of an economic evaluation (Byford and Raftery, 1998).

Effective wheelchair provision can have a range of potential societal benefits, for instance enabling disabled people to access education and work and reduce their need for caregiver assistance. Therefore, as part of a full economic evaluation in this area of research a societal perspective would be beneficial. For the purpose of this chapter, I wanted to focus specifically on intervention cost data as I was not conducting an economic evaluation. Although patient accrued costs can be used as part of economic analyses, a societal perspective was deemed beyond the remit of this particular chapter due to the core focus on wheelchair service costs. The aims specifically addressed costs associated with wheelchair services, and thus a more refined perspective was deemed appropriate.

4.4.2.1. Capital costs

All of the partnered wheelchair providers were able to give a comprehensive breakdown of wheelchair costs and customisation costs for each participant. I was therefore able to make relatively precise estimates of capital equipment costs. For three of the participants recruited from WK total costs were provided but the breakdown of costs was not available. I therefore sought recommended retail prices for the wheelchairs from manufactures and subtracted this price from the total cost in order to give an estimated customisation cost. All costs are native to the year the wheelchair was supplied and have not been uprated to 2013/2014 prices as this could have skewed cost data unfavourably.

4.4.2.2. Staff time

Due to time constraints it was not possible to monitor staff time precisely, for instance using "time and motion" monitoring. As each service had different care pathways I instead choose to consult with occupational therapists, managers and clinical leads within each service to establish estimates of time taken to supply each wheelchair (see table 4.1).

Table 4.1: Estimated staff time (hours) per wheelchair supplied by each provider

	Occupational therapist time (hrs)			Technician/engineer time (hrs)			Support staff time (hrs)
	Admin	**Assessment**	**Handover**	**Admin**	**Assessment**	**Handover**	**Admin**
BIME	0.75	2.5	0.25	-	2.5	-	0.75
WK	1	2.5	1.5	-	2.5	1.5	1.88
NHS	3	4	1.5	1	4	1.5	0.25

Staff pay scales were obtained from managers. I was not able to calculate exact salaries for NHS and WK staff due to the variance in progression along pay bands, I therefore chose pay points in the

middle of pay bands to estimate staff hourly rates. For BIME staff I was able to use exact hourly rates.

Sensitivity analysis was used to adjust staff time taken to supply a wheelchair. WK were unable to provide estimates of administrator time due to the way their service is centralised. Average admin staff time for WK was calculated by dividing the number of wheelchairs supplied per year (WK, 2013b) by total staff time for that year (based on two administrative staff working full-time). Overhead rates were calculated using PSSRU NHS unit cost guidance (Curtis, 2011) and the WK 2013 financial statement. An actual overhead rate for BIME was obtained from the commercial manager.

4.4.2.3. Overheads

It is important to factor in overheads for all direct staff costs in order to take account of additional support required by any professional to effectively carry out their work. Overheads include a range of factors, such as costs for management staff, staff expenses, utilities, rental/premises rates, professional training and so on (Curtis, 2011). An overhead rate of 60.7% was applied to all NHS staff costs (Curtis, 2011) and 54% for BIME staff costs (as directed by BIME finance manager). An explicit overhead rate was not available for WK, however I was able to calculate an estimated overhead rate using the WK financial statement for 2013 (WK, 2013a). In 2013 WK expenditure on direct staff costs associated with mobility services totalled £1,011,000. Additional non-direct staff costs associated with WK mobility services (including support staff costs, rent, travel and expenses etc.) totalled £587,000, giving an estimated overhead rate of 58.1%.

4.4.2.4. Maintenance and repairs

Estimated maintenance costs for children's wheelchairs are not available, therefore adult costs reported in the PSSRU unit cost guidance were used (Curtis, 2013); £114 per PWC repair and £29 per MWC repair. For buggies the manual wheelchair maintenance cost has been used. Following consultation with the partnered wheelchair suppliers an average wheelchair use period of four years per child was estimated. Due to the unique nature of the BIME Wizzybug, estimated total use time was two years per child; the Wizzybug can only be used up to age 5, thus length of time using it is limited. It was assumed that each NHS and WK wheelchair would require one repair/maintenance each year. BIME staff estimated a one-off cost of £100 per repair, per supplied Wizzybug.

4.4.2.5. Refurbishment and wheelchair recycling

It is estimated that 50% of all wheelchairs supplied by the NHS are recycled (Curtis, 2013), it is therefore important to factor in the impact of refurbishment and recycling on wheelchair provision

costs. The partnered NHS service stated that they were able to recycle a wheelchair up to two times. After consultation with the partnered clinical lead refurbishment cost was estimated to be 25% of the basic wheelchair cost. Sensitivity analysis was conducted in order to estimate cost savings associated with recycled wheelchairs . For wheelchairs that are recycled the overall cost of the wheelchair has been divided by the number of recycles, assumed length of life of the wheelchair and with the subsequent refurbishment costs added on for each recycle. Three cost scenarios were developed for the sensitivity analysis:

1. Scenario 1 (no recycle): each wheelchair is supplied to only one child for a 4 year period and then scrapped.
2. Scenario 2 (one recycle): each wheelchair is supplied to two children consecutively for 4 years each and then scrapped. Wheelchair is refurbished before second use, with an estimated refurbishment cost of 25% of the basic wheelchair cost for WK and NHS, and a flat rate of £500 for the BIME Wizzybug.
3. Scenario 3 (two recycles): each wheelchair is supplied to three children consecutively for 4 years each and then scrapped. Wheelchair is refurbished before second and third use, with an estimated refurbishment cost of 25% of the basic wheelchair cost for WK and NHS, and £500 for the BIME Wizzybug.

4.4.2.6. Annuitisation of costs

Capital costs were annuitised to account for the number of years those costs are incurred by the wheelchair service, according to the assumed length of life of the wheelchair. In scenario 1 the assumed wheelchair length of life was four years, for scenario 2 it was eight years and scenario 3 twelve years (two, four and six years respectively for the BIME Wizzybug). I worked under the assumption that each new recipient of a recycled wheelchair would require a full re-customisation of the wheelchair and thus customisation costs would be incurred in full for each new recipient (using the average for wheelchair type). All customisation capital costs were annuitised separately over four years to account for the assumed length of life of the customisation. The following equation was used for annuitisation calculations:

$$C = \left[P - S * \frac{1}{(1+r)^t}\right] * (AF)^{-1} \qquad \text{Where AF=} \left[1 - \frac{1}{(1+r)^t}\right] r^{-1}$$

C= calculated equivalent annual cost of the unit; P= cost of purchasing the unit; S= scrap value of the unit after t years of service; r= discount rate; AF= annuity factor

Annuity factors (at a 3.5% discount rate [HM Treasury, 2013] and £0 scrappage value) were calculated as follows: 2 years: 1.899; 4 years: 3.673; 6 years: 5.329; 8 years: 6.874; 12 years: 9.663. Example:

> *Wheelchair costs £2000, customisation costs £300 and refurbishment costs 25% of wheelchair cost (£500). The chair is recycled once (expected length of life of wheelchair is 8 years) and customised twice (expected length of customisation is therefore 4 years).*
>
> *Wheelchair = £2000/6.874 = £290.99 annuitised cost per year*
>
> *Customisation = £300/3.673 = £81.68 annuitised cost per year*
>
> *Refurbishment = £500/8years = £62.50 per year (not annuitised)*
>
> *Annual cost = £290.99 + £81.68 + £31.25 = £435.17 annuitised annual cost per recipient*

4.4.2.7. Total costs

In order to estimate the total cost per patient, annuitised capital costs were multiplied over the assumed length of wheelchair use (four years). This took into account differences in assumed length of wheelchair life and associated refurbishment/repair costs. Under all scenarios it was assumed that each supplied wheelchair per recipient would incur staff costs for the original provision of the wheelchair and four subsequent follow-ups/reviews. Likewise, it was assumed that each non-recycled wheelchair would incur four repair/maintenance costs over the four years of provision. For recylced wheelchairs it was assumed that three repairs/maintenance procedures would be needed, as a refurbishment would be carried out in the final year in place of maintenance. Finally, for recycled wheelchairs a refurbishment cost of 25% of the basic wheelchair cost was incurred per recycle, along with full re-cusomisation costs (annuitised over four years).

4.5. Results

4.5.1. Response rate and sample size

A total of 125 study invitation packs were distributed across England and Wales by the three recruitment sites: 61 to parents of children aged 5 or under; 29 to children (and their parents) aged 6 to 15; and 35 to children (and their parents) aged 16 to 18. 38 questionnaires were returned by participants (30.4% response rate), therefore giving an overall sample size of 38.

4.5.2. Demographic characteristics

A comprehensive breakdown of demographic details are presented in table 4.2. The majority of children had a diagnosis of cerebral palsy (63.2% [N=24]) and used their wheelchair(s) 'all of the

time' (57.9% [N=22]). 63.2% (N=24) of participants were male and almost all were white British (94.7% [N=36]). Half of the participants were recruited from WK and half used both a manual and a powered wheelchair, costing was based on the most recent wheelchair they had received from their wheelchair service. The wheelchair definitions were thus expanded to account for variance in equipment costs. The wheelchair types categories were adult active MWCs, child active MWCs, child standard MWCs, buggies, PWCs, and PWCs made specifically for young children (the Wizzybug). Adult active MWCs were included as adolescents may benefit from progression into these chairs once child MWCs become too small. See table 4.2 for number of participants per wheelchair type category.

Table 4.2: Demographic characteristics of sampled children

Demographic characteristics	Number (%)
Study site	
NHS	6 (15.8)
WK	19 (50)
BIME	13 (34.2)
Gender	
Female	14 (36.8)
Male	24 (63.2)
Age	
0-5 years	19 (50)
6-15 years	11 (28.9)
16-18 years	8 (21.1)
Ethnicity	
White British	36 (94.7)
Other Asian background	1 (2.6)
Other mixed background	1 (2.6)
Diagnosis	
Cerebral Palsy	24 (63.2)
Spinal Muscular Atrophy	4 (10.5)
Muscular Dystrophy	3 (7.9)
Rett Syndrome	1 (2.6)
Chromosome Deletion	1 (2.6)
Lissencephally	1 (2.6)
Porencephaly	1 (2.6)
Hemiplegia / stroke	1 (2.6)
Spina Bifida	1 (2.6)
Global developmental delay	1 (2.6)
Frequency of equipment use	
A little of the time	1 (2.6)
Some of the time	6 (15.8)
Most of the time	5 (13.2)
All of the time	22 (57.9)
Did not answer	4 (10.5)
Type of equipment used	
MWC	11 (28.9)
PWC	6 (15.8)
Buggy	2 (5.3)
MWC and PWC	19 (50)

It should be noted that WK offer two distinct wheelchair services: charity funded mobile/regional clinics and WK contracted NHS services. All of the WK participants had received wheelchairs through the charity WK services, and thus the results do not represent the costs of WK led NHS services.

4.5.3. Capital costs

Like-for-like comparisons of specific wheelchair models was not possible as participants from different services did not use the same model wheelchairs. The results therefore relate to types of wheelchairs and not specific makes or models. The results do not indicate differences in costs between services for exact models of wheelchairs, and thus should only be viewed as making higher level comparisons of costs for different types of wheelchairs.

In total, wheelchair costs came to £125,178 across the cohort, with an additional £29,234 in costs for optional extras and customisation. The mean cost for a wheelchair was £3294.15 per child, with customisation costing an average of £769.31 per child and making up 18.9% of capital costs. Adult active MWCs had the highest proportion of customisation costs compared to wheelchair costs, with an average of 60.3% (£1985.81) of adult active MWC capital costs associated with customisation. On average the BIME Wizzybug (Under 5's PWC) had the lowest customisation cost to wheelchair cost ratio, with 5.7% (£171.92) of capital costs associated with customisation. See figure 4.1 for a breakdown of average wheelchair and customisation costs by wheelchair type.

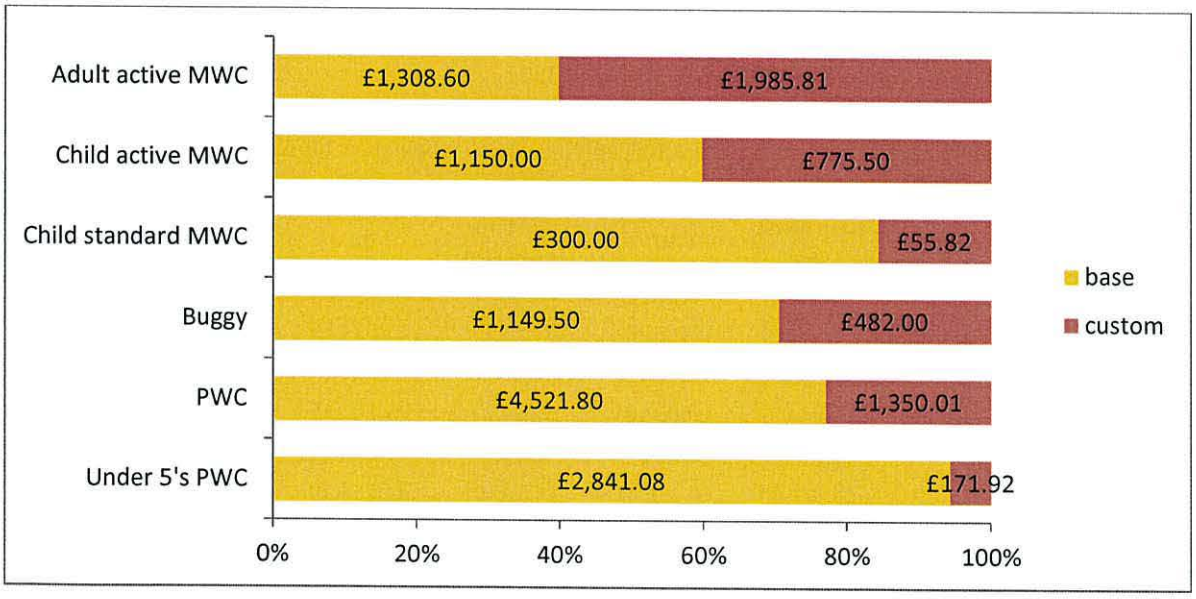


Figure 4.1: Comparison of mean wheelchair (base) and customisation (custom) capital costs by wheelchair type

Customisation costs were higher for WK wheelchairs as compared to NHS wheelchairs for all equivalent types of wheelchairs, see figure 4.2 for a full comparison. This perhaps demonstrates the higher level of customisation available from WK as compared to the NHS.

Figure 4.2: Comparison of NHS and WK mean MWC and customisation capital costs by wheelchair type

On average PWCs had the highest wheelchair costs (£4,421.80) and total costs (£5871.81). Adult active MWCs had the highest customisation costs (£1985.81). The lowest cost wheelchairs were child standard MWCs (£300), which also had the lowest customisation costs (£55.82). The highest cost single wheelchair was £15,500 for a WK supplied PWC. The highest cost single customisation was £2996.25 for e-fix wheels (a specialist electric drive wheel customisation) on a WK supplied adult active MWC. The highest cost overall customisation of a wheelchair (taking into account all accessories) was £4,206 on a WK supplied adult active MWC with a basic value of £1700. See table 4.3 for a full breakdown of capital costs by wheelchair type.

Table 4.3: Mean wheelchair, customisation and total capital costs by wheelchair type.

	N	Base mean cost	SD	Custom mean cost	SD	Total mean cost	SD
Adult active MWC	5	£1,308.60	£558.82	£1,985.81	£1,968.32	£3,294.41	£2,104.64
Child active MWC	3	£1,150.00	£412.28	£775.50	£1,037.79	£1,925.50	£987.91
Child standard MWC	5	£300.00	£7.07	£55.82	£53.00	£355.82	£48.03
All MWC	13	£884.08	£606.10	£964.20	£1,503.15	£1,848.28	£1,854.97
Buggy	2	£1,149.50	£388.20	£482.00	£32.53	£1,631.50	£355.67
PWC	10	£4,521.80	£4,449.41	£1,350.01	£1,254.10	£5,871.81	£4,094.52
Under 5's PWC	13	£2,841.08	£100.27	£171.92	£72.10	£3,013.00	£157.00

Taking into account wheelchair supplier, on average WK supplied PWCs had the highest average wheelchair cost (£4521.80) and total cost (£5871.81), while WK supplied standard MWCs had the lowest average wheelchair cost (£292.50). WK supplied adult active MWCs had the highest associated customisation cost (£1713.01) while NHS supplied child standard MWCs had the lowest

average customisation and total costs (£24.70 and £329.70 respectively). See table 4.4 for a comparison of mean NHS and WK capital costs by wheelchair type.

Table 4.4: Comparison of NHS and WK mean MWC, customisation and total capital costs.

		N	Base mean cost	SD	Custom mean cost	SD	Total mean cost	SD
Adult active MWC	NHS	2	£950.00	£282.84	£349.01	£252.26	£1,299.01	£535.10
	WK	3	£1,248.83	£608.47	£1,713.01	£1,803.21	£2,961.84	£1,442.15
Child active MWC	NHS	1	£885.00	NA	£42.50	NA	£927.50	NA
	WK	2	£1,282.50	£484.37	£1,142.00	£1,161.07	£2,424.50	£676.70
Child standard MWC	NHS	3	£305.00	£0.00	£24.70	£42.78	£329.70	£42.78
	WK	2	£292.50	£3.54	£102.50	£17.68	£395.00	£21.21

4.5.4. Annual capital costs

The annual capital costs (over four years) for all types of wheelchairs across all three suppliers were predictably lowest in scenario 3 (2 recycles) and highest in scenario 1 (no recycle), see table 4.5. Taking into account cost of wheelchair, customisation, annual repair and refurbishment, the highest estimated annual capital cost was for non-recycled WK PWCs (£1712.61) closely followed by non-recycled BIME Wizzybugs (£1636.04). The lowest estimated annual capital cost was for once and twice recycled NHS child standard MWCs (£89.63 and £76.82 respectively).

Table 4.5: Annual capital costs by estimated length of viable wheelchair usage

		No recycle	1 recycle	2 recycles
Adult active MWC	NHS	£382.66	£291.91	£242.12
	WK	£835.37	£716.07	£663.63
Child active MWC	NHS	281.51	196.97	159.81
	WK	689.07	566.56	512.71
Child standard MWC	NHS	118.76	89.63	76.82
	WK	136.54	108.60	96.32
Buggy	WK	473.18	363.37	315.10
PWC	WK	1712.61	1280.66	1090.78
Under 5's PWC	BIME	1636.04	1038.99	840.35

Taking into account cost of wheelchair, customisation, annual repair and refurbishment over the assumed length of life of each wheelchair, total annuitised capital costs for non-recycled WK PWCs were again found to be the most costly (£6850.43) while NHS child standard MWCs were the least costly (£307.27), see table 4.6. Costs were again cheapest for wheelchairs recycled twice, see figures 4.3-4.5.

Table 4.6: Total annuitised capital costs by estimated length of viable wheelchair usage

		No recycle	1 Recycle	2 Recycles
Adult active MWC	NHS	£1,530.62	£1,167.63	£968.47
	WK	£3,341.46	£2,864.29	£2,654.52
Child active MWC	NHS	£1,126.05	£787.90	£639.24
	WK	£2,756.29	£2,266.25	£2,050.83
Child standard MWC	NHS	£475.04	£358.50	£307.27
	WK	£546.16	£434.39	£385.26
Buggy	WK	£1,892.71	£1,453.49	£1,260.41
PWC	WK	£6,850.43	£5,122.66	£4,363.12
Under 5's PWC	BIME	£3,272.09	£2,077.97	£1,680.69

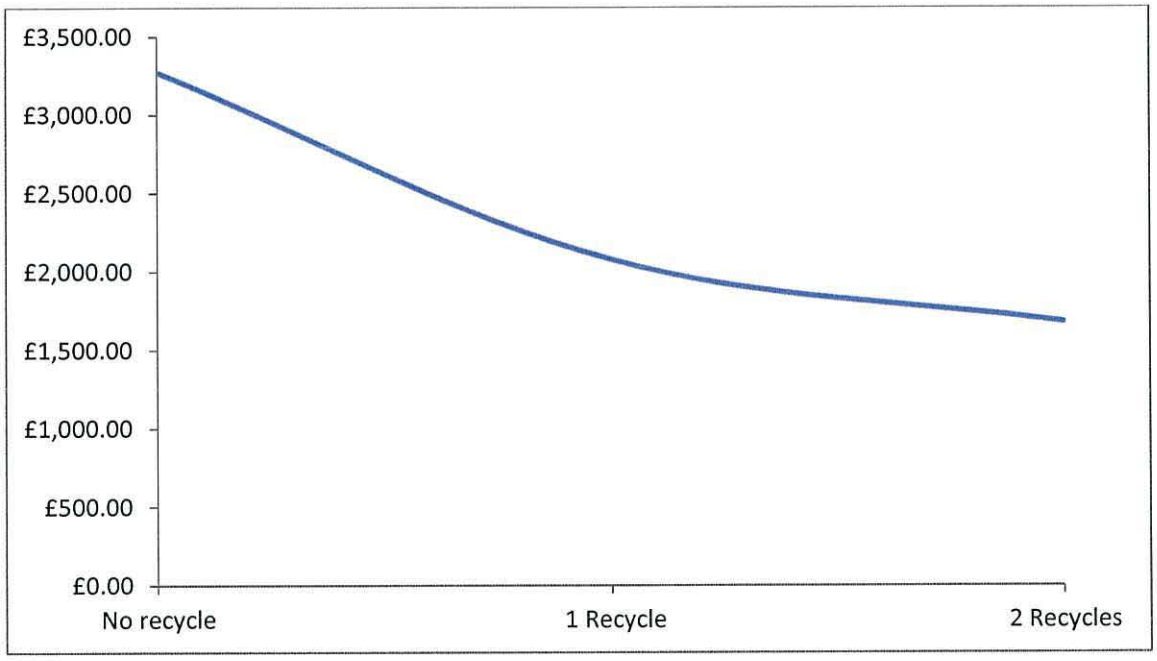


Figure 4.3: Total annuitised BIME under 5's PWC capital cost by estimated length of viable wheelchair usage

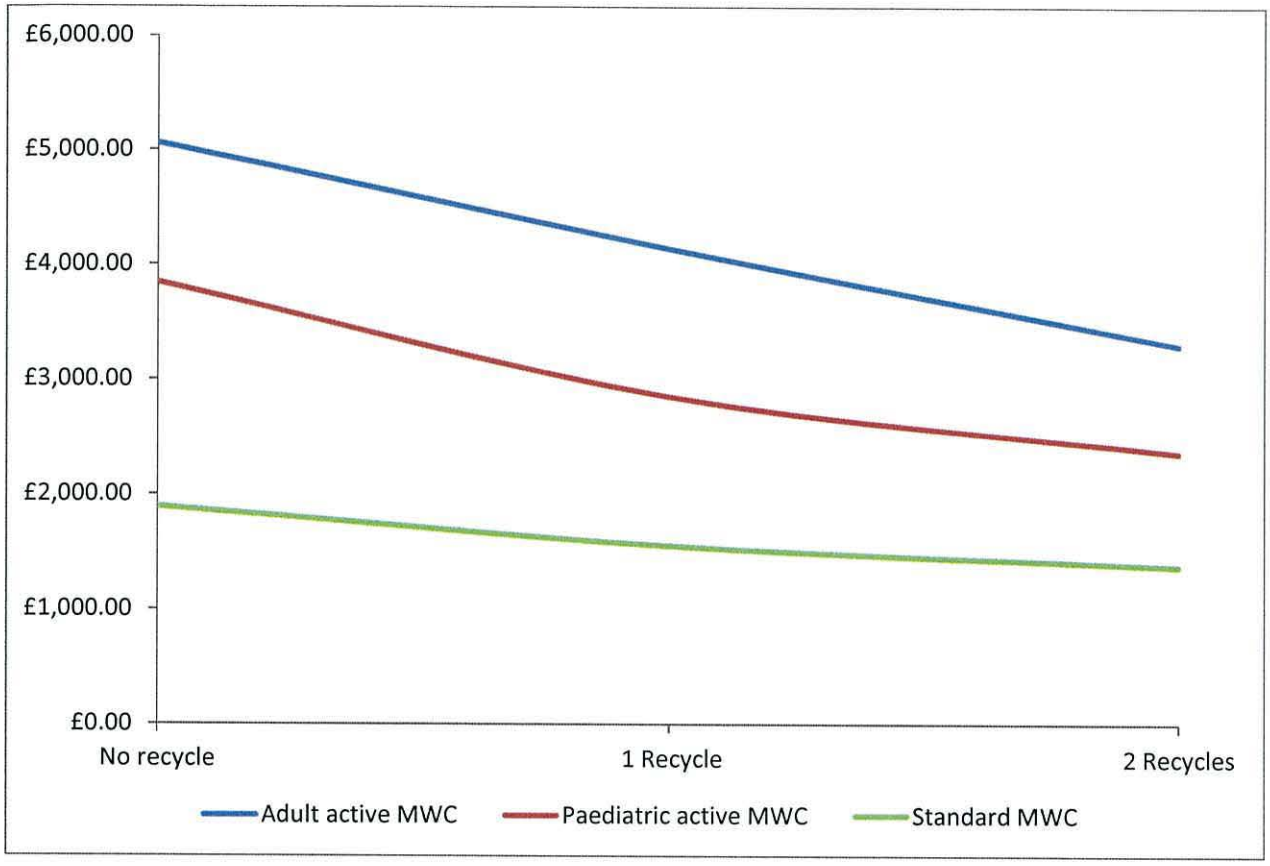


Figure 4.4: Total annuitised NHS capital costs by estimated length of viable wheelchair usage

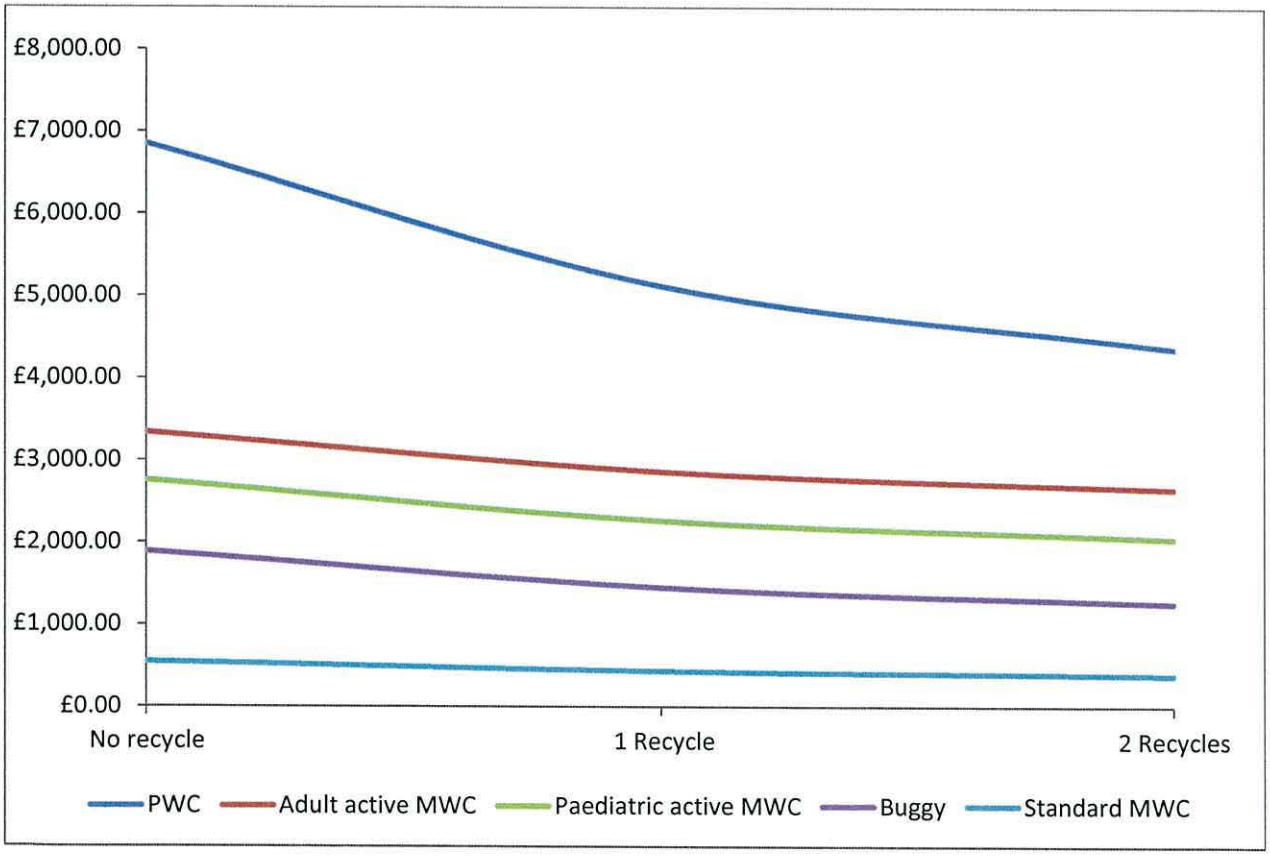


Figure 4.5: Total annuitised WK capital costs by estimated length of viable wheelchair usage

4.5.5. Staff time costs

The total staff cost of supplying a wheelchair was relatively similar across the three suppliers, see table 4.7. There was some variance due to differing staff time per wheelchair supply and staff salaries. Based on assumptions regarding staff time and salary, it appears that BIME staff costs were lowest per wheelchair provision (£259.74) while NHS were highest (£306.61). This was mainly due to differences in length of time spent on work and consultations directly associated with provision of a single wheelchair.

Table 4.7: Estimated staff costs per wheelchair provided

		NHS	WK	BIME
Occupational therapist (OT)	£/H	£15.22	£18.17	£26.56
	Time (hr)	8.5	5	3.5
Technician	£/H	£9.10	£14.58	£25.78
	Time (hr)	6.5	4	2.5
Administrative support	£/H	£9.10	£11.25	£15
	Time (hr)	0.25	1.88	0.75
	Total	£190.80	£170.32	£168.66
Total with overhead costs		**£306.61**	**£269.28**	**£259.74**

Follow-up appointments were estimated to be an hour long and involving an OT and technician. This resulted in an estimated per follow-up cost of £80.60 for BIME, £51.78 for WK and £39.08 for NHS. Repair costs for NHS and WK wheelchairs were estimated using published costs; £114 per PWC repair and £29 per MWC repair (Curtis, 2013), while a one-off repair of £100 per Wizzybug recipient was recommended by BIME.

4.5.6. Total costs

The final part of the analysis was to combine annuitised costs, staff time for provision/annual reviews, repair costs and refurbishment in order to estimate the total cost of supplying a wheelchair to a single child for a 4 year period (2 years for BIME). Sensitivity analyses were used to account for differences in staff time: Scenario B deducted 30mins from each staff members estimated time (15mins less for admin staff) and Scenario C added an extra 60mins per staff member (30mins for admin staff), see table 4.8 (Scenario A was the base case). Staff time was adjusted to account for different levels of assessment needs depending on wheelchair type. After consultation with the partner services, it was estimated that PWC users would require the greatest amount of time for assessment (staff time Scenario C) and child standard MWC users to require the least (staff time Scenario B). All other wheelchair users were estimated to require the previously estimated average (staff time Scenario A).

Table 4.8: Staff costs per wheelchair provided sensitivity analysis

	Scenario A	Scenario B	Scenario C
NHS	£306.61	£287.07	£353.00
WK	£269.28	£238.94	£329.95
BIME	£259.74	£213.66	£351.89

As observed in the previous steps of the analysis all single-user wheelchairs were more expensive than equivalent recycled wheelchairs, see table 4.9. WK supplied PWCs had the highest cost per recipient (£4780.19 to £7379.51) while NHS supplied standard MWCs had the lowest cost per recipient (£721.67 to £918.43). For almost all equivalent types of wheelchairs, WKs total costs were more than double those of the NHS (see figure 4.6).

Table 4.9: Total cost per patient receiving wheelchair over expected length of use

			Annual capital cost			Staff costs			Total cost		
		N	No recycle	1 recycle	2 recycles	Provision	Follow-up/ review	Ann. repair	No recycle	1 recycle	2 recycles
Adult active MWC	NHS	2	£353.66	£262.91	£213.12	£306.61	£39.08	29	£1,993.57	£1601.57	£1402.41
	WK	3	£806.37	£687.07	£634.63	£269.28	£51.78	29	£3,817.88	£3311.68	£3101.92
Child active MWC	NHS	1	£252.51	£167.97	£130.81	£306.61	£39.08	29	£1,588.97	£1221.81	£1073.17
	WK	2	£660.07	£537.56	£483.71	£269.28	£51.78	29	£3,232.68	£2713.64	£2498.24
Child Stand. MWC	NHS	3	£89.76	£60.63	£47.82	£287.07	£39.08	29	£918.43	£772.91	£721.67
	WK	2	£107.54	£79.60	£67.32	£238.94	£51.78	29	£992.22	£851.46	£802.34
Buggy	WK	2	£444.18	£334.37	£286.10	£269.28	£51.78	29	£2,369.12	£1900.88	£1707.8
PWC	WK	10	£1,598.61	£1,166.66	£976.78	£329.95	£51.78	112	£7,379.51	£5539.71	£4780.19
Under 5's PWC	BIME	13	£1,586.04	£988.99	£790.35	£259.74	£80.60	100	£3,693.02	£2,498.92	£2,101.64

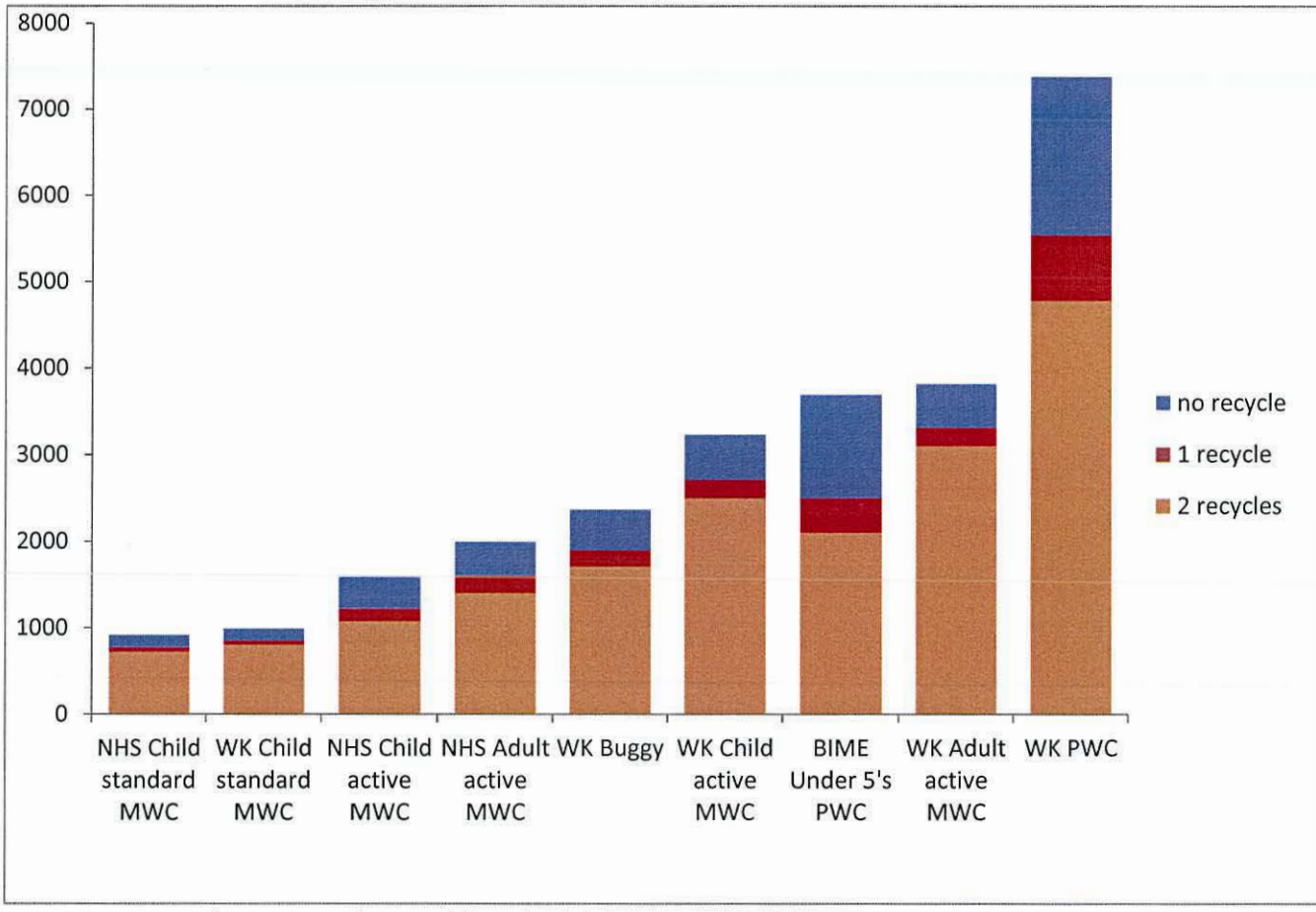


Figure 4.6: Total cost per patient receiving wheelchair over expected length of use

4.6. Discussion

In this cohort of disabled children the costs associated with the provision of a wheelchair varied greatly depending on the type of wheelchair provided, the level of customisation required, the supplier of the wheelchair and the assumed length of viable use of the wheelchair. Sensitivity analysis of number of wheelchair recycles showed that twice recycled wheelchairs (i.e. provided to three consecutive children) potentially offer the lowest capital cost per child. In practice the ability to recycle a wheelchair relies on many different factors, including the condition of the wheelchair on return, the suitability of the wheelchair for another child and the subsequent improvements in technology since the original purchase of the wheelchair.

4.6.1. Potential cost savings associated with wheelchair refurbishment

The PSSRU unit costs guidance estimates that 50% of NHS supplied wheelchairs are recycled (Curtis, 2013). Assuming that 25% of these chairs are recycled once and 25% are recycled twice, an overall total cost saving of between 9% and 14% could be achieved by the NHS for MWCs compared to a 'no-recycle' scenario. In reality it is unlikely that any NHS wheelchair service would adopt a 'no-recycle' policy, however these findings certainly support the use of refurbished wheelchairs,

assuming the quality of provision is not impacted by such practices. In general WK do not refurbish and recycle wheelchairs as often as NHS services. Assuming the same recycling scenario as outlined above, an overall total cost saving of between 8% and 15% could be achieved compared to a 'no-recycle' scenario. It may be beneficial for wheelchair services to invest more in repair and maintenance services so that wheelchairs are in better condition when returned, and thus more likely to be recyclable. At present some WK recipients often pay for their own repair and maintenance, which may in turn be reducing the condition of wheelchairs and their subsequent recyclability.

4.6.2. Cost comparisons between services

Published estimates of adult NHS wheelchair costs are relatively similar to those found in this chapter: £700-£3000 for a PWC, £100-£650 for an MWC (Curtis, 2013), although these lack direct comparison with the child results in this chapter as they are based on adult wheelchairs. Placing these costs in the context of mainstream mobility equipment, such as buggies and prams for babies and infants, demonstrates that basic but essential mobility equipment for disabled children is relatively inexpensive by comparison; even basic prams can cost parents in excess of £500.

In general I found that WK spent more than the NHS on wheelchairs and customisation, although it is important to be clear that like-for-like comparisons of wheelchair costs were not possible as WK and NHS participants did not use the same model wheelchairs. Many families go to WK because they have been unable to access appropriate mobility equipment through the NHS (WK, 2011). It is therefore unsurprising that WK equipment was generally more expensive in this sample as children with complex needs are more likely to access WK services. The NHS tends to cater well to children with basic needs, and thus costs are likely to be lower as they are more likely to supply basic, low cost wheelchairs. This raises some interesting questions about the level of provision provided by these two different services. Children and families seek wheelchairs through charities such a WK because NHS provision does not meet their need. This can often occur because NHS services are not able to fund particularly expensive pieces of equipment. For instance, one PWC provided by WK had a basic wheelchair cost of £15,500. It is unlikely that a wheelchair of this price would be funded by the NHS due to budget constraints. However, a child could potentially have better outcomes and better QoL if the NHS were able to provide more expensive equipment on the same scale as WK.

The DoH have stated that they are committed to improving the outcomes of disabled children (DoH Commissioning Team, 2010). Furthermore the Equality Act 2010 states that public authorities have an obligation to promote equality of opportunity for all disabled people (Davis, 2012; Office of

Disability Issues, 2006). In the context of disabled children with mobility impairments the first step towards equality is to provide the best mobility equipment at the right time to promote the most benefits. This highlights the need to build the economic case for wheelchair interventions for disabled children and to examine the cost-effectiveness of different types of wheelchair interventions.

4.6.3. PWCs for young disabled children

The cost of PWCs made specifically for young children can be exceptionally high, with some in the region of £10,000 before customisation. By comparison the BIME Wizzybug is relatively low cost. The BIME Wizzybug was purposefully included in this study as it is one of the only PWC devices in the UK designed specifically for children under the age of 5. It also represents a relatively affordable PWC entry point for young children accessing NHS wheelchair services. At present many NHS wheelchair services still restrict access to PWCs for young children due to safety concerns (WK, 2011). However, early provision of powered mobility has been shown to have various benefits (Furumasu et al, 2008; Bottos et al, 2001; Jones et al, 2003). The results indicate the Wizzybug has greater capital costs than the NHS issued MWCs, but lower costs than the WK issued adult active MWCs and PWCs over a hypothetical equipment loan period. With additional research into the effectiveness of the Wizzybug it may be possible to provide clear cost-effectiveness evidence to support the provision of PWCs for children under the age of 5 in the UK, or contrary depending on the results.

4.6.4. Study limitations

The results from this chapter must be examined with care. It should be taken into account that the aim of the study reported in this chapter was to examine the costs associated with the supply of a wheelchair for a disabled child, not to make broader conclusions about overall costs more generally to the NHS, WK and BIME. I sought to apply health economics methods of evaluation to costing a case-study of wheelchair services. Thus there are issues with making conclusions from this data. This cohort is too small to make accurate assessments of the quality of the wheelchairs supplied. Without direct comparisons of children with similar needs and wheelchairs it is difficult to assess which service is spending more on equipment for comparative conditions and levels of need. Likewise, directly comparable models of wheelchairs were not available between the services to compare costs.

A number of assumptions had to be applied to the data. These were based on expert opinion and published data, however a full economic analysis would require a more robust approach to costing wheelchair interventions, for instance "time and motion" monitoring, client service-receipt

inventories and micro-costing to examine associated health service use costs before and after wheelchair provision.

Other issues included the lack of diversity in the sample, for instance almost all participants were white British (93.2%) and the majority had cerebral palsy (64.7%). The NHS sub-group sample was particularly small (N=6) making it impossible to make broader assumptions about NHS services. Furthermore, no NHS PWC or buggy users were recruited thus limiting full comparison with the WK sub-group sample. NHS wheelchair services in the UK vary greatly in terms of contract prices for wheelchairs and assessment criteria (Goddard, 2008) therefore the results are relevant to the individual patients included in this study. It should also be noted that half of the participants used both a powered and manual wheelchair, which is important to consider in future research as use of a secondary wheelchair may be a confounding variable.

4.6.5. Implications for conceptual framework

In the previous chapter I presented a conceptual framework to guide the development of cost-effective wheelchair services for children in the UK. The results from this chapter specifically highlight the need for appropriate outcome measures to enable robust evaluation of wheelchair intervention cost-effectiveness. The collection of cost data alone cannot be used to guide resource allocation and service development. The applicability of commonly used generic measures of utility has not been established in previous research, thus additional research is required to test specific outcome measures for the purpose of economic evaluation in this context.

The methods used in this chapter could be adapted to examine streamlining of services and cost-saving procurement strategies, for instance the expansion of maintenance and recycling procedures. The economic analysis of wheelchair services and interventions requires a number of factors to be taken into account (such as level of customisation) due to the variance in interventions for each individual child. This chapter provides an adequate costing prototype for future economic analyses in this field.

The findings in this chapter demonstrate the importance of maintenance and recyclability of wheelchairs. Additional maintenance of wheelchair stock could improve the function and condition of equipment and potentially extend the life of equipment. Additional maintenance procedures could be a measure adopted by services to save costs and improve rapidity of services. The conceptual framework highlights the need for cost-savings, timely services and streamlined service pathways, which could all be facilitated by improving the quality of stock wheelchairs and increasing the useable life of a wheelchair.

Although this may not be appropriate for children with more complex needs, the expansion of maintenance procedures could benefit children eligible for more basic wheelchairs, as they would have quick access to good quality stock wheelchairs which could be customised with appropriate seating and postural support to meet their individual needs. Reflecting on the conceptual framework, this study highlights the need for innovative approaches to cost-saving strategies which promote the needs of the service user whilst reducing long-term expenditure within services.

4.7. Conclusion

Without a full economic evaluation it is not possible to assess whether the greater expenditure by WK provides better outcomes for disabled children. Assessment of clinical outcomes and HRQOL is required to understand the impact of different wheelchair interventions and their incremental cost-effectiveness. Without appropriate outcome measures it is difficult or even impossible to know if higher cost interventions equate to better outcomes for disabled children. It is therefore important to examine whether current standard measures of HRQoL and utility are fit for purpose in this context. Furthermore, the use of discrete choice experiment methods can be used to measure the relative importance of different service attributes, such as maintenance and review procedures. I will explore these considerations in greater detail in the subsequent chapters of this thesis.

This chapter demonstrates that costing wheelchair interventions requires a number of factors to be taken into account. The expected life of each wheelchair and the cost of customisation are particularly important as they appear to have a large impact on the total cost of a wheelchair. Future research could apply the methods incorporated in this case-study into a full economic evaluation alongside a trial, for instance using the Wizzybug as the intervention and a child standard MWC as the comparator.

Figure 4.7: The Wizzybug PWC for young children, designed by DesigAbility (BIME)

Chapter Five: Prioritising wheelchair services for children: a pilot discrete choice experiment to understand how young wheelchair users and their parents prioritise different attributes of wheelchair services

5.1. Chapter summary

The primary aim of this chapter is to explore how disabled children and their parents prioritise different attributes of wheelchair services. Secondly, I aim to compare priorities between disabled children and parents, and estimate marginal rate of substitution (MRS) between attributes of wheelchair services.

DCEs are a tool used by health economists to understand how individuals prioritise different attributes of healthcare services or treatments. I conducted the first pilot DCE study to explore how disabled children (aged 11-18) and their parents prioritise different attributes of hypothetical wheelchair services. A total of 30 parents of disabled children and 11 disabled children were recruited. A generic forced choice design was used. Participant's preferences were based on five key attributes: comprehensiveness of wheelchair assessment, cost contribution for wheelchair, level of training provided by wheelchair service, waiting time for delivery of wheelchair and frequency of wheelchair reviews. A mixed-level orthogonal array was used to produce eight pairwise choice tasks. For each pairwise choice, participants were asked to choose which hypothetical service scenario (service A or B) they preferred.

The most important service attribute for both the disabled children and parents was comprehensiveness of wheelchair assessment. The results indicate that DCE methods can be used effectively to examine wheelchair service preferences of disabled children (aged 11 and over) and their parents.

5.2. Introduction

In principle, assessing the benefit of health care services in monetary value poses profound challenges to health economists (Ryan et al, 2008). Alternative measurement techniques are required, as preferences for goods and services cannot always be directly observed from market patterns of buying and selling (Ryan et al, 2008). Two techniques for valuing monetary benefit have arisen from economic theory: revealed preference and stated preference. Revealed preference is observed in the action of individuals in the market, while stated preference is based on individuals stating which alternative they would prefer in a hypothetical situation (Mark and Swait, 2004).

Revealed preference has limited use in healthcare, as healthcare is not traded explicitly and is often free at the point of care (or subsidised by insurance). Furthermore, healthcare providers act as both the supplier and the agent of healthcare, creating an imperfect market balance through asymmetry of information (Brazier et al, 2007). Practical application of revealed preference cannot be controlled

in the same way that stated preference, as existing alternatives can only be included (Mark and Swait, 2004). Although stated preference lacks the validity and reliability of revealed preference, it has grown more popular in the valuation of healthcare benefits as it can be designed to address predetermined hypotheses (Ryan et al, 2008).

DCEs are an established method of conjoint analysis used in health economics to elicit stated preferences for different services or different attributes of services. DCE are a form of attribute-based stated preference valuation. A DCE is designed as a number of hypothetical scenarios arranged into paired choice scenarios. These paired choice scenarios have a set number of attributes (e.g. cost, time, distance) with varying levels (e.g. £50 or £150) chosen by the researcher based on previous knowledge and research (e.g. literature review, focus groups of stakeholders, qualitative interviews). Individuals are asked to make trade-offs between the attributes in the DCE by comparing the variation of levels between pairwise choices, and then choosing between the two or more competing hypothetical scenarios, thus revealing their relative preference for different attributes (Gidman et al, 2007).

Although service user feedback regarding wheelchair services has been reported previously, there is no published evidence as to how wheelchair service users prioritise different attributes of wheelchair services either explicitly or implicitly, thus the relative importance of these attributes to service users is not currently known.

5.2.1. Areas for wheelchair service development

Disabled children and their parents should be engaged in shaping wheelchair services at the local level (HM Treasury and Department for Education and Skills, 2007; Bray et al, 2014). Wheelchair services should therefore be designed around the child and their family (Barnardos & WK, 2006; HM Treasury and Department for Education and Skills, 2007), and should support service users to make informed decisions about treatment, care and support (DoH, 2004). Active engagement in the development of wheelchair services is a key priority (DoH Commissioning Team, 2010; CSIP, 2006). In order for this to be achieved it is important to understand how service users prioritise the different attributes of wheelchair services, which in turn will inform how service development should be planned and prioritised.

5.3. Aims and objectives

The overarching aim was to explore the preferences of disabled children and their parents for different attributes of wheelchair services. Secondary objectives were:

- To compare the preferences of disabled children and their parents for different attributes of wheelchair services.
- To calculate hypothetical marginal rate of substitution values for different configurations of wheelchair services using cost-contribution as the denominator.
- To evaluate the use of DCE methods in disabled children in relation to wheelchair services.

5.4. Methods

See chapter two for details on recruitment, data protection and ethical considerations.

5.4.1. Design of the DCE

The attributes and levels in this pilot DCE were derived from a mixed-method systematic review of the literature (see chapter three) and through discussion with young wheelchair users (aged 11 to 18) and healthcare professionals working within wheelchair services. A list of possible attributes/levels was developed from the systematic review findings and then refined through discussion with experts in wheelchair provision. The wider supervisory research team discussed in detail the DCE attributes and levels throughout the development stages of the DCE study. Once the DCE had a preliminary design it was presented to a small sample (N=10) of young wheelchair users (aged 11 to 18) at a children's wheelchair charity beneficiary meeting (the WK Kidz Board) in order to gage their understanding of the DCE method and the appropriateness of the attributes, levels and questionnaire design.

Subsequent to the feedback provided by the Kidz Board members, the design and layout of the DCE was refined in order to make it easier to understand for children from age 11. This included developing pictorial representations of the attributes and levels to increase ease of use (see appendix E.1). Two versions of the DCE questionnaire were developed to allow for slight differences in wording of questions for parents and children. The overall design, layout, attributes and levels remained the same.

Five key attributes were identified during the process outlined above: (1) comprehensiveness of wheelchair assessment, (2) cost contribution for wheelchair, (3) level of training provided by service, (4) waiting time for delivery of wheelchair and (5) frequency of wheelchair reviews. Of these five attributes, four were assigned two levels (e.g. wait 1-3 months or 6-12 months for delivery) and one had four levels (e.g. pay nothing, £50, £150 or £300). This combination of attributes and levels produced a full factorial design of 64 hypothetical service scenarios. For ease of completion, an appropriate mixed-level orthogonal array was used to reduce the number of scenarios down to eight

with efficient design (Sloane, 2010). Coding of attribute levels for the eight scenarios was obtained from an appropriate mixed-level orthogonal array (Sloane, 2010). Scenarios were mirrored in a foldover design, so that each of the eight scenarios had a mirrored alternative with opposite attribute levels, giving a total of 16 scenarios. Mirrored scenarios were then paired to produce eight pairwise scenarios/choices, see figure 5.1 for an example pairwise choice. This ensured that there was minimum overlap and attribute levels were not repeated across pairwise choices. For each pairwise choice participants were asked to choose which of the two hypothetical service scenarios (service A or service B) they preferred. An example of a pairwise choice task is presented in figures 5.1 and 5.2. An effect code was assigned to each attribute's level to allow analysis (see table 5.1).

Service A	Service B
Your child's **health, school and social life needs** will be considered in the wheelchair assessment	Your child's **health needs** will be considered in the wheelchair assessment
The service will be **free**	You will have to **contribute £50** for your child's wheelchair
Your child will receive **wheelchair and life skills** training	Your child will receive **wheelchair skills** training
It will take between **6 and 12 months** for your child's chair to arrive	It will take between **1 and 3 months** for your child's chair to arrive
Your child's needs and wheelchair will be reviewed every **6 months**	Your child's needs and wheelchair will be reviewed every **12 months**

Which service would you prefer?

☑ **OR** ☐

Please tick **only ONE box**

Figure 5.1: Example of parent DCE pairwise choice

Table 5.1: Full list of attributes, levels and effect codes (child version)

Attribute	Level	Definition (effect coding)
Comprehensiveness of wheelchair assessment	Health needs	Your health needs will be considered in the wheelchair assessment (0)
	Health, school and social life needs	Your health, school and social life needs will be considered in the wheelchair assessment (1)
Cost (£) contribution for wheelchair	No cost	You will not have to contribute any money for your wheelchair (0)
	£50	You will have to contribute £50 for your wheelchair. This would be a one-off payment for each new wheelchair (50)
	£150	You will have to contribute £150 for your wheelchair. This would be a one-off payment for each new wheelchair (150)
	£300	You will have to contribute £300 for your wheelchair. This would be a one-off payment for each new wheelchair (300)
Level of training provided by service	Wheelchair skills training	You will receive wheelchair skills training as part of the service. Wheelchair skills training will include wheelchair driving techniques, road safety and maintaining your wheelchair (0)
	Wheelchair and life skills training	You will receive wheelchair skills training and life skills training as part of the service. Wheelchair skills training will include wheelchair driving techniques, road safety and maintaining your wheelchair. Life skills training will include work placements, learning independence and ambassador groups (1)
Delivery time for delivery wheelchair	Between 1 and 3 months	It will take between 1 and 3 months for your wheelchair to be delivered after the final assessment (0)
	Between 6 and 12 months	It will take between 6 and 12 months for your wheelchair to be delivered after the final assessment (1)
Frequency of wheelchair review	At least every 6 months	Your needs and wheelchair will be reviewed every 6 months. This will include a reassessment of your needs and a review of your wheelchair for any maintenance or repairs it requires (6)
	At least every 12 months	Your needs and wheelchair will be reviewed every 12 months. This will include a reassessment of your needs and a review of your wheelchair for any maintenance or repairs it requires (12)

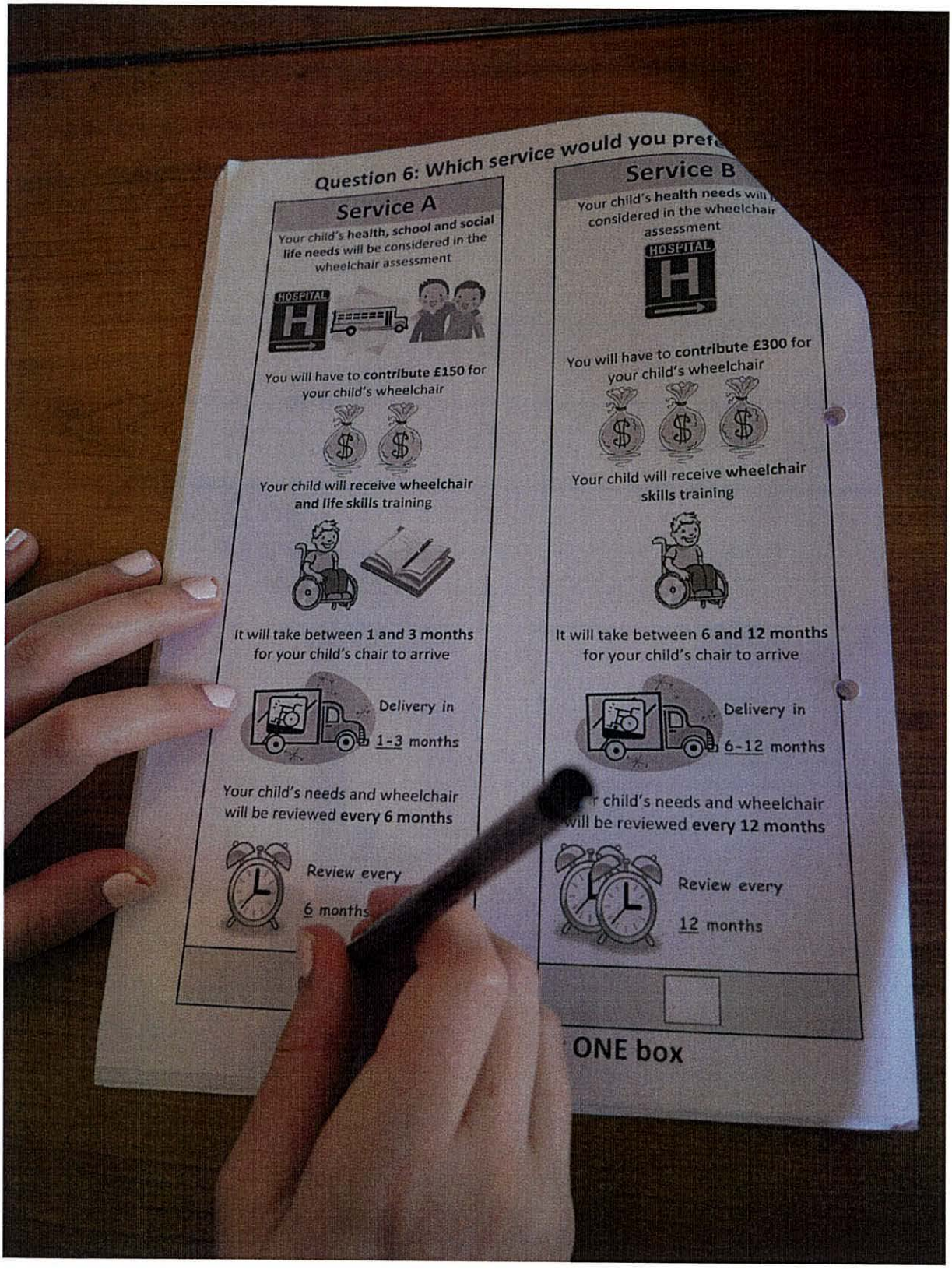


Figure 5.2: Example of parent DCE questionnaire being completed

5.4.2. Data collection

The DCE questionnaire was presented to the participants as part of an interview and explained by the interviewer. Participants completed a self-administered DCE questionnaire, with the interviewer present to answer any questions. A small number of participants (N=6) chose not to take part in the interview but did agree to complete a DCE questionnaire, which was instead posted to them. The DCE questionnaire contained an attribute ranking task and eight pairwise choice tasks, each with five attributes with varying levels. Instructions on how to complete the DCE questionnaire were presented to the participants at the beginning of the questionnaire, with an example answer (see appendix E.2). A supplementary notes section was included with the questionnaire for further information on the attributes and levels (see appendix E.2)

5.4.3. Data analysis

SPSS v20.0 and Stata v10.1 were used to analyse the data. DCE data were analysed using the conditional logit model logistic regression technique (Ryan et al, 2008; Ryan and Gerard 2003), see appendices E.3 for model calculation. Using this technique the magnitude of the β-coefficient is relative to the change in utility as a result of change in the attribute's level. A positive β-coefficient indicates that as the level increases so does the likelihood of a participant choosing it. Likewise, a negative β-coefficient indicates that as the level decreases, the likelihood of participants choosing it increases.

It was hypothesised that a positive β-coefficient would be observed for the comprehensiveness of wheelchair assessment attribute and level of training provided by service attribute, as participants were expected to prefer to have additional in-depth assessment of needs (including assessment of health, education and social needs) and additional training (wheelchair and life skills training). For the other attributes it was hypothesised that a negative β-coefficient would be observed, as participants were expected to prefer lower cost contribution, shorter waiting time for delivery and more regular wheelchair reviews.

As there were a mixture of quantitative and qualitative attributes, attributes were not directly comparable on the same scale, thus MRS was calculated to attain common scale for all attributes. This allowed comparison between attributes to be made. MRS is the amount of a given attribute that a person is willing to forgo to obtain one additional unit of another attribute. For instance, an individual may be willing to contribute towards the cost of the wheelchair in order to reduce the delay in wheelchair delivery. Cost contribution (a quantitative scale) was used as the denominator to calculate the MRS for a one-unit change in each of the remaining attributes. By dividing the other

attributes' coefficients by the cost contribution coefficient the MRS was indirectly estimated. 95% confidence intervals for the β-coefficients were estimated using non-parametric bootstrapping methods, run on 5000 iterations using Stata v10.1.

5.5. Results

5.5.1. Response rate and sample size

A total of 125 study invitation packs were distributed across England and Wales. These contained initial questionnaires for disabled children and parents. Disabled children and parents were recruited from the same household where possible (and appropriate). Of the disabled children who returned an initial questionnaire (N=15), 13 met eligibility criteria (aged >10) and were invited to complete the DCE questionnaire. A total of 11 disabled children completed the DCE questionnaire (84.6% response rate). All returned DCE questionnaires were completed in full with no major data omissions. 35 initial questionnaires were returned by parents (28% response rate), who were then invited to complete the DCE questionnaire. Of that number, 30 parent DCE questionnaires were completed in full (85.7% response rate).

5.5.2. Demographic characteristics

Demographic details of the samples are presented in tables 5.2 and 5.3. In the disabled child sample (N=11), 63.6% (N=7) were male, 63.6% (N=7) were aged 16 to 18 and 81.8% (N=9) had cerebral palsy. In the parent sample (N=30), 86.7% (N=26) of respondents were women and aged between 30 and 49. Of the sampled parents 66.7% (N=20) had a child with cerebral palsy and half had a child under the age of 5, illustrating a wider variance in child age than the disabled child sample. There is a lack of ethnic diversity in both samples with the vast majority of respondents being white-British.

Table 5.2: Demographic characteristics of the disabled child sample (n=11)

Demographic characteristics	Number (%)
Study site	
NHS Wheelchair Service	2 (18.2)
Charity	9 (81.8
Gender	
Female	4 (36.4)
Male	7 (63.6)
Age	
11-15 years	4 (36.4)
16-18 years	7 (63.6)
Ethnicity	
White British	11 (100)
Education	
High school	4 (36.4)
College	5 (45.5)
University	1 (9.1)
Home schooled	1 (9.1)
Child's condition	
Cerebral Palsy	9 (81.8)
Muscular Dystrophy	1 (9.1)
Hemiplegia / stroke	1 (9.1)
Frequency of equipment use	
Most of the time	1 (9.1)
All of the time	10 (90.9)
Type of equipment used	
Manual	3 (27.3)
Manual and powered	8 (72.8)

Table 5.3: Demographic characteristics of the parent sample (n=30)

Demographic characteristics	Number (%)
Study site	
NHS Wheelchair Service	5 (16.7)
BIME	10 (33.3)
Whizz-Kidz	15 (50.0)
Gender	
Female	26 (86.7)
Male	4 (13.3)
Age	
21-29 years	2 (6.7)
30-39 years	14 (46.7)
40-49 years	12 (40.0)
50-59 years	2 (6.7)
Ethnicity	
White British	29 (96.7)
White & Asian	1 (3.3)
Marital status	
Married	23 (76.7)
Co-habiting	3 (10.0)
Single	2 (6.7)
Separated	1 (3.3)
Divorced	1 (3.3)
Education	
Higher	14 (46.7)
Further (e.g. A Level)	3 (10.0)
GCSE/O level	7 (23.3)
Other	1 (3.3)
None	5 (16.7)
Annual household Income	
£5000-15,000	3 (10.0)
£16,000-£25,000	5 (16.7)
£26,000-£35,000	3 (10.0)
£36,000-£50,000	10 (33.3)
£51,000-£75,000	4 (13.3)
£75,000 or more	4 (13.3)
Missing	1 (3.3)
Employment status	
Full-time	5 (16.7)
Part-time	12 (40.0)
Unemployed / stay at home parent	13 (43.3)
Child's condition	
Cerebral Palsy	20 (66.7)
Spinal Muscular Atrophy	2 (6.7)
Muscular Dystrophy	3 (10.0)
Chromosome deletion	1 (3.3)
Hemiplegia / stroke	1 (3.3)
Lissencephally	1 (3.3)
Rett syndrome	1 (3.3)
Porencephaly	1 (3.3)
Child's age	
5 years or under	15 (50.0)
6-15 years	10 (33.3)

Table 5.3 (parent sample demographics) continued

16-18 years	5 (16.7)
Frequency of child's equipment use	
A little of time	1 (3.3)
Some of the time	6 (20.0)
Most of the time	4
All of the time	18 (60.0)
Missing	1 (3.3)
Type of equipment used by child	
Powered	2 (6.7)
Manual	10 (33.3)
Manual and powered	17 (56.7)
Waiting for first wheelchair	1 (3.3)

5.5.3. DCE results: Disabled child sample (N=11)

Table 5.4 shows the results for the two samples. The β-coefficients of three of the five attributes were statistically significant ($P<0.05$): comprehensiveness of wheelchair assessment ($P=0.009$), waiting time for delivery of wheelchair ($P=0.041$) and cost contribution for wheelchair ($P=0.019$). The remaining two attributes were non-significant: level of training provided by service ($P=0.924$) and frequency of wheelchair reviews ($P=0.519$). Based on the β-coefficients and MRS values, comprehensiveness of wheelchair assessment was of greatest importance (β-coefficient=1.4247, MRS= £152.61), followed by waiting time for delivery of wheelchair (β-coefficient=-0.9221, MRS=£98.77) and cost contribution for wheelchair (β-coefficient =-0.0093). Preference was shown for comprehensive wheelchair assessments (of health, education and social needs), shorter waiting time for delivery of wheelchair and lower cost contribution. For the remaining two non-significant attributes, disabled children preferred (if everything being equal) wheelchair and life skills training and less frequent wheelchair reviews.

5.5.4. DCE results: Parent sample (N=30)

The β-coefficients of two of the five attributes were statistically significant ($P<0.05$). These were: comprehensiveness of wheelchair assessments ($P=0.000$) and waiting time for wheelchair delivery ($P=0.000$). This indicates that these two attributes were significant factors in parental choices. The remaining three attributes were non-significant: cost contribution for wheelchair ($P=0.092$), level of training provided by service ($P=0.371$) and frequency of wheelchair reviews ($P=0.260$). Based on the β-coefficients and MRS values, comprehensiveness of wheelchair assessment was of greatest importance (β-coefficient=1.5329, MRS= £548.29), followed by waiting time for delivery of wheelchair (β-coefficient=-1.3699, MRS=£490.02). Preference was shown for comprehensive wheelchair assessments (of health, education and social needs) and shorter waiting time for delivery

of wheelchair. For the remaining three non-significant attributes, parents preferred (if everything being equal) lower cost contribution, basic wheelchair skills training and more frequent wheelchair reviews. As cost contribution was not significant, parental MRS values are not reliable.

5.5.5. Comparison of disabled child and parent DCE results

Both samples showed preference for comprehensive wheelchair assessments and shorter wheelchair delivery times, in that order for both samples. The cost contribution attribute was only significant for the child sample, who showed preference for lower cost contribution. MRS values were higher for parents (£548.29 [CI £353.38 to £1435.45] for wheelchair assessment and £490.02 [CI £313.29 to £1326.77] for delivery waiting time) than for disabled children (£152.61 [CI £133.20 to £182.53] for wheelchair assessment and £98.77 [CI £81.93 to £121.32] for delivery waiting time), suggesting the parent sample placed higher importance on these attributes than the disabled child sample. However, as the cost contribution attribute was not significant for parents, it is difficult to make direction comparisons with the disabled child data.

The disabled child and parent samples differed in direction of coefficient preference for level of training provided by service and frequency of wheelchair reviews: the β-coefficients for both attributes indicate that, everything being equal, parents preferred basic wheelchair skills training (β-coefficient =-0.1557) and more frequent wheelchair reviews (β-coefficient=-0.0390), while disabled children preferred wheelchair and life skills training (β-coefficient =0.0306) and less frequent wheelchair reviews (β-coefficient =0.0364). However, the β-coefficients for these attributes were not significant.

Table 5.4: Results from the conditional logit model: disabled child study sample (n=11) and parent study sample (n=30)

	Disabled child sample (n=11)					Parent sample (n=30)				
Attribute	**β-coefficient**	**95% CI****	***P*-value**	**MRS values*** (cost)**	**95% CI****	**β-coefficient**	**95% CI****	***P*-value**	**MRS values***△ (cost)**	**95% CI****
Comprehensiveness of wheelchair assessment	1.4247*	1.4153 to 2.0824	0.009	£152.61	£133.20 to £182.53	1.5329*	1.4507 to 2.1633	0.000	£548.29	£353.38 to £1435.45
Cost contribution for wheelchair	-0.0093*	-0.0138 to -0.0089	0.019	--	--	-0.0028	-0.0060 to 0.0005	0.092	--	--
Level of training provided by service	0.0306	-0.1955 to 0.2858	0.924	--	--	-0.1557	-0.4002 to 0.0311	0.371	--	--
Waiting time for delivery of wheelchair	-0.9221*	-1.4086 to -0.8442	0.041	£98.77	£81.93 to £121.32	-1.3699*	-1.9859 to -1.3104	0.000	£490.02	£313.29 to £1326.78
Frequency of wheelchair reviews	0.0364	-0.0022 to 0.0749	0.519	--	--	-0.0390	-0.0813 to 0.0032	0.260	--	--
Number of observations= 88 Number of individuals = 11 Log likelihood function = -26.64 Log likelihood ratio (5) = 33.85						Number of observations= 240 Number of individuals = 30 Log likelihood function = -64.51 Log likelihood ratio (5) = 114.86				

*Significant attribute [$P < 0.05$]

**95% confidence intervals generated using non-parametric bootstrapping (5000 replications)

***Marginal rate of substitution values = β-coefficient for attribute/β-coefficient for cost attribute

△ Though the cost contribution attribute was not significant to parents (P=0.092 [>0.05]), everything being equal, parents preferred lower cost contribution; the parents' MRS values were calculated using the cost contribution attribute as the denominator to show how parents trade-off the cost contribution attribute against the other attributes. This allowed comparison with the disabled child sample MRS values.

5.5.6. Sub-group analysis: Matched-pairs of disabled children and their parents

As the original sample of parents (N=30) contained a diverse child age range, some differences between parent and disabled child preferences may be due to different service needs at different stages of development. Half of the parents (N=15) in the original sample analysis had a child aged 5 or under, and thus parental service preferences may have been skewed towards parents of younger disabled children. Likewise, all sampled children (N=11) were aged 11 or over, with 63.6% aged 16 to 18 (N=7). This highlights issues with making direct comparisons between the service preferences of these two samples. In order to test preferences a sub-group analysis was performed using only the data from matched-pairs of disabled children (N=9) and their parents (N=9), see table 5.5 for results. A smaller distribution of child age was observed, with all children aged 11 or over (63.6% [N=7] aged 16 or over).

Similarly to the main analysis, both the disabled child and parent samples showed significant preference for comprehensive wheelchair assessments (β-coefficients= 1.6194 [*P*=0.015] and 2.1893 [*P*=0.010] respectively). The cost contribution attribute was not significant for the parent sample but was borderline significant for the child sample, who showed preference for lower cost contribution (β-coefficients= -0.0095 [*P*=0.050]). Using cost contribution as the denominator, the MRS value for wheelchair assessment was higher for parents (£307.14 [CI £252.53 to £472.02]) than for disabled children (£170.03 [CI £150.41 to 201.86]), suggesting at face value that the parent sample were willing to contribute more financially to receive comprehensive assessments for their children. However, as the cost contribution attribute was not significant for parents, it is difficult to make direction comparisons with the disabled child data. This result indicates that parental service preference was not significantly impacted by cost contribution, which was also observed in the main analysis.

Unlike the findings from the main analysis, the matched disabled children and parents did not differ in direction of coefficient preference on any of the attributes. This indicates that, everything being equal, both sub-group samples had a significant preference for comprehensive wheelchair assessments (of health, education and social needs), and for the remaining four non-significant attributes, both the disabled child and parent samples showed preference for lower cost contribution (borderline significant for children [0.05]) (β =-0.0095 and -0.0071 respectively), basic wheelchair skills training (β =-0.1998 and -0.3497 respectively), shorter waiting time for delivery (β =-1.0104 and -1.2671 respectively) and less frequent wheelchair reviews (β =0.0433 and 0.0040 respectively).

Table 5.5: Sub-group analysis: Results from the conditional logit model of disabled child (n=9) and parent (n=9) matched-pairs

	Disabled child sample (n=9)					Parent sample (n=9)				
Attribute	**β-coefficient**	**95% CI****	***P*-value**	**MRS values*** (cost)**	**95% CI****	**β-coefficient**	**95% CI****	***P*-value**	**MRS values***△ (cost)**	**95% CI****
Comprehensiveness of wheelchair assessment	1.6194*	1.5745 to 2.2935	0.015	£170.03	£150.41 to £201.86	2.1893*	2.2088 to 3.2599	0.010	£307.14	£252.53 to £472.02
Cost contribution for wheelchair	-0.0095▲	-0.0139 to -0.0087	0.050	--	--	-0.0071	-0.0192 to 0.0049	0.246	--	--
Level of training provided by service	-0.1998	-0.4645 to 0.0210	0.588	--	--	-0.3498	-1.2170 to 0.5176	0.429	--	--
Waiting time for delivery of wheelchair	-1.0104	-2.0454 to 0.0246	0.056	--	--	-1.2671	-2.5926 to 0.0713	0.064	--	--
Frequency of wheelchair reviews	0.0433	-0.0813 to 0.1680	0.495	--	--	0.0040	-0.0452 to 0.0429	0.955	--	--
Number of observations= 72						Number of observations= 72				
Number of individuals = 9						Number of individuals = 9				
Log likelihood function = -22.51						Log likelihood function = -16.84				
Log likelihood ratio (5) = 27.38						Log likelihood ratio (5) = 41.01				

* Significant attribute [$P < 0.05$]

**95% confidence intervals generated using non-parametric bootstrapping (5000 replications)

***Marginal rate of substitution values = β-coefficient for attribute/β-coefficient for cost attribute

▲Borderline significant attribute [P=0.05]

△Though the cost contribution attribute was not significant for either sample, everything being equal, both samples preferred lower cost contribution. MRS values were calculated using the cost contribution attribute as the denominator to show how participants trade-off cost contribution against the other service attributes.

5.6. Discussion

This chapter reports the first study to elicit and compare the preferences of disabled children and their parents for different attributes of wheelchair services using DCE methods. The findings in this chapter illustrate that DCEs can be used successfully in potentially vulnerable samples of the population (such as disabled children) in the assessment of healthcare services. If appropriately powered, DCE results can be used as a valuable asset in priority setting, service development and healthcare decision-making, as they allow different attributes of services to be ranked by importance, and their relative monetary value to families calculated using MRS.

5.6.1. Main findings from primary analysis

For this sample of 11 disabled children and 30 parents of disabled children comprehensiveness of wheelchair assessment was the most important attribute of wheelchair services, followed by wheelchair delivery time. The β-coefficients for these attributes indicate that both the sampled disabled children and parents had preference for services with comprehensive wheelchair assessments (assessment of health, education and social needs) and shorter wheelchair delivery times. The results from the disabled child sample also indicated that cost contribution was an important attribute and lower cost contribution was preferred. The remaining two attributes (level of training provided by service and frequency of wheelchair reviews) were not statistically significant ($p>0.05$) for either sample, and thus they did not impact service preferences.

Both samples showed preference for services that offered assessments which focused on the health, education and social needs of children, as opposed to just health needs. NHS wheelchair services tend to focus on clinical health needs in wheelchair assessment and provision, which may neglect to consider other important aspects of disabled children's lives (National Assembly for Wales, 2010).

5.6.2. Marginal rate of substitution

As cost was a significant attribute for the sampled disabled children it could be used as a denominator in MRS calculations for a single unit change in the other attributes. As cost was a non-significant attribute for the parent sample it could not be used for MRS calculations; however, assuming everything being equal, the cost coefficient did appear to indicate that sampled parents preferred services with lower cost contributions, as would be expected. As an exploratory exercise to allow comparison with the disabled child sample MRS values, cost was used as a denominator for the parent sample and MRS

analysis was performed for both samples, bearing in mind the non-significance of cost contribution to the sampled parents. Willingness to pay was not calculated as the cost attribute referred to cost contribution rather than the full cost of the service. Furthermore, as the sample sizes were small and the cost contribution attribute was only significant for children it was not deemed appropriate to do full willingness to pay calculations beyond MRS calculations.

The MRS values of the wheelchair assessment and delivery time attributes were different for the two samples, with parental MRS values higher for both attributes. This would suggest that the sampled parents were willing to contribute more money to attain preferred levels in these attributes for their child, compared to the disabled child him/herself. However, it is important to reiterate that cost contribution was not a significant attribute for sampled parents, while it was for sampled disabled children. Assumptions made regarding MRS should be viewed with caution.

The significance results of the cost contribution attribute would suggest that for sampled parents cost contribution does not influence service preference, but it did for sampled disabled children. This may reflect that the parent sample were willing to pay more to obtain the best suited services for their child, thus contribution cost did not influence their preferences. The sampled disabled children may have felt some degree of burden on their parents and therefore may have been influenced by the cost contribution attribute. As children rely on their parents for financial support, the sampled disabled children may not have been as aware of the value of money or may have been more wary of putting further financial pressure on their parents.

Identical cost contribution levels were used for both samples, which did not take into account differences in how the two groups value money, particularly as the sampled disabled children would have expected to spend family money rather than their own. The cost contribution attribute levels were based on a number of sources, including the deposit cost for a BIME Wizzybug (£200) and the average cost of a standard MWC from the NHS (£270 [Curtis et al, 2013]), I also wanted to factor in the voucher scheme which allows service users to request a voucher towards the cost of a privately funded wheelchair, thus reducing service user expenditure (Sanderson et al, 2000). With this in mind the attribute levels were set relatively low (£0, £50, £150, £300) in order to reflect a reasonable service user contribution. Higher cost levels may have made the cost contribution attribute too predictable and thus may have skewed preferences. In hindsight sampled parents may have felt that contributing up to £300 for a wheelchair was relatively good value for money, while sampled disabled children may have considered this to be a significant amount of money. It is of note that 60% (N=18) of sampled parents

had a household income of over £36,000 per year, which may have impacted their willingness to contribute financially to receive a better service for their child.

A future DCE in this field should consider using considerably higher cost contribution attribute levels to test these issues, for instance setting the levels at retail prices for different types of wheelchairs (e.g. £0, £500, £1500, £3000), or conversely using a more child-friendly approach to cost contribution, such as proportion of income/pocket money. A larger sample would be beneficial as it would enable additional sub-group analyses, such as analysing the effect of household income on preferences and MRS values.

5.6.3. Attribute β-coefficient directions

Most of the attributes' β-coefficient directions are reflective of *a priori* hypotheses, although the coefficient directions for frequency of wheelchair reviews for disabled children and level of training for parents were contradictory to these hypotheses. It is interesting that the parent sample showed preference for their child to receive just wheelchair skills training as opposed to wheelchair and life skills training, although this was not statistically significant. The coefficient direction for the level of training attribute may indicate that sampled parents did not feel it was the responsibility of wheelchair services to provide life skills alongside wheelchair skills training, or potentially that the provision of life skills training may impact on essential wheelchair skills training. Future research may benefit from defining life skills training based on age (e.g. play skills for children under 5). Likewise, more appropriate terminology may be required for young people with profound learning impairments to make the attributes more relevant to their specific circumstances.

For the frequency of wheelchair reviews attribute the disabled child sample preferred less frequent reviews as opposed to more frequent reviews, although this was also non-significant. This may indicate that the sampled disabled children did not necessarily see the benefit of more frequent reviews of their needs, or they may not enjoy reviews and thus would prefer them to be less frequent. The non-significant attributes did not influence the wheelchair service preferences of the samples, thus interpretations about preferences are limited.

5.6.4. Sub-group analysis

Sub-group analysis was used to examine differences in preferences between matched-pairs of disabled children (N=9) and their parents (N=9). The aim of this additional analysis was to examine the potential influence of child age specifically on parental preferences, as the service needs of disabled children and

their parents are likely to change with child age. The results demonstrate similarities between child and parent preferences for children over the age of 11.

Comparing these results to the main study sample shows variation in parental preferences for frequency of wheelchair reviews and the significance of delivery time on service preference. Interestingly, the sub-group of parents with children aged 11 or over preferred less frequent reviews (as did their children), while the full sample of parents had preference for more frequent reviews. As half of the parents in the full sample had a child aged 5 or under this is not entirely surprising, as younger children need more frequent reviews due to their rapidly changing needs associated with growth and development. Interestingly, only comprehensiveness of assessment was found to be a significant attribute in all samples, and thus is the most influential attribute on the service preferences of the participants in this study.

Level of training was not found to be significant in either the full or sub-sample of parents. β-coefficient directions indicated that in general parents had preference for basic training, which is particularly interesting in the sub-sample analysis. I previously theorised that the β-coefficient direction in the full parent sample may have been skewed by parents of younger children, but in fact the preference for basic training was observed across the age range. This may indicate that parents believe that life skills training is beyond the remit of wheelchair services, or that a focus on basic wheelchair skills training would be more beneficial than also incorporating other forms of training.

These findings reinforce that wheelchair services must be age-specific and able to adapt to changes in needs over time. The key issue is that assessments take into account the changing holistic needs of children and not just their clinical needs.

5.6.5. Policy and health service implications

To some extent the results reinforce the findings from previous research and recommendations from government and NFPO reports. Wheelchairs are important interventions for disabled children to enhance independence, social inclusion and participation (Evans et al, 2007; Wiart et al, 2003; Wiart et al, 2004; Home and Ham, 2003; Bottos et al, 2001). It is thus important that wheelchair provision supports optimised physical, cognitive and social development (DoH, 2004), and that wheelchairs are useable in all places required (Welsh Assembly Government, 2005; DoH, 2004; CSIP, 2006). A holistic approach to assessment and performance measures should be employed to cater for the clinical, social, educational and lifestyle needs of service users (DoH Commissioning Team, 2010; National Assembly for

Wales, 2010). In order for disabled children to achieve the best outcomes, wheelchairs must be delivered quickly and within set timelines (Muscular Dystrophy Campaign, 2010; NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2005; HM Treasury and Department for Education and Skills, 2007; Welsh Assembly Government, 2005; DoH, 2004; DoH Commissioning Team, 2010; National Assembly for Wales, 2010), and should be reviewed at least annually (Welsh Assembly Government, 2005).

5.6.6. Study limitations and critique of the method

This underpowered DCE study is a pilot with small sample sizes, and was in part designed to test the methodology in this particular population group. A revised DCE study with a larger sample size in the same population group would be informative and beneficial for guiding wheelchair service development. The results in this chapter indicate that DCEs can be used effectively with disabled children aged over 11 to elicit preferences for different attributes of wheelchair services. Parents of younger disabled children (aged under 11) felt that the questionnaire was not suitable for their child due to the complexity of the questionnaire or due to the nature of their child's disability. I therefore cannot comment on the suitability of using DCE methods in children under the age of 11 or with children with profound learning impairments. Future research would benefit from testing the understanding of child respondents to ensure that they truly understand the DCE task.

It is interesting to note that all participants completed the DCE questionnaire in full without error or missing data. Participants appeared to understand the instructions given and completed the questionnaire with relative ease. This may be due to the presence of an interviewer to explain the questionnaire and to answer any questions participants may have had. Time was taken to ensure materials were appropriate for both children and adults. Pictorial representations, appropriate language and accessible layout were used to improve clarity and facilitate accurate completion (see appendix E.1). Advice from stakeholders (through the WK Kidz Board) allowed me to adapt the layout, instructions and attributes/levels for the target population. Optimising the design of the DCE may have facilitated accurate completion by participants, and thus informative elicitation of preferences. It should be noted that all child participants had mental capacity and were able to consent to take part in the study, thus a convenience sample was used. It therefore cannot be stated that the results are representative of children with learning impairments.

Recruitment for this DCE was relatively simple, particularly as the sample sizes were small. I had originally intended to have more child participants, but due to child ages and level of cognitive ability it was difficult to do so. Recruitment from the NHS wheelchair service proved to be the most difficult, which may reflect how engaged NHS service users feel as compared to users of charity-led wheelchair services. It is interesting that the results from the two samples were relatively similar, particularly in the sub-group analysis of matched-pairs. Although parents and their children completed the DCE questionnaire at the same time, they did so without conferring and thus were not able to influence one another. Participants appeared to have little difficulty understanding the concept of the questionnaire, which may be in part due to the way the scenarios were laid out and the use of illustrated representations of attribute levels.

Due to the size of the samples and their demographic characteristics, the results are not generalisable to the wider population of disabled children who use wheelchairs and their parents. The vast majority of parent participants were white-British mothers of children with cerebral palsy. All of the disabled child participants were white-British and the majority had cerebral palsy. It is important to also consider the impact of household income on MRS values. The samples were relatively self-selective, thus the important views of disengaged or unmotivated individuals may have been missed. Future research should focus on achieving a more representative sample and should include subset analysis to analyse differences in preferences between groups (e.g. socioeconomic status).

Understanding the appropriateness of the DCE method in specific settings, such as wheelchair services, is important to researchers and decision-makers. Due to small sample sizes subgroup analyses by age, developmental level, cognitive ability and disability prognosis were not possible. The age range of the child sample (11-18 years) could be considered too vast to draw together the results and make wider conclusions, particularly with such a small sample. Likewise, the full parent sample had children ranging from age 2 to 18 years, and thus priorities for wheelchair services would rightly be different depending on the child's age, ability and condition. Furthermore I was unable to take into account important factors such as whether the child had a life-limiting condition or the purpose of the mobility equipment for very young children. For instance, the families recruited by BIME used a Wizzybug PWC for very young children (<5 years), which is conceptualised with a dual purpose: mobility equipment and a toy to learn independent movement. This would therefore impact preference for attributes that are not necessarily relevant for this group of very young children, for instance life skills training. Given the small

sample sizes there is a danger of child age causing aggregation to the mean by, for example, including a parent of a 2 year old in the same analysis as a parent of an 18 year old.

The differences between the child and parent groups in terms of child age-related needs and cognitive development are also difficult to compare and different outcomes should be expected. The child age range is too wide and the samples are too small to make any tangible conclusions irrespective of statistical significance. Making comparisons between child and parent samples also raises some interesting issues. As a general rule children are not expected to take full responsibility for what happens in their lives; it is up to their parents to take this responsibility, particularly for young children. It is therefore not surprising that there were differences between child and parent preferences. Conversely in the sub-group analysis child and parent preferences were relatively similar and comparable, particularly in terms of β-coefficient directions. This raises some interesting questions as to whether children and parents influenced each other's preferences, or whether they genuinely had a shared sense of service preference. This sub-group analysis allowed the influence of child age to be removed to some extent, and thus gave a more refined understanding of the relationship between child and parent service preferences for disabled children aged 11 to 18. Due to sample size it is not possible to make definite conclusions, although these results appear to compliment the findings from the primary analysis.

5.6.7. Further research and methodological implications

The greatest implication of this research is perhaps not the particular preferences of individuals but that the sampled children had the cognitive ability to understand the process and methodology. Multiple longitudinal completion of DCEs over time would likely be important to record differences in preferences between age groups and disabilities. For children not expected to live until adulthood due to life-limiting conditions, long term outcomes such as life skills are likely to be less important compared to getting the right wheelchair and quickly. In addition, children and parents look to services and professionals to give them the benefit of their expertise and to provide them with a fit for purpose service, especially when a child's illness trajectory is uncertain and there is no expertise to draw on in the family.

It is difficult to see how policy decisions about service attributes could be made based solely on this type of DCE involving disabled children and their parents (even with adequate numbers and statistical power). DCE data in this scenario would need to be supplemented with evidence of effect to see if

additional service attributes improve age-related outcomes. At present this data is limited, and thus additional research into many aspects of wheelchair provision for disabled children is needed. If service commissioners were to decide to follow lean principles and strip away attributes of wheelchair services based on the results of a large scale DCE, they would be doing so based on the testimonies of families who potentially had not been exposed to aspects of the service they were being asked to state preference for. I can therefore only conclude that the use of DCE methods in this population group is feasible. I therefore regard this pilot DCE study as a success as it raises both methodological and service commissioning questions which are essential to developing the best wheelchair services for disabled children.

5.6.8. Implications for conceptual framework

The findings in this chapter have specific relevance to the conceptual framework developed in chapter three. Continued service development through consultation with service users is necessary to ensure that all NHS wheelchair services meet the needs of service users. DCE methods could be used to elicit service user preferences on a national scale in order to identify the key areas for service development. This could refocus service provision based on the preferences of disabled children and their parents. Consultation with service users is therefore of key priority, and DCE methods would allow preferences to be measured quantitatively and could produce actionable results relatively quickly.

The conceptual framework highlights that continued service development should involve collaboration with service users. The use of DCE methods could enable the preferences of service users to be elicited in a robust and relatively simple manner. This study demonstrates the potential benefits of using DCE methods in this population. In the context of wheelchair service development, the needs and priorities of service users are key to effective development. As demonstrated in chapter 3, many policy reports have recommended the involvement of services users in service development (DoH, 2004; Barnardos and WK, 2006; HM Treasury and DES, 2007; DoH Commissioning Team, 2010).

The results of this DCE indicate that holistic assessment of needs is a key priority for service users. The conceptual framework indicates that services should focus on outcomes beyond health, including psychosocial needs. In order to do so, appropriate outcome measures are needed. Therefore, the applicability of existing outcomes must be tested, or new measures should be developed. In order to appropriately measure the impact of a wheelchair appropriate outcome measures are needed, and likewise these measures should reflect the holistic needs of disabled children. Furthermore, timely

delivery of equipment should be made a priority to ensure that children get the most out of their equipment.

Balancing the clinical needs of disabled children and the wider benefits of appropriate mobility equipment is key for wheelchair services. The traditional focus on clinical needs does have some benefits, as it is imperative that health, posture and function are considered as part of wheelchair provision. However, the importance of other outcomes must not be diminished, particularly outcomes which reflect the desires of disabled children, for instance developing independence and social interaction. Likewise, consideration of age and how outcomes and preferences change over time must be taken into account.

These results highlight the importance of appropriate outcomes to enable services to measure change and improvement. The subsequent chapters will explore the appropriateness of generic measures of utility in this context, and how disabled children define QoL in relation to wheelchair use.

5.7. Conclusion

The results from this chapter cannot be generalised to the wider population of disabled children and parents due the small sample sizes and unrepresentative demographic characteristics. However, the results indicate that for this cohort of disabled children and their parents the most important wheelchair service attributes were comprehensiveness of wheelchair assessment and wheelchair delivery time. These results do show congruence with previous literature, which indicates that the key priorities in wheelchair services should be holistic assessment of wheelchair needs of disabled children and wheelchair delivery in a timely manner. The results indicate that sampled disabled children and parents were willing to contribute financially to receive preferred attribute levels of wheelchair services, although cost contribution was not shown to be an important attribute to the parent sample, and thus did not have an impact on their service preferences. Future research could utilise larger and more representative samples. More research is needed into the effective measurement of outcomes from wheelchair provision, particularly addressing social, education and independence needs of disabled children.

DCE methods can be used effectively to examine wheelchair service preferences of disabled children (aged 11 and over) and their parents. Care must be taken to ensure that DCE methods are used appropriately, for instance taking into account the layout, language and presentation of the DCE

questionnaire. Consideration of methodological implications is required when comparing child and parent preferences.

Chapter Six: Measuring health-related quality of life of young wheelchair users: Testing agreement and correlation between the EQ-5D-Y and HUI outcome measures using self-reported and proxy outcomes.

6.1. Chapter summary

As discussed in the previous chapter, service user preferences cannot guide wheelchair service development alone, thus robust cost-effectiveness evidence is also needed. Chapter four highlighted the issues of costing wheelchair interventions for children. In order to perform cost-utility analysis both costs and utilities are required. NICE advises NHS funding allocation based on the clinical and cost-effectiveness of interventions. NICE technology appraisal guidance recommends use of the QALY as a primary outcome, furthermore this guidance specifically recommends the EQ-5D as the favoured tool for eliciting HRQoL utility data. The HUI outcome measures are a well-established alternative to the EQ-5D in child populations. In order to accurately calculate QALY estimates for wheelchair interventions for children it is important to understand whether standard HRQoL outcome measures are accurately measuring the HRQoL of young wheelchair users, and whether proxy reports can be used in circumstances where children are unable to self-report. In this chapter I report HRQoL correlation and agreement between the HUI and EQ-5D-Y measures and between children and parents in order to assess the applicability of these measures.

15 children and 36 parent proxies participated in the study, with 13 matched-pairs of child/parent proxy data. Overall I found there to be limited agreement between measures in both cohorts. Parents undervalued their child's HRQoL, but importantly did so in a consistent manner. Measuring correlation alone is insufficient to understand the true relationship between outcome measures and respondent types. At the least, child value sets are required to fully understand the HRQoL of children using the EQ-5D-Y.

6.2. Introduction

6.2.1. Health-related quality of life outcome measures and the QALY

Generic, preference-based measures of HRQoL are used by health economists to assess the utility outcomes of clinical interventions. Utility refers to the subjective level of wellbeing experienced in different health states (Robinson, 1993). Each possible health state is assigned a quality weight based on the desirability of that state, for instance ranging from death to perfect health (Neumann et al, 2000). Cost-utility analysis allows comparison between varied and unrelated interventions as relative benefits can be assessed based on a single comparable measure. The most commonly used utility measure is the

quality adjusted life-year (QALY), which is calculated by combining length and quality of life. For instance, two years of life in a health state rated at 50% QoL would equate to one QALY (2 x 0.5).

A great deal of research is undertaken to develop descriptive systems and to assign utility weights for individual HRQoL measures such as the EQ-5D and HUI (EuroQoL Group, 1990; Saigal et al, 1994; Dolan et al, 1996; Torrance et al, 1996; Kind et al, 1999; Feeny et al, 2002; Horsman et al, 2003; Feeny et al, 2004; Pogany et al, 2006). Utility weights are usually based on the health state preferences of general population samples, and thus can make measures less suited to specific clinical settings. For this reason disease-specific measures of QoL can be more suited to specific disease-states and conditions (Patrick and Deyo, 1989). However, disease-specific measures are rarely suitable for economic analysis for a number of reasons: firstly their scoring systems cannot always be directly utilised for QALY calculations; secondly they are not necessarily preference-based; and finally their specificity limits comparisons across disease areas (Patrick and Deyo, 1989). It is important to establish to what extent generic measures can be applied to specific conditions and interventions, such as in the case of disabled children and wheelchair interventions.

Previous literature shows that chronic illness and disability can have a detrimental impact on the HRQoL of children (Varni et al, 2007). For instance, severity of cerebral palsy is related to reductions in HRQoL (Varni et al, 2005b; Vargus-Adams, 2005; Dobhal et al, 2013). To date evidence on the applicability of generic, preference-based measures of HRQoL for the purpose of cost-utility analysis in disabled children has been limited. The appropriateness of standard, NICE approved measures such as the EQ-5D is currently unknown in this population.

6.2.2. Proxy reporting of HRQoL

Wherever possible it is best to obtain direct result from patients, however this is not always appropriate due to issues with age, ability and capacity. Proxy reports are a suitable substitute in circumstances where the patient is unable to self-complete, although issues of using proxy reports have been found in disabled child populations (Varni et al, 2005b; Bray et al, 2010). Many HRQoL measures have been adapted specifically for proxy respondents, such as the Health Utility Index (HUI) measures. Understanding the relationship between proxy and self-report outcomes is important to assess their relative validity and their potential use in clinical settings (Eiser and Morse, 2001). For instance, parents often underestimate their child's QoL, but understanding how parents and children differ can reveal

important information about how parental perception of QoL changes with child age and disease severity (Eiser and Morse, 2001).

When implemented correctly QALYs can be an essential tool to help guide funding allocation in an evidence based manner. In order for QALYs to be calculated accurately, outcome measures such as the EQ-5D must be sensitive to the specific intervention or population. It is therefore important to understand the applicability of outcome measures in specific settings and to test their usefulness.

6.3. Aims and objectives

The overarching aim of this study was to assess the appropriateness of the EQ-5D-Y and HUI instruments for eliciting accurate HRQoL estimates from disabled children and their parents by proxy. Secondary objectives were:

- To compare the HRQoL results of disabled children and their parents by proxy.
- To assess correlation between child and parent proxy measures.
- To assess the construct validity of the EQ-5D-Y and HUI measures, with consideration of validity between measures and respondent type (child and parent proxies).
- To assess the agreement between the EQ-5D-Y, and HUI measures, and respondent type (child or parent proxy).

6.4. Methods

See chapter two for details on recruitment, data protection and ethical considerations.

6.4.1. Data collection

Data was collected using questionnaire surveys. Separate surveys were given to children and their parents, with slight changes in wording to account for differences. Questionnaires contained the EQ-5D-Y, a visual analogue scale (VAS) and the HUI measure (see appendix E.4). Child questionnaires contained self-administered versions of the EQ-5D-Y and HUI measures, while parent questionnaires contained proxy versions. A range of demographic data was collected, including child/parent age, gender and ethnicity. Furthermore, relevant information about the child's disability and wheelchair use, including diagnosis, length of time using a wheelchair, type of wheelchair used and frequency of wheelchair use was also collected.

6.4.2. Measures

The EQ-5D is a generic, validated HRQoL measure. The EQ-5D-Y is an adapted version validated for use in children and parent proxies. It is based on a descriptive system containing five domains: mobility; self-care; usual activities; pain and discomfort; and anxiety and depression (EuroQol Group, 1990). Respondents are asked to rate their health today by indicating their level on each domain using one of three options: no problems, some problems, a lot of problems. A five digit health state is generated from participants responses, for instance 11111 indicates a state of health with no problems, while 21211 indicates a state health with some problems in mobility and usual activities. Using this classification system 243 potential health states are possible. Health states are converted to a single summary index score by weighting the levels of each domain and deducting these weights from 1 (perfect health). A pre-existing UK time-trade off (TTO) value set was used to assign weights for domain levels (Dolan et al, 1996). This value set was derived from a general population sample, with TTO used to value levels. At present no specific value sets for children or parent proxies are available.

The HUI is a generic, validated HRQoL measure containing the HUI2 and HUI3 systems (Horsman et al, 2003). It comprises a 15-question self-completion questionnaire, with each question having between four and six levels. The HUI2 comprises 7 attributes: sensation, mobility, emotion, cognition, self-care, pain and fertility (fertility assumed at level 1 as per the HUI guidelines); and the HUI3 comprises eight attributes: vision, hearing, speech, ambulation, dexterity, emotion, cognition and pain. A comprehensive health state is first developed for the HUI2 and HUI3 using the individual attribute levels for each system. The attribute levels are defined by responses to single questions or specified sets of questions, with the HUI3 results being used to generate HUI2 levels codes for certain attributes. For instance, the HUI3 vision attribute level is defined by two vision questions, while the HUI2 sensation attribute level is defined by a combination of three HUI3 attributes (vision, hearing and speech). Overall HRQoL utility scores are calculated using utility functions for the HUI2 and HUI3 attributes. For this study the multi-attribute utility functions (MAUF) developed for the HUI2 (Torrance et al, 1996) and HUI3 (Feeny et al, 2002) were used to assign utility scores to attribute levels, on a death to perfect health scale (0 to 1). Using these weighted utility functions an overall utility score was calculated for participants. A UK value set was available, however the traditional MAUF data (developed from Canadian samples) was used instead as this is well established within the literature and recommended for primary analysis of the HUI measures (Feeny et al, 2002). At present the HUI3 is the HUI inc. recommended measure for primary analyses due to the more detailed descriptive system and full structural independence (Feeny et al,

2002). Thus study presents both HUI systems in order to assess their relative usefulness in this population.

The VAS is typically presented alongside EQ-5D measures. It is used to measure self-rated health status on a scale from worst imaginable to best imaginable health (EuroQoL Group, 2013). VAS results were used as a quantitative measure of self-reported health and used comparatively against the measures of HRQoL. In order to aid comparison with the EQ-5D-Y and HUI measures the VAS scoring system was converted from a 0 to 100 scale to a 0 to 1 scale where needed.

6.4.3. Analysis

6.4.3.1. Statistical analysis of mean scores

Using the Shapiro-Wilk test I found that the data was not sufficiently normally distributed, therefore non-parametric statistical tests were used. Results for tests of normality are presented in appendix F.1. As the independence of observations between the child and proxy data could have potentially impacted the suitability of certain tests of significance, two techniques were used to analyse statistically significant differences between the two groups. Mann-Whitney U tests were used for each measure to evaluate the difference between child and parent proxy rank total scores across the whole dataset. As these groups could not be defined as being wholly independent (as the child's HRQoL score goes up, the parent proxy score is likely to go up as well due to familiarity between the parent and child) Wilcoxon signed-rank tests were also used to evaluate differences in total score mean ranks for matched-pairs of children and parent proxies. Using both tests allowed differences between child and proxy results to be tested both as a whole group and as matched-pairs, furthermore the data could be analysed under two assumptions: that the groups were independent and related.

6.4.3.2. Correlation between child and parent proxy total scores

Normality of data varied depending on the outcome measure and variable (child age, child gender, type of wheelchair used). In light of these findings, Spearman's rank-order (a non-parametric test) was used to test correlation, as normal distribution was not observed throughout the data. Spearman's rank-order is used to measure association between two ranked variables, for example the utility scores of children and parent proxies. This correlation technique is more robust when dealing with outliers (Mukaka, 2012), and thus was fitting for this data.

Correlation was between matched-pairs of child and parent proxy total scores to examine whether they were associated. In order for child and proxy measures to be considered sufficiently associated correlation coefficients had to be defined as moderate or strong. In the interest of uniformity the strength of correlations was defined as absent (r_s <0.20), weak (r_s =0.20 to 0.35), moderate (r_s =0.35 to 0.50) and strong ($r_s \geq 0.50$) (Juniper et al, 1996). There are issues with only reporting significant results from correlation analysis, therefore both significant and non-significant correlations are presented in this chapter.

6.4.3.3. HRQoL domain construct validity

Convergent and divergent validity between the HRQoL domains (e.g. mobility, pain etc.) on the EQ-5D-Y and HUI measures was estimated using correlation coefficient analysis (Scalone et al, 2011). As the data was not sufficiently normally distributed a non-parametric test of correlation was needed, therefore Spearman's rank-order correlation was used to calculate coefficients of responses between measures. HRQoL domains considered conceptually equivalent between the measures were expected to exhibit moderate to strong correlations, thus indicating convergence. Likewise, for domains considered unrelated absent or weak correlations were expected to indicate divergence (Scalone et al, 2011).

Convergence was tested by the strength of correlations between domains expected to converge and diverge, for instance for a domain to be considered conceptually valid the strongest correlation had to be with the equivalent domain and divergence exhibited with all other domains. Strength of correlations was defined as stated above for the correlation analysis (Juniper et al, 1996).

6.4.3.4. Agreement between measures- Bland-Altman plots

Analysing correlations between measures and domains provides an indication of the degree to which they associate, but does not tell us if they are in agreement. In some cases high correlation can be observed in measures which in fact have low agreement, thus their true association is not clear from correlation alone (Bland and Altman, 1986). In order to understand agreement between measures I chose to perform additional analyses. When comparing equivalent measures none can be definitively judged to provide a wholly accurate measurement (Bland and Altman, 1986), thus assessing their degree of agreement provides a better understanding of how the results relate to one another and whether clinically comparable data are produced. This can be particularly useful when assessing proxy subjective measures such as those used in QoL assessment, as it allows evaluation of whether proxy results are in agreement with self-reported results (Gabbe et al, 2010).

Bland-Altman plots were used to assess agreement between children and parents and the different HRQoL measures used in this study. The average of paired results from two separate measures are plotted on the x-axis and the difference between the paired results are plotted on the y-axis. The overall mean difference in values (defined as the bias) is plotted as a solid line to indicate the average discrepancy between measures. The standard deviation of all paired measure differences is used to indicate variability (also described as repeatability) and define limits of agreement (Hanneman, 2008). Assuming differences are normally distributed, 95% of differences will lie between the established limits of agreement (mean difference ± 1.96 SD) (Bland and Altman, 1986), represented as dashed lines on the plot.

These plots can be used to determine if there are clinically important discrepancies between measures, and whether these differences are impacted by changes in the means of the paired measure results (Bland and Altman, 1986). A clinically important discrepancy is represented by bias or limits of agreement beyond those deemed acceptable for clinical use, for instance if comparable measures are showing important differences which demonstrate that they are not equally sensitive to a particular phenomenon, setting or population. In the case of HRQoL measurement, this could be demonstrated by one measure valuing a health state at 0.75 and another at 0.25; the difference between these measures is wide enough to raise concerns about their validity, and thus impact clinical decision making based on the results. In this example the measures couldn't be used interchangeably in a clinical setting due to wide differences in outcomes. Furthermore, consideration would be needed as to which measure would of greatest benefit for the given sample.

For the purpose of the agreement analyses in this chapter, a confidence limit of 0.50 was chosen, with any confidence limit falling above 0.50 considered a clinically important discrepancy, and thus an unacceptable level of disagreement between the two measures in question. The EQ-5D-Y and the HUI measures are natively scored on a 0 (death) to 1 (perfect health) scale, so to enable comparability VAS was converted accordingly. The EQ-5D-Y and HUI measures can be scored below 0, as a health state may be considered worse than death and therefore below 0 on the death to perfect health scale. The lowest possible scores for each of the measures are -0.594 for the EQ-5D-Y (UK TTO value set) (Dolan et al, 1996), -0.03 for the HUI2 and -0.36 for the HUI3 (Horsman et al, 2003). Considering that these measures all have ranges greater than 0 to 1, a confidence limit of 0.50 was sufficient to show at least a basic level of agreement between them. In clinical practice a lower limit would be needed to ensure direct

comparability between the measures, however I chose to be relatively conservative due to the small sample sizes.

Traditionally, Bland-Altman plots have been used to assess agreement between new measures and those currently used as standard. The EQ-5D is recommended for use by NICE, however the HUI is highly validated and was developed specifically for children, thus it is not possible to recommend one over the other based solely on the Bland-Altman plot. For this reason the VAS was used as an additional measure of agreement and a base level of current health which could be used to examine the relationship between self-rated health and calculated utility.

6.5. Results

6.5.1. Response rate

A total of 125 study invitation packs were distributed across England and Wales by the three recruitment sites: 61 to parents of children aged 5 or under; 29 to children (and their parents) aged 6 to 15; and 35 to children (and their parents) aged 16 to 18. 36 initial questionnaires were returned by parents (29% response rate). Of the 64 packs sent to eligible disabled children (i.e. over the age of 5) 15 were returned (23% response rate). Two child participants took part in the study without their parents.

6.5.2. Sample size and missing data

In total, 15 children and 36 parents participated in the study. Two child and parent participants were excluded from the EQ-5D-Y analyses due to missing data, giving sample sizes of 13 and 34 respectively for EQ-5D-Y analyses. Five parent participants were excluded from both the HUI2 and HUI3 calculations due to missing data, giving a sample size of 31 for HUI2 and HUI3 analyses. All child participants completed the HUI2 and HUI3 measures in full. All participants completed the VAS. A total of 13 sets of child/parent paired data were obtained, although two were excluded from the EQ-5D-Y analyses due to missing data (N=11).

6.5.3. Demographic characteristics- Full dataset

Demographic details for the full dataset are presented in tables 6.1 and 6.2. In the parent sample 88.9% (N=32) of respondents were female, 80.5% (N=28) were aged between 30 and 49 and 63.9% (N=23) had a child with cerebral palsy. The children of the parents in the parent sample tended to use either a MWC (33.3% [N=12]) or a manual and a powered wheelchair (50% [N=18]), with the majority using their

wheelchair 'all of the time' (55.6% [N=20]). The demographic data indicates that parent participants were generally well educated (52.7% [N=19] had further or higher education) and most had a household income of £36,000 or more (61.1% [N=22]); above the national mean household income of £28,200 (Office for National Statistics, 2013).

In the disabled child sample 66.7% (N=10) were male, 53.3% (N=8) were aged 16 to 18, 80% (N=12) had cerebral palsy, and most participants were in high school or college (73.3% [N=11]). All respondents either used a MWC (33.3% [N=5]) or a manual and a powered wheelchair (66.7% [N=10]). Additionally, the vast majority of participants used their wheelchair 'all of the time' (86.7% [N=13]).

There is a lack of ethnic diversity in both samples with the vast majority of respondents being white-British (94.4% [N=34] of parents and 100% [N=15] of children).

Table 6.1: Demographic characteristics of the disabled child sample (n=15)

Demographic characteristics	Number (%)
Study site	
NHS Wheelchair Service	3 (20)
Whizz-Kidz	12 (80)
Gender	
Female	5 (33.3)
Male	10 (66.7)
Age	
6-15 years	7 (46.7)
16-18 years	8 (53.3)
Ethnicity	
White British	15 (100)
Education	
Primary school	2 (13.3)
High school	5 (33.3)
College	6 (40)
University	1 (6.7)
Home schooled	1 (6.7)
Diagnosis	
Cerebral Palsy	12 (80)
Muscular Dystrophy	1 (6.7)
Hemiplegia / stroke	1 (6.7)
Spina Bifida	1 (6.7)
Frequency of equipment use	
Most of the time	2 (13.3)
All of the time	13 (86.7)
Type of equipment used	
Manual	5 (33.3)
Manual and powered	10 (66.7)

Table 6.2: Demographic characteristics of the parent sample (n=36)

Demographic characteristics	Number (%)
Study site	
NHS Wheelchair Service	5 (13.9)
BIME	13 (36.1)
Whizz-Kidz	18 (50)
Gender	
Female	32 (88.9)
Male	4 (11.1)
Age	
21-29 years	5 (13.9)
30-39 years	16 (44.4)
40-49 years	13 (36.1)
50-59 years	2 (5.6)
Ethnicity	
White British	34 (94.4)
White & Asian	1 (2.8)
Chinese	1 (2.8)
Marital status	
Married	28 (77.8)
Co-habiting	4 (11.1)
Single	2 (5.6)
Separated	1 (2.8)
Divorced	1 (2.8)
Education	
Higher	16 (44.4)
Further (e.g. A Level)	3 (8.3)
GCSE/O level	10 (27.8)
Other	2 (5.6)
None	5 (13.9)
Annual household Income	

Table 6.2 (parent sample demographics) continued

Less than £5000	1 (2.8)
£5000-15,000	4 (11.1)
£16,000-£25,000	5 (13.9)
£26,000-£35,000	4 (11.1)
£36,000-£50,000	12 (33.3)
£51,000-£75,000	5 (13.9)
£75,000 or more	4 (11.1)
Missing	1 (2.8)
Employment status	
Full-time	5 (13.9)
Part-time	16 (44.4)
Unemployed / full-time parent	15 (41.7)
Child's diagnosis	
Cerebral Palsy	23 (63.9)
Spinal Muscular Atrophy	4 (11.1)
Muscular Dystrophy	3 (8.3)
Other	6 (16.7)
Child's age	
5 years or under	19 (52.8)
6-15 years	11 (30.6)
16-18 years	6 (16.7)
Frequency of child's equipment use	
A little of time	1 (2.8)
Some of the time	6 (16.7)
Most of the time	5 (13.9)
All of the time	20 (55.6)
Missing	4 (11.1)
Type of equipment used by child	
Powered	5 (13.9)
Manual	12 (33.3)
Manual and powered	18 (50)
Waiting for first wheelchair	1 (2.8)

6.5.4. Demographic characteristics- Matched-pairs of children and parents

Demographic details for the matched-pairs of disabled children and parents are presented in table 6.3. The majority of parents were female (92.3% [N=12]), white British (100% [N=13]), aged between 40 and 49 (61.5% [N=8]) and married (76.9% [N=10]). Parent education levels were lower than the full dataset, with 46.1% [N=6] having further and/or higher education. Although few parents were in full-time employment (7.7% [N=1]), household income was generally high; 69.2% [N=9] had a household income of £36,000 or more. The majority of child participants were male (61.5% [n=8]), diagnosed with cerebral palsy (84.6% [N=11]), in high school or college (69.2% [N=9]) and white-British (100% [N=13]). All child respondents either used a MWC (33.3% [N=4]) or a manual and a powered wheelchair (66.7% [N=9]). Additionally, the vast majority of child participants used their wheelchair 'all of the time' (84.6% [N=11]).

Table 6.3: Demographic characteristics of matched-pairs of children/parents

Demographic characteristics	Number (%)
Study site	
NHS Wheelchair Service	2 (15.4)
Whizz-Kidz	11 (84.6)
Parent gender	
Female	12 (92.3)
Male	1 (7.7)
Parent age	
30-39 years	3 (23.1)
40-49 years	8 (61.5)
50-59 years	2 (15.4)
Parent ethnicity	
White British	13 (100)
Parent marital status	
Married	10 (76.9)
Co-habiting	1 (7.7)
Single	1 (7.7)
Divorced	1 (7.7)
Parent education	
Higher	4 (30.7)
Further (e.g. A Level)	2 (15.4)
GCSE/O level	2 (15.4)
Other	3 (23.1)
None	2 (15.4)
Annual household Income	
£5000-15,000	1 (7.7)
£16,000-£25,000	1 (7.7)
£26,000-£35,000	1 (7.7)
£36,000-£50,000	6 (46.2)
£51,000-£75,000	2 (15.4)
£75,000 or more	1 (7.7)
Missing	1 (7.7)
Parent employment status	
Full-time	1 (7.7)
Part-time	6 (46.2)
Unemployed / stay at home parent	6 (46.2)
Child's condition	
Cerebral Palsy	11 (84.6)
Hemiplegia/Stroke	1 (7.7)
Muscular Dystrophy	1 (7.7)
Child age	
6-15 years	7 (58.8)
16-18 years	6 (46.2)
Child gender	
Female	5 (38.5)
Male	8 (61.5)
Child ethnicity	
White British	13 (100)
Child education	
Primary school	2 (15.4)
High school	5 (38.5)
College	4 (30.7)
University	1 (7.7)
Home schooled	1 (7.7)
Frequency of child's equipment use	
Most of the time	2 (15.4)
All of the time	11 (84.6)
Type of equipment used by child	
Manual	4 (33.3)
Manual and powered	9 (66.7)

6.5.5. HRQoL total score results

Results for the EQ-5D-Y and HUI2 indicate that for all HRQoL domains parent proxies reported greater proportions of problems as compared to self-reporting children (see tables 6.4 and 6.5). For the HUI3, parent proxies reported greater proportions of problems for most domains, although a higher proportion of children reported problems in ambulation and pain (see table 6.6).

Table 6.4: Proportion of different levels on EQ-5D-Y by domain, respondent and child age group (%)

EQ-5D-Y domains/levels*		Child reported 6-15	16-18	Total	Parent proxy ≤5	6-15	16-18	Total
Mobility	1	0.0	0.0	**0.0**	0.0	9.1	0.0	**2.9**
	2	0.0	100.0	**46.1**	5.23	0.0	0.0	**2.9**
	3	100.0	0.0	**53.9**	94.7	90.9	100.0	**94.1**
Self-care	1	0.0	0.0	**0.0**	0.0	0.0	0.0	**0.0**
	2	57.1	75	**66.7**	10.5	36.4	66.7	**27.8**
	3	42.9	25	**33.3**	89.5	63.6	33.3	**72.2**
Usual activities	1	42.9	0.0	**20**	0.0	9.09	0.0	**2.8**
	2	28.6	87.5	**60**	10.5	36.4	66.7	**27.8**
	3	28.6	12.5	**20**	89.5	54.6	33.3	**69.4**
Pain / discomfort	1	57.1	37.5	**46.7**	15.8	36.4	0.0	**19.4**
	2	42.9	62.5	**53.3**	84.2	54.6	100.0	**77.8**
	3	0.0	0.0	**0.0**	0.0	9.1	0.0	**2.8**
Anxiety / depression	1	85.7	87.5	**86.7**	52.6	63.6	66.7	**58.3**
	2	14.3	0.0	**6.7**	47.4	27.3	33.3	**38.9**
	3	0.0	12.5	**6.7**	0.0	9.1	0.0	**2.8**

**Problems associated with HRQoL domain increase as level increases*

Table 6.5: Proportion of different levels on HUI2 by domain, respondent and child age group (%)

HUI2 domains/levels*		Child reported 6-15	16-18	Total	Parent proxy ≤5	6-15	16-18	Total
Sensation	1	28.57	62.50	**46.67**	18.75	27.27	50.00	**27.27**
	2	28.57	25.00	**26.67**	12.50	9.09	33.33	**15.15**
	3	42.86	12.50	**26.67**	25.00	54.55	16.67	**33.33**
	4	0.00	0.00	**0.00**	43.75	9.09	0.00	**24.24**
Mobility	1	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	2	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	3	0.00	12.50	**6.67**	26.32	9.09	16.67	**19.44**
	4	100.00	87.50	**93.33**	52.63	81.82	83.33	**66.67**
	5	0.00	0.00	**0.00**	21.05	9.09	0.00	**13.89**
Emotion	1	85.71	75.00	**80.00**	68.42	63.64	83.33	**69.44**
	2	14.29	25.00	**20.00**	15.79	27.27	16.67	**19.44**
	3	0.00	0.00	**0.00**	10.53	0.00	0.00	**5.56**
	4	0.00	0.00	**0.00**	5.26	9.09	0.00	**5.56**
	5	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**

Cognition	1	42.86	50.00	**46.67**	37.50	45.45	66.67	**45.45**
	2	57.14	50.00	**53.33**	18.75	45.45	33.33	**30.30**
	3	0.00	0.00	**0.00**	12.50	9.09	0.00	**9.09**
	4	0.00	0.00	**0.00**	31.25	0.00	0.00	**15.15**
Self-care	1	0.00	25.00	**13.33**	0.00	9.09	0.00	**2.78**
	2	28.57	12.50	**20.00**	0.00	0.00	16.67	**2.78**
	3	0.00	12.50	**6.67**	0.00	9.09	0.00	**2.78**
	4	71.43	50.00	**60.00**	100.00	81.82	83.33	**91.67**
Pain	1	42.86	0.00	**20.00**	26.32	27.27	0.00	**22.22**
	2	57.14	100.00	**80.00**	63.16	54.55	83.33	**63.89**
	3	0.00	0.00	**0.00**	5.26	18.18	16.67	**11.11**
	4	0.00	0.00	**0.00**	5.26	0.00	0.00	**2.78**
	5	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**

**Problems associated with HRQoL domain increase as level increases*

Table 6.6: Proportion of different levels on HUI2 by domain, respondent and child age group (%)

		Child reported			Parent proxy			
HUI3 domains/levels*		**6-15**	**16-18**	**Total**	**≤5**	**6-15**	**16-18**	**Total**
Vision	1	100.00	100.00	**100.00**	50.00	45.45	66.67	**51.52**
	2	0.00	0.00	**0.00**	25.00	36.36	33.33	**30.30**
	3	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	4	0.00	0.00	**0.00**	6.25	9.09	0.00	**6.06**
	5	0.00	0.00	**0.00**	12.50	9.09	0.00	**9.09**
	6	0.00	0.00	**0.00**	6.25	0.00	0.00	**3.03**
Hearing	1	57.14	75.00	**66.67**	89.47	90.91	100.00	**91.67**
	2	42.86	25.00	**33.33**	5.26	0.00	0.00	**2.78**
	3	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	4	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	5	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	6	0.00	0.00	**0.00**	5.26	9.09	0.00	**5.56**
Speech	1	57.14	87.50	**73.33**	31.58	45.45	83.33	**44.44**
	2	28.57	0.00	**13.33**	0.00	27.27	0.00	**8.33**
	3	14.29	12.50	**13.33**	15.79	27.27	16.67	**19.44**
	4	0.00	0.00	**0.00**	10.53	0.00	0.00	**5.56**
	5	0.00	0.00	**0.00**	42.11	0.00	0.00	**22.22**
Ambulation	1	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	2	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	3	0.00	0.00	**0.00**	5.26	9.09	0.00	**5.56**
	4	0.00	12.50	**6.67**	21.05	0.00	16.67	**13.89**
	5	14.29	12.50	**13.33**	10.53	18.18	0.00	**11.11**
	6	85.71	75.00	**80.00**	63.16	72.73	83.33	**69.44**

Dexterity	1	14.29	25.00	**20.00**	15.79	27.27	16.67	**19.44**
	2	14.29	12.50	**13.33**	0.00	18.18	0.00	**5.56**
	3	14.29	12.50	**13.33**	0.00	0.00	0.00	**0.00**
	4	28.57	37.50	**33.33**	31.58	18.18	66.67	**33.33**
	5	28.57	12.50	**20.00**	21.05	27.27	16.67	**22.22**
	6	0.00	0.00	**0.00**	31.58	9.09	0.00	**19.44**
Emotion	1	100.00	100.00	**100.00**	73.68	72.73	83.33	**75.00**
	2	0.00	0.00	**0.00**	26.32	18.18	16.67	**22.22**
	3	0.00	0.00	**0.00**	0.00	9.09	0.00	**2.78**
	4	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	5	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
Cognition	1	42.86	50.00	**46.67**	37.50	45.45	66.67	**45.45**
	2	42.86	0.00	**20.00**	12.50	27.27	0.00	**15.15**
	3	14.29	37.50	**26.67**	0.00	9.09	0.00	**3.03**
	4	0.00	12.50	**6.67**	6.25	9.09	33.33	**12.12**
	5	0.00	0.00	**0.00**	18.75	9.09	0.00	**12.12**
	6	0.00	0.00	**0.00**	25.00	0.00	0.00	**12.12**
Pain	1	42.86	0.00	**20.00**	36.84	27.27	0.00	**27.78**
	2	28.57	75.00	**53.33**	47.37	54.55	83.33	**55.56**
	3	28.57	25.00	**26.67**	15.79	18.18	16.67	**16.67**
	4	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**
	5	0.00	0.00	**0.00**	0.00	0.00	0.00	**0.00**

**Problems associated with HRQoL domain increase as level increases*

Descriptive statistics are presented in table 6.7. The overall mean scores on all of the measures were higher for child self-reports than for parent proxies (see figure 6.1). Child scores were also higher than parent proxies for all equivalent age groups. Trends in scores between measures were somewhat equivalent for children and parent proxies: The VAS had the highest overall mean score for children and parent proxies (78.93 [SD 14.12] and 71.75 [SD 19.70] respectively), followed by the HUI2 (0.54 [SD 0.07] and 0.42 [SD 0.16] respectively). Children scored the EQ-5D-Y higher than the HUI3 (0.37 [SD 0.18] and 0.23 [SD 0.09] respectively), while parent proxies scored the EQ-5D-Y lower than the HUI3 (-0.04 [SD 0.14] and 0.10 [SD 0.23] respectively).

Table 6.7: Outcome measure results and descriptive statistics by child age group and respondent

		Child reported			Parent proxy			
		6-15	16-18	Total	≤5	6-15	16-18	Total
EQ-5D-Y	Mean	0.26	0.49	**0.37**	-0.08	-0.02	0.04	**-0.04**
	SD	0.10	0.18	**0.18**	0.08	0.22	0.04	**0.14**
	Median	0.22	0.57	**0.36**	-0.10	0.00	0.04	**-0.08**
	25th	0.18	0.37	**0.22**	-0.17	-0.13	0.00	**-0.15**
	75th	0.36	0.63	**0.57**	-0.01	0.10	0.07	**0.03**
	N	7	6	**13**	19	11	4	**34**
VAS	Mean	84.29	74.25	**78.93**	69.37	75.00	73.33	**71.75**
	SD	15.92	11.30	**14.12**	23.41	15.49	14.72	**19.70**
	Median	90.00	78.50	**80.00**	75.00	80.00	75.00	**77.50**
	25th	85.00	70.00	**73.50**	50.00	70.00	66.25	**57.50**
	75th	91.00	80.00	**89.50**	90.00	87.50	83.75	**90.00**
	N	7	8	**15**	19	11	6	**36**
HUI2	Mean	0.51	0.57	**0.54**	0.36	0.45	0.52	**0.42**
	SD	0.08	0.07	**0.07**	0.17	0.16	0.06	**0.16**
	Median	0.49	0.55	**0.54**	0.34	0.43	0.54	**0.43**
	25th	0.45	0.54	**0.49**	0.22	0.41	0.48	**0.32**
	75th	0.54	0.61	**0.58**	0.53	0.49	0.56	**0.54**
	N	7	8	**15**	14	11	6	**31**
HUI3	Mean	0.19	0.25	**0.23**	0.00	0.18	0.18	**0.10**
	SD	0.09	0.09	**0.09**	0.24	0.24	0.06	**0.23**
	Median	0.21	0.27	**0.22**	-0.09	0.12	0.21	**0.12**
	25th	0.14	0.22	**0.21**	-0.16	0.06	0.17	**-0.09**
	75th	0.22	0.30	**0.29**	0.12	0.26	0.22	**0.21**
	N	7	8	**15**	14	11	6	**31**

Variations in N due to missing data

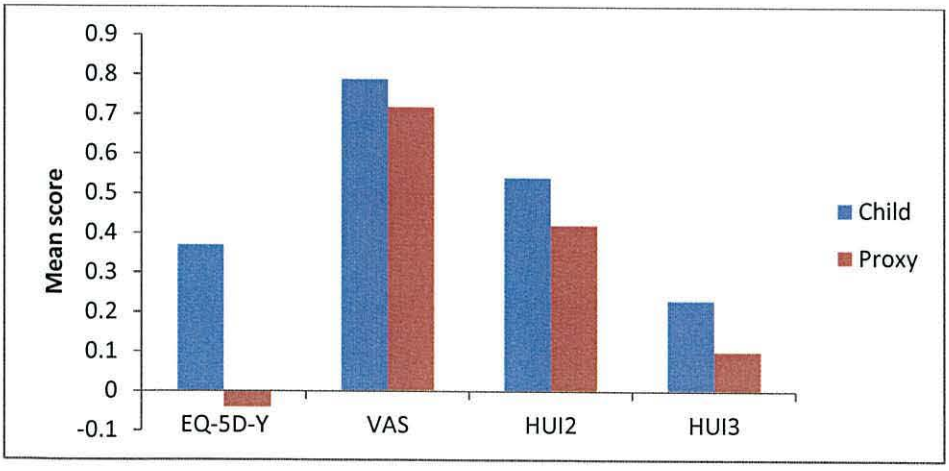


Figure 6.1: Outcome measure mean scores (child and parent proxy)

6.5.6. Comparisons with population norm reference scores

At present there are no published EQ-5D-Y reference scores for children, largely due to there being no validated value sets for calculating reference scores for children. However, unpublished child norm scores for the EQ-5D-Y and VAS were available based on a cohort of over 3000 school children aged between 7 and 19 (Noyes, 2004). The reported child population norm score for the VAS was 83.17 and compared to the other measures shows the greatest similarity to the results in this study, with child self-reported scores in this study 5.4% less (78.93, SD 14.12) and proxy scores 15.9% less (71.75, SD 19.70) than the norm score. Conversely, the mean EQ-5D-Y reference score was 0.89, compared to much lower means observed in this cohort of children and parent proxies; 0.37 (SD 0.18) and -0.04 (SD 0.14) respectively.

UK MAUF population norm reference scores for children were not available for the HUI measures, thus Canadian HUI norm reference scores were used as a comparator. The reported Canadian HUI2 reference score for children aged 8 and between 12-16 is 0.95 (Saigal et al, 1994; Feeny et al, 2004); 43.2% and 55.8% higher than the child self-reported (0.54 [SD 0.07])and parent proxy (0.42 [SD 0.16]) results in this cohort respectively. HUI3 population reference scores exhibit more variation with age: 0.92 at age 5-12; 0.90 at age 13-15; and 0.85 at age 16-19 (Pogany et al, 2006). This cohort scored considerably lower at roughly equivalent age ranges, with 6-15 year olds scoring an average of 0.19 [SD 0.09] and 16-18 year olds scoring 0.25 [SD 0.09]. The mean parent proxy HUI3 score for age ranges 6-15 and 16-18 was 0.18 (SD 0.24 and 0.06 respectively).

6.5.7. Statistical analysis of HRQoL scores- Mann-Whitney U test

Using Shapiro-Wilk analysis, it was found that the data was no sufficiently normally distributed, thus non-parametric tests were used (Wilcoxon signed rank and Mann-Whitney U). Normality results are presented in appendix F.2.

A significant effect of respondent type was found for all measures besides the VAS (see table 6.8). Child self-reported scores were significantly higher on all measures besides the VAS (scores higher for children but not significant): The mean ranks of children and parent proxies on the HUI2 were 31.53 and 19.61 respectively (U=112, Z=-2.830, p=.005); the mean ranks of children and parent proxies on the HUI3 were 31.53 and 19.61 respectively (U=112, Z=-2.825, p=.005); the mean ranks of children and parent proxies on the EQ-5D-Y were 35.92 and 19.44 respectively (U=66, Z=-3.705, p=.000); and the mean ranks of children and parent proxies on the VAS were 29.50 and 24.54 respectively (U=217.5, Z=-1.091, p=.275).

These results demonstrate that there were statistically significant differences between the responses of children and parent proxies in the full dataset on all measures besides the VAS, with children scoring their HRQoL significantly higher than parents by proxy.

Table 6.8: Mann-Whitney U significance test results by measure

		N	Mean Rank	Sum of Ranks	Mann-Whitney U	Wilcoxon W	Z	Asymp. Sig. (2-tailed)
EQ-5D-Y	Child	13	35.92	467	66	661	-3.705	0.005*
	Proxy	34	19.44	661				
	Total	47	-	-				
VAS	Child	15	29.5	442.5	217.5	883.5	-1.091	0.275
	Proxy	36	24.54	883.5				
	Total	51	-	-				
HUI2	Child	15	31.53	473	112	608	-2.83	0.005*
	Proxy	31	19.61	608				
	Total	46	-	-				
HUI3	Child	15	31.53	473	112	608	-2.825	0.005*
	Proxy	31	19.61	608				
	Total	46	-	-				

* Significant at 0.01 level

6.5.8. Statistical analysis of matched-pair HRQoL scores- Wilcoxon signed-rank test

See table 6.7 for median score results and table 6.9 for significance results. Similar results to the Mann-Whitney test were observed in the Wilcoxon signed-rank test for the matched-pair child and parent proxy data, with statistically significant differences for all measures besides the VAS. The median scores for matched children and parent proxies on the EQ-5D-Y were 0.232 and -0.076 respectively (Z=-2.524, p=.012, N=11); 80 and 77.5 respectively on the VAS (Z=-1.483, p=.138, N=13); 0.540 and 0.431 respectively on the HUI2 (Z=-2.310, p=.021, N=13); and 0.223 and 0.118 respectively on the HUI3 (Z=-2.599, p=.009, N=13). These results indicate that there were statistically significant differences between matched pairs of children and their parents by proxy on nearly all measures (VAS non-significant), with children scoring their HRQoL significantly higher than their parents by proxy.

Table 6.9: Wilcoxon signed-rank significance test results by measure

Child - Proxy		N	Mean Rank	Sum of Ranks	Z	Asymp. Sig. (2-tailed)
EQ-5D-Y	-ve Ranks	0[a]	0	0	-2.524[#]	0.012*
	+ve Ranks	8[b]	4.5	36		
	Ties	3[c]	-	-		
	Total	11	-	-		
VAS	-ve Ranks	4[d]	3.25	13	-1.483[#]	0.138
	+ve Ranks	6[e]	7	42		
	Ties	3[f]	-	-		
	Total	13	-	-		
HUI2	-ve Ranks	1[g]	3	3	-2.310[#]	0.021*
	+ve Ranks	8[h]	5.25	42		
	Ties	4[i]	-	-		
	Total	13	-	-		
HUI3	-ve Ranks	1[j]	2	2	-2.599[b]	0.009*
	+ve Ranks	9[k]	5.89	53		
	Ties	3[l]	-	-		
	Total	13	-	-		

[#] Based on negative ranks.
* significant at 0.05 level
a. Child EQ-5D index score (TTO) < Parent EQ-5D index score (TTO)
b. Child EQ-5D index score (TTO) > Parent EQ-5D index score (TTO)
c. Child EQ-5D index score (TTO) = Parent EQ-5D index score (TTO)
d. Child VAS < Parent VAS
e. Child VAS > Parent VAS
f. Child VAS = Parent VAS
g. Child HUI2 Utility function < Parent HUI2 Utility function
h. Child HUI2 Utility function > Parent HUI2 Utility function
i. Child HUI2 Utility function = Parent HUI2 Utility function
j. Child HUI3 Utility function < Parent HUI3 Utility function
k. Child HUI3 Utility function > Parent HUI3 Utility function
l. Child HUI3 Utility function = Parent HUI3 Utility function

6.5.9. Correlation between child self-report and parent proxy measures (matched-pairs)

Correlation coefficients are presented in table 6.10. Expected convergent measures are highlighted in grey. Significant large (>0.05) correlations coefficients were found between matched-pairs of child and parent proxy results for the EQ-5D-Y (r_s=.665, p=.026), HUI2 (r_s=.728, p=.005) and HUI3 (r_s=.842, p=.000). There was also significant correlation between the child HUI3 and parent proxy HUI2 (r_s=.567, p=.043); the child HUI2 and parent proxy HUI3 (r_s=.932, p=.000); and the child EQ-5D-Y and parent proxy HUI2 (r_s=.637, p=.039). It is of importance to consider non-significant correlations as significance can be

misleading in smaller samples using Spearman's rho. With this in mind, there was also strong correlation between the child and parent proxy VAS results (r_s=.545, p=.054). Weak non-significant correlation was found between the child EQ-5D-Y and parent HUI3 (r_s=.290, p=.388); and the child HUI2 and parent EQ-5D-Y (r_s=.279, p=.406).

Convergence between equivalent child and parent proxy measures appears to be good, with only the parent HUI3 and child HUI2 exhibiting stronger correlations with non-equivalent measures. Weak/strong negative correlations between the child VAS and the parent proxy HUI2/3, and a weak negative correlation between the parent proxy VAS and child HUI3 indicate unexpected relationships between these measures, where as one increases the other decreases. In general the child self-report and parent proxy measures are to some extent associated for all equivalent measures.

Table 6.10: Correlations between child self-report and parent proxy results

	Child EQ-5D-Y	Child VAS	Child HUI2	Child HUI3
Parent EQ-5D-Y	**.665***	-.177	.279	-.167
Parent VAS	.075	.545	-.187	-.298
Parent HUI2	**.627***	-.329	**.728****	**.567***
Parent HUI3	.290	-.537	**.932****	**.842****

**Correlation is significant at the 0.01 level (2-tailed).
*Correlation is significant at the 0.05 level (2-tailed).

6.5.10. Construct validity

Construct validity correlation results for children and parents are presented in table 6.11.

6.5.10.1. Child self-reported measures: EQ-5D-Y and HUI2

Due to missing data sample size varied depending on domain; N=13 for all EQ-5D-Y mobility correlations and N=15 for all other correlations. Expected convergent HRQoL domains are highlighted in grey. For children the HUI2 emotion domain was strongly significantly correlated with the anxiety and depression domain of the EQ-5D-Y (r_s=.782, p=.001), and the HUI2 self-care domain was strongly significantly

correlated with the self-care and anxiety/depression domains of the EQ-5D-Y (r_s=.557, p=.031; r_s=-.548, p=.034 respectively). Significant convergent validity was therefore observed for the self-care and emotion domains of the HUI2 and EQ-5D-Y. Including non-significant correlations, moderate convergence was observed between the HUI2 sensation domain and the EQ-5D-Y mobility and self-care domains (r_s=.493, p=.061; r_s=.488, p=.091 respectively); weak/moderate convergence was observed between mobility domains (r_s=.312, p=.300).

Divergence was not as expected for some of the domains, for instance the EQ-5D-Y pain and discomfort domain had the highest correlation with the HUI2 emotion domain (r_s=.468, p=.079). Unexpected moderate/strong negative correlations were observed between HUI2 pain and EQ-5D-Y mobility domains (r_s=-.507, p=.077); the HUI2 self-care domain and the EQ-5D-Y pain/discomfort and anxiety/depression domains (r_s=-.316, p=.251; r_s=-.548, p=.034 respectively); and the EQ-5D-Y self-care domain and the HUI2 emotion and cognition domains (r_s=-.354, p=.196; r_s=-.472, p=.075 respectively).

In summary, the child reported HUI2 showed good convergence with the EQ-5D-Y in some respects, but also exhibited interesting differences, particularly in terms of divergence and negative correlations.

6.5.10.2. Child self-reported measures: EQ-5D-Y and HUI3

The HUI3 speech domain was strongly significantly correlated with the self-care domain of the EQ-5D-Y (r_s=.569, p=.027). Non-significant moderate correlations were observed between the pain domains (r_s=.494, p=.061) and the HUI3 dexterity domain and the EQ-5D-Y self-care domain (r_s=.370, p=.175). A weak correlation was found between the EQ-5D-Y and HUI3 mobility/ambulation domains (r_s=.252, p=.407), however moderate correlation was observed between the HUI3 ambulation domain and the EQ-5D-Y self-care domain (r_s=.352, p=.199). Interestingly there was a strong negative correlation between the HUI3 cognition and EQ-5D-Y self-care domains (r_s=-.525, p=.045) and the HUI3 dexterity and EQ-5D-Y anxiety and depression domains (r_s=-.558, p=.030), indicating as these HUI domains increase the EQ-5D-Y domains decrease.

Convergent validity between the child EQ-5D-Y and HUI3 was therefore relatively limited, with unexpected divergence and negative correlations. As the vision and emotion domains could not be tested for correlations, full assessment of the measures was not possible.

6.5.10.3. *Parent proxy measures: EQ-5D-Y and HUI2*

For construct validity correlation results for parent proxies see table 6.11. Due to some missing data sample size varied depending on domain correlations, ranging from N=31 to N=36. Parent proxy results indicated that equivalent pain domains were significantly moderately/strongly correlated (r_s=.499, p=.002) and the HUI2 sensation domain was strongly significantly correlated with the usual activities domain of the EQ-5D-Y (r_s=.575, p=.000). Furthermore, moderate significant correlations were also found between the mobility domains (r_s=.406, p=.017). The HUI2 self-care domain was significantly correlated with the EQ-5D-Y mobility (r_s=.349, p=.043), usual activities (r_s=.433, p=.008) and pain/discomfort domains (r_s=.348, p=.038) (largest correlation with usual activities). Significant convergent validity was therefore observed for mobility, sensation/usual activities, emotion and pain domains for the parent proxy HUI2 and EQ-5D-Y. Non-significant weak convergence was also observed for the self-care domains (r_s=.274, p=.106) and between the HUI2 cognition and EQ-5D-Y usual activities domains (r_s=.332, p=.059), although these domains were more correlated with divergent domains.

Considering convergence and expected divergence, the parent proxy HUI2 and EQ-5D-Y showed excellent overall convergence and construct validity, although correlation coefficients were not as strong as those observed for the child equivalent measures.

6.5.10.4. *Parent proxy measures: EQ-5D-Y and HUI3*

Parent proxy results indicated strong significant correlations between equivalent pain domains (r_s=.540, p=.001) and between the HUI3 speech domain and the EQ-5D-Y usual activities domain (r_s=.529, p=.001). Furthermore, moderate/strong significant correlations were also found between the HUI3 and EQ-5D-Y dexterity and self-care domains (r_s=.450, p=.006); the HUI3 ambulation domain and the EQ-5D-Y mobility domain (r_s=.492, p=.003); and equivalent emotion/anxiety/depression domains (r_s=.451, p=.006). Non-significant weak correlations were observed between the HUI3 cognition domain and the EQ-5D-Y usual activities domain (r_s=.229, p=.091); and the HUI3 vision domain and EQ-5D-Y pain and discomfort domain (r_s=.321, p=.068).

Convergent validity was therefore observed for mobility, usual activities, pain, self-care/dexterity and emotion domains for parent proxy HUI3 and EQ-5D-Y results, indicating excellent convergence and construct validity between these two measures.

Table 6.11: Construct validity between EQ-5D-Y and HUI measures

		Child reported					Parent proxy reported				
		MOBILITY	SELF CARE	USUAL ACTIVITIES	PAIN AND DISCOMFORT	ANXIETY AND DEPRESSION	MOBILITY	SELF CARE	USUAL ACTIVITIES	PAIN AND DISCOMFORT	ANXIETY AND DEPRESSION
HUI2	SENSATION	.488	.493	.249	.116	-.140	.011	.263	**.575****	.238	.174
	MOBILITY	.312	.189	.000	.286	.105	**.406***	.150	.133	-.031	-.104
	EMOTION	-.225	-.354	.000	.468	**.782****	-.055	-.114	.209	.261	**.393***
	COGNITION	-.098	-.472	.000	-.071	.366	-.051	.155	.332	.197	-.008
	SELF-CARE	.407	**.557***	-.069	-.316	**-.548***	**.349***	.274	**.433****	**.348***	.056
	PAIN	-.507	-.354	.000	.200	.196	.175	-.185	.138	**.499****	.310
HUI3	VISION	a	a	a	a	a	.062	.030	.165	.321	.204
	HEARING	.098	-.200	-.224	.094	.111	.078	-.044	.199	.120	.151
	SPEECH	.503	**.569***	.267	-.040	-.233	-.038	.297	**.529****	.057	.151
	AMBULATION	.252	.352	.245	.221	.195	**.492****	-.018	-.043	.058	-.163
	DEXTERITY	.278	.370	-.150	-.413	**-.558***	.039	**.450****	.176	.068	-.304
	EMOTION	a	a	a	a	a	-.116	-.095	.119	.085	**.451****
	COGNITION	-.368	**-.525***	.000	-.050	.278	.001	.142	.299	.193	.021
	PAIN	-.310	-.270	.322	.494	.229	.146	-.107	.088	**.540****	.320

*. Correlation is significant at the 0.05 level (2-tailed).
**. Correlation is significant at the 0.01 level (2-tailed).
a. Cannot be computed because at least one of the variables is constant.

6.5.10.5. Child and parent proxy measures (matched-pairs): EQ-5D-Y

For EQ-5D-Y construct validity correlation results for child and parent proxy matched-pairs, see table 6.12. Due to missing data sample size varied depending on domain; N=11 for all EQ-5D mobility correlations and N=13 for all other correlations. Significant strong correlations were found for the self-care (r_s=.854, p=.000), usual activities (r_s=.675, p=.011), pain and discomfort (r_s=.559, p=.047) and anxiety and depression (r_s=.776, p=.002) domains, indicating convergent validity for these domains

between child a parent proxy measures. Mobility domain convergence could not be tested as the parent mobility domain was constant and thus could not be computed. Unexpectedly, the parent proxy usual activities and child reported self-care domains were also significantly strongly correlated (r_s=.950, p=.000), as was the parent proxy anxiety/depression domain and the child reported pain/discomfort domain (r_s=.592, p=.033). Furthermore, negative correlations were observed between parent proxy anxiety/depression and child reported mobility (r_s=-.386, p=.241) and self-care (r_s=-.433, p=.139); parent proxy pain/discomfort and child reported mobility (r_s=-.280, p=.404); and child reported anxiety/depression and parent proxy self-care (r_s=-.393, p=.184) and usual activities (r_s=-.255, p=.400).

In summary, a good degree of convergence was found between the EQ-5D-Y results of children and parents by proxy, although divergence was not as expected.

Table 6.12: Construct validity between child self-reported and parent proxy EQ-5D-Y domains

		Child reported				
		MOBILITY	SELF-CARE	USUAL ACTIVITIES	PAIN AND DISCOMFORT	ANXIETY AND DEPRESSION
Parent proxy	MOBILITY	a.	a.	a.	a.	a.
	SELF-CARE	.571	**.854****	.356	.071	-.393
	USUAL ACTIVITIES	.280	**.950****	**.675***	.278	-.255
	PAIN AND DISCOMFORT	-.280	.208	.230	**.559***	.140
	ANXIETY AND DEPRESSION	-.386	-.433	.084	**.592***	**.776****

Parent mobility domain excluded as variable was constant and could not be computed
** Correlation is significant at the 0.01 level (2-tailed).
* Correlation is significant at the 0.05 level (2-tailed).

6.5.10.6. Child and parent proxy measures (matched-pairs): HUI2

See table 6.13 for HUI2 construct validity correlation results for child and parent proxy matched-pairs, N=13 for all other correlations. Significant strong correlations were found for the sensation (r_s=.924,

p=.000), mobility (r_s=.736, p=.004), emotion (r_s=.778, p=.002) and self-care (r_s=.642, p=.018) domains for child reported and parent proxy measures, and a weak non-significant correlation for cognition (r_s=.238, p=.433) and pain (r_s=.329, p=.272). These results indicate a good degree of convergent validity between child reported and parent proxy measures. A strong significant negative correlation was observed between parent proxy emotion and child reported self-care domains (r_s=-.654, p=.015), indicating that as parent proxy assessment of emotion increased child reported self-care decreased. A number of other negative correlations were also observed, although most were absent or weak correlations. The most surprising of these non-significant negative correlations was the child pain domain, which was moderately negatively correlated with the parent mobility domain (r_s=-.465, p=.109).

In general convergent validity was good but limited by the convergence of the cognition and pain domains. Divergence was generally well observed, thus overall correlation was adequate.

Table 6.13: Construct validity between child self-reported and parent proxy HUI2 domains

		Child HUI2					
		SENSATION	MOBILITY	EMOTION	COGNITION	SELF-CARE	PAIN
Parent HUI2	SENSATION	**.924****	.042	-.053	.067	.293	-.159
	MOBILITY	.222	**.736****	.000	.000	.271	-.465
	EMOTION	-.211	.123	**.778****	.395	**-.654***	.234
	COGNITION	.087	-.312	-.141	.238	.095	-.225
	SELF-CARE	.481	-.123	-.233	-.393	**.642***	.310
	PAIN	.177	.000	.329	.000	-.277	.329

**. Correlation is significant at the 0.01 level (2-tailed).
*. Correlation is significant at the 0.05 level (2-tailed).
a. Variables constant and could not be computed

6.5.10.7. *Child and parent proxy measures (matched-pairs): HUI3*

For the HUI3 (N=13), significant strong correlations were found for the speech (r_s=.795, p=.001), ambulation (r_s=.736, p=.004), dexterity (r_s=.824, p=.001) and pain (r_s=.816, p=.001) domains for child reported and parent proxy measures, indicating good convergent validity (see table 6.14). The parent proxy vision domain and child reported hearing domain were also highly correlated (r_s=.981, p=.000), as was the parent proxy dexterity domain and the child reported speech domain (r_s=.773, p=.002). Divergence between the measures was generally as expected. Strong negative correlations were observed between parent proxy ambulation and child reported cognition domains (r_s=-.493, p=.087); parent proxy emotion and child reported dexterity domains (r_s=-.642, p=.018); parent proxy cognition and child reported ambulation and pain domains (r_s=-.598, p=.031; r_s=-.564, p=.045 respectively); and parent proxy pain and child reported dexterity (r_s=-.569, p=.042). Other incidences of negative correlation were observed, although these were generally absent or weak non-significant correlations.

In summary child and parent proxy HUI3 measures were relatively convergent, although negative correlations raise concerns. Not all domains could be tested due constant variables.

Generally across the measures good convergent validity between equivalent domains was observed. Divergence was somewhat unexpected, and negative correlations raise interesting considerations of construct validity. In general the child reported and parent proxy measures appear to be well correlated using the Spearman's rho correlation, although the child measures did not exhibit as much convergence as the proxy equivalents.

Table 6.14: Construct validity between child self-reported and parent proxy HUI3 domains

		Child HUI3							
		VISION	HEARING	SPEECH	AMBULATION	DEXTERITY	EMOTION	COGNITION	PAIN
Parent HUI3	VISION	a.	**.981****	-.250	-.096	-.152	a.	.287	.123
	HEARING	a.	a.	a.	a.	a.	a.	a.	a.
	SPEECH	a.	.000	**.795****	.327	.219	a.	-.160	.174
	AMBULATION	a.	-.365	.189	**.736****	-.119	a.	-.493	.000
	DEXTERITY	a.	-.221	**.773****	-.128	**.824****	a.	-.430	-.364
	EMOTION	a.	.101	-.280	.181	**-.642***	a.	.303	.314
	COGNTION	a.	.162	-.096	**-.598***	.253	a.	.340	**-.564***
	PAIN	a.	.285	-.091	.354	**-.569***	a.	.118	**.816****

a. Child vision and emotion and parent hearing domains excluded as variables constant and could not be computed
**. Correlation is significant at the 0.01 level (2-tailed).
*. Correlation is significant at the 0.05 level (2-tailed).

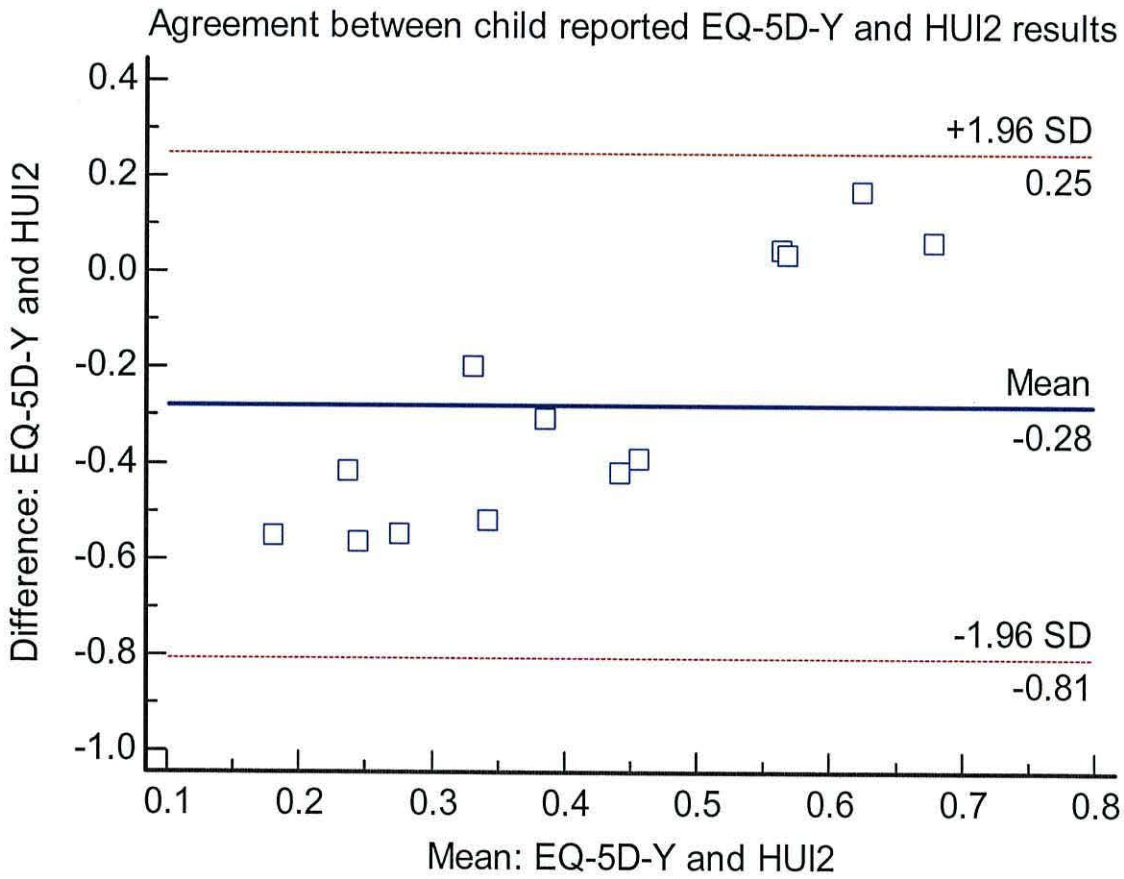
6.5.11. Agreement between measures and respondents

6.5.11.1. Child self-reported measures

All Bland-Altman plots are presented in appendix G, for illustrative purposes one is presented below with statistical interpretation (see figure 6.2).

The mean (± SD) child reported HUI2 outcome score was 0.55 (±0.08) compared with 0.27 (±0.29) for the child reported ED-5D-Y. The bias (mean difference) of the two measures was -0.28 (95% CI -0.44 to -0.12), with children scoring the EQ-5D-Y lower than the HUI2 on average (see figure 6.2). Precision (difference standard deviation) was 0.27 (95% confidence limit from –0.81 [95% CI -1.09 to -0.52] to 0.25

[95% CI -0.04 to 0.54]) with an overall confidence limit of 1.06. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.81 to 0.25; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 1.06 on a maximum utility scale from -0.594 to 1. In practice these discrepancies could include variance from perfect state of health to death. Therefore, the child reported data from the EQ-5D-Y and HUI2 were not in agreement for this cohort. The Bland-Altman plot appears to indicate a proportional error, as difference appears to increase in proportion to mean score increases, although the sample size is too small to make clear precise judgements about error.



The child self-reported EQ-5D-Y had an overall confidence limit ranging from 1.06 to 1.39 depending on the comparator measure (see table 6.15), indicating clinically important discrepancies between it and all other measures. A confidence limit of 0.73 was observed in the VAS and HUI3 analysis, again indicating

important disagreement between the measures. Only the VAS and HUI2 analysis produced an acceptable confidence limit (*CL*=0.42). Therefore all measures besides the VAS and HUI2 showed clinically important discrepancies with one another and thus are insufficiently agreeable to be used interchangeably in this cohort (see table 6.15). All relevant Bland-Altman plots for the child self-report measures are presented in appendix G.1.

Table 6.15: Agreement between child self-reported measures

		Mean difference	SD difference	95% agreement lower and upper limits	Confidence limit
EQ-5D-Y	VAS	-0.52	0.36	-1.21 to 0.18	1.39
	HUI2	-0.28	0.27	-0.81 to 0.25	1.06
	HUI3	0.04	0.29	-0.53 to 0.61	1.14
VAS	HUI2	0.25	0.18	0.11 to 0.60	0.42
	HUI3	0.54	0.19	0.18 to 0.91	0.73

6.5.11.2. Agreement between parent proxy measures

Overall confidence limits for the parent proxy measures fell between 0.62 and 1.04 depending on the comparator measures (see table 6.16). Taking into account the pre-determined cut-off of 0.50, all measures therefore showed clinically important discrepancies with one another and thus are insufficiently agreeable to be used interchangeably in this cohort (see table 6.16). All relevant Bland-Altman plots for the parent proxy measures are presented in appendix G.2.

Table 6.16: Agreement between parent proxy measures

		Mean difference	SD difference	95% agreement lower and upper limits	Confidence limit
EQ-5D-Y	VAS	-0.76	0.24	-1.22 to 0.30	0.92
	HUI2	-0.46	0.16	-0.77 to -0.15	0.62
	HUI3	-0.14	0.2	-0.53 to 0.26	0.80
VAS	HUI2	0.29	0.19	-0.09 to 0.66	0.75
	HUI3	0.61	0.27	0.09 to 1.13	1.04

6.5.11.3. Agreement between child reported and parent proxy measures (matched-pairs)

All relevant Bland-Altman plots for the child/parent proxy measures are presented in appendix G.3. Sufficient agreement was found between the child and parent proxy HUI2 (*CL*=0.22), HUI3 (*CL*=0.22) and

VAS (*CL*=0.32) (see table 6.17), with the HUI measures showing the most agreement between child and parent scores. These results indicate that reports from either the child or their parent by proxy in this cohort could potentially be used interchangeably for the HUI and VAS measures. The EQ-5D-Y exhibited clinically important discrepancies between child and parent proxy responses (*CL*=1.04) and thus are insufficiently agreeable to be used interchangeably in this cohort.

Table 6.17: Agreement between child reported and parent proxy measures

	Mean difference	SD difference	95% agreement lower and upper limits	Confidence limit	Wilcoxon signed rank test	Spearman's Rho
EQ-5D-Y	0.23	0.27	-0.29 to 0.75	1.04	0.012*	0.665*
VAS	0.04	0.08	-0.12 to 0.20	0.32	0.138	0.842**
HUI2	0.05	0.06	-0.06 to 0.15	0.22	0.021*	0.545
HUI3	0.16	0.06	-0.06 to 0.18	0.22	0.009*	0.728**

**Significant at the 0.01 level
*Significant at the 0.05 level

6.6. Discussion

6.6.1. Differences between child and parent proxy results

The results indicate that for the sampled children and parents, the children scored all measures higher than parents on average. The VAS elicited the highest score for both children and parents, while the EQ-5D-Y and HUI3 had the lowest scores. Statistically significant differences were found between parent and child scores on all equivalent measures besides the VAS, with child scores significantly higher than parents on the EQ-5D-Y and HUI measures. Furthermore, HRQoL scores varied greatly from reported population norms and were to a large extent lower, except the VAS.

Generally good correlation was found between child and parent proxy measures, with strong correlation between all equivalent measures, although convergence was atypical for the child HUI2 and parent HUI3. Agreement was sufficient for equivalent child/proxy measures, besides the EQ-5D-Y.

Previous research has demonstrated the issues of using proxy HRQoL data in disabled child populations (Varni et al, 2005b). Self-reported data should be used whenever it is possible and ethical to do so, however proxy measures are essential in situations where self-reporting is not possible. The results from this study suggest that the proxy VAS could provide a robust and reliable alternative to self-reported data in this population. The HUI measures also show sufficient correlation and agreement between child

self-reports and proxy reports, although differences between these measures were significantly different. The EQ-5D-Y exhibited the least association and comparability between child and parent proxy results.

6.6.2. Differentiating correlation and agreement

It is important to take into account apparent discrepancies between analytical tests. I found significant differences between child and parent measures, but also found that they were correlated. These two concepts would appear to be mutually exclusive, but in fact demonstrate the issue with using correlation to assess agreement between measures. Statistically significant difference in mean scores demonstrates that the scores are sufficiently different to be significant, however this does not indicate the relationship between scores and whether they increase or decrease in parallel between children and parents. Furthermore, correlation is an indication of association, for instance as a child's score goes up so does their parents, but it does not given an indication as to whether these scores are in agreement, as there could be a large difference between child and parent scores but they could vary in a similar way. These results indicate that the parent scores were significantly lower than their children's but that they were also correlated. This would suggest that parental scores were lower but their variance was equivalent to children's.

In order to further understand this relationship between child and proxy results in the study agreement between child and parent measures was tested. Agreement between the child and parent EQ-5D-Y was insufficient, but all other measures showed to some extent acceptable agreement between child and proxy measures. This is, however, a case of personal interpretation and some researchers or clinicians may find that the variance between child and proxy reports causes practical issues. The results indicate that the child and parent VAS/HUI measures could be used interchangeably (with consideration of their differences) but caution would be needed when using the EQ-5D-Y due to discrepancies in agreement. Although equivalent HUI measures were significantly different between children and parents, their agreement and correlation indicates that they may be sufficiently associated. Reviewing the mean scores by age (see table 6.7) indicates that the parent scores for the under 5 group were lower than for the other age groups, which may account for large differences in overall mean scores, although this does not account for the significant differences found in the paired Wilcoxon signed-rank test.

It is important to consider correlation and agreement between measures within groups. There was insufficient agreement between all parent proxy measures to allow them to be used interchangeably.

Similarly, only the child reported VAS and HUI2 measures showed sufficient agreement. Therefore, there appears to be little agreement between measures in both cohorts. This indicates that the measures are potentially measuring different things and thus are not wholly comparable within groups. This is potentially expected for the VAS as it is not a direct measure of HRQoL, but it is interesting that the EQ-5D-Y and HUI measures lack sufficient agreement to be used in place of one another as HRQoL measures within groups.

As the child HUI2 showed the most agreement with the VAS, it appears to be the most recommendable measure. The VAS gives an indication of overall health state classification as it is a self-rating scale of health on the day of completion, it is therefore a good comparator for more complex measures because it allows comparison between societal and personal valuation of health states. The VAS can be used to analyse how an individual personally values the health state assigned to them (or their child) by another measure (Whynes, 2008), such as the EQ-5D-Y and HUI. This gives an indication of how variables and attributes not included in the preference-based measures independently affect health status (Whynes, 2008), therefore providing an indication of the validity of the measures in relation to personal valuation of health status. VAS can be more helpful than the EQ-5D when identifying lesser changes in health status (Wolfe and Hawley, 1997), and the VAS allows freedom for respondents to report health status according to their own views (Davies et al, 2003). The VAS was a good comparator for understanding the relationship between the value sets and personal valuations of health status, and therefore provided an adequate means to assess the overall applicability of measures in this population.

As an example, the parent VAS mean score was relatively incomparable with the HUI3 and EQ-5D-Y parent proxy measures, and thus raises questions as to the validity of these measures, as they may not be sufficiently measuring HRQoL in accordance with the way parents define overall health status for their children. This is particularly interesting as the HUI3 is generally recommended before the HUI2 due to the more detailed descriptive system. The results indicate that for this cohort the extra detail in the descriptive system of the HUI3 may actually have encompassed attributes or levels that negatively impacted HRQoL scores of participants. Additional research is required to understand how these two measures differ within this population.

6.6.3. Construct validity

In order to better understand the relationship between the EQ-5D-Y and HUI measures, construct validity between the measures was also analysed. Convergence between the two HUI measures was not assessed as their constructs are interlinked and it would be difficult to assess validity in a meaningful way.

Construct validity was sufficient between the EQ-5D-Y and HUI2 for both children and parents (and the HUI3 for just parents). Construct validity between the child reported EQ-5D-Y and HUI3 was relatively limited. It should be noted that the vision and emotion HUI3 domains for children could not be tested due to constant variables, and thus full convergence could not be assessed. Although construct validity was not entirely acceptable, in general there was sufficient convergence between measures on the different HRQoL domains. Therefore for most equivalent questions the measures are assessing similar constructs. This would indicate that lack of agreement between measures comes from the differences in scope. The EQ-5D-Y is a simple questionnaire based on five domains of HRQoL with only three levels for each domain. Conversely, the HUI2 and HUI3 measures have six and eight domains respectively and up to six levels per domain. It is potentially the broader scope of the HUI measures that sets them apart from the EQ-5D-Y.

Construct validity between children and parents on equivalent measures was also found to be satisfactory, although there was some unexpected divergence. The mobility domain could not be assessed due to constant variables. The results indicate satisfactory construct validity between equivalent child and proxy measures and thus they can be assumed to be measuring the same HRQoL constructs.

6.6.4. Implications for future economic evaluations

Based on these results the use of HRQoL measures in children with disabilities and their parents by proxy requires much consideration as to the most appropriate way to elicit accurate data. Although construct validity was sufficient between measures, the agreement between measures on overall index scores was limited. This may in part reflect the scope of the measures, but may also highlight the inadequacy of standard HRQoL measures in child and disabled populations. In general, parents estimated their child's HRQoL to be significantly lower than the child by self-report. It is difficult to presume why this would be the case, but may be due to HRQoL appearing worse as an outside observer or the prejudice of an able-bodied observer. The variation in HRQoL scores between measures is

somewhat alarming, for example between the parent proxy EQ-5D-Y and HUI2. This large difference in mean score indicates that these 2 measures cannot be assessing HRQoL in the same manner, or that one is much more sensitive than the other.

Evaluating HRQoL of children using the EQ-5D-Y is difficult as value sets for children are not currently available. Using adult value sets may be an appropriate alternative but does not offer a true reflection of the HRQoL of children specifically. To improve accuracy further research is needed to develop child EQ-5D-Y value sets and potentially further adapt the EQ-5D-Y to reflect the key domains of HRQoL that are important to children and young people.

The domains of the EQ-5D-Y appear to converge with those of the HUI measures, which is a strength of these measures. However, the levels on the EQ-5D-Y may lack sensitivity for disabled and child populations. For instance, the mobility domain has no consideration for mobility beyond walking and thus automatically discounts the HRQoL of a mobility impaired child because they are unable to walk, even though they may be mobile by other means. This is also the case for the HUI measures, even though they have additional levels. Likewise, the self-care domain may lack appropriate options for children who are currently learning to care for themselves, and the usual activities domain may be difficult to interpret for a disabled children. It is also worth considering that these measures would likely be insensitive to change over time relating to wheelchair interventions as they lack the scope to capture improvements in independence, functional mobility (other than walking) and other benefits associated with correct wheelchair provision.

6.6.5. Health state preference weights for children and young people

At present the EQ-5D-Y is validated for proxy reporting from age four (Scalone et al, 2011) and child self-reporting from age eight (Wille et al, 2010). In studies where participant age ranges from 12 (or below) to 18 EuroQoL currently recommends the use of just the EQ-5D-Y to allow comparability across the age ranges (EuroQoL Group, 2014). Likewise, the HUI is validated for proxy use from age five, and child self-reporting from age eight (Horsman et al, 2003). In total 52.8% (N=19) of the parent sample had a child aged five or under, 52.5% of which (N=10) were aged under four. The youngest child in the parent sample was two years old, therefore three years below the minimum age limit of the HUI measures. This raises some methodological issues, as these measures are not designed for use in such young children, and therefore the domains of HRQoL cannot be assumed to be accurate. Although this is a legitimate limitation, this chapter presents an exploratory application of utility measures in disabled

children, including children younger than five. The purpose was to understand the applicability of such measures, with specific focus on evaluating PWC interventions for young children; a key area of contention in NHS wheelchair provision. I therefore believe that the inclusion of parents of such young children was a strength rather than a limitation.

Early provision of appropriate wheelchairs for disabled children can have a range of benefits, and in order for appropriate evaluations of effectiveness and cost-effectiveness to be undertaken there needs to be consideration of how health economics methods of evaluation can be applied in this group. Part of that is about learning how to elicit utility data for young children, whether that is from the child or a proxy, as there are real gaps in the current methodological toolbox. There must be consideration of how to value the health states of young children. For instance, it is currently unclear whether preference weights for different health states can be adequately valued by young children, or whether it is appropriate to use preference weights from wider society or proxy reports (Wille et al, 2010). These are serious methodological considerations that must be addressed in order to appropriately apply utility measurement to young children. The lack of appropriate measures for young children under the age of five highlights the difficulty of measuring health status, QoL and utility of young children for the purpose of economic evaluation.

As an example of these issues, the adult and youth versions of the EQ-5D are essentially the same but with slight changes in wording, however they are technically classed as two different instruments and cannot be assumed to be the same. Accordingly, the use of adult preference weights is not appropriate for the EQ-5D-Y (Wille et al, 2010), thus until an appropriate value set is available the EQ-5D-Y should not be used for utility measurement (Canaway and Frew, 2012). For the purposes of the analyses carried out in this chapter an assumption had to be made that adult preference weights would suffice, similarly the EQ-5D-Y was used in an unvalidated age group both to increase the sample size and to examine the impact of age on HRQoL. In a clinical setting these assumptions would likely be inappropriate, indicating that the lack of appropriate tools and measures necessitates a nuanced approach to HRQoL evaluation in children.

In order to account for some of these issues additional analysis of matched-pairs of children and parents was conducted, where child age was suitable for the measures used. This allowed analysis of the full sample to be compared with those of validated age ranges on certain tests. For instance, Mann-Whitney U (full sample) and Wilcoxon signed-rank tests (matched-pairs) were used to analyse

respondent type effect and both found similar results; respondent type had a significant effect on all measures besides the VAS, with children rating their HRQoL higher on all measures.

6.6.6. Relating findings to NICE guidance

In recent years NICE has expanded its remit to cover public health and social care alongside healthcare. NICE states that evidence of cost-effectiveness is required before an intervention can be recommended for NHS use, and that QALYs are the preferred outcome measure of choice (NICE, 2008). In order to calculate QALYs utility data is required, thus at present the application of health economics methods of evaluation to young children and disabled people is particularly difficult as appropriate tools and measures to elicit utility data are not available. The concern is that lack of evidence may inhibit appropriate NICE recommendations, and therefore wheelchair interventions for children and young people may be unfairly marginalised.

In a recent NICE press release the Citizens Council concluded that additional social values were needed to guide health and social care resource allocation, including: the right to health and welfare for all, independence, respect, choice and safeguarding the vulnerable (NICE press release, 08/08/2014). All of these additional values apply to children and disabled people. It is the role of bodies like NICE to ensure that all members of society are equally considered in the allocation of health and social care resources. In order to do so appropriate measures of effect and evaluation processes are required. The evidence in this chapter demonstrates the ongoing issues of applying health economics to children and disabled people.

6.6.7. Generic vs. condition-specific outcome measures

There is a potential that condition-specific measures of HRQoL would be more suitable in this population. However, these bring issues of incompatibility and impracticality, especially as most are not preference-based. In a clinical trial setting it would not be realistic to include an outcome measure for every possible condition or disability related to wheelchair use, and it would be even more difficult to produce a sufficient analysis incorporating a range of outcome measures which could number in the hundreds if every potential mobility impairing disability was considered.

In this study I specifically included generic preference-based measures recommended by NICE and previous research as I wanted to understand how to measure HRQoL for the purpose of economic evaluation of wheelchairs. Therefore, I required measures that could be used to calculate cost per QALY

estimates. I also wanted to choose measures that have been widely used in other areas to allow comparison with populations norms. Although the EQ-5D-Y has been adapted for use in younger populations, it was not originally designed specifically for children. This perhaps accounts for the issues observed in this chapter. The HUI on the other hand was developed specifically for use in paediatric oncology and thus is more applicable to children and young people. The use of child-specific measures may be the way forward for performing economic analyses in this population, however most child-specific measures are not preference-based and thus cannot be used in cost-utility analysis (Chen et al, 2014).

One example of a potentially suitable, preference-based child HRQoL measure is the CHU-9D. This measure was developed through qualitative research directly with children and thus reflects how children define HRQoL (Stevens, 2010). Additionally, a preference weight scoring system has been developed using a sample of adolescents rather than adults (Ratcliffe et al, 2011).

Although the CHU-9D currently represents one of the best tools for utility measurement in children and adolescents, its applicability to disabled children is currently unknown, furthermore it is not validated for use in children under the age of 7 (although validation for younger ages is ongoing). At present, child-specific HRQoL measures such as the HUI2 and CHU-9D are the only suitable tools for economic evaluation of paediatric healthcare interventions. Specifically, any measure that has been developed through consultation with children and adolescents in both the development of the descriptive system and valuation sets. The EQ-5D-Y is not a viable option at present because an appropriate value set is not available for children.

6.6.8. Measuring HRQoL in young children

There are still issues with measuring HRQoL of children under the age of 5; to my knowledge there are no validated child-specific preference-based measures which have been validated below the age of 4. The issue in this age group is that descriptive systems and value sets cannot be accurately developed using young children alone. An appropriate alternative would be to develop a proxy-specific HRQoL outcome measure for young children, using both appropriately aged children and parent/carer proxies to develop a descriptive system and preference weights. The use of just parents would lose the voice of the child and would bias the measure towards an adult understanding of HRQoL, likewise only using older children would perhaps bias the measure towards an adolescent understanding of HRQoL. Another important consideration is whether a generic or disability-specific measure would be needed.

This would require testing the applicability of measures such as the CHU-9D in disabled populations, for example using qualitative research to examine the extent to which such measures reflect how a disabled child defines health and QoL. In the next chapter I will apply this methodology in order to understand how disabled children and their parents define health and QoL.

Perhaps looking beyond the QALY is needed, for instance adopting the capability approach to outcome measurement. At present, however, there are no capability measures appropriate for use in children.

6.6.9. Implications for conceptual framework

One of the key areas for development in wheelchair services is using appropriate outcome measures to assess the effectiveness of wheelchair interventions. If this data was available it could guide procurement strategies, scope of provision and potentially improve outcomes for service users. The results in this chapter highlight the difficulty in developing outcome measures that are preference-based, generic and suitable for children. Strict eligibility criteria is one of the most contentious issues related to NHS wheelchair services for children, particularly with regards to powered mobility for young children on the basis of safety concerns. With appropriate cost-effectiveness evidence arbitrary equipment restriction based on age or equipment type could be limited, as provision could be guided by evidence. The results in this chapter offer a guide to applying and developing appropriate outcome measures and indicates where future research should be directed; specifically preference-based HRQoL measures designed for children, and tested in disabled populations, or alternatives such as the capability approach.

The evidence in this chapter demonstrates that the development and utilisation of appropriate outcome measures requires a great deal of consideration. The relevance of existing outcome measures needs to be examined before implementation to ensure that they appropriately measure the area of interest. For example, these findings demonstrate the potential usefulness of the HUI2, however more work is needed to test the applicability of other measures (such as the CHU-9D) and the wider application of the HUI2. The EQ-5D-Y appears to be the least appropriate in this setting. Another important finding was that proxy reporting could be used if self-reported data is not available due to issues of capacity or health.

Reflecting on the conceptual framework, more work is needed to test and develop outcome measures which are appropriate in this population group. Furthermore, there is a need to implement a knowledge translation framework to facilitate the translation of evidence into practice. This could be particularly

important for ensuring that the most appropriate outcome measures are being utilised based on best available evidence.

6.6.10. Study limitations

In the interest of transparency I acknowledge the limitations of this research. First, the sample sizes are small and thus lack power to make wider assumptions about HRQoL of disabled children. Secondly, the demographic characteristics are not representative and thus the research lacks generalizability. Thirdly, I did not include any condition-specific measures for comparison, however this would have been beyond the scope of this relatively small project. Fourthly, the VAS is not a HRQoL measure, and thus making comparisons between it and the other measures is not wholly appropriate, although this was a good method to compare HRQoL to self-rated health status which are inextricably linked. Finally, missing data was excluded which reduced the power of the analysis. In future research a large sampling frame could be utilised and a wider range of HRQoL measures used. Furthermore, in a larger sample missing data could be more easily handled and potentially imputed to maintain the sample size. It may also be of interest to examine before and after HRQoL results for wheelchair interventions to test measure sensitivity to change.

6.7. Conclusion

Parents reported their child's HRQoL to be significantly lower than the self-report of the child on all measures. The VAS elicited the highest scores, while the EQ-5D-Y and HUI3 had the lowest. Although measures within groups were relatively well correlated they exhibited low agreement and thus were not particularly comparable in terms of HRQoL measurement. Construct validity between measures and between children and parents was good, thus the measures could be assessed as measuring the same HRQoL domains generally. If assuming the VAS to be a relatively realistic comparator for the other measures, the child self-report HUI2 appears to be the most accurate measure for children with disabilities. However there still remains uncertainty as to the validity of both the child and proxy versions of each measure and their applicability in this specific setting.

The EQ-5D-Y in particular needs to be updated in order to improve applicability to children with disabilities. This would potentially require rewording or restructuring of levels. Furthermore, child value sets are required to fully understand the HRQoL of children using the EQ-5D-Y. In order for the EQ-5D-Y to be used effectively in children with mobility impairment further validation work is required.

Future research must focus on developing generic, preference-based measures which are validated for children, and applicable to more varied clinical groups; this includes developing generic descriptive systems which do not discriminate against specific functional abilities (such as mobility impairment). Furthermore, validated value sets for children are essential to allow QALYs to be calculated appropriately. Without such data it remains difficult for appropriate economic analyses to be conducted in order to inform resource allocation. Use of correlation to assess agreement between measures should be supplemented with additional analyses of agreement as correlation alone can be misleading with regards to assessing agreement between measure and respondent types.

Chapter Seven: A qualitative exploration of health-related quality of life in young wheelchair users: Are generic, preference-based health-related quality of life measures suitable for children with mobility impairments?

7.1. Chapter summary

In the previous chapter I found that the appropriateness of the EQ-5D-Y and HUI measures for eliciting utility outcomes might be limited for disabled children. In order to further explore the use of these measures in this population, and more generally the use of preference-based utility measures, it is important to understand how disabled children and their parents (by proxy) define QoL.

In this chapter I present an exploratory descriptive study, utilising a qualitative framework analysis approach. Disabled children and parents of disabled children were asked take part in an interview about how they define QoL in relation to wheelchair use and the suitability of the HUI and EQ-5D-Y outcome measures. Data was collected through face-to-face semi-structured interviews. 11 children and 24 parents participated in the study; seven children and parents took part together. Three analytical themes were derived from the data which encompassed how participants defined QoL, these were: participation and positive experiences; self-worth and feeling fulfilled; health and functioning. For the purpose of economic evaluation it appears that the EQ-5D-Y in particular is not appropriate to elicit reliable utility scores for disabled children. Child-specific HRQOL measures or the capability approach may be more suited in this population.

7.2. Introduction

7.2.1. Disability and equality

The United Nations convention on the rights of persons with disabilities specifically states that all possible steps must be taken to ensure that disabled children experience the same fundamental human rights and freedoms as all other children (Schulze, 2010). This includes facilitating disabled children to express views regarding all matters which affect them personally. Furthermore, disabled children must not be excluded from opportunities to play, learn, seek specialist care/assistance or participate in society (Schulze, 2010).

The UK Equality Act 2010 states that public authorities have an obligation to eliminate discrimination and promote equality for all disabled people (Davis, 2012). Public services such as the NHS have a duty to tackle inequality and promote equal opportunities for disabled people (Office of Disability Issues, 2006). The DoH is committed to improving the outcomes of disabled children by ensuring that relevant outcomes are used in healthcare, and that the views of disabled children and their families are taken into account (DoH Commissioning Team, 2010).

7.2.2. Identifying appropriate outcome measures for children's wheelchair services

Generic, preference-based measures of HRQoL could offer a relatively simple solution to the issue of identifying and utilising appropriate outcome measures in wheelchair services. Traditional approaches to measuring outcomes have been service based, such as monitoring time to wheelchair assessment and delivery, which suits the needs of the service rather than the patient. The use of HRQoL outcome measures is well documented within the discipline of health economics. These types of measures are used to assess the utility (or health state preference) outcomes of clinical interventions. Utility refers to the level of wellbeing associated with a specific health state (Robinson, 1993). Accordingly, comparisons can be made between interventions and patient groups as utility provides a global measure by which to assess benefit (Phillips, 2009).

Preference-based HRQoL outcome measures such as the EQ-5D are built on many years of research and validation. Some measures are designed and validated for specific populations, for instance the HUI was originally developed for use in paediatric oncology and as such is one of the only preference-based generic HRQoL measures recommended for use in children (Brazier et al, 1999). These measures are comprised of a descriptive system (questions and possible responses) and utility weights for each possible response. Utility weights are usually based on the health state preferences of general population samples, and thus can sometimes be insensitive for certain patient groups.

The alternative is to use disease-specific measures which are tailor-made to be sensitive in specific disease states and populations (Patrick and Deyo, 1989). However, for the purpose of economic evaluation these measures are rarely suitable for a number of reasons: their scoring systems cannot naturally be used for QALY calculations; they are not necessarily preference-based; and they cannot be used to make comparisons across disease areas (Patrick and Deyo, 1989).

For the purpose of economic evaluation it is therefore imperative to understand the extent to which generic preference-based measures of HRQoL are suitable in specific population groups. Without this information comparisons between intervention outcomes may inappropriately favour one intervention over another, or may be entirely incorrect. In this chapter I address these important issues, using an explorative qualitative approach to explore the applicability and content validity of the EQ-5D-Y and HUI measures in this specific setting.

7.2.3. Qualitative methods in health economics

Qualitative methods in health economics facilitate the practical application of health economics research, such as assessing the validity of outcome measures (Smith et al, 2009). Qualitative methods allow researchers to observe a topic or theme at the individual level within its own context (Coast et al, 2004), and thus demonstrate how perspectives of an individual fit into a wider societal context. In this chapter I used qualitative methods to understand how disabled children and their parents define QoL in relation to wheelchair use and how their definition relates to widely used HRQoL measures. Such data could influence how standard measures of HRQoL are applied in marginal groups such as disabled children.

7.3. Aims and objectives

The aim of this study was to explore the application of health economics methods of utility data collection to wheelchairs for disabled children by determining how disabled children and their families define QoL in relation to wheelchair use and disability. Furthermore, I aimed to determine whether standard, NICE approved HRQoL measures are appropriate methods to facilitate economic evaluation in this population. I approached this research from an extra-welfarist, societal and social model of disability perspective, underpinned by principles of disability equality and utility theory. The objectives were:

- To understand the key domains of QoL defined by disabled children and their parents in relation to wheelchair use and mobility impairment.
- To examine differences in how disabled children and parents define QoL in relation to wheelchair use and mobility impairment.
- To explore the extent to which generic preference-based HRQoL measures, such as the EQ-5D-Y and HUI2/3, reflect how disabled children and their parents define HRQoL in relation to wheelchair use.

Research questions:

A. What are the key domains of QoL defined by disabled children and their parents?
B. To what extent do generic HRQoL measures reflect how disabled children and their parents define QoL in relation to wheelchair use?

7.4. Methods

See chapter two for details on recruitment, data protection and ethical considerations.

7.4.1. Qualitative research reporting standards

To acknowledge the importance of explicit and comprehensive reporting of qualitative research, this chapter follows the COREQ checklist for qualitative reporting standards (Tong et al, 2007). In the interest of explicitly stating my perspective and background, I present personal characteristics deemed relevant to the subsequent analysis and interpretation of findings: I was solely responsible for conducting, coding and analysing all interviews. Wider discussion of the data with the supervisory research team was used to shape and test interpretations and to ensure internal validity. At the time of conducting the research I was a PhD student at Bangor University, studying Health Economics. I have completed Masters level modules in qualitative data collection and analysis. I approached this research from the perspective of an able-bodied father to an able-bodied child. All participants were unknown to me prior to conducting the interviews, and they were made aware of my personal goals for completing the research.

7.4.2. Design

This is an exploratory descriptive study, using a qualitative framework analysis approach. Qualitative framework analysis is a popular approach in health-related research as it aims to meet specific information needs (Lacey and Luff, 2009). Qualitative framework analysis was developed by Ritchie and Spencer (1994) specifically for use in applied policy research. Unlike more traditional methods of qualitative analysis, such as those used in grounded theory, qualitative framework analysis has defined stages allowing a systematic and transparent approach to qualitative analysis (Ritchie & Spencer, 1994). Although this approach to analysis has inductive processes, the use of deductive *a priori* methods allows specific themes and issues to be examined in targeted populations using pre-specified aims and objectives (Pope et al, 2000). In the context of health-related research this allows researchers to focus on a particular area of interest or phenomena, whilst maintaining systematic and transparent processes (Gale et al, 2013). The benefit of qualitative framework analysis is that researchers can identify trends in the data and focus on relationships between themes in order to draw explanatory conclusions (Smith and Firth, 2011).

Traditional approaches to qualitative research, such as grounded theory and phenomenology, are underpinned by both philosophical and theoretical principles which distinctly guide the research

process. Qualitative framework analysis on the other hand is generally devoid of these intrinsic values, and thus offers a flexible approach to theme generation that can be applied in a wide range of settings (Gale et al, 2013). An approach such as grounded theory would not have been appropriate in this context as *a priori* objectives and themes were defined explicitly (Lacey and Luff, 2009). Furthermore, qualitative framework analysis has been found to be particularly useful in health research with clear aims, such as developing HRQoL items for outcome measures (Stevens and Palfreyman, 2012), which was particularly relevant to the objectives of this chapter.

7.4.3. Data collection

Data was collected through face-to-face interviews conducted in participants' homes. A semi-structured interview approach was used, guided by an interview schedule (see appendix H). The interview schedule was used as a guide to ensure that key topics were discussed, although natural discussion was encouraged to keep dialogue unrestricted. Each interview lasted for around an hour. The interview schedule was reviewed after each interview and slight modifications were made where necessary as new and interesting topics were identified. This included adding specific themes and topics which arose frequently, and making slight amendments to questions in order to streamline the schedule and to ensure that key themes were covered in each interview. The core interview schedule was not changed drastically, as the amendments were used to ensure that the interviews were reflexive to the key themes and areas of discussion important to the participants, rather than to change the nature of the interviews.

Participants were encouraged to speak openly around the interview topics. The interview schedule was developed from the findings of a previous systematic review (chapter three; Bray et al, 2014), discussion within the supervisory research team and with consideration of the EQ-5D-Y and HUI HRQoL measures. The EQ-5D-Y and HUI were chosen specifically as the EQ-5D is recommended by NICE for cost-effectiveness analysis (Brazier et al, 1999) and the HUI is one of the only preference-based HRQoL measures suitable for use in young children. The questions were designed to allow participants to consider how they define QoL in relation to wheelchair use and to reflect on the appropriateness of the HRQoL measures to capture this definition. The EQ-5D-Y and HUI measures were completed by participants prior to interview (self-complete and/or proxy where applicable). Parents were asked to discuss health and QoL in relation to their child.

Use of child-centred interview techniques facilitated child participation in an age-appropriate way. This included using age appropriate language and props to engage younger children. Although it is now common to involve children as active participants, previous research on children's views has tended to be collected from adults, such as parents, carers or teachers. Although data from parents provides useful evidence, also including the views and opinions of children is important (Kyronlampi-Kylmanen & Maatta, 2011). When aiming to understand the impact of an intervention on a child their views and experiences should be seen as paramount. Qualitative research methods have been shown to be an effective and appropriate method in understanding the perspectives of disabled children (Curtin & Clarke, 2005)

Children's level of participation in the interview was made on a case by case basis. For some children this meant being interviewed on their own, while others were present whilst their parent was being interviewed and had limited input into the interview. No children under the age of five were included in the interviews at the request of their parents. Field notes were recorded throughout each interview to supplement the interview transcripts.

7.4.4. Questionnaire and measures

Participants completed a questionnaire containing demographic questions and HRQoL outcome measures (ED-5D-Y and HUI) prior to the interview. Age appropriate versions were completed by children and proxy versions by parents.

7.4.5. Data handling and analysis

After each interview the digital recordings were transcribed verbatim. Identifiable data was deleted from the transcripts to maintain confidentiality. Transcripts were not returned to participants for comments or corrections due to time constraints. The software nVivo v9.2 was used for qualitative data handling and analysis of the interview transcripts. The qualitative framework analysis approach was applied to organise and synthesise views and experiences into analytical themes (Ritchie & Spencer, 1994). Qualitative framework analysis has five key stages; familiarisation, identifying a thematic coding framework, indexing, charting and mapping/interpretation (Ritchie & Spencer, 1994).

During the familiarisation stage I became immersed in the data through listening to the interviews and reading the transcripts in detail. A sample of interviews was also reviewed by the wider supervisory research team and discussed in detail to develop the thematic coding framework collaboratively. An *a*

priori thematic coding framework was developed from a number of sources: the interview schedule; familiarisation with the interview transcripts; research team discussion; and the HRQoL domains of the EQ-5D-Y and HUI measures. The *a priori* thematic coding framework was applied flexibly so that it could be adapted to reflect the emerging categories and themes (Ritchie & Spencer, 1994). The emergent *a priori* thematic coding framework was discussed by the wider research team and refined in order to ensure internal validity. It was then piloted on a sample of four transcripts to check applicability. The results from this pilot were discussed by the supervisory research team and the thematic theory was further refined.

In the full analysis all transcripts were line-by-line coded using the finalised *a priori* thematic coding framework. This included indexing of predetermined codes and inductive codes which arose during the coding process. The emerging codes were grouped into categories, which were subsequently refined into higher order analytical themes giving a broader understanding of the coded transcripts. These themes were discussed by the wider supervisory research team to ensure that interpretations were representative of the data. Using charts and maps the data was integrated to gain a better understanding of the phenomena. Child and parent responses were analysed separately to account for their different but equally valid perspectives. Child age was also considered in analyses, with age groups defined as under 5's, 6 to 15 years olds and 16 to 18 year olds.

Once the coding was completed the emergent analytical themes were charted by theme and participant. This facilitated a deeper understanding of how each theme was interlinked, as relationships between themes could be observed within subjects and between other themes. The data was then used to build an understanding of how disabled children and their parents define QoL with respect to mobility impairment, and the subsequent applicability of standard measures of HRQoL.

7.5. Results

In the results section participant quotes are presented as informative and clear representations of specific analytical themes. Irrelevant information has been replaced with ellipses [...] to facilitate ease of reading. Repetitive speech and linguistic fillers (such as 'um') have been removed. Where there is more than one respondent presented in a single quote the following tags have been used to ease reading: 'R:' for researcher, 'C:' for child, 'M:' for mother and 'F:' for father. A small amount of participant information is provided for each quote to place them in context i.e. gender and age. Participant ID numbers are also provided so that multiple quotes from participants can be identified.

7.5.1. Response rate and sample size

A total of 125 study packs were distributed across England and Wales by the three recruitment sites. 38 initial HRQoL/demographic questionnaires were returned (initial response rate of 30.4%). Of the 38 child/parent dyads invited to take part in the interview ten declined. 27 interviews were conducted (one of which contained two child participants from the same family), giving a secondary response rate of 73.7%. An overall interview response rate of 22.4% [N=28] was observed for all of the 125 invitation packs sent out.

In total 17 parents decided to take part on their own, either because their child was too young to participate [N=12] or because they felt that it was not suitable for their child [N=5]. Four children over the age of 16 took part on their own and seven child/parent dyads took part in the interview together. In total 24 parents and 11 children were interviewed. Demographic details are presented in tables 7.1 and 7.2.

Table 7.1: Child demographic characteristics (parent and child samples)

Demographic characteristics	Parents N=17 (%)	Children N=4 (%)	Both N=7 (%)
Study site			
NHS Wheelchair Service	3 (17.6)		2 (28.6)
BIME	7 (41.2)		
Whizz-Kidz	7 (41.2)	4 (100)	5 (71.4)
Child gender			
Female	6 (35.3)	1 (25)	3 (42.9)
Male	11 (64.7)	3 (75)	4 (57.1)
Child age			
5 years or under	12 (70.6)		
6-15 years	5 (29.4)		4 (57.1)
16-18 years		4 (100)	3 (42.9)
Child ethnicity			
White British	16 (94.1)	4 (100)	7 (100.0)
Other mixed background	1 (5.9)		
Child diagnosis			
Porencephaly	1 (5.9)		
Cerebral Palsy	11 (64.7)	3 (75)	6 (85.7)
Muscular Dystrophy	2 (11.8)	1 (25)	
Rett syndrome	1 (5.9)		
Lissencephally	1 (5.9)		
Chromosome deletion	1 (5.9)		
Hemiplegia / stroke			1 (14.3)
Child Frequency of equipment use			
A little of the time	1 (5.9)		
Some of the time	4 (23.5)		
Most of the time	3 (17.6)		1 (14.3)
All of the time	9 (52.9)	4 (100)	6 (85.7)
Child Type of equipment used			
Powered	2 (11.8)		
Manual	7 (41.2)	1 (25)	3 (42.9)
Manual and powered	8 (47.1)	3 (75)	4 (57.1)

Table 7.2: Parent sample demographic characteristics

Demographic characteristics	Parents N=17 (%)	Parents and children N=7 (%)
Parent gender		
Female	15 (88.2)	7 (100.0)
Male	2 (11.8)	
Parent age		
21-29 years	2 (11.8)	
30-39 years	11 (64.7)	1 (14.3)
40-49 years	4 (23.5)	5 (71.4)
50-59 years		1 (14.3)
Parent ethnicity		
White British	16 (94.1)	7 (100.0)
White & Asian	1 (5.9)	
Parent marital status		
Married	11 (64.7)	7 (100.0)
Co-habiting	3 (17.6)	
Single	2 (11.8)	
Separated	1 (5.9)	
Parent education		
Higher	10 (58.8)	2 (28.6)
Further (e.g. A Level)	1 (5.9)	1 (14.3)
GCSE/O level	5 (29.4)	1 (14.3)
None	1 (5.9)	2 (28.6)
Other		1 (14.3)
Annual household Income		
£5000-15,000	3 (17.6)	
£16,000-£25,000	3 (17.6)	
£26,000-£35,000	2 (11.8)	1 (14.3)
£36,000-£50,000	5 (29.4)	4 (57.1)
£51,000-£75,000	2 (11.8)	2 (28.6)
£75,000 or more	2 (11.8)	
Parent employment status		
Full-time	3 (17.6)	
Part-time	6 (35.3)	3 (42.9)
Unemployed / stay at home parent	8 (47.1)	4 (57.1)

7.5.2. Defining QoL

In total, 15 categories of codes were used by participants to define QoL. The most commonly identified themes across the cohort were independence, social interaction and activities/participation. The 15 categories were synthesised to form 3 analytical themes, these were: participation and positive experiences; self-worth and feeling fulfilled; health and functioning. See table 7.3 and figure 7.1 for a breakdown of codes, categories and analytical themes.

Table 7.3: Breakdown of codes, inductive categories and analytical themes

Pre-determined codes	Inductive categories	Analytical themes
Defining QoL	Activities and participation Happiness Independence Social	**Participation and positive experiences**
	Achievement and fulfilment Being able to adapt Emotional wellbeing Equality Feeling normal	**Self-worth and feeling fulfilled**
	Cognition Communication Health Mobility Pain Self-care	**Health and functioning**
Usual activities	EQ-5D-Y relevance	**HRQoL measure relevance**
Mobility/ambulation Self-care Pain/discomfort Emotion/mental health	EQ-5D-Y and HUI relevance	
Sight Hearing Communication Cognition Dexterity	HUI relevance	

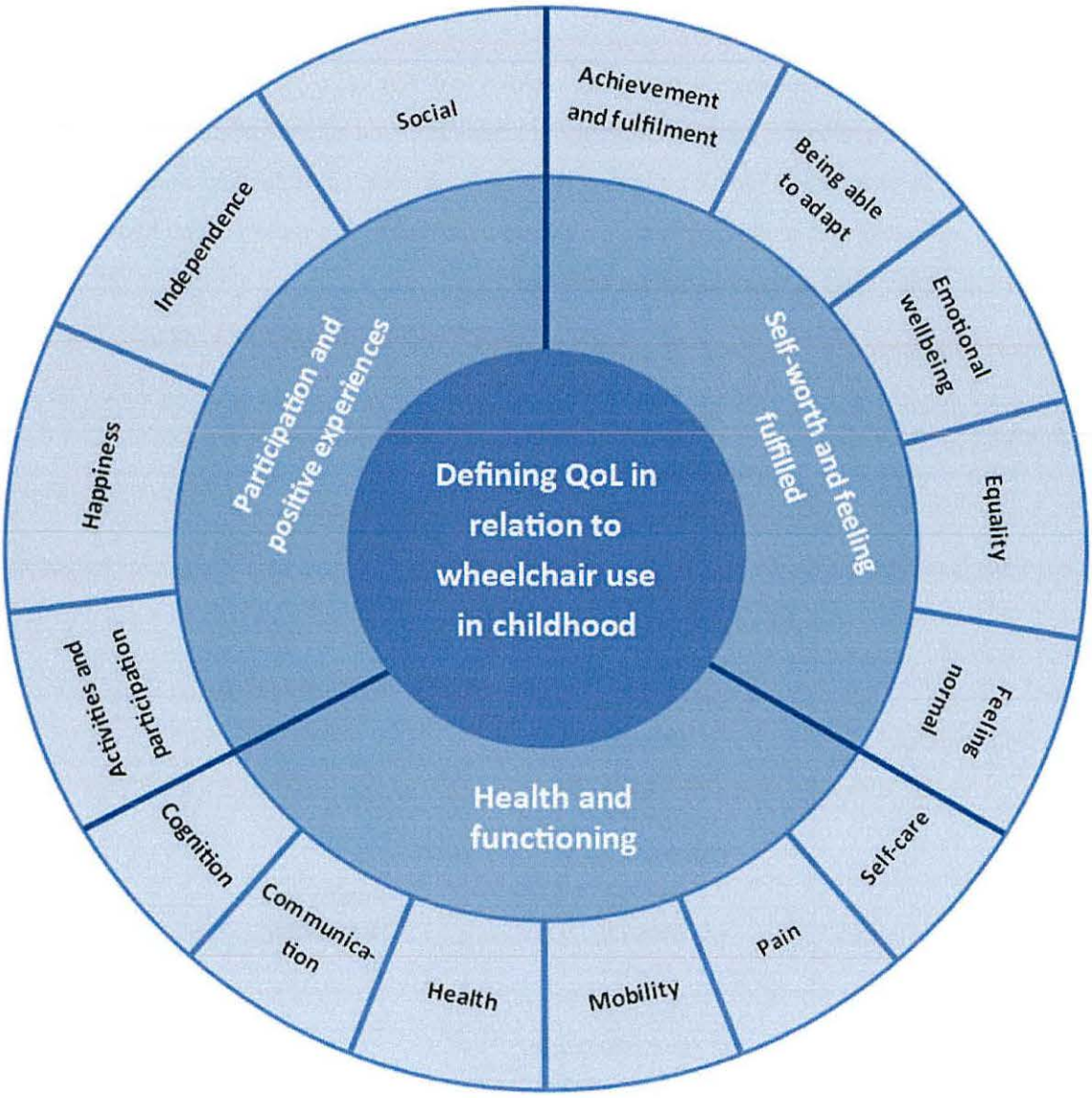


Figure 7.1: Defining QoL in relation to wheelchair use in childhood- a thematic summary and map

7.5.2.1. Participation and positive experiences

Being able to take part in positive and enjoyable experiences was an important aspect of defining QoL for children and parents across all age ranges. Furthermore, the categories of independence and activities/participation were the only common categories found across all sub-groups (i.e. 3 child age groups; children and parents). A key aspect of participation for children and parents alike was disabled children being able to do activities that are meaningful to them without restriction.

Mother of 4 year old boy (S1-21)*: R: What does the term "quality of life" mean to you in relation to [CHILD'S NAME] and other children with disabilities?*

M: Just that they get to have the opportunities and enjoyment of other children, really. And that what he is doing is meaningful to him.

Similarly, children highlighted the need to maintain usual activities and social interactions in order to maximise QoL.

13 year old girl (S3-19): *Make sure I can enjoy my life and do things that I want to do rather than just sitting in the house all the time, like actually going out places and doing things and meeting new friends.*

Disabled children often face barriers to participation caused by inadequate facilities or poor access for wheelchair users. Adaptations and equipment such as wheelchairs play a vital role in removing barriers to participation and finding alternative ways to take part in activities.

Mother of 10 year old girl (S2-28): *QoL is them being able to do anything they want to be able to do. And not being prevented from doing that by something stupid like not being able to access it because there's no ramp. Or no lift or something like that.*

Older children (16-18 years old) and parents did not view disability or wheelchairs as a barrier. They perceived barriers to be caused by inadequate provision to facilitate participation. This conceptualisation of disabling barriers reflects the central tenets of the social model of disability (Oliver, 1998). With appropriate adaptations in place some negative QoL impacts of disability can be limited. Issues arise when access is inadequate for wheelchair users, or when there is inappropriate provision to support disabled children.

Mother of 4 year old boy (S3-26): *Even going to the park, everything is access issues or things not designed for wheelchairs or people with disabilities, you know. Because he's, in his head, the same as anybody else so he wants to go to theme parks and things like that...I think as well he just wants to be doing what everybody else is doing, and he can't access it.*

Closely related to activities and participation was social interaction. The ability to interact with friends and participate in social situations was of particular importance to older children. Parents did not place

as much emphasis on social interaction, and tended not to differentiate this from other forms of activities and participation. For children, particularly older children, friends and social groups are an important aspect of self-identity and independence, and thus children place greater emphasis on social interaction. For older children socialising was also about taking opportunities and not being afraid to try new things.

> 18 year old boy (S2-20): *Yeah because if I didn't have a WC at all I'd just be sitting down all day really. And that wouldn't be as good because I wouldn't be able to get out and about and make friends.*

The importance of independence underpins these key aspects of QoL. Children and parents described how wheelchairs and other adaptations positively impact independence by providing freedom of movement and the ability for children to make their own decisions. It is therefore imperative that children are provided with equipment that is appropriate for their age and abilities and that maximises their independence.

> 17 year old girl (S2-29): *Everywhere I went I was pushed around in this buggy when I went out. I was like 10 [years old] in a buggy, if you know what I mean. I felt left out quite a lot, there's just no independence just sitting in a buggy. I think it's really good that little ones can have [PWCs] younger.*

Taking control of their own lives and being in charge of their own decisions was particularly important for older children who were transitioning from dependence on their parents/carers to some degree of self-sufficiency. For many children independence was about being able to socialise and to do activities that were important to them, without feeling like they were restricted by their mobility impairment. Although self-sufficiency was important, it was accepted that assistance would be required. The key component of this was that children felt in control of how assistance and support was provided to them.

> 17 year old boy (S3-24): *A good QoL for me I think is being, not necessarily being entirely independent, but being in charge of my own doings, so to some extent that means I'm able to move myself about in my chair but in other respects where I require assistance, like I have an assistant in college, but I'm still in charge of asking him for help, asking him to get my books out. I am in charge of my own life, I'm not being dictated to by other people or really by my disability.*

The concept of independence varied between participants, and was defined in relation to the abilities and potential of each child. For instance, for one child independence would be living alone and being completely self-sufficient, while for another it would be something as simple as being able to communicate their needs appropriately. Although children were striving for different levels of independence, they and their parents all sought to develop as much personal control as was possible for them (or their child).

> Mother of 10 year old girl (S2-28): *For some children, they aren't really going to reach a terribly high level of independence but for other children they'll reach an incredibly high level of independence...And I think, personally, that's what you should be striving for all the time, for them to be able to do as much as they want to do and the activities they want to do, and be as independent as they want to be.*

Wheelchairs play an important role in developing independence and giving children some control over their movement. For disabled children and their parents a child's ability to make decisions about their own movement was not necessarily about being mobile in the traditional sense, but finding alternatives modes of movement using adaptations such as wheelchairs. Achieving independence was therefore perceived as a key priority in wheelchair provision.

> Mother of 10 year old girl (S2-28): *They need to measure: is the child meeting its potential, what it thinks is its potential, and is the child being able to do everything it wants to do and access everything it wants to do using that? Is the child as independent as it possibly can be? Is it able to go to the places it wants to go to? And I think that would give you a much more accurate measure than "it's in a WC and it's doing this..." do you know what I mean? Or whether it's stopped the spine curving...I think that's quite a big thing. As long as they can emotionally feel they are doing everything then I think that's a pretty good indicator that they've got a good QoL.*

Achieving the correct wheelchair intervention was not the only area of importance in relation to wheelchair provision and adaptation, as participants also indicated that modifying the home to fit the needs and abilities of each child also impacts independence. Without proper access children can be restricted in their ability to learn independence and self-sufficiency. Therefore, access in the home and the wider environment has some part in defining QoL in relation to independence and wheelchair use.

13 year old girl (S3-19): *I think [to develop independence] is most of the reason why I have the kitchen extension done, because in the older house I wasn't doing the stuff I could learn how to do. So when I had the house done it was easier to learn how to be independent.*

7.5.2.2. ***Self-worth and feeling fulfilled***

The importance of self-worth and feeling fulfilled was expressed through a number of categories by both children and parents. Interestingly, this analytical theme was almost exclusively expressed by parents of children under the age of 5 and by older children. Self-worth was defined in a number of ways by children, including ability to achieve goals and be fulfilled; feeling equal and free from prejudice; being able to adapt and feel normal; and good emotional wellbeing. These attributes of QoL are augmented by the child's ability to experience positive things in their lives (through independence, social interaction and participation) and by their ability to function and maintain good health and happiness.

Happiness was not a concept discussed explicitly by all participants, which may be in part due to abstract discussion of happiness through other concepts, such as friendship and independence. However, for parents of children under the age of 5 happiness was an important priority when defining QoL.

Mother of 4 year old girl (S1-03): *People define it by health, and about being well. And it's a lot about that, I define it in a way of happiness really.*

For many parents this was their first child with a disability and thus presented a whole new understanding of childhood and parenthood. They therefore had to redefine happiness with regards to their child's disability, and thus placed importance on this as a nebulous concept rather than specific areas of life that can bring happiness. For the parents of older children and for children themselves, the ambiguous concept of happiness was not discussed in great detail. This may have been due to better understanding of how they were able to achieve happiness through specific aspects of their lives, such as socialising and taking part in fulfilling activities. As children get older it appears that parents shift their attention away from happiness as a global concept and instead focus on specific aspects of life which bring happiness.

16 year old girl (S2-07): *R: So what do you think the term good QoL means?*

C: Like having a good life, enjoying your life

Achievement and fulfilment was almost exclusively discussed by older children. For older children these were important attributes when defining QoL, specifically being able to set and achieve goals. This category linked with independence and autonomy, as children are able to express themselves through setting personal goals and deciding what is important for them. Achievement was described as being able to make the most of opportunities, regardless of perceived barriers to access or participation.

> 18 year old boy (S2-20): *It's also just sort of trying to give yourself as many opportunities as you can and take advantage of them because there's certain opportunities that you might only get once. Take advantage of it. To be honest when I was a bit younger some of the things I was able to do I can't do now but I'm pleased that actually I did do them because I've enjoyed doing them and at least I know I've had that experience.*

One parent of a young child also discussed the importance of achievement. For them QoL was not about specific abilities, such as walking and talking, but about facilitating a child to achieve all that they were personally capable of achieving. They defined QoL as being about subjective abilities and making the most of an individual's situation regardless of ability or disability.

> Mother of 4 year old girl (S1-03): *I think that QoL is really hard because I do define it completely differently because I just don't...people think about it as like "walking and talking", and actually you realise there's so much more to life than that. And I feel with [CHILD'S NAME] that it's about making a difference in her life. It's giving her access to everything but in a different way. There's so much more to life, you can still achieve things but in a different way and it doesn't matter if you're not walking or talking or if you can't hear.*

Emotional wellbeing was a common theme for parents of young children. They expressed that QoL was about children having a happy and enjoyable childhood free from worry. Interestingly, this was not a theme discussed by older children or their parents. Much like the concept of happiness, it appears that many participants did not speak directly about emotional wellbeing as they perhaps alluded to it through other concepts which have an impact on emotional wellbeing, such as social interaction and achievement.

> Mother of 2 year old girl (S1-06): *It's a child being a child and just not having to worry about, not that she will be at this age, but not having to worry about how they look or feel. She's just enjoying being a child because she can move around.*

Although older children and their parents did not discuss emotional wellbeing directly in great detail, they did refer to coping with disability. Coming to terms with a disability and the limitations it may impose can impact QoL, as ability to cope with and accept a disability to some extent defines how limitation will affect QoL. Coping mechanisms and resilience are therefore influential factors in defining QoL for disabled children. For many of the older children and their parents adaptations such as wheelchairs were seen as an important tool in the process of coming to terms with disability and impairment.

> 17 year old boy (S2-25): *If you weren't able to deal with your disability appropriately and if you weren't able to deal with that fact, then they would... disability is something that you cannot get away from, and if you weren't able to deal with that fact you would quickly become very depressed, very helpless and you'd quickly think "my life isn't worth living."*

Equality was an important aspect of QoL for parents of young children. They identified that QoL was in part about their child having equal opportunities to participate and not being excluded from anything, which links with being able to participate and enjoy activities.

> Mother of 3 year old boy (S1-04): *R: So for you, what does the term "QoL" mean in relation to [CHILD'S NAME]?*
>
> *M: Just that he's given every opportunity to enable him to do his best, really. And making sure that he gets the opportunity and that when he gets it he can do it as well, whatever it is it's been adapted so that he can do it.*

This concept was not widely discussed by other subgroups, although one older child did state that their definition of QoL was about not being stereotyped or discriminated against because of their disability, and another child indicated that they wanted to be treated the same as other children regardless of their disability.

> 17 year old boy (S2-25): *One of the big things for me is not being stereotyped, not being discriminated against, not having to fight for things, I sometimes feel really under pressure from people.*

7.5.2.3. ***Health and functioning***

The role of health and function in defining QoL was found to be of key importance to parents of younger children, but was less important for older children and their parents. This indicates a clear difference in the way parents define QoL as their child ages. Parents of younger children may still be coming to terms with their child's disability and the future impacts it may have on their health and function, thus they place greater importance on these aspects. Older children and their parents potentially have more awareness of disability prognosis and the potential changes in health and function over time. They therefore have more awareness or expectation for what the future holds. For this reason they may be able to focus on other aspects of their life, such as activities and socialising, which have a direct impact on QoL and are within the control of the parent and the child. It is this understanding of adaptation and how to work around limitations which encourages older children and their parents to focus on self-worth and positive experiences. This indicates that defining QoL is relative to current ability and potential future ability, mediated by age.

Parents of younger children indicated that the ability to communicate was a key aspect in defining QoL. For these parents the ability to communicate was not simply about talking, but about being understood by whatever means are available to each child. It was therefore deemed important for young children to be able to express their needs through a medium of communication appropriate for them.

> Mother of 3 year old boy (S1-04): *Alleviating frustration is a big problem for us because I 'get' [CHILD'S NAME], but some people don't and sometimes he can get quite frustrated. And things like trying to understand what they're saying if they're really trying and someone's not understanding...they can then become withdrawn if people aren't understanding them and upset and sad.*

Being able to express basic needs, such as hunger, thirst and needing the toilet are important aspects of communication. Without the ability to do so children may feel frustrated, upset and isolated. In some circumstances children aren't able to verbalise but can find other ways of communicating, such as using their wheelchair to gain attention and to show their parent what it is they are interested in.

> Mother of 2 year old girl (S1-06): *She communicates very well with [her PWC]. When she wants your attention, your legs!*

Traditional definitions of functional abilities, such as walking and self-care, were not widely used to define QoL. Self-care was only discussed by one child and the relative importance of mobility was only discussed by two parents of children under the age of 5. They stated that walking and freedom of movement with adaptations are basic needs which can affect QoL when restricted, although this was not a widely stated opinion.

> Mother of 4 year old boy (S3-26): *People think "well he's never done it, he's never walked, he won't miss it". It's not true, you have a basic need to get up on your feet and walk from being 6months old, you have that, you need to do it. And he's needed to do it, he's needed to feel his feet and things like that, so it's completely, you know, he sees kids walking past and running and he cries, you know. There's no getting round it, even though he's the happiest kid in the world, he feels it, you know, he wants to do it. So yea, it's a massive part. You can't do anything, you can't go anywhere that you want to go, you know. You want to go and get a drink, you want to pick that toy up, you want to go over to your friends.*

However, the ability to be mobile by other means (such as wheelchairs) was seen as an important aspect of defining QoL for disabled children, and for most participants being able to adapt was considered more important than basic mobility, particularly for children who are unlikely to achieve full independent movement by walking.

> Mother and father of 12 year old boy (S2-10): *F: A basic wheelchair allows him to actually live for a bit, but the PWC takes it to that next level and gives him independence, this difference is just as vast. The thought of having life without a wheelchair doesn't bare thinking about, it's impossible isn't it, it's like he'd have a 0.5 QoL. In a wheelchair he's probably got a 5 out of 10 QoL and then with the independence you'd probably look at 8 out of 10 QoL. It gives him ...*
>
> *M: [PWC] gives him a choice doesn't it, where he can make the decisions, whereas when he's in the MWC everyone makes the decision for him.*

Being able to find alternatives when limitations cause restrictions allows children to overcome barriers that may negatively impact their QoL. Parents tended to focus on what their child could achieve as opposed to what they couldn't. QoL was defined as being able to do everything possible even if that meant that adaptations were required. In the context of disabled children, parental understanding of

the QoL benefits of adaptations demonstrates the importance of focussing on individual ability rather than expectations derived from wider society and able-bodied peers.

> Mother of 5 year old boy (S2-08): *I suppose under the mobility side, it wouldn't be about walking. It would be about independence in their chair. Because the biggest frustration he has is keeping up with others and doing what they're doing, and so you don't necessarily have to be walking to keep up with them. And it's like, as well, writing, he can't write, probably will never functionally write, and it's taken him a year to realise he doesn't have to write, he can use other ICT [information and communications technology]. So he doesn't have the same life but he does have the same, it would be the same QoL.*

All subgroups discussed the beneficial effects of adaptations, specifically the role of wheelchairs in raising QoL. For disabled people who require adaptations, equipment such as wheelchairs can be an intrinsic part of defining QoL.

> Mother of 5 year old boy (S2-08): *For people with disabilities, QoL does come down to your adaptations and how you adjust your life to make it quality, if that makes sense.*

Adaptations exist as external items which facilitate particular activities or abilities, therefore their users are acutely aware of their value. Adaptations become integral to that individual's ability to function in a particular way, and thus to some extent their QoL is tied into that piece of equipment.

> 16 year old boy (S2-16): *As long as there is a way for somebody to communicate or to get themselves from A to B, or even if there's something as simple as maybe getting them a little hook thing so they can dress themselves or something like that. As long as they're doing stuff that they want to do.*

Several children and parents across all age groups stated that with the correct adaptations the QoL of a disabled child can be equivalent to that of an able-bodied child. Although their abilities and needs are different, if disabled children are able to find a balance between ability and use of adaptations they can maximise their QoL. This requires correct provision of adaptations which promote the best functional ability for that child.

> Mother of 3 year old boy (S1-04): *I've had conversations with friends who are completely able-bodied and they've sort of said "Do you wish you could do some of the things I can do?"*

but I said "But do you wish you could put a basketball ball in the basket like I can?". It's like, everybody's got things they can do better than other people. It's like, they can walk better than I can but I can push a wheelchair better than you can. It's one of those things that, I've just always thought everybody's got things they're good at. Everybody's got things they're not very good at and I'm just not very good at walking. But I'm really good at pushing a wheelchair and things like that, and I've had friends who have nothing wrong with their body but as soon as they get in my wheelchair their coordination with the chair is terrible and they can't even push it in a straight line even after practice and things like that.

However, parents and children both stated that there would likely be a difference between the QoL impact of a disability experienced from birth and one acquired at a later date. It was generally agreed that acquired disability would be more detrimental to QoL than a disability from birth, as an individual would be able to miss the skill they once had. In terms of defining QoL, it appears that the impact of disability on individual QoL is relative to that individual's abilities, both past and present.

17 year old boy (S2-25): *Because when you have it from birth you almost don't have to deal with it, because you know...you don't know any different, and when you do start knowing any different you deal with that again. The difference is your Mum and Dad are there to feed you, make sure you're alright. When a disability happens that say, when your parents are not around, or when you've not had it from [birth]...you kind of know what you've lost.*

A small number of participants in each subgroup remarked that there is a balancing act between maintaining normal functioning and deciding to use adaptations. For some children continuing with normal functioning can have a detrimental impact on health and QoL compared to accepting adaptations. For instance, a child may physically be able to continue walking but may experience more pain doing so. Alternatively they could use a wheelchair and potentially have less pain and have more functional mobility. It is therefore counterproductive to assume that QoL is defined by normal functioning in this situation, as QoL may be maximised by reducing that normal functioning. In this respect, defining QoL is about maximising functioning regardless of ability or the requirement for adaptations.

17 year old boy and mother (S3-24): *M: [CHILD'S NAME] has friends at the moment who are at varying stages of their conditions and at the moment they're still at the stage where their QoL would be affected by removing walking because they're not in enough pain and discomfort to give it up.*

R: So it's relative to the person and the stage?

M: And the stage of their condition really. Because we've seen lads give it up and be absolutely fine and say it's just so much easier, and other lads who've got the same condition but at a different stage who say "Oh God I couldn't give up my walking" but that's because they're not suffering as much, they're not struggling as much. You were at a stage of struggling so much there was no...

C: There's a tipping point where you go "It's not worth it anymore, it hurts too much, it's not practical, I'm too tired".

Health was only explicitly used to define QoL by two parents of younger children. Health was seen as secondary to other areas of QoL, and separate to disability. The lack of focus on health may be due to the implicit desire for good health, or potentially due to parents' and children's augmented understanding of health in relation to disability and QoL.

Mother of 4 year old girl (S1-03): *She has problems with absolutely everything, but I define quality of life as more in... It's more in making every day count, but also, you know, accessing everything you can, and it's a bit about attitude. Especially when people have a baby they think "Oh I want my child to be healthy" and I think "Do you know what, [CHILD'S NAME] is really healthy but she has a lot of problems with other things".*

Like health, pain was not a topic expressed explicitly in great detail, as most participants did not use freedom from pain to define of QoL. This could again be due to the implicit importance of being free from pain, much like good health. Pain was linked to health, as it was seen partly as a symptom of other health conditions, such as seizures.

Mother of 2 year old boy (S1-01): *I think just being well, because we've had a lot of problems with various things. So his health, at the minute, is the main thing. Just to see that he's not in pain. He has quite bad seizures which are controlled at the minute.*

7.5.3. Content validity: Relevance and accuracy of EQ-5D-Y and HUI measures

All of the five HRQoL domains measured on the EQ-5D were considered relevant to children with disabilities by all sub groups. In particular, the emotion (anxiety/depression), usual activities and pain/discomfort domains were considered appropriate for this group of children across the age ranges, both in terms of content and possible responses.

It is of note that participant definitions of QoL and their assessment of outcome measure content validity produced a number of contrary results. For instance, many participants agreed with the pain/discomfort and self-care domains on the HRQoL measures, but did not use these to define QoL when prompted to consider QoL in relation to wheelchair use. These contradictions highlight the differences between how individuals define core components of QoL and how they believe HRQoL should be measured.

7.5.3.1. ***Emotion***

Participants across all sub-groups were in consensus that mental health and emotion (EQ-5D anxiety/depression domain and HUI emotion domain) are important to HRQoL. For some children worrying can have a large impact on their HRQoL and can be related to underlying anxiety about their health and disability.

> Mother of 5 year old boy (S2-08): *I would include mental health into that. In terms of feeling sad, worried or unhappy, generally he doesn't until he compares himself and then he can sometimes feel a bit worried about things that he can't do.*

Several participants stated that mental health and emotion are related to other aspects of HRQoL, for instance being unable to do usual activities may cause sadness. Emotional wellbeing was described as a by-product of meeting other individual needs, and thus was considered to be interlinked with other aspects of HRQoL. In this respect emotional wellbeing can be a defining factor of HRQoL and a by-product of fulfilling other factors of HRQoL. This perspective, however, does not take into account the impact of mental illness and the indiscriminate way in which it may manifest.

> Mother of 10 year old girl (S2-28): *R: And then finally feelings of being worried, sad or unhappy?*
>
> *M: Yeah it would have an impact on your QoL obviously. I think one would hope if you get the other bits right then that bit would follow on as ok.*

R: So you see them as interconnected then?

M: Yeah.

7.5.3.2. ***Pain and discomfort***

Participants across all sub-groups stated that pain and discomfort is important to HRQoL and an important domain to consider when measuring HRQoL. Participants agreed that not being able to control pain can have a large impact of HRQoL, which is especially relevant for children with certain forms of disability, such as CP, which are associated with pain and stiffness.

18 year old boy (S2-20): *Yeah it can be annoying if you have pain. I'm quite lucky that I'm not in pain like, a lot of pain. I do get aches from time to time and things like that and certain things that you get only because you're disabled. But I think I'm lucky in a lot of senses with regard to that because being...I know there are other people who are in a lot more pain.*

7.5.3.3. ***Self-care***

Participants across all groups were generally in consensus that self-care does impact HRQoL, particularly for older children. The parents of younger children felt that self-care, as worded in the EQ-5D-Y, was not completely relevant as young children wouldn't necessarily be expected to care for themselves at that age. At present the EQ-5D-Y is not validated for children below the age of 4 (EuroQoL Group, 2014) which may account for this.

Mother of 5 year old boy (S1-23): *R: And would you say that his issues with being able to look after himself impact on his QoL or not?*

M: No. No I don't, I don't think they do because I think that [CHILD'S NAME] doesn't know any different. It's not like he could do that and now he can't. [CHILD'S NAME] has always been washed and dressed and bathed and showered and everything so I don't feel that the thinks "I wish I could do this by myself"

Some participants stated that the self-care domain could also include expanded definitions of self-care, for instance being able to communicate needs so that someone else can provide care, or the use of adaptations to make self-care easier.

Mother of 4 year old girl (S1-03): *And I think "looking after myself", you know, she will never be able to do things like washing or dressing without help. But actually, she could potentially when I get the house done, take herself upstairs in the lift, turn a bath on. That actually might be a massive step for her, for her independence really.*

7.5.3.4. ***Usual activities***

Participants across all sub-groups agreed that the usual activities domain was appropriate. A large proportion of participants stated that being able to do usual activities is an important part of defining HRQoL, and thus should be considered a high priority when measuring HRQoL. This reflects earlier findings regarding the importance of activities and participation in defining QoL.

18 year old boy (S2-20): *R: The next one is usual activities so being able to go to work, going to school, doing hobbies, things like that. Would you say that has a impact on QoL?*

C: Yeah that's probably more important than [mobility and self-care] probably. It's just important being able to do something, even if it's just small things and like being able to do your hobbies. It can be a bit disappointing when you want to do certain hobbies and then you find out it's often making it more difficult for you then to continue with that.

A small number of divergent cases stated that the term 'usual activities' was too broad and could potentially be broken down with separate questions for independence and socialisation. This reflects the relative importance of these domains of HRQoL and the broad nature of 'usual activities'.

16 year old girl (S2-07): *R: Would you say there's anything else you would add to that if you were measuring QoL?*

C: Going out and seeing your friends

R: So more about socialising?

C: Yeah

One young man stated that socialising does not impact QoL but education does, thus the usual activities domain could potentially be too broad to capture such differences.

17 year old boy (S2-25): *When you put going to school on the question the question takes on a very different sort of tack. Going to school is important for QoL but socialising isn't.*

Socialising is an optional thing, you can probably sit at home and not do anything. The point at which this question becomes tricky is the educational argument, so I'd say really it isn't important in the sense of socialisation but it is important in the sense of educational performance.

7.5.3.5. ***Ambulation/mobility***

Parent and child participants in all age groups stated that mobility is relevant to HRQoL

11 year old boy (S2-11): *Mobility is the most important thing because you wouldn't be able to get around without it.*

However, the wording of the EQ-5D-Y mobility domain levels were considered inappropriate as mobility beyond walking is not taken into account. Participants felt that the response options were not appropriate for disabled children as they intrinsically devalue HRQoL based on ability to walk. For these children independent walking was limited, difficult or impossible, and thus a wheelchair was an essential tool to enable mobility. They stated that mobility was key to good HRQoL, but that the benefits of mobility were not confined to walking.

Participants discussed the role of adaptations (such as wheelchairs) and the ability they have to raise HRQoL to that of an able-bodied person. The EQ-5D-Y automatically discounts HRQoL due to inability to walk, and therefore does not account for the benefits of wheelchairs. Furthermore this measure would be inappropriate for assessing change over time from a wheelchair intervention as the aim of a wheelchair for long-term use is to provide mobility by other means, not to enhance walking ability.

Mother of 10 year old girl (S2-28): *Mobility needs to be expanded if they really want to be able to measure wheelchairs, because mobility just as walking about will never ever measure what a wheelchair can do for a child.*

Similarly to the EQ-5D-Y, many participants highlighted that although mobility is important to HRQoL, the wording of the HUI ambulation/mobility domain is not sensitive enough to account for mobility beyond walking and the positive HRQoL impact of alternative mobility. The HUI measures allow more choice with regards to mobility, including consideration of adaptations and supported walking. However the measure automatically assumes that walking is the pinnacle of mobility for all individuals. For disabled children who potentially have lifelong walking impairment it is important for them that mobility is defined more broadly.

17 year old boy and mother (S3-24): *M: I remember this part [HUI Q9: Ambulation/mobility]. That was a really bad one...doesn't make any sense!*

C: Because about the walking and things. I can do standing transfers and things like that, so...

M: This says "Unable to walk alone even with walking equipment", "Able to walk short distances with the help of another person"...

C: I essentially use my mum as walking equipment.

M: And "Requires a wheelchair to get around the neighbourhood". That doesn't make sense does it? And I remember that one because I went "What?!" The beginning made sense to us. And that's why you ended up putting "Unable to walk at all" but you can sort of stagger around with the help of somebody.

7.5.3.6. ***Dexterity***

Dexterity was considered to be an important domain of HRQoL. Limited dexterity in the hands or fingers can cause frustration and can limit abilities, which in turn may impact HRQoL.

13 year old girl (S2-17): *R: And then being able to use your hands and fingers, do you think that impacts QoL?*

C: Yeah because if I wasn't able to use my hands and fingers I wouldn't be able to do work and stuff, and to communicate with my friends and to play games

Although many participants across the subgroups felt that this was an appropriate domain, parents in particular commented on the lack of sensitivity in the question and answers to account for the abilities of disabled children. For children who have issues with one hand or one side of their body the HUI dexterity domain is unable to account for differences in ability. Many wheelchair users, particularly those with cerebral palsy, have limited ability with only one side of their body. The HUI dexterity domain refers to 'hands or fingers' without differentiating ability to use one or both hands. Selecting the most appropriate answer was therefore difficult for participants and often led to them rating ability worse than it actually was.

Mother of 6 year old boy (S3-15): *The one in particular was about his left, not being able to do things with your hands, because he can but he also can't. He can do plenty of things with his right hand, but as soon as it comes to two-handed tasks it's more difficult. So you can't say he can do this, because he can't do that either. I felt like I was short-changing him because he can do lots of things, but to answer the question accurately I had to say "no he can't". There wasn't enough options really.*

Adaptations also have a role in augmenting the HRQoL impact of dexterity problems. Although the questionnaire accounts for use of 'special tools' and adaptations, it assumes that the use of adaptation automatically impacts HRQoL. This reflects the difference in the way children with disabilities and their parents define HRQoL and the role that adaptation has in improving HRQoL rather than signifying poorer HRQoL.

Mother of 5 year old boy (S2-08): *He can pick things up, he can feed himself, but he can't write. The finer the motor skill gets the worse his... mobility gets...but again, that one comes back to what I was saying before about adaptations in life. So yes, he has those limitations but with the iPad and other things he has access to, it doesn't come in on his QoL. It's not going to be a factor for him in our day and age. It might have been 10 or 20 years ago, but it's not today.*

7.5.3.7. ***Cognition***

Children stated that cognition does impact HRQoL, specifically being able to learn and retain information. They emphasized the importance of education and school, and the impact cognition has on these important aspects of their lives. Several children discussed the impact cognition has on other usual activities, such as being able to remember names and other important information.

13 year old girl (S2-17): *Yeah, because if you couldn't remember things like passwords it would make it difficult, or numbers or something, I reckon that would make it difficult. You wouldn't be able to call someone or someone's name because you wouldn't be able to call them by their name.*

Although parents also felt that cognition impacts HRQoL, the parents of younger children did not unanimously agree that cognition has a specific role in defining HRQoL. They stated that children with learning disabilities can achieve a good HRQoL and that other aspects of HRQoL have more importance

than cognition. For instance communication was perceived to have a specific impact on HRQoL regardless of cognitive ability.

Mother of 10 year old girl (S2-28): *R: And then the final one would be memory and problem solving – do you think those have an impact on QoL?*

M: I think so because it's all tied up with the feelings thing, things you remember evoke different feelings and also your learning ability probably. What you get out of stuff is what you've learnt and everything. I guess it does, having said that, again I know plenty of children who perhaps have what you would define as learning disorders but they are perfectly happy with how they are, you know, and so they would say they've got a good QoL you know.

This difference between parents and children may reflect the variance in the cognitive impact of disabilities. The children who participated in the study were cognitively able to do so and thus had a certain level of ability, while the young children of the parents participating in the study had a greater variance in ability. Parents who have a child with a pronounced form of intellectual disability may have a better understanding of the impact cognition has on HRQoL, and thus are able to reconcile their child's cognition and HRQoL. Older children who perhaps have little experience of cognitive disablement were only able to reflect on the domain in relation to their own ability. In some respects this is reminiscent of the way in which mobility was previously perceived to be defined from a specifically able-bodied perspective.

7.5.3.8. ***Communication***

There was a high level of consensus across all subgroups that communication has an important role in defining HRQoL. Several participants, particularly parents of young children, stated that lack of communication can cause frustration and subsequent reduction in HRQoL. Older children focussed on the perceived wider impacts of commination, such as not being able to socialise and make friends.

Mother of 7 year old boy (S2-18): *Being able to be understood, he used to get really annoyed and it would show, and he was quite cranky probably at that point, whereas now he can communicate...we've always repeated back to him what he's said so he knew that I understood, and then if I got it wrong he'd keep going, he'd keep going and keep going*

until you did get it right! But there was a big big change I think in him from when he couldn't communicate...to being able to be understood.

As well as lack of communication, many participants also discussed the impact of being understood. For children who lack clarity in their speech or who communicate through other means (such as sign language) being able to communicate clearly and effectively can be challenging, particularly when trying to communicate with strangers. The HUI measure captures this differentiation between being understood by people who know the person well and those who do not.

13 year old girl and mother (S2-17): *M: She gets frustrating sometimes when her [speech] clarity really goes, 'cause if you're really worried your clarity goes a little bit doesn't it?*

C: Yeah

M: And then people can't understand as much

C: And when I'm tired

A small number of parents of younger children noted that the HUI focuses on speech rather than other forms of communication, such as sign and text. This reflects the need to ascertain individual ability and the role it has in defining subjective HRQoL.

Mother of 2 year old boy (S1-01): *M: I would say it's still quite... it's sort of basing everything on speaking or not speaking, not necessarily communicating.*

R: So its definition of communication is too limited?

M: Yes. I think so.

7.5.3.9. ***Senses***

Hearing and vision were both deemed appropriate in measuring HRQoL across all sub-groups. Children again focused on the perceived wider impact of senses on HRQoL, such as being able to interact with others and maintaining independence.

18 year old boy (S2-20): *It probably depends on how you deal with it because it probably would be really bad if somebody couldn't hear and somebody couldn't see both at the same time...Sometimes people that can't see but they can hear, or they can't hear but they*

can see. But it must be hard if you're wanting to talk to your friends but you can't hear them talking to you. That must be really hard. Not being able to see things must be hard.

A number of parents and children stated that impaired senses do not necessarily impact HRQoL if appropriate adaptations are in place. This again relates back to the impact that adaptation and subjective experience have on HRQoL. For individuals without sight or hearing impairment the loss of senses is perceived to have a major impact on HRQoL, much like the perception of reduced mobility. The actual lived experience of HRQoL differs from perception, particularly when that perception is affected by high-functioning subjectivity. It is therefore difficult to accurately measure HRQoL in disability from the perspective of an able-bodied person. The implicit able-bodied viewpoint discriminates against any loss of function caused by disability.

Mother of 10 year old girl (S2-28): *This is a hard one because me being able to see and hear, I would say yes [senses impact QoL], however, I know a couple of people who are visually impaired and they have a great QoL so actually I'm not sure. It's the same as with WC users and walking around. It's exactly the same sort of thing, you can still have a good QoL without perhaps having all the senses that people think you should have.*

7.6. Discussion

7.6.1. The importance of independence

The results in this chapter illustrate that independence has a clear impact on the health and QoL of disabled children. Wheelchair provision should enable and sustain independence (Muscular Dystrophy Campaign, 2011) and promote physical, cognitive and social development (DoH, 2004). Without the ability to move independently children are unable to have full control over their social interactions and their ability to participate in activities. Furthermore, without appropriate access to enable independent movement children are restricted in their ability to socialise and participate. The desired level of independence, socialisation and participation for each child varies, and each child (and their parents) will have different expectations for levels of independence. Children and parents seek an appropriate level of independence relative to the child's abilities, and this relates to removing barriers that oppose independence. Wiart et al (2004) found that mothers believed that wheelchairs enabled their disabled child to take part in age-appropriate activities and reduce their need for assistance.

An important part of defining QoL for disabled children is enhancing and facilitating independence (in accordance with individual ability) to promote and maintain social interaction and participation in activities. Children and parents showed consensus about the importance of participation, activities and independence, although children placed greater emphasis on social interaction, particularly older children. Evans et al (2007) found that after provision of a PWC children experienced increased independence, which led to increased socialisation and participation.

7.6.2. Self-worth and fulfilment

Self-worth and feeling fulfilled was exclusively important for parents of children under the age of 5 and older children. Participants defined this theme using a number of key categories, including: the ability to achieve goals; to be considered equal and normal; and being able to adapt. Self-worth and fulfilment was closely linked with independence, social interaction, participation and ability to function, all of which contributed to a positive feeling of self-worth. Interestingly, child participants and parents of older children generally refrained from using terms such as happiness and emotional wellbeing. This indicates the different QoL perspectives associated with age, as nebulous terms such as happiness become replaced with specific activities or functioning which provide happiness and wellbeing. This may indicate a gradual learning process for parents of disabled children who become more aware of their child's capabilities as they get older and thus are better able to define happiness in the context of their child's abilities. This reflects findings from Wiart et al (2004), who found that for mothers of disabled children there is a period of coming to terms with the changing mobility and equipment needs of their child.

Achievement was identified as being both linked and separate to independence and autonomy, as children are able to express themselves through setting personal goals and become fulfilled by achieving them. The sense of achievement was both in the outcome and the process of overcoming the barrier. Coping mechanisms and resilience are key to HRQoL in disabled children, and equipment such as wheelchairs are a part of that development.

7.6.3. Differences between child and parent perceptions of health and function

The greatest divergence in defining QoL was the theme of health and functioning. Parents, particularly those of younger children, clearly stated that health and functioning has a significant role in defining QoL, however as child age increased this theme became less prominent. Potentially this indicates the there is a learning process for parents of disabled children, who become more aware of their child's

capabilities as they get older. Parents place greater importance on their child's health and functioning when they are uncertain about their child's future. As awareness of ability increases so does emphasis on other domains of QoL, such as activities and socialising.

Traditional functional abilities such as walking and basic self-care were not widely used to define HRQoL. Instead participants focussed on mobility by other means (such as wheelchairs) and ability to adapt. This indicates that positive abilities and functioning are much broader than traditional definitions, such as being able to walk. Achieving mobility by other means was key to defining QoL in this cohort. This linked with the concept of overcoming barriers and achieving fulfilment through adaptation. In the context of functioning, QoL for disabled children is therefore about individual ability rather than expected norms. Several participants stated that disabled children can achieve a QoL on par with an able-bodied child if the correct adaptations are in place to facilitate this.

7.6.4. The impact of acquired disability on HRQoL

It is of note that across all sub-groups participants felt that disabilities acquired later in life would have a greater impact on HRQoL than congenital mobility impairments. The loss of abilities and functioning was perceived to be more detrimental to HRQoL than having never experienced them. This links with the perception that QoL is defined by personal ability, and thus a change in that ability detrimentally impacts QoL. The important factor is thus the baseline level of ability and the ability to cope with subsequent change. Focusing on 'normal' functioning can potentially detrimentally impact wheelchair users, as their HRQoL may in fact be increased by reducing walking and favouring wheelchair mobility. This reflects Sen's (1993) differentiation of functionings and capabilities, as focussing simply on achieved functioning presupposes optimal capabilities.

There was a perception that having and then losing a functional ability would impact HRQoL more than never having had that ability, for instance walking. However, participants did not explicitly discuss deterioration in health and QoL as a result of illness or disability. It is of note that participants perceived a reduction in ability to impact QoL but did not relate this to degenerative illness or disability. This may indicate that participants felt that being able to come to terms will degenerative illness or disability and being able to prepare for changes in ability reduces the impact on HRQoL, whereas disability acquired later in life through acute illness or injury does not allow for physical or emotional preparation. To some extent this indicates the importance of emotional wellbeing and facilitating disabled children to find ways to cope with disability.

7.6.5. Relevance of the EQ-5D-Y and HUI measures

Participants were asked to review the content validity of the EQ-5D-Y and HUI measures and to discuss the relevance of each question to them (or their child). The general consensus was that the domains of HRQoL were relevant to some extent. However, often the available options for each question were insufficient or the question wording was not sensitive enough for their abilities, particularly the mobility/ambulation domains. For instance, participants stated that although issues of ambulation, dexterity and impaired senses can impact HRQoL, these issues can essentially by negated by appropriate adaptations. Furthermore, all participants indicated that although mobility does impact HRQoL, mobility should not just be defined as walking. For the most part participant perceptions regarding the relevance of the measures was in-keeping with their previous definitions of QoL. However, Participants did tend to agree with domains on the measures which they had not themselves included when defining QoL. This may reflect the measures' specific focus on HRQoL and the participants' focus on global QoL. It also indicates that personal definition of QoL may not be all encompassing and thus more specific impacts on HRQoL can be ignored or forgotten.

Although the measures were considered generally relevant, the available options were not varied enough to capture the nuances of HRQoL for disabled people, therefore making completion difficult. This was particularly relevant for the EQ-5D-Y which has only three levels for each question. The simple nature of the EQ-5D-Y is intended to make it easy and quick to complete, but because of this simplicity it also becomes less suitable for people with abilities and functioning's which do not adhere to expected norms.

> 17 year old boy (S3-24): *I think as far as the five columns or categories are concerned, they kind of cover the broadest range but the actual three answers that you're given for each one kind of, I don't think, to me at least, it was difficult for me to fill it out. I find these things very difficult because you try and be extremely positive as possible about things, and there seem to be gaps almost where I fit. I fit in the gaps which is... I found it difficult to fill out just because you think "I don't fit here, or I don't fit here".*

7.6.6. QoL and age

A common theme running throughout the data was QoL needs change with age. For instance, parents of younger children placed much greater importance on health and function, while social interaction became more influential as children aged. This calls into question the appropriateness of generic

measures such as the EQ-5D-Y. The EQ-5D was not originally designed for use in children, and although the youth version has been validated in younger age groups, the domains and levels of the descriptive system were not designed around the testimony of children, let alone people with disabilities. These results reflect the findings in the previous chapter showing that the EQ-5D-Y may not be suitable for this population. A potential solution would be to develop value sets based on the preferences of children and young people, but this would not help to understand which domains of HRQoL should be included in a child-specific measure, or whether general population health state preferences are suitable for disabled populations. Child-specific measures, such as the HUI and CHU-9D would therefore be preferable, although additional research is needed verify their applicability for disabled children more generally.

7.6.7. HRQoL and fluctuating health status

Another important consideration is the appropriateness of generic measures in populations where health and QoL is likely to fluctuate or degenerate rapidly. For instance, if HRQoL fluctuates on a day-to-day basis due to changing functional abilities, or if health decreases rapidly in a short space of time, then basing HRQoL estimates on a single time point could cause validity and reliability issues. Likewise, if regular retests were used to counter this then the measure would need to be highly sensitive to change over time. The simple descriptive system used in the EQ-5D-Y may be insufficiently sensitive to detect change, particularly with regards to mobility. In this respect a patient-generated outcome measure might prove to be more efficient. For instance the My QuOL-T allows users to identify and prioritise aspects of their life or illness which affect their QoL, and then monitor these on a daily basis (Health Foundation, 2012). This measure was designed specifically for use by children and young people and has been applied effectively in paediatric palliative care. From a clinical perspective this is an innovative approach to measuring effect, however from a health economics perspective patient-generated outcomes are not generic enough for utility measurement. Each individual would have very different needs and priorities, thus making comparisons between people and groups would be difficult. Furthermore, it would not be possible to generate a generic health state preference for every possible scenario. Likewise, identifying preferences at the individual level would prove too time consuming.

7.6.8. Implications for future economics evaluations

The overall indication is that although the domains are potentially relevant, the available responses are not. The EQ-5D-Y was especially difficult to use in this disabled population. The other issue is in health

state preferences. For instance, the preference of an able-bodied person for a state of immobility is clearly going to be low. However, as stated by participants in this study and others (Bartonek et al, 2012; Burström et al, 2014), inability to walk does not automatically discount HRQoL. This is a key distinction to make when considering the use of general population value sets in disabled populations; their preferences will always be for a state of ability rather than disability. From the perspective of the medical model of health this is an entirely understandable and acceptable perspective. However, taking into account the social model of disability and the perspectives of disabled people, it is fundamentally wrong to apply an able-bodied perspective to a disabled phenomenon. It is also inappropriate to assume that all disabled people would achieve better HRQoL if they could achieve normal mobility functioning.

If value sets could be generated for specific sub-groups preference weights would potentially be more representative of that specific sub-group. But this would create a specific preference-based measure rather than a generic one. The comparability of results would thus become limited. This leaves somewhat of a dilemma as to how to value utility for the purpose of economic evaluations in young wheelchair users.

The gold standard measures recommended by NICE are potentially not fit for purpose in this group as subsequent utility scores would reflect a general population perception of HRQoL rather than the individual's. Although this would be representative of the wider societal view of the health states, it would not be representative of the population that was intended to be studied. A potential solution would be to forego utility measurement and to identify appropriate clinical effectiveness measures to facilitate cost-effectiveness analysis. However, measuring the myriad benefits of wheelchairs is difficult, and use of solely clinical outcomes would not truly capture the effects of wheelchair provision. Furthermore, utility is needed to calculate QALYs, which are key to NICE HTA guidance on commissioning and funding within the NHS. Perhaps the solution is to think beyond the QALY and clinical effectiveness.

7.6.9. Considering the capability approach

The capability approach states that evaluation of outcomes should consider actual ability to achieve valuable functioning (Robeyns, 2003). Under the Pareto principle interpersonal comparisons of utility are of little benefit and difficult to analyse, however by examining outcomes beyond utility, such as health or capability, it is possible to make meaningful interpersonal comparisons (Brouwer et al, 2008).

In the context of wheelchair provision the capability approach (rather than health maximisation) could potentially be a more favourable approach to measuring outcomes.

The capability approach stems from Sen's theory of capabilities (Sen, 1993), which states that with justice-related issues capabilities are the most relevant outcome or comparator (Nussbaum, 2003). If we consider healthcare and resource allocation to be an issue of equity then the capability approach is appropriate for use in health related research and health economics. One of the issues with utility is that minority groups and the socially repressed develop "adaptive preferences" which reflect their lower status in society, thus their preferences or utilities do not have equal status (Nussbaum, 2003). This approach reflects the central beliefs of the social model of disability that disability is a social issue rather than a personal pathology (Lang, 2007), and thus the capability approach may in fact account for issues of inequality faced by disabled people.

7.6.10. Capability and children

Biggeri et al (2004) proposed 14 central capabilities specific to children: Life and physical health, Love and care, mental well-being, bodily integrity and safety, social relations, participation, education, freedom from economic and non-economic exploitation, shelter and environment, leisure activities, respect, religion and identity, time-autonomy, and mobility. Interestingly, mobility as a capability was judged to only be relevant from age 11 upwards, furthermore mobility was not specifically defined as being able to walk (Biggeri et al, 2004). At present the capability approach lacks a strong theoretical foundation when applied to children and therefore has some limitations, but with further development it could become the central theoretical basis for measuring child wellbeing (Biggeri et al, 2004).

7.6.11. Capability and disability

The capability approach can also be used in disabled populations as a normative framework to evaluate wellbeing. Disability can be defined as an individual being deprived of opportunities due to impairment (Mitra, 2006). The benefit of this approach is that to some extent it views disability in the social context of the individual (Bakhshi and Trani, 2006). Disability can therefore be seen on two levels: capabilities and actual functioning. From this perspective potential disability impacts practical opportunities (capabilities), but actual disability is determined by the level of functional restriction caused by impairment (Mitra, 2006). This differentiation takes into account the difference between expected and experienced disability, for instance the difference between being immobile and using a wheelchair. Identifying disability as the difference between actual functioning and an individual's ideal capability

does not predefine disability and instead creates a personal continuum of capability (Trani et al, 2011). Trani et al (2011) defined 15 capabilities specific to disabled people: emotional relations, freedom of choice, physical integrity, communication, social participation, political participation, education and knowledge, work, mobility, leisure activities, choice of residence, religion, respect, self-care, and financial autonomy.

7.6.12. Mapping the emergent analytical themes on to the capability approach

Considering these important approaches to capability and wellbeing, a qualitative analysis framework was used to map the emergent themes of HRQoL from this study onto the central tenets of the child/disability capability approaches, the EQ-5D-Y/HUI domains of HRQoL, and additionally the conceptual attributes of the ICECAP-O capability measure (Grewal et al, 2006), see table 7.4. Although the ICECAP-O is designed for older adults, it was used as a proxy for the theoretical application of the capabilities approach in this setting as it allowed comparison with an applied use of the capability approach. The ICECAP-O proposes five conceptual attributes of capability relevant for older people: attachment (love and friendship), security (thinking about the future without concern), role (doing things that make you feel valued), enjoyment (enjoyment and pleasure), and control (independence) (Grewal et al, 2006). An adult ICECAP measure is also available (the ICECAP-A), however the ICECAP-O has a more extensive evidence base and was therefore deemed more appropriate for this analysis. It is important to clarify that the capability approach was not used in the qualitative interview analysis phase and did not form part of the *a priori* coding framework. This subsequent cross-examination of the themes is therefore independent of the previous framework analysis

Table 7.4: Comparison of analytical themes with different capability approaches and HRQoL measures

'Defining HRQoL' analytical themes	'Defining QoL' categories	Child capability tenets (Biggeri et al, 2004)	Disability capability tenets (Trani et al, 2011)	ICECAP-O conceptual attributes (Grewal et al, 2006)	EQ-5D-Y HRQoL Domains	HUI 2 and 3 HRQoL Domains
Participation and positive experiences	Activities/participation Happiness Independence Social	Participation; Leisure activities Love and care Time autonomy Social relations	Leisure activities - Freedom of choice Social participation	Enjoyment - Control Attachment; Enjoyment	Usual activities - - -	- - - -
Self-worth and feeling fulfilled	Achievement/fulfilment Being able to adapt Emotional wellbeing Equality Feeling normal	Education - Mental wellbeing Respect Respect	Education / knowledge - Emotional relations Respect Respect	Role Security - Security; Role; Equality Security	- - Anxiety/depression - -	- - Emotion - -
Health and functioning	Cognition Communication Health Mobility Pain Self-care	Life/physical health; Education - Life and physical health Mobility - -	Education / knowledge Communication - Mobility - Self-care	- - - - - -	- - - Mobility Pain/discomfort Self-care	Cognition Communication - Ambulation/mobility Pain Self-care
Others:		1. Bodily integrity and safety 2. Freedom from exploitation 3. Religion and identity 4. Shelter and environment	1. Physical integrity 2. Financial autonomy 3. Religion 4. Choice of residence 5. Political participation 6. Work			1. Senses 2. Dexterity

The analytical themes of defining HRQoL found in this study mapped well on to the capability conceptual attributes of the three approaches. The majority of the conceptual attributes had a parallel theme or category in this study. As was expected the 'health and functioning' theme did not map on to the ICECAP-O as this measure focuses on capabilities rather than facilitators to capabilities, for example being able to socialise rather than good health facilitating socialising. Conversely, the central tenets of the child and disability capability approaches did make reference to health and functioning. The child capability tenets that could not be mapped on to the themes from this study tended to focus on basic needs (such as shelter, safety, freedom from exploitation) and thus may have been taken for granted by the participants in this study. Furthermore, the adult-centric tenets of the disability capability approach (such as work and political participation) were not relevant to the child-specific themes found in this study.

Examining the evidence from the interviews highlights that participants tended to focus on actual functioning and capabilities rather than the underlying factors, such as appropriate wheelchair provision for example. This indicates that focusing on what can be achieved as a consequence of meeting basic needs may be beneficial. With regards to wheelchair users, moving away from setting a baseline level of ability (benchmarked against population norms) may help to focus on what the individual wants to achieve and is capable of achieving. In this sense the outcome is both compatible with individual and wider comparisons of HRQoL and capability.

Table 7.4 demonstrates that the EQ-5D-Y and HUI measures tend to focus on health and functioning, which highlights why these measures missed some of the important definitions QoL used by participants in this study. It was found that most participants discussed the activities and abilities which gave them happiness rather than discussing happiness and wellbeing as more abstract concepts. This illustrates that capabilities and achieved abilities were more important to the participants than the base level of ability. For instance, one young man discussed the importance of what he could achieve in relation to what he couldn't-

> 16 year old boy (S2-16): *It's like, everybody's got things they can do better than other people. It's like, they can walk better than I can but I can push a wheelchair better than you can. It's one of those things that, I've just always thought everybody's got things they're good at. Everybody's got things they're not very good at and I'm just not very good at walking. But I'm really good at pushing a wheelchair and things like that*

It should be noted that the child and disability capability approaches defined mobility as being able to move around freely, while the HRQoL measures specifically refer to optimal mobility in terms of walking. This differentiation is acutely important when examining the impact of mobility on the health, QoL and wellbeing of an individual with mobility impairment.

It is important to clarify that the conceptual attributes of the ICECAP-O were developed empirically and specifically with older people (Grewal et al, 2006), and thus their relevance to disabled children is limited. However, this novel conceptual mapping exercise indicates that the various conceptualisations of the capability approach can be appropriate for measuring outcomes from wheelchair interventions for disabled children. Additional research is required to explore exactly which conceptual attributes of capability are of greatest importance to young wheelchair users. The biggest barrier to using the capability approach in this population is the current lack of a capability measure validated or designed specifically for children. At present there are no suitable capability measures that could be used in this specific setting, and thus this would be the first essential step towards developing a capability measure that is suitable for disabled children.

7.6.13. Implications for conceptual framework

Much like the previous chapter, the results presented in this chapter relate to the issue of appropriate outcome measures in wheelchair provision for children. The results in this and the previous chapter demonstrate that using existing HRQoL outcome measures may have limitations, as the needs of disabled children are not reflected in the preferences of the general public. It is first important that additional child-specific measures (such as the CHU-9D) are tested, as these must be validated for use in disabled children. This requires additional research to understand the applicability of measures like the HUI2 and the CHU-9D in larger disabled populations. The capability approach offers an alternative to HRQoL measurement, but cannot be used for QALY calculations and currently there are no validated measures available for children.

The findings presented in this chapter highlight the need to involve service-users in service development, as illustrated by the concepts of the conceptual framework. The focus on independence, socialising and positive activities demonstrates that services need to look beyond clinical outcomes and address the specific social and lifestyle needs of disabled children as well. This relates to the need for appropriate outcome measures which take into account the psychosocial needs of service users. Furthermore, environmental barriers to wheelchair use need to be addressed in order to facilitate

involvement in activities and social interaction. The conceptual framework highlights that health needs are important, but should not be seen as the only outcomes in wheelchair provision. Appropriate HRQoL outcome measures could bridge this gap but, as demonstrated in this chapter, existing measures such as the EQ-5D-Y and HUI may lack applicability for disabled children. Therefore, additional research is needed to adapt or develop measures which reflect the views of disabled children.

7.6.14. Study limitations

In the interest of transparency I have considered the limitations of the study. Only a small proportion (14.3%) of participants were children taking part on their own. It could therefore be argued that the results capture the voice of the parents more than the children. Furthermore, 25% of interviews were conducted with the parent and child present at the same time, which may have influenced both parent and child answers. Most participating children were aged 16 or over (72.7%) and thus this study potentially misses the perspectives of younger wheelchair users. There were inherent problems with including young children in the interviews due to parental concern, child ability and communication skills. Future research would benefit from focusing on younger children and utilising a greater array of child friendly research methods to elicit responses.

The demographic characteristics of the sample should also be taken into account, as there was a distinct lack of ethnic diversity (100% of participating children were white-British). Furthermore most children had a diagnosis of cerebral palsy, thus the results may not be representative of all other disabilities. This raises questions as to the generalisability of the subsequent findings. It would have been beneficial to include additional outcome measures in the interview schedule so that comparisons could have been made between generic and disease-specific measures. However, as the focus was on utility measurement it was not deemed necessary to expand the scope of the interviews in this way.

Considering the results from this chapter critically, it is important to address issues of transferability, credibility, confirmability and dependability (Hannes, 2011). Due to the study demographics and the relatively small sample size it is difficult to judge whether these results are transferable to other comparative settings. I have provided a clear and thorough breakdown of participant demographics to give specific context for these results. The analytical themes generated from the interviews are reflective of the opinions expressed by participants, highlighted by the illustrative quotes throughout this chapter. I became immersed in the data throughout the process of data collection and analysis, and used supervisory research team meetings as a means to test my understanding and interpretation of the

data. Furthermore, I reviewed my interview schedule after each interview so that I could be reflexive to new and emerging themes. I therefore believe that the findings presented in this chapter are a true representation of what the participants discussed and provides dependable insight. Due to time constraints participants were not involved in analysis and did not have the opportunity to verify transcripts, which potentially may have affected the credibility of the findings. However, all interviews were transcribed verbatim and double-checked for errors prior to formal analysis. The use of qualitative framework analysis allowed for a transparent and flexible approach to the data analysis, providing a clear pathway as to how conclusions were made based on the available data.

7.7. Conclusion

Disabled children and their parents define QoL in relation to wheelchair use through three distinct but interrelated concepts: participation and positive experiences; self-worth and feeling fulfilled; and health and functioning. Children and their parents showed general consensus when defining QoL, however parents of younger children placed greater emphasis on health and functioning. For the purpose of economic evaluation it appears that the EQ-5D-Y in particular may not be appropriate to elicit reliable utility data from disabled children. Future economic research in this field must consider the role of capabilities in disabled children as this has interesting implications for applying health economics in this population.

At present there are no capability measures made specifically for children, furthermore the concept of capability in children is difficult to define fully and requires extensive research to truly understand. Further research is needed to develop a child-specific capability measure and to test subsequent relevance to disabled children. For the time being cost-effectiveness or cost-utility analysis would therefore be a favourable approach to economic evaluation, but identifying appropriate measures of effect is also a difficult task. Child-specific preference-based utility measures may be appropriate in this population and should be used as a basis for cost-utility analysis. This requires additional research to understand the applicability of measures such as the CHU-9D in disabled populations, and potentially the development of value sets specific to wheelchair users. The HUI2 is a suitable interim measure to test methods of economic evaluation in this specific setting, but potentially lacks validity and reliability for a full economic analysis to inform NICE guidance.

Chapter Eight: Discussion and conclusions

8.1. Chapter summary

This thesis presents one of the first applications of health economic methods and approaches to wheelchair interventions for disabled children. In this discussion chapter I summarise the findings from the previous chapters and draw synthesised conclusions using an adapted thematic synthesis technique (Oliver et al, 2005). I discuss implications for the commissioning of wheelchair services and future research in this field, and I relate the conclusions to the conceptual framework developed in chapter three. In the interest of reducing repetition, a breakdown of thesis findings by original research objectives is presented in appendix J.

8.2. Summary of findings

In the third chapter of this thesis I presented the findings from a systematic review of wheelchair effectiveness, service user perspectives, policy and economic evidence. The findings were synthesised to develop a conceptual framework for optimal wheelchair service provision for children, based on the development of cost-effective services. The conceptual framework was used as an interpretative lens for thesis and as such I believe that the findings in each chapter offer novel insights into conducting economic evaluations of wheelchair interventions and in disabled populations more generally. In section 8.3 I will revisit the conceptual framework in relation to the thesis findings.

Using a mixed-method systematic review technique proved to be an excellent means to understand the wider context of the research problem and existing literature. I found that UK policy and NFPO recommendations were reflective of the perspectives of young wheelchair users and their families, but that there was a lack of effective translation of policy and evidence into practice. This was evidenced by common issues arising in several reports spanning several years, for instance the need for joined-up working between NHS, social services and education authorities in wheelchair provision (Audit Commission, 2002; DoH, 2004; Prime Minister's Strategy Unit, 2005; Welsh Assembly Government, 2005; Care Services Improvement Partnership, 2006; Scottish Executive, 2006; DoH Commissioning Team, 2010; National assembly of Wales, 2010).

I found little high-quality effectiveness evidence regarding wheelchairs for disabled children and practically no robust cost-effectiveness evidence within this field. Although evidence of effect was found, it was of generally low quality and thus the findings had to be assessed accordingly. Only one RCT was found in the intervention evidence searches (Jones et al, 2012). The lack of high-quality research

highlights the issues of measuring outcomes associated directly with wheelchair interventions for disabled children, and the ethical issues of conducting paediatric and disability research. Despite the lack of high quality evidence, a number of beneficial effects of appropriate wheelchair interventions were found, including improved social and play skills (Furumasu et al, 2008; Tefft et al, 2011), functional mobility (Jones et al, 2003; Tefft et al, 2011; Jones et al, 2012) and developmental gains (Bottos et al, 2001; Jones et al, 2003; Jones et al, 2012).

One of the most important findings from the systematic review was that for children and young people wheelchairs offer more than mobility, including a wide range of clinical, social and developmental benefits. The holistic benefits of wheelchairs are therefore of key importance when assessing needs and outcomes (DoH Commissioning Team, 2010; National Assembly for Wales 2010*)*. This was reiterated in the DCE findings of chapter five, as participants showed significant preference for wheelchair services which assessed social and educational needs alongside clinical needs. The aim of the DCE was twofold; firstly to see if DCEs could be completed accurately by young wheelchair users and secondly to understand how wheelchair service users and their parents prioritise different attributes of wheelchair services. de Bekker-Grob et al (2010) and Cunningham et al (2011) demonstrate that DCEs can be used effectively to elicit the preferences of children, but to date few published DCEs have studied the views of children and no published DCEs have specifically sampled disabled children. The United Nations states that disabled children have the right to express their views on all matters which affect their lives, including health services (Schulze, 2010), likewise the Children's Act (2004) states that children should be involved in the development of services and processes designed to support and care for them. In order to focus on the views and opinions of children it is imperative that children are included in research (Kyronlampi-Kylmanen & Maatta, 2011). The inclusion of children in this research is one of the major strengths of the thesis overall.

The results of the DCE indicated that for parents and children comprehensiveness of wheelchair assessment was the most important attribute of wheelchair services, followed by wheelchair delivery time. Interestingly, the level of training provided and frequency of wheelchair reviews did not impact service preference. As this was a pilot, the sample size was too small to make generalisable conclusions. The importance of this study was in determining that DCE methods can be conducted appropriately in this population. Future research utilising a larger sample could consider the impact of age, condition, socioeconomic status and type of wheelchair on preferences for service attributes.

Although DCE data can help to understand service user preferences, a range of evidence is needed to fully address the issues of wheelchair services, including evidence of effectiveness and cost-effectiveness. Furthermore, an appropriate knowledge translation framework may be required to implement change across wheelchair services in an evidence-based way. The Knowledge to Action Process framework (Graham and Tetroe, 2009) and the NHS Institute for Innovation and Improvement 'Spread and Adoption' tool (2012) offer potential approaches for translating knowledge into practice, however more evidence is needed in this topic area first. Building a knowledge translation framework was beyond the scope of this thesis, however the conceptual framework offers a good starting point for identifying gaps in knowledge and research priorities.

The initial research priorities identified in the systematic review included developing appropriate outcome measures, applying health economic methods and exploring the use of HRQoL as part of cost-effectiveness analysis in this setting. The incorporation of generic preference-based measures into routine data collection would facilitate economic evaluation of wheelchair services in line with NICE recommendations (NICE, 2013). The economic concept of utility as an outcome measure showed potential in this setting due to preferences for health states being accounted for. However, the application of standard HRQoL outcome measures, such as the EQ-5D-Y and HUI, in this population was limited in the literature and necessitated further investigation as part of this thesis.

QALYs are an integral part of making comparisons across interventions and one of the biggest influences on NICE decisions (Dakin et al, 2014). In order to conduct cost-utility analysis for the purpose of QALY calculation, preference-based measures are needed to value health states (Stevens and Palfreyman. 2012), thus their applicability in specific settings needs to be understood. In chapters six and seven I examined the usefulness of generic, preference-based HRQoL measures for utility measurement in disabled children.

In chapter six I found that VAS scores were highest for both children and parents, while HUI3 and EQ-5D-Y scores were lowest respectively. In agreement with previous research (Varni et al, 2005b; Bray et al, 2010), disabled children scored their HRQoL higher than their parents on all measures, with only the VAS exhibiting a non-significant difference between children and proxies. Interestingly, child and proxy results showed generally good correlation in terms of convergent validity despite significant differences. This illustrated the potential issue of using correlation in this manner, as correlation is an indication of association rather than agreement (Bland and Altman, 1986). Using Bland-Altman plots I found that most child and parent equivalent measures were in fact in agreement; only the EQ-5D-Y showed

insufficient agreement. Conversely, most measures within the two groups did not show sufficient agreement, highlighting the potential differences between HRQoL measures with different descriptive systems and health state valuations. This indicated that equivalent self-reported and proxy measures were suitably related (accounting for significant differences), while different measures within groups were not, therefore the EQ-5D-Y and HUI measures were not in agreement about HRQoL in this cohort.

Although the HUI2 showed some potential for measuring utility in this group, overall it was difficult to conclude that any of the measures were appropriate, particularly the EQ-5D-Y. The VAS scores were drastically different to the HRQoL measures, thus indicating disparity between how the participants rated their own health state and how the measures rated their HRQoL. The VAS acts as a good comparator for more complex preference-based measures as it allows comparison between societal and personal valuation of health states (Krabbe and Weijnen, 2003). Overall, the results from chapter six call into question the potential use of these measures for the purpose of economic evaluation in this setting. I conclude that additional research is needed to test the applicability of additional child-specific measures such as the CHU-9D. At present the results indicate that the HUI2 could be used as an interim outcome measure, but may be unsuitable for full economic analyses of wheelchair interventions.

In order to further investigate the use of HRQoL measurement in this setting, I used qualitative methods to examine how disabled children and their parents define QoL in relation to wheelchair use. Qualitative methods in health economics can provide many benefits, such as observing a societal issue from an individual perspective (Coast et al, 2004). The aim was to determine whether HRQoL measures are an appropriate tool for measuring outcomes of wheelchair interventions by examining how QoL is impacted by wheelchair use at a personal level. In order to do so I wanted to examine how disabled children and their parents defined QoL and to ascertain their perspectives on the relevance of the EQ-5D-Y and HUI measures.

Participants defined QoL in relation to wheelchair use through three distinct but interrelated concepts: participation and positive experiences; self-worth and feeling fulfilled; and health and functioning. Corroborating aspects of this, a number of previous studies have identified the social (Home & Ham, 2003; Wiart et al, 2003; Wiart et al, 2004; Lawlor et al, 2006; Evans et al, 2007; Furumasu et al, 2008), independence (Bottos et al, 2001; Home & Ham, 2003; Wiart et al, 2004; Lawlor et al, 2006), functional (Jones et al 2003, Benedict et al, 1999), developmental (Jones et al, 2003; Jones et al, 2012) and participatory (Home & Ham, 2003; Wiart et al, 2003; Wiart et al, 2004; Lawlor et al, 2006; Evans et al, 2007) benefits of appropriate wheelchair interventions. The importance of self-worth and fulfilment was

novel, as this theme had not been explicitly discussed in the existing effectiveness or qualitative literature, although related topics such as self-confidence (Home & Ham, 2003; Wiart et al, 2003) and dignity (Wiart et al, 2004) were previously identified.

It was interesting to find a good degree of consensus between child and parent responses, particularly considering the significant differences between child and proxy HRQoL reporting in chapter six. The qualitative findings relate to the acceptable levels of agreement and construct validity found between child and proxy measures, indicating relatively good consensus in terms of how participants completed the measures, even if subsequent HRQoL ratings were significantly different. Participants stated that both the EQ-5D-Y and HUI measures were reflective of how they defined health and QoL, but that they lacked sensitivity and thus would not be wholly appropriate in this population. As an example, some participants indicated that walking would have a worse impact on their HRQoL than wheelchair use due to associated pain and limited functional mobility. These nuances are difficult to pick-up using generic measures.

The descriptive systems of the measures did not accommodate the abilities and functioning of disabled children, for instance optimal mobility is only defined as 'walking' on the EQ-5D-Y. Research by Bartonek et al (2012) and Burström et al (2014) found that people with mobility impairments did not consider mobility to significantly affect their HRQoL, yet due to limited descriptive systems and normative value sets these measures heavily discount HRQoL due to mobility impairment. Again, this is reflective of the findings from chapter six and the appropriateness of the measures. In this population, definitions of HRQoL need to consider the potential benefits of assistive technology and the role assistive technology can have in enhancing HRQoL, even if functioning remains outside of what would be considered normal. Many participants stated that with appropriate wheelchairs and other assistive technology they could reach a HRQoL on par with an able-bodied child.

Considering the evidence from chapters six and seven, for the purpose of economic evaluation it appears that the EQ-5D-Y in particular is not appropriate to elicit reliable utility scores for disabled children. The HUI measures also have limitations. Generic, preference-based measures appear to lack sensitivity in this population. The valuation of disabled health states by the general population demonstrates a medical model of health economics, which is directly at odds with the social model of disability. The capability approach provides a potential alternative to utility measurement for the purpose of economic evaluation. However, at present there are no capability measures specifically for children, furthermore the concept of capability in children is difficult to define (Biggeri et al, 2004). The

key research imperative is to firstly understand what capability is in children, and secondly to develop a generic capability measure that could be used with children for the purpose of economic evaluation. As capability is an essentially generic concept it would allow comparisons across interventions. Other child-specific HRQoL measures also need to be tested in this population, and additional value sets or amended descriptive systems may be needed to improve applicability. Due to the differentiation of functioning and capability, capability approach offers the best alternative in this unique setting.

As well as examining some of the issues of outcome measurement for the purpose of economic evaluation in this population, I conducted a case-study of costing wheelchair provision in chapter four to find out how best to cost wheelchair interventions for the purpose of economic evaluation. By better understanding these two key aspects of economic evaluation, my aim was to inform future cost-effectiveness analysis in this field.

Costing wheelchair interventions requires a number of factors to be considered. It was particularly interesting to observe the relatively high cost of customisation associated with the supply of a wheelchair, which accounted for almost 19% of capital costs in wheelchair provision. This reflects the vast differences in each wheelchair intervention and the need to make precise judgements about associated costs. Seating, posture and customisation needs can vary greatly due to condition and severity (Lau et al 2008), all of which have financial implications. It is therefore not possible to make broad judgements about wheelchair costs. For instance, even with the supply of a standard MWC the variance in costs between patients can be high due to fluctuations in individual needs. Furthermore, the expected life of each wheelchair can impact costs, as recycled wheelchairs are likely to cost less in the long term. The costing of wheelchair intervention therefore requires a close attention to detail in order to capture all associated costs. This is imperative in order to fully account for the cost implications of wheelchair provision. Specific attention should be paid to the cost of customisation, the number of times the wheelchair is recycled and the associated refurbishment and repair costs. Additional micro-costing in this setting would be beneficial.

8.3. Revisiting the conceptual framework:

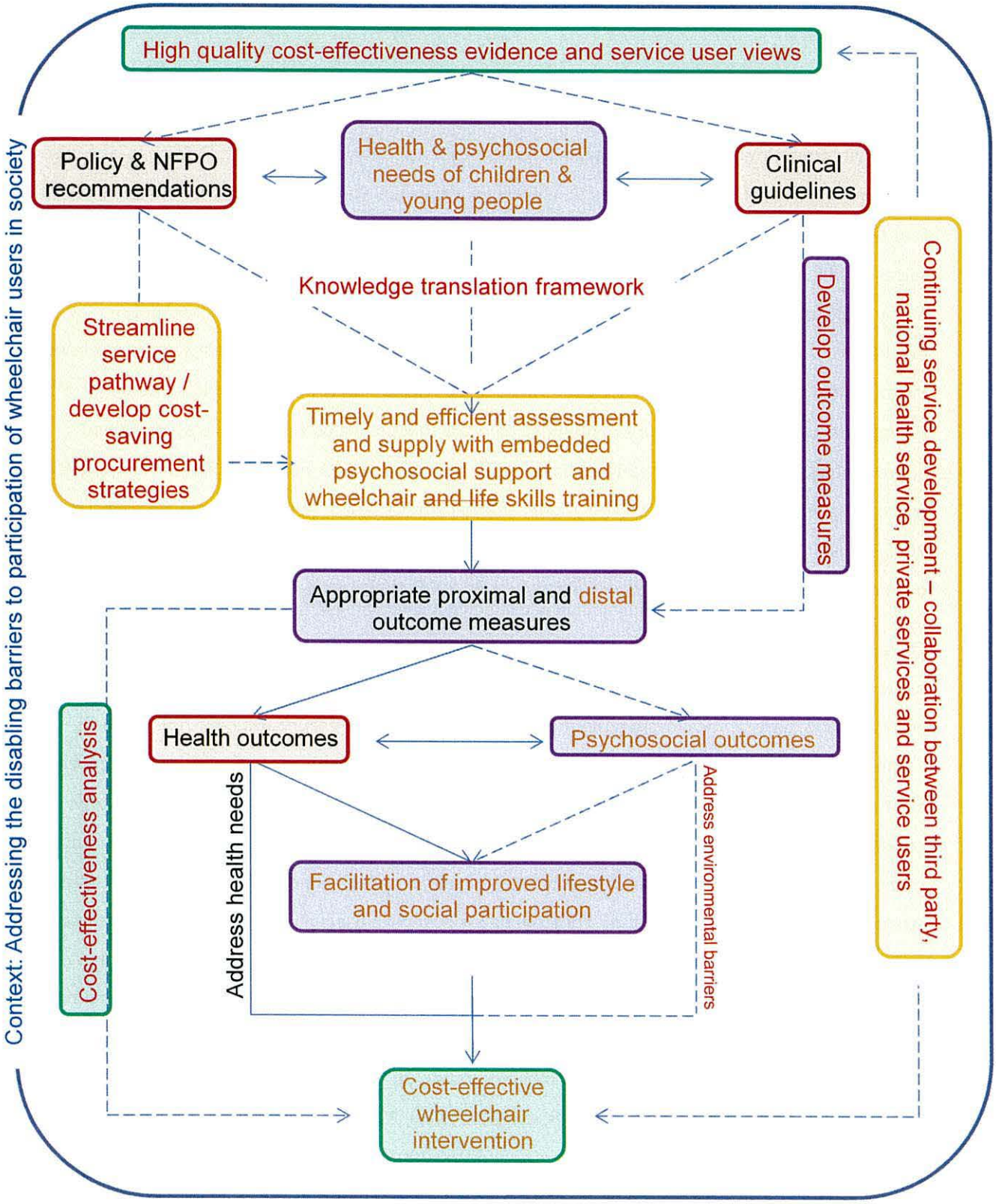
The novel conceptual framework presented in chapter three, and developed throughout the thesis, provides a structured and interpretive lens for the development and analysis of cost-effective wheelchair services. In developing the conceptual framework, my aim was to highlight stages for development and the research priorities needed to facilitate better outcomes for disabled children and

more efficient services. In light of the findings presented in this thesis, the conceptual framework provides a thorough account of where research should be targeted to improve wheelchair services and promote better analysis of outcomes for disabled children in relation to wheelchair use. The studies reported in this thesis address some of these issues and set an agenda for future research and development. The conceptual framework was developed to identify areas which require actions and stages of development. The key areas for action can be grouped into three key concepts:

- Measuring cost-effectiveness
    - High quality evidence of effectiveness
    - Cost-effectiveness analysis; Cost-effective wheelchair interventions
- Developing and utilising appropriate outcome measures
    - Health & psychosocial needs of children & young people; psychosocial outcomes
    - Develop outcome measures; Appropriate proximal and distal outcome measures
    - Facilitation of improved lifestyle and social participation
- Service development and cost-savings
    - Streamline service pathway / develop cost-saving procurement strategies
    - Timely and efficient assessment and supply with embedded psychosocial support and wheelchair and life skills training
    - Continuing service development – collaboration between third party, national health service, private services and service users

The 'measuring cost-effectiveness' and 'developing and utilising appropriate outcome measures' concepts are interrelated, as appropriate measures are required to measure effect, and consequently cost-effectiveness. I therefore acknowledge their relationship as distinct but related concepts in the conceptual framework.

As this PhD was aimed at the application of health economics in this area of research, the findings do not map against all issues identified in the conceptual framework; specifically, exploring environmental barriers and developing a knowledge translation framework were beyond the remit of this PhD. However the findings do have relevance for the other key areas of development, which are illustrated in figure 8.1. Policy/NFPO recommendations, clinical guidelines and health outcomes were all identified in the systematic review; although they are stages of the conceptual framework they do not require additional research as a priority. I have therefore focussed on the three key areas outlined above.



Key

Framework stages	---------->	Area for development	Relevant evidence from thesis
Required actions	———>	Direction of progression	Chapter 3
Development stages	<———>	Interaction	Chapters 4, 5 & 7
Overarching context			Chapters 4, 6 & 7
			Chapters 5, 6 & 7

Figure 8.1: Revisited conceptual framework for developing cost-effective wheelchair services for disabled children

8.3.1. Revisiting the conceptual framework: Service development and cost-savings

The results from three chapters map against issues of service development and cost-savings (see yellow highlighted boxes in figure 8.1 for specific aspects of conceptual framework). The results from chapter four demonstrate the importance of maintenance and recyclability of wheelchairs. A potential approach to cost-saving could be the development of additional maintenance procedures for supplied wheelchairs and wheelchair stock in order to improve the function and condition of equipment, and potentially extend the useable life of each wheelchair. I estimate potential NHS wheelchair cost savings of between 9% and 14% if at least half of all wheelchairs are reused. This can only be achieved if adequate maintenance is provided to ensure that wheelchairs are functioning correctly and are maintained appropriately. A number of publications have highlighted the need for better and faster maintenance procedures in wheelchair services (NHS Modernisation Agency, 2005; Prime Minister's Strategy Unit, 2005; Welsh Assembly Government, 2005; Muscular Dystrophy Campaign, 2010;), and the need to promote recyclability (Prime Minister's Strategy Unit, 2005; Welsh Assembly Government, 2005).

Consultation with service users is necessary to ensure that all NHS wheelchair services meet the needs of service users, and has been recommended by a number of government and NFPO reports (Barnardos and WK, 2006; Care Service Improvement Partnership, 2006; HM Treasury and DES, 2007; DoH Commissioning Team, 2010). Chapter five demonstrates that DCE methods could be used effectively to elicit service user preferences for different arrangements of wheelchair services. If done on a large enough scale such results could be used to guide national developments to wheelchair services based on service user opinions. Furthermore, the attributes of services which are the least preferential could be streamlined to reduce costs whilst meeting the needs of service users. For example, the results from chapter five demonstrate that additional training procedures, including life skills, could be limited due to service user preferences for basic wheelchair skills training. The results also substantiate the need for holistic assessment of needs (DoH, 2004; Care Services Improvement Partnership, 2006; DoH Commissioning Team, 2010; National Assembly for Wales, 2010; Muscular Dystrophy Campaign, 2011) and improved waiting times (Audit Commission, 2002; DoH, 2004; Prime Minister's Strategy Unit, 2005; Welsh Assembly Government, 2005; Barnardos and WK, 2006; HM Treasury and DES, 2007; DoH Commissioning Team, 2010; National Assembly for Wales, 2010). Although the sample is too small to make definitive judgements about service preferences, the results indicate that the DCE method could be used effectively to gather this data and subsequently develop and streamline services.

The findings presented in chapter seven reiterate the DCE results, as participants focused on independence, socialising, participation and self-wroth when defining QoL in relation to mobility impairment and wheelchair use. There is clearly a need to involve service-users in decisions about outcomes and effectiveness. Services need to look beyond clinical outcomes and also address the specific social and lifestyle needs of disabled children. Evidence demonstrates that wheelchair can have a number of significant beneficial effects on social and play skill (Furumasu et al, 2008; Tefft et al, 2011), and that appropriate wheelchair interventions can improve independence (Bottos et al, 2001; Home and Ham, 2003; Wiart et al, 2004; Lawlor et al, 2006) and reduce need for assistance (Benedict et al, 1999; Lawlor et al, 2006).

8.3.2. Revisiting the conceptual framework: Measuring cost-effectiveness

The results from three chapters map against issues relating to the need for high quality evidence of effectiveness and cost-effectiveness, and the application of cost-effectiveness analysis techniques (see green highlighted boxes in figure 8.1 for specific aspects of conceptual framework). In chapter four I presented a case study of costing wheelchair interventions for children. The process of costing the interventions was particularly important for this area of development, as it demonstrated the need for close attention to customisation costs, maintenance and recycling procedures. The need to collate accurate cost data is paramount for robust and reliable cost-effectiveness data (Mogyorosy and Smith, 2005). Chapter four could be used as a guide for costing wheelchair interventions for the purpose of larger scale cost-effectiveness analyses in this area.

The results from chapter four also show the importance of differentiating types of wheelchairs in future economic analyses, as the cost of chairs, customisation and staff time varied greatly by wheelchair type. For instance, total average costs between different types of NHS MWCs varied by up to £1000. The provider of the wheelchair is also relevant, as the charity funded wheelchairs tended to be higher in cost; in some cases double the cost of equivalent types of NHS equipment. Future economic evaluations could examine the relative cost-effectiveness of comparable wheelchairs between NHS and charity or private wheelchair providers to see if additional expenditure on more expensive equipment and customisation improves outcomes in a cost-effective manner.

The results from chapters six and seven provide guidance on the application of standard measures of effectiveness for the purpose of economic evaluation, to be precise HRQoL outcome measures for the purpose of QALY calculations. Cost-effectiveness evidence is still the single biggest influence on NICE

decisions (Dakin et al, 2014), and NICE specifically recommend the QALY as primary outcome measure is cost-effectiveness analyses (NICE 2013). Therefore, in order for evidence to have the most influence on technical guidance and subsequent funding allocation, QALY data is required. At the least, another form of robust cost-effectiveness evidence is required if QALYs cannot be calculated.

In chapter six analysis of generic HRQoL measures showed significant differences between the EQ-5D-Y, EQ-VAS and HUI measures, meaning that they were potentially measuring and valuing HRQoL in different ways. These results indicate that generic preference-based HRQoL measures may lack applicability in this population group due to descriptive systems which do not account for the abilities of disabled children and utility weights which are too grounded in normal functioning to be applied to people with disabilities. This was further verified in chapter seven, where participants discussed the inadequacies of the descriptive systems of the EQ-5D-Y and HUI measures when applied to disabled children.

The key priority is the development of valid and representative outcome measures which reflect the needs of disabled children, and which can be used for cost-effectiveness analysis. Ideally these should be preference-based and comparable to standard measures such as the EQ-5D-Y so that QALYs can be calculated. At present NICE recommended measures, such as the EQ-5D (NICE, 2013), may be too focussed on normal functioning to account for the interplay between HRQoL and disability. The EQ-5D-Y in particular lacked applicability in this cohort, while the HUI2 showed some potential. However, additional research is needed to test these measures, and other child-specific measures such as the CHU-9D, in larger samples of disabled children. HRQoL may be impacted by disability (Varni et al, 2007; Dobhal et al, 2013), but existing generic preference-based measures are not sufficiently designed to elicit accurate HRQoL data for disabled children, and thus accurate and robust cost-effective evidence cannot be generated using current generic measures.

I advocate the testing of child-specific preference-based HRQoL measures in future economic evaluations to assess their applicability. If the applicability of these measures cannot be adequately established then alternative methods of effectiveness measurement are required. The capability approach could offer an alternative, although the application of this approach in children is currently limited due to a lack of appropriate measures. Therefore, in the short-term I propose that cost-effectiveness analysis should be conducted using disability and child specific QoL and HRQoL measures in order to build a pool of economic evidence relating to wheelchair interventions for children. In the long-term, preference-based HRQoL and capability measures with descriptive systems relevant to

disabled children need to be developed, and value sets relevant to this population produced for existing generic measures. These could be used across services for disabled children and adults for economic analyses of a range of different interventions.

Another important consideration for cost-effectiveness analyses in this setting is the use of proxy data. In chapter six I found that parents reported their child's HRQoL to be significantly lower than the child when self-reporting; reiterating issues of using proxy data for disabled children reported in previous literature (Varni et al, 2005b; Bray et al, 2010). However, construct validity and agreement between children and proxies was acceptable. This indicates that proxy data could be used appropriately in this population when children are unable to self-report. Significant differences between parent proxy and child reports would need to be accounted for and possibly weighted accordingly; understanding the relationship between proxy and self-reported data is essential. Eiser and Moore (2001) state that there needs to be a better understanding of how children interpret QoL questions and how this differs between children and adults, and between children of different ages. At present few measures are targeted at specific child age groups, although QoL is not a static concept in childhood (Clarke and Eiser, 2004).

The relationship between child and parent results can be complex; Upton et al (2008) found that parents of healthy children overestimated their child's HRQoL while parents of children with health conditions underestimated their HRQoL. Therefore more research is needed to understand the relationship between child and proxy reporting, and also to determine an age at which a child is determined to be a reliable respondent (Matza et al, 2004). I believe that the results from chapter seven offer some insight into these issues in disabled children.

8.3.3. Revisiting the conceptual framework: Developing & utilising appropriate outcome measures

The results from three chapters address the issues of developing and utilising appropriate outcome measures (see purple highlighted boxes in figure 8.1 for specific aspects of conceptual framework). The results of the DCE presented in chapter five indicate that holistic assessments of need are a key priority for service users, and thus outcomes beyond health must also be considered, for instance QoL (NHS Modernisation Agency, 2005), psychosocial needs (DoH, 2004) and independence (Care Services Improvement Partnership, 2006). This also relates to service development, as services need to focus on outcomes beyond health when assessing the wheelchair and mobility needs of disabled children (DoH Commissioning Team, 2010; National Assembly for Wales, 2010). The negative emotional impacts of

disability could be identified and treated if HRQoL issues were addressed in the management and treatment of children with disabilities such as cerebral palsy (Vogels et al, 1998). Therefore, a balance is needed between the clinical needs of disabled children and the wider benefits of appropriate wheelchair interventions. In order to do so appropriate outcome measures are needed, thus the applicability of existing measures needs to be tested or new measures developed.

Health, posture, functional abilities and other clinical outcomes should still be considered an important part of wheelchair assessment and provision. Alongside clinical outcomes, other important outcomes should be prioritised, particularly outcomes which reflect the desires of disabled children. This requires focus on independence, participation and social interaction according to the results presented in chapter seven. Consideration of age and how outcomes and preferences change over time is also important, as definitions of QoL varied by age group to some extent.

Previous literature found that disability has a detrimental impact on the HRQoL of children. For instance, Varni et al (2005a) found that of the chronic conditions they evaluated, cerebral palsy had the biggest impact on HRQoL of children. Research by Vargus-Adams (2005) reiterated this and found that the physical function domains of HRQoL were particularly affected, likewise Dobhal et al (2013) found that cerebral palsy mainly impacted child HRQoL in the domains of physical independence, mobility and social integration. The evidence for other conditions and disabilities is limited; Grootenhuis et al (2007) reported one of the first studies to describe the HRQoL of people with muscular dystrophy, and found that the HRQoL of children and adults with muscular dystrophy was significantly worse than healthy controls. However, existing qualitative literature also shows that appropriate wheelchair provision can improve QoL (Home and Ham, 2003; Tefft et al, 2011), and adult EQ-5D data (with an appropriately adapted 'mobility' domain for wheelchair users) showed a significant improvement in adult HRQoL after PWC provision (Davies et al, 2003).

The findings presented in chapter six show large differences on all measures between child population norms scores and the disabled children participating in this study. Analysis of these scores in chapter six raised concerns about the appropriateness of generic measures, particularly the EQ-5D-Y, for eliciting accurate HRQoL data in this population. The qualitative findings in chapter seven confirmed that the descriptive systems of the EQ-5D-Y and HUI measures lacked applicability and sensitivity for disabled children. The development of appropriate child HRQoL measures lags behind adult measures due to the challenges of designing appropriate descriptive systems and taking account of developmental changes in childhood (Bjornson and McLaughlin, 2001).

Existing HRQoL outcome measures may therefore have limited applicability in this population, as disabled children's understanding of HRQoL in relation to wheelchair use is not represented in standard descriptive systems. As previously stated, the first step is to test other child-specific measures such as the CHU-9D, as these must be validated for use in disabled children. As the CHU-9D doesn't use mobility as a dimension for HRQoL (Canaway and Frew, 2013) it may be useful in this setting, as many participants indicated that the mobility dimensions of the EQ-5D-Y and HUI measures were particularly problematic. Larger sample sizes are needed to gain a broader picture of the applicability of these measures. Alternative methods, such as the capability approach, should also be considered.

The results presented in chapter seven demonstrate that psychosocial outcomes are particularly important to disabled children and their parents; independence, social interaction, participation and self-worth were perceived as the key indicators for QoL in relation to wheelchair use. Therefore, wheelchair services need to ensure that assessments of needs and outcomes take account of the clinical, social, independence, emotional and environmental needs of disabled children. As stated previously, the capability approach may therefore provide an appropriate means of assessing outcomes if a suitable measure become available. Mapping the emergent QoL analytical themes on to various definitions of capability (Biggeri et al, 2004; Grewal et al, 2006; Trani et al, 2011) showed congruence between the qualitative findings and the tenets of capability.

Research priorities should focus on developing a child-specific capability measure, as well as an appropriate HRQoL measure/value set. Return on investment analysis may also have some benefits in the short term while appropriate measures are being developed, as illustrated by Frontier Economics (2011) who successfully used social return on investment to evaluate a new approach to wheelchair service management. The QALY is perhaps too narrow to be used appropriately in this population, however additional research is needed to make definitive statements about the applicability of the QALY framework in this setting.

8.4. Synthesis of evidence from each chapter

In order to understand the overall context and develop final conclusions from this thesis, I conducted a final over-arching synthesis of the findings from each chapter. I used the same method of qualitative framework synthesis (Oliver et al, 2005) employed in the chapter three over-arching synthesis, as it proved to be a useful method for drawing together different sources of evidence. To facilitate this synthesis I considered the overarching aims of the thesis, which were to explore the application of

health economics to wheelchair interventions for disabled children, and specifically to understand how best to apply methods of economic evaluation to the assessment of paediatric wheelchair intervention cost-effectiveness. During the process of the thesis two key areas of interest became clear: wheelchair service development and outcome measures for the purpose of economic evaluation of wheelchair services/interventions. The synthesis was therefore focussed on addressing the overarching aim of the thesis in the context of service development and applying health economics in this field. The overarching synthesis is presented in table 8.1. A number of key findings were elicited from the synthesis:

A. A holistic approach to wheelchair assessment is required, which factors in a range of concepts, including clinical, social, developmental, independence and educational needs. Assessments should take account of changing QoL with age, and thus tailor interventions to the needs of each child rather than restriction through eligibility criteria. For instance, children under the age of 5 should not be prohibited from accessing appropriate powered mobility solely because of their age. Furthermore, PWCs for children under the age of 5, like the Wizzybug, are not necessarily prohibitively expensive. Additional cost-effectiveness evidence could build the case for routine provision of PWCs to younger children.
B. In the analysis of wheelchair interventions and services, a wide range of outcomes beyond clinical effect should be embedded in wheelchair services. Specifically, independence, self-worth and social interaction were highlighted by both the systematic review and qualitative interview data. Outcomes should reflect what is important to disabled children, and not be unduly guided by the expectations of the general population.
C. Results from the statistical analysis of HRQoL measures and the qualitative interview data illustrate that current generic measures of HRQoL may have limited relevance in this group due to limitations in the descriptive systems and value sets. Child-specific measures may be more appropriate than measures like the EQ-5D-Y which have been converted from adult to child measures. Future research should focus on developing value sets representative of disabled populations; furthermore a disability-specific measure with an appropriate descriptive system may be appropriate. This, however, would raise issues of comparability with standard measures of HRQoL.
D. Alternative methods of evaluation such as the capability approach may be more valid than utility measurement. The themes used by participants to define QoL in relation to wheelchair used were more closely associated with capability tenets than the EQ-5D-Y and HUI HRQoL domains. However, capability has not yet been applied appropriately in children. Return on investment analysis could be

used as a complimentary method to account for outcomes that are difficult to measure/value using traditional methods. This may have particular relevance to children under the age of 5.

Table 8.1: Over-arching synthesis of thesis findings

	Chapter 3: Systematic review	Chapter 4: Costing case-study	Chapter 5: DCE	Chapter 6: Analysis of HRQoL measures	Chapter 7: Defining QoL	Synthesis of findings and recommendations
Applying principles of health economics to wheelchair provision for disabled children	Outcomes beyond clinical benefits should be considered, e.g. psychosocial and independence outcomes. Distinct lack of economic evidence in this field.	Costing must consider customisation, maintenance and refurbishment costs of wheelchairs. Potential for cost savings through recycling and refurbishment of wheelchairs.	Highest service priority was expanding remit of wheelchair assessments beyond health e.g. consider social and education needs in wheelchair provision. Marginal rate of substitution analysis proved to be useful method for analysing service priorities DCEs can be used effectively in this population to explore service preferences.	HUI2 offers best option for HRQoL assessment, although limited by the descriptive system relevance; additional measure/validation required. Parent proxy and child measures correlate and show agreement, but are statistically different. Child self-reports should therefore be used where possible.	Independence enhances HRQoL through participation and positive experiences. Health and function important for parents of younger children, but other factors more important as child ages. Self-worth and feeling fulfilled important HRQoL domain.	Outcomes should consider more than health, specifically independence, self-worth and social interaction. Current generic measures of HRQoL have limited relevance in this group, additional child-specific measures should be trialled. Capability may offer a better approach to outcome measurement, but has not yet been applied appropriately in children. Return on investment analysis could be used as a complimentary method to account for outcomes that are difficult to measure and value using traditional methods. This may have particular relevance to children under the age of 5.
Considerations for wheelchair service development	Outcomes beyond clinical benefits should be considered, e.g. psychosocial and independence outcomes. Focus on individual need rather than eligibility criteria. Reduce waiting times and improve joint funding/multi-agency approach. Improve maintenance and aftercare procedures.	Powered mobility for under 5's can be relatively cheap (compared to PWCs for older children) e.g. Wizzybug. Recycling wheelchairs and regular maintenance may provide cost savings.	Highest service priority was expanding remit of wheelchair assessments beyond health e.g. consider social and education needs in wheelchair provision. Timely delivery of wheelchair (1-3 months) significant factor in service preference. Parents and children differ with regards to preferences for review frequency and level of training, although both non-significant.	Appropriate outcome measures should be embedded in wheelchair services. Child self-reported HRQoL data should be prioritised over proxy data wherever possible.	Services should focus on developing child independence, social interaction and participation through appropriate wheelchair provision. Health and function important outcomes in younger children, but secondary to other outcomes as child ages.	A holistic approach to wheelchair assessment is required. Waiting times have been an issue in NHS wheelchair services for many years, and delivery time significantly impacted service preferences. Regular maintenance can improve the longevity of wheelchairs and provide cost savings through recycling. Appropriate outcome measures, which factor in independence and social interaction, should be embedded into services. PWCs for children under the age of 5, like the Wizzybug, are not necessarily prohibitively expensive. Additional cost- effectiveness evidence could build the case for routine provision of PWCs to younger children.

8.5. Strengths and limitations

In the interest of transparency, it is important to discuss some of the strengths and limitations of this thesis and the separate studies presented. Considering sampling and recruitment, there was a lack of diversity in the samples overall which affects the generalisability of the findings. In the full recruited sample almost all participants were white British (94.7%), most children had cerebral palsy (63.2%) and half were aged 5 or under. Although the project was restricted by time and funding, recruitment in general was relatively simple because the sample sizes were small and the participants were enthusiastic to participate. This reflects that the sample was relatively self-selective, therefore disengaged or unmotivated individuals may have been missed. Furthermore, average household income was above the national average; in the DCE sample 61% of participants had an annual household income of £36,000 or more, compared with a national average of £28,200 (Office for National Statistics, 2013) at the time of data collection.

All children who participated (i.e. completed questionnaires or took part in the interview) had mental capacity. The sample of child participants is therefore not representative of children with learning impairments. This relates to some issues with comparisons of the full samples rather than matched pairs, as the parental sample contained parents of children with both physical and cognitive impairments and with a wider age range (2 to 18 years compared to 6 to 18 years and 11 to 18 years depending on the study). Due to small sample sizes subgroup analyses by age, developmental level, cognitive ability and disability prognosis were not possible.

Recruitment from the NHS site was the most difficult and led to an under representation of NHS participants (N=6). For instance, no NHS PWC or buggy users were recruited which affected full comparisons with the WK sub-group sample in the costing case study. I believe this difficulty in NHS recruitment reflects the service satisfaction of participants; most of the charity-led wheelchair service participants had previously accessed NHS services but had then chosen to seek equipment elsewhere due to NHS restrictions. They were therefore motivated to discuss their experiences and to 'give something back' to the charity services.

Moving on to the individual studies, the original aim of the systematic review was to examine wheelchair interventions more generally, however due to the focus of the literature and apparent lack of evidence, the results are more applicable to PWC interventions than MWC interventions. Furthermore, over half of the intervention studies looked specifically at children with cerebral palsy,

therefore limiting the generalisability of the findings to other conditions and disabilities. As noted previously, the evidence was generally of low quality therefore the conclusions have to be viewed with caution due to risk of bias and underpowered analyses.

In defence of the systematic review, all studies and publications considered relevant to the predefined review questions and inclusion criteria were included, therefore the evidence presented is an accurate representation of the existing evidence base. A comprehensive list of search terms was devised and tested to ensure that all relevant literature was found. The synthesis of mixed-method evidence across a variety of types of evidence was particularly important for understanding the wider context of wheelchair interventions and services. This is an approach which is relatively novel in health economics, and this review demonstrates that looking beyond economic evidence can help to build a broader picture of the topic area. Although high quality evidence was lacking, I believe that the inclusion of several types of evidence helped to provide a rich overview of existing literature in this topic area. Only evidence written or translated into English was included in this review, which may have excluded valuable research written in other languages. However, it was beyond the scope of this PhD to conduct searches in other languages or to translate studies.

The aim of the wheelchair costing case-study presented in chapter four was to examine the costs associated with the supply of a wheelchair for a disabled child. Broader conclusions about overall costs more generally to the NHS, WK and BIME could not be made due to the small sample size and the lack of comparable makes/models of wheelchairs. Furthermore, due to sample size, comparisons between children with equivalent wheelchairs or conditions could be not conducted. NHS wheelchair services in the UK vary greatly in terms of contract prices for wheelchairs and assessment criteria (Goddard, 2008) therefore the results are relevant to the individual patients included in this study and the specific NHS wheelchair service they were recruited from.

Due to a lack of available data, some assumptions had to be made regarding staff time and costs. I was explicit about the assumptions made and used a basic form of deterministic sensitivity analysis to adjust assumptions and account for uncertainty. Furthermore, all assumptions were based on expert opinion and published evidence. Although compromises had to be made and the results lack generalisability, I believe that chapter four offers a wealth of practical information by providing a template for future costings exercises for the purpose of economic evaluation in this field. The aim of chapter four was not to present a full economic evaluation but to examine the costs associated with the supply of a wheelchair, and thus the aims have been met.

The DCE presented in chapter five was designed as a pilot, thus a larger sample size would have produced more generalisable and informative results. The attributes and levels of the DCE were developed using the systematic review findings and through consultation with wheelchair service professionals and young wheelchair users. It would have been beneficial to conduct additional qualitative research to inform the development of the DCE in a more valid way (Coast et al, 2012). A strength of the DCE was eliciting the preferences of both children and parents and making comparisons between them. This provided a novel perspective regarding the differences between the service preferences of children and their parents.

One of the key aims of the DCE was to establish if the DCE method could be used appropriately in this population. Importantly, all participants completed the DCE questionnaire in full without obvious error or missing data, demonstrating the ease of completion of the questionnaire and the applicability of the approach. When presented with the instructions participants showed adequate understanding and subsequently completed the questionnaires with little need for additional clarification or guidance. I believe that the design of a user-friendly and child-centred questionnaire helped to ensure materials were appropriate for both children and adults. Optimising the clarity of the questionnaire took a number of iterations, and was guided by input from young wheelchair users. I believe the use of pictures and appropriate language made the DCE easy to understand and therefore relatively easy to complete (see appendix E.1). Much like the HRQoL questionnaire, I focused on making the DCE user friendly. I therefore decided that the number of pairwise choice tasks should be limited to eight to reduce burden on respondents. Although this meant that the number of attributes and levels had to be limited, it also meant that the questionnaire was easier and quicker to complete and therefore more likely to hold the attention of children. This is an important consideration in the development of DCEs. Parents and their children completed the DCE questionnaire at the same time, but I was present I to make sure that there was no conferring or influencing of responses.

For the studies presented in chapters six and seven it would have been beneficial to test a wider range of measures, including child and condition-specific measures, to allow a more thorough comparison of construct/content validity and agreement. In particular it would have been interesting to include the CHU-9D and potentially an adult capability measure for exploratory application of the capability approach. The EQ-5D-Y and HUI measures were chosen explicitly because the EQ-5D is considered the gold standard in HRQoL measurement for economic evaluations (NICE, 2013), and the HUI is one of the most well established measures of child HRQoL (Horsman et al, 2003). The inclusion of additional

measures was considered, but due to the age range of participants this could have potentially placed too much burden on respondents, and therefore may have led to more missing data. In the interest of creating a questionnaire that was relatively quick and easy to complete, I chose to focus on the two most prominent generic preference-based measures. Furthermore, I wanted to maintain comparability between children and parents so equivalent measures were used in both groups.

It is worth noting that the VAS is not a preference-based HRQoL measure, and thus making comparisons between it and the other measures does have limitations, however as stated previously the VAS is a good means to compare HRQoL and self-rated health status (Woolfe et al, 1997; Davies et al, 2003; Krabbe and Weijnen, 2003; Whynes, 2008), which are inextricably linked. In chapter six missing data was excluded as the sample size was too small to accurately impute missing data. This further reduced the sample size and therefore the power of the analyses. If more time and funding had been available it would have been interesting to use a larger sample and to examine before and after HRQoL results for wheelchair interventions in a pilot cost-effectiveness analysis. However, the remit of the PhD was to understand how to apply health economics in this setting, therefore the knowledge gathered in this thesis was needed prior to committing to a larger scale economic evaluation. The use of correlation, construct validity and agreement analyses produced a thorough account of the relationship between the measures and respondents. Regression analysis would also have been complementary, but time restrictions limited which analyses could be conducted.

For the qualitative study presented in chapter seven I had originally intended to include more children, but due to time constraints and parental concerns about their child's capacity to take part this was not possible. This meant that only 14% of interviewees were children taking part on their own, therefore potentially skewing the results towards the opinions of the parents. However, 25% of interviews were conducted with the parent and child, and analysis was separated by respondent so that voice of the child could be analysed separately to the parent. Although the presence of the parent may have influenced the child's responses, I believe that a strength of this study was the separate analyses of parent and child responses. Another consideration was the age of child participants, as 73% were aged 16 or over, therefore focussing on older children rather than younger children. In order to comply with ethical procedures I allowed parents of younger children to decide if their child was able to take part in the study. As some parents felt that it wasn't suitable for their child this meant that generally only older children were interviewed, this was also somewhat true for the HRQoL and DCE questionnaires. Child age, cognitive ability, health and severe communication impairment also impacted involvement in the

research. Due to time constraints participants were not asked to verify transcripts or provide input into the analysis and interpretation of results. In the next sections I will discuss the issues of reflexivity and rapport in more detail.

I believe that overall this thesis presents a thorough exploration of the topic area and tackles a number of key issues. There are areas where it could have been strengthened, for instance the inclusion of different types of economic analysis (for instance social return on investment, cost-benefit and cost-minimisation) and the piloting of an economic analysis. Due to time and funding constraints this was not possible. Furthermore, I chose to focus on cost-effectiveness analysis and the QALY as these are particularly influential for NICE and NHS service commissioning.

8.5.1. Reflecting on the qualitative methods: Reflexivity

It is important to reflect on my influence as a researcher on the qualitative data collection, analysis and interpretation. Reflexivity is a continuous process of evaluating how one's values, perceptions and behaviours influence the process of qualitative research (Lambert et al, 2010). Knowledge is both partial and situated (Malterud, 2001), thus the researcher must account for their own influence in qualitative research. Malterud (2001) states that reflexivity is a process of identifying preconceptions a researcher may hold which could influence their approach to the research. These may include personal and professional experiences, motivations and theoretical perspectives. In the interest of reflexivity and being transparent about how my preconceptions, experiences and background have influenced the qualitative aspects of the thesis, I present a summary of pertinent information about myself and my background.

It is first important to state that I did not know the participants before undertaking the research, and thus I did not have a previous relationship with the participants. I was not responsible for any aspect of participants' care and therefore the relationship built during the research process was more equitable than if the research had been conducted by wheelchair service staff member. Furthermore, I disengaged after data collection and had no further contact apart from feeding back findings in a child centred way.

A range of other factors are also of note. I am an able-bodied white male, and at the time of data collection and analysis I was in my late 20's. Before starting this qualitative research I completed an MSc in public health and health promotion (including modules in health economics), and worked as a research officer for a clinical research network based in North Wales. It was during my MSc that I became interested in health economics and decided it was a field of research that I wanted to pursue.

As a research officer I worked on a number of paediatric research projects, as well as research in the fields of diabetes, dementia and primary care. My undergraduate degree was in Psychology and I am a father to an able-bodied child. My professional interests lie in health economics, paediatric research and disability research. I approached the qualitative research from the perspective of a health economist, but also as a father and an able-bodied adult. I became aware of this topic of research after the publication of the National Assembly for Wales (2010) report regarding the urgent need for the wheelchair service development in Wales. At the time I was supporting a number of paediatric clinical research projects and was interested by the issues raised in the report. I had previously worked with disabled children whilst completing my undergraduate studies and was interested in continuing work in this area, thus when I was approached by my supervisors regarding a PhD in this field I considered it to be an excellent opportunity for personal and professional development.

I approached this work from the perspective of a health economist, and thus my approach to data collection and analysis reflects this. My theoretical perspective is underpinned by extra-welfarism, therefore I support: the use of outcomes beyond utility such as happiness, social interaction and pain; interpersonal comparisons of wellbeing; and valuation extended beyond those affected, for instance general population health state valuations. Furthermore, I accept that the QALY is an adequate proxy for health maximization in the assessment of healthcare efficiency. I am also a proponent of the capability approach, and believe that differentiating capabilities and actual functioning is particularly important in disabled populations, reflecting the SMD.

The qualitative interviews were conducted after the systematic review but before the other analyses presented in this thesis. Prior to conducting the qualitative analyses I had begun to analyse the utility, DCE and costings data also presented in this thesis (chapters four, five and six). The systematic review facilitated a rich and varied understanding of the topic area, including how service users feel about wheelchair services and the impact of wheelchairs on their lives. My preconception was that wheelchairs are a necessity for disabled children in order to achieve postural support and independent mobility. From the literature it was apparent that wheelchairs offer a number of beneficial effects to development, health and quality of life of disabled children, however high quality evidence was lacking. From the qualitative and policy evidence found in the review I deduced that NHS wheelchair services were currently unable to meet the holistic needs of all disabled children due to budget restrictions and strict eligibility criteria. The conceptual framework helped to organise my understanding of where evidence was needed to promote cost-effective wheelchair services. Due to my role as a health

economist, I was particularly interested in the lack of economic evidence and the reasons behind this. To me this demonstrated the difficulties in applying methods of economic evaluation in this setting and the apparent lack of attention given to this patient group.

As an able-bodied person, I have no personal experience of living with a disability. In order to gain insight into the experiences of disabled children I attended a number of WK Kidz Board meetings and met with young wheelchair users. I used these meetings to informally discuss wheelchair use, services and living with a disability. Furthermore, I attended national assistive technology and disabled living events, such as Kidz Up North, in order to meet young wheelchair users and their families. Attending these events throughout the course of my PhD helped me to formulate my own understanding of childhood disability and wheelchair use.

My theoretical understanding of disability stems from the work of Oliver (1998), Shakespeare and Watson (2002), Connors and Stalker (2007) and Lang (2007). This literature, grounded in the SMD, had a profound impact on my understanding of disability and the role society has in exacerbating disability. The SMD approach to understanding disability through disabling societal contexts was reflected in much of the qualitative research found in the systematic review. Disability exists as both a personal and societal construct, whereby social oppression and discrimination disables those with impairments through societal barriers to participation and independence (Oliver, 1998). Furthermore, the negative attitudes of society towards disability creates a 'subordinate' disabled group in society, which in turn causes institutional discrimination and an internalised perception of reduced capability and self-efficacy amongst disabled people (Lang, 2007).

Related to my theoretical stance on defining disability, I believe that as a progressively tax-funded public institution, the NHS (alongside social services and education authorities) has a duty of care to help disabled people to live full and satisfying lives, facilitated by the supply of adequate equipment to promote participation in society, education and work. The United Nations (1993) states that all countries should provide disabled people with adequate access to assistive technologies to promote independence and mobility, furthermore WHO (2008c) have stated that appropriate wheelchair provision can improve access to education, work and social activities. I believe that health economics and cost-effectiveness evidence can help to promote the development of healthcare services in an evidence-based manner. Robust cost-effectiveness evidence could be used to prioritise the most cost-effective interventions, potentially reducing expenditure and/or improving outcomes for service users. It is perhaps of note that my understanding of wheelchair effectiveness is informed by the limited

research in this field, therefore I accept that at present the true holistic effectiveness of wheelchairs is not fully understood, and thus additional research is needed.

I was solely responsible for conducting, coding and analysing all interviews. Wider discussion of the data with the supervisory research team was used to shape and test interpretations and to ensure internal validity. This included reviewing of audio files and transcripts and then discussing as a group the thematic content of the data.

I divulge these details about my personal and professional background in the interest of transparency and reflexivity. I believe that my theoretical stance and perspectives naturally influence my approach to this research. If bias is the presence of undesirable or hidden skewness, then to some extent it can be limited by identifying and accounting for the effect of the researcher (Malterud, 2001). Although preconceptions influence my role as a qualitative researcher, being able to identify my own subjectivity is key to avoiding bias and increasing reflexivity (Malterud, 2001). Qualitative research cannot be conducted in a vacuum; the influence of the researcher must therefore be understood and accounted for (Lambert et al, 2010).

8.5.2. Reflecting on the qualitative methods: Building rapport

The building of rapport in qualitative research is an important consideration, and starts from the first contact with participants (Dickson-Swift et al, 2007). Rapport is defined as a 'harmonious relationship' between the interviewer and interviewee, built on a sense of trust which facilitates a free exchange of information (Spradley, 1979). It encourages the exchange of meaningful dialogue and an understanding of the social world and lived experiences of the interviewee (Dundon and Ryan, 2010). Building rapport requires an interviewer to respond to the language and culture of the interviewee. Good rapport can help retain interviewee attention and elicitation of pertinent information (Lavin and Maynard, 2001), however researchers must strike a balance between rapport building and adhering to the protocol of the interview (Lavin and Maynard, 2001). At the most basic level, rapport and trust can be built through politeness, courtesy and facilitating interviewees to talk without fear of judgement or criticism (Silverman, 2006). On a more complex level, other concepts of confidence, empathy, commonality and respect must be considered (Dundon and Ryan, 2010). Russell et al (2002) collated practical advise on how to achieve rapport, which included adapting self-presentation to downplay differences between the interviewer and interviewee, including clothing and use of language; participating in common activities

relevant to the interviewee and environment; and building a degree of common ground using prior knowledge and any biographical similarities.

When interviewing children additional techniques are required, such as specifically establishing rapport before beginning the interview in order to make the child comfortable and thus improve trust and honesty (Gill et al, 2008). This can be achieved by adopting an informal and relaxed manner and adopting language relevant to the child. Researchers should react to the child's cues and follow their lead to find a sense of common ground (Punch, 2002). The interview process is likely to be foreign or even confusing for younger children, therefore time should be taken to explain the purpose of the research, what will happen in the interview and how their information will be used (Gill et al, 2008). Researchers should also aim to build rapport with parents, as they are the 'gatekeeper' to discussions with the child (Punch, 2002). Children should be encouraged to state if they do not know how to answer a question or to ask for further clarification, as this will limit guesswork. The interviewer must be mindful to use appropriate levels of language and to ask clear questions, but also to not be patronising, or feign commonality (Punch, 2002).

Reflecting on the interview process, participants were highly engaged and enthusiastic to take part in the interviews. It is difficult to know if participant enthusiasm facilitated the development of good rapport or was a by-product of already established rapport, although I believe it was mixture of the two. I believe that the excellent enthusiasm of most participants may be related to the recruitment sites, as both BIME and WK were offering services outside of the NHS and were doing so either at a low cost (BIME low deposit for wheelchair loans) or no cost (WK charity funded equipment). Participants expressed gratitude towards these services and a desire to 'give something back'. This was also demonstrated by the ease of recruiting participants from BIME and WK, whilst NHS participants were much more difficult to recruit.

Many participants stated that they had been let down by previous NHS services and wanted to discuss their experiences. This often felt like a form of catharsis; for many participants this was the first time they had been given a forum to explicitly discuss their experiences of wheelchair services. In some respects this was perhaps a hindrance as well, as the study was not an examination of wheelchair services but of QoL in relation to wheelchair use. This required me to be careful to direct participants back to the topic area without making them feel like I was ignoring them or interrupting them. In order to promote rapport, I allowed discussions to be directed by the participant, but used my skills as an interviewer to guide the interviews back to the topic area, for instance by redirecting questioning or

relating current topics of discussion back to the interview schedule. By doing so, participants were able to discuss the issues that were important to them but I was also able to gather the relevant data I required. During the data collection phase the supervisory team reviewed a sample of interview audio tapes and provided feedback on my interview technique and conduct.

Each interview started with a brief explanation of what we would be talking about and an explanation of the overall goals of the research. I made sure to use appropriate language levels, particularly with children, so that participants felt confident about the interview process and objectives. I treated the beginning of each interview as a means to establish rapport by engaging positively with parents and children, using 'small talk' and general discussion to build a relationship with each individual and to find common ground. Participants were informed that they were free to discuss whatever they felt was relevant to the questions, and thus were not restricted. I did not interrupt participants, which helped to facilitate engagement and openness in the conversation.

Most participants were enthusiastic about their participation and appeared to enjoy the opportunity to discuss their experiences. Parents in particular demonstrated a desire to express their thoughts and feelings about wheelchair services and the benefits of appropriate wheelchair provision. Prior to conducting the interviews all participants received a patient information sheet with my picture on, which I felt helped to put the participants at ease when we met in person. I also phoned each interviewee to arrange a date for the interview, which aided in establishing some context and rapport before the actual interview.

For all interviews I dressed informally to reduce differences between myself and the participants. For younger children I engaged in play and followed their lead on discussion. I used a tablet computer to engage younger children by asking them to take a picture of their wheelchair and then to draw on the picture to indicate what they liked and didn't like about their wheelchair. Although this did not produce additional data, the children simply enjoyed the act of drawing on the tablet computer and so it helped to put them at ease. It also aided in focussing the children on the topics of the interview and making them feel comfortable in the novel situation.

When interviewing children and parents together I attempted to aim most of my questioning at the child so I could prioritise their discourse. I also explicitly directed individual questions at either the child or the parent so that there was no confusion about who should be answering each question. In a small number of interviews parents interrupted their child on some questions, I dealt with this by then

rephrasing certain questions so they were relevant only to child, for instance asking for the child to answer first followed by the parent. For the majority of interviews both children and parents were respectful of one another and allowed communication without interruption.

One of the biggest challenges I faced was learning how to interview children without either being patronising or being too adult. I found it to be quite a fine line between engaging children on their level and talking down to them. I found the best way to achieve this was to first observe their behaviour and language, and then to incorporate that into my own methods of communication in a natural way. I discussed with children their interests and hobbies (such as videogames, TV programmes and sports) in order to build some common ground. This helped to build a level of trust and allowed commonality to form. Children were encouraged to let me know if they didn't know how to answer a question, and I would try to rephrase it for additional clarity.

In the majority of cases the participants appeared to be comfortable, engaged and enthusiastic during the interviews. Many participants appeared to enjoy the process and were grateful for their inclusion in the study. I had originally intended to include more young children in the interviews, but this was not possible in practice. Many parents of younger children felt that the interview process would either be too difficult for their child or that their child did not have the capacity to contribute due to communication problems or intellectual impairment. In order to respect their wishes I did not pursue the inclusion of children if the parent explicitly stated reservations about their involvement. In order to maintain rapport I wanted to be agreeable to parental wishes about involvement. For the young children who did participate I let them and their parents decide on the level of involvement so that they felt comfortable to take part.

8.6. Implications for future economic evaluations

8.6.1. Measuring outcomes: HRQoL or capability?

Due to NICE's focus on the QALY (Dakin et al, 2014), it is the most influential outcome measure in UK health economics. The starting point for QALY calculation is the use of generic, preference-based HRQoL measures to assess utility. Measures such as the EQ-5D have become vastly popular due to their wide-ranging applicability in most populations. In the treatment of cerebral palsy and other chronic conditions QoL is often one of the most important outcomes (Bjornson and McLaughlin, 2001). A key concept in rational disinvestment is ensuring that when budget cuts are unavoidable, potential benefits are

maximised whilst potential harm is minimised (Donaldson et al, 2010). Measures like the EQ-5D facilitate this by allowing outcome comparisons to be made across disparate interventions and disease-groups using a single comparable measure. As illustrated in the results presented in this thesis, the applicability of these generic preference-based measures for disabled children is limited, therefore calling into question the validity of making comparisons across diverse populations. In particular, the use of HRQoL in children under the age of 5 is essentially impossible due to the lack of validated measures and value sets. In chapter six I concluded that current methods for eliciting utility data are not appropriate in this population, particularly the EQ-5D-Y. Although parental proxy reporting of HRQoL was significantly different to child self-reports, there was agreement and correlation between equivalent measures. In the absence of appropriate measures, there is a risk that certain interventions, such as PWCs for children under the age of 5, have become marginalised with regards to policy, guidance and commissioning. It is therefore imperative that methods for gathering utility data or alternative methods of economic analysis are employed.

Using health state preferences derived from the general public presents a utilitarian approach to health state valuation. The actual applicability of these preferences to specific groups is uncertain. For instance, disabled people indicate that mobility does not have a major impact on their HRQoL (Bartonek et al, 2012; Burström et al, 2014), and yet general population EQ-5D value sets heavily discount HRQoL when mobility is reduced. This is not to say that loss of mobility does not affect HRQoL, the issue is that general population preferences are always likely to discount health status based on perceived normal functioning, and thus measurement of the HRQoL of disabled people will be judged unfairly. Furthermore, as found in chapter seven, the descriptive systems of these measures are not representative of how disabled children define health and QoL, which can lead to measures being insensitive to change (Harding, 2001) and invalid for measuring HRQoL. Disease or condition-specific measures are a viable alternative in child populations (Matza et al, 2004) but they lack the comparability of generic measures (Eiser, 1997), therefore alternative approaches to generic data collection are required, such as the capability approach.

The term capability is used in a person-centric manner; it defines wellbeing as an individual's potential and actual ability to function (Robeyns, 2003). It therefore frames wellbeing around individual needs and abilities. In this respect it doesn't presuppose the benefit of 'normal' functioning to the same extent as HRQoL measures such as the EQ-5D. Valuable functionings are therefore of key importance, for instance being able to be independent and take part in activities that are important to the individual. To

some extent this approach therefore mirrors the social model of disability, which states that disability should not automatically be seen as a medical problem in need of medical intervention (Oliver, 1998).

There is still uncertainty about how best to apply the capability approach in health economics. For instance, it is unclear whether capability should be used as an alternative means of evaluation whilst maintaining health maximisation goals, or whether it should be used specifically to evaluate capability and/or equity goals (Coast et al, 2008). Furthermore, it is not possible to apply capability to children as the concept of child capability has not been fully defined (Biggeri et al, 2004). Accordingly, there are currently no validated child-specific measures of capability for the purpose of economic analysis. EuroQoL explicitly state that the EQ-5D adult version can be used from age 16 upwards, and that between ages 12 to 15 a hybrid of adult and child EQ-5D tools can be used (EuroQoL Group, 2014). Applying this same logic to the ICECAP-A capability tool, there is potential for it to be applied from age 18. Although this does not account for younger children, it does provide a potential route to collecting preliminary capability data in this population and testing the application of this method.

One of the critical issues arising from the evidence presented in this thesis is the age-appropriateness of wheelchair provision and outcome measures. Whether considering utility or capability, the application of health economic methods to the various age groups clearly varies, and each age group presents different issues in terms of wheelchair needs and data collection methods. Above 5 years of age there is an acceptance of powered mobility and appropriate outcome measures become increasingly available. For instance, from age 16 adult approaches to health economics can be applied with relative ease (although validity is still an issue). Therefore, there must be consideration of age-appropriateness with regards to evaluating utility, capability and other outcome measures in economic evaluation.

8.6.2. Future research

Griebsch et al (2005) argue that there are four potential approaches to applying cost-utility analysis in child populations; the first is to develop generic measures appropriate for children and adults; the second is to solely use adult measures; the third is to focus on child-specific measures and accept a lack of comparison with adult data; and the final potential approach is to move away from single generic measures entirely. The issues of measuring QALYs in children are also relevant for disabled people, especially disabled children, as their conceptualisation of HRQoL is different to that of able-bodied people (as demonstrated in chapter seven). From the evidence presented in this thesis it appears that the use of generic measures is limited in both child and disabled populations due to unrepresentative

descriptive systems and value sets. However, I believe that the QALY approach of combing quantity and quality of health state still has merits, even if it cannot be applied in a generic way. The lack of universal comparability raises issues with the 'QALY is a QALY is a QALY' argument, but it doesn't necessarily mean that such approaches are redundant. When measured correctly, health state valuations can provide a holistic approach to assessing outcomes, as factors beyond clinical symptoms can be accounted for (Matza et al, 2004). This is important in the assessment of wheelchair intervention and other assistive technology effectiveness due to the wide range of clinical, social and developmental benefits facilitated by adaptive technologies. It does, however, raise serious concerns about comparing QALY gains across populations and diseases (Griebsch et al, 2005), particularly if each population sub-group requires a different conceptualisation of HRQoL, health and subsequent health state valuations.

In order to examine which of these four approaches is required, a number of research developments are needed to enable robust economic evaluation of wheelchairs for disabled children. Firstly, child-specific preference-based HRQoL measures, such as the CHU-9D and HUI2, need to be fully validated in this population, with value sets calculated from a representative sample of disabled children and parents for proxy measures; secondly, capability needs to be fully conceptualised for children and used to create an appropriate and validated child-specific capability measure; and finally, there is potential for a disability-specific preference-based HRQoL to be developed, which could factor in the impact of adaptations on HRQoL health state valuation.

A range of quantitative and qualitative evidence is required to tackle some of these issues. Validated HRQoL measures need to be tested in a larger sample to assess applicability. If applicability cannot be verified then additional research is needed to either develop a new measure for disabled people (or specifically disabled children) or to adapt an existing generic measure. This would require qualitative research to develop a new measure or to adapt the descriptive system of an existing measure. I believe that the evidence presented in chapter seven offers a sound foundation for the development of a new disabled child-specific descriptive system. Additional research would also be needed to develop appropriate value sets in this population. This could be approached either from a HRQoL or capability perspective, although additional research is first needed to fully understand capability in children before outcome measures are developed.

In my opinion the first step towards developing robust economic evidence is to use child-specific measures, such as the CHU-9D and HUI2, to perform a pilot economic analysis of wheelchair interventions for children. Additional validation would be required and caution would be needed to

ensure that HRQoL was not unfairly discounted. A pilot economic analysis using these measures could provide useful data and help to understand the applicability of generic measures in both child and disabled populations. Although these measures may not be wholly suitable in this population, there is much to be learnt from trialling methods and establishing where further research is needed. Current generic measures of HRQoL may have limitations, but they are still useful for measuring the effectiveness of different assistive technologies while appropriate alternatives are not available (Bjornson and McLaughlin, 2001).

A possible approach would be to measure the HRQoL of children before and after supply of their wheelchair as part of a clinical trial or even a modelling exercise. A number of additional factors would need to be taken into account, including the type of wheelchair, level of customisation, accessories, other wheelchairs owned and condition/severity. As children require wheelchairs for a vast range of conditions and disabilities, and wheelchair interventions vary between individuals, it would be important to differentiate the sample into sub-groups for analysis, which would require a large sample in a trial setting. As part of sensitivity analyses, sub-groups could be arranged by condition, severity, wheelchair type and level of customisation. For the purpose of a clinical trial it would be important to focus on a single intervention, for instance a specific wheelchair model, so that the intervention was comparable within the sample. A control group would also be required for QALY calculations. The control could include children who did not receive a wheelchair or who received a standard wheelchair compared to a more technologically advanced wheelchair (for example MWC vs. PWC, or standard MWC vs. active MWC). Consideration of ethical implications would be needed to ensure that essential equipment was not being withheld in an unethical manner.

Unfortunately this approach would not be suitable for assessing interventions for children under the age of five as there are currently no validated measures for this age group. Therefore, there is a need to develop a proxy HRQoL measure which could be validated for under 5's. Again, this would require extensive research to build or adapt a descriptive system and to generate proxy health state valuations. If such a measure was available it would be possible to gather HRQoL data before and after the supply of a child's first wheelchair as part of a clinical trial, which could then be used to calculate or model effectiveness estimates for PWCs for under 5's. In the short term, an alternative approach to analysis is therefore needed, such as return on investment analysis.

8.6.3. Return on investment as an alternative means of evaluation

To date NICE has produced no HTA guidance relating to wheelchair interventions for children. As the results presented in this thesis indicate, there are fundamental issues with using NICE recommended health economics tools in diverse populations such as disabled children. The lack of HRQoL evidence in disabled children inhibits the economic evaluation of wheelchairs for disabled children, leading to a distinct lack of evidence to guide commissioning and provision. The lack of NICE attention to interventions such as wheelchairs reflects a medical model of health and disability, whereby medical treatment takes precedent over rehabilitation and non-clinical outcomes. The effectiveness evidence for wheelchair interventions for children is generally of low quality, but is sufficient to illustrate the wide ranging benefits of supplying the right equipment at the right time.

Due to the inherent issues of measuring utility in disabled children, particularly children under the age of five, it may be beneficial in the short term to use alternative approaches to assessing the economic costs and benefits of appropriate wheelchair provision. For instance return on investment analysis (Cabinet Office, 2009) offers a complementary approach to traditional economic analysis techniques and is highly relevant to public policy development. Using social return on investment the wider personal and social benefits of appropriate wheelchair provision could be analysed, for instance the financial benefits of supporting a child to be independent, leading to employment and social contribution through taxation. The benefit of social return on investment analysis is that it encourages identification of outcomes, mapping of change and valuation of outputs. Furthermore organisations or services are facilitated to quantify wider contribution to society and develop services accordingly (Arvidson et al, 2010),

However, this also raises a number of subsequent issues, such as valuing the return on investment of a wheelchair for a child with a life-limiting illness. These are important considerations which must be taken into account. Frontier economics (2011) demonstrated that the social return on investment approach can be used effectively in this setting, providing encouraging evidence for the future application of this method. Establishing which outcomes to measures and how to quantify certain benefits requires thorough consideration (Arvidson et al, 2010), but there is certainly scope for further application of this method in wheelchair intervention and service assessment.

8.6.4. Implications for QALY calculation

A central principal of the QALY is that all QALYs are equal, therefore they can be judged across all members of society without bias (Rawlins and Culyer, 2004). This allows the QALY to be used as a

universal outcome. QALYs are designed to be weighted the same, regardless of the disease or individual, and yet NICE has altered the valuation of health gains in certain populations, such as disadvantaged groups, to address issues of equity (Shah et al, 2013). NICE has pledged to take special account of the needs of disabled people. This perhaps confuses the issue of whether to prioritise equity or cost-effectiveness.

Utility data is only as useful as the measure that has been used to generate it. If the applied measure is not reliably measuring HRQoL then the subsequent utility data is flawed, and thus any calculated QALYs are not 'equal'. Many generic measures and value sets are not applicable to subset populations (e.g. children, wheelchair users) because the health state preferences and subsequent weights are derived from general public perceptions of health state valuation. Furthermore, the domains of HRQoL on generic measures may be too broad and therefore insensitive to changes experienced by people with specific diseases or conditions (Harding, 2001).The use of generic HRQoL measures in marginal groups is therefore problematic, for instance application of QALY measurement in child populations is underdeveloped due to a lack of appropriate instruments to classify health states (Griebsch et al, 2005).

In chapter six I concluded that the EQ-5D-Y and HUI measures potentially undervalue the HRQoL of disabled children; they proved to be especially insensitive when used as proxy measures and when used in young disabled children. Furthermore, in chapter seven I concluded that the descriptive systems of these measures did not fully reflect how disabled children define QoL in relation to wheelchair use. These results raise serious concerns about the usefulness of standard preference-based measures of HRQoL for calculating QALYs for disabled children. The use of cost-utility analysis and the QALY has been applied to disabled children in previous research, but authors noted the pitfalls of using a generic measure such as the EQ-5D to measure utility in disabled children (Hoving et al, 2008). There is a real possibility that outcomes and subsequent QALYs are undervalued by using inappropriate or insensitive methods of utility data collection.

The DoH states that the outcomes of disabled children can be improved if correct outcome measures are used (DoH Commissioning Team, 2010), and yet there are still issues with measuring the effectiveness of interventions such as wheelchairs due to lack of validated measures. The results from this thesis demonstrate that outcomes must look beyond clinical effect and health, and include wider benefits important to each child, such as ability to socialise and individual freedom. In this respect looking beyond the QALY and cost-utility analysis may facilitate economic evaluation of wheelchairs, even though the results will lack comparability more generally.

8.6.5. How this thesis fits into the wider context of wheelchair provision for disabled children

The overarching aim of this PhD thesis was to apply health economics methodologies to disabled children who use wheelchairs. The importance of health economics in guiding service development and resource allocation cannot be underestimated. This PhD was started at a time when NHS wheelchair services were in urgent need of improvement, particularly with regards to waiting times and eligibility criteria (WK, 2011). However, the issues around wheelchair services have received little attention since the 2010 National Assembly for Wales report (National Assembly for Wales, 2010). The intervention of WK in some wheelchair services in England has demonstrated that with effective planning and procurement strategies wheelchair services can supply better equipment and at lower costs (Frontier Economics, 2011). At present there is no evidence to demonstrate the cost-effectiveness of wheelchair services and interventions, and thus there is a clear gap in knowledge for commissioners. Furthermore the long-term viability of the WK model of service provision is yet to be seen, likewise it is difficult to predict if the success of WK can be replicated by the NHS independently.

Policy and practice has to some extent moved on since the conception of this thesis. There is now an NHS tariff for wheelchair provision in England which sets out minimum service level requirements, and a new NHS standard contract for wheelchair services (NHS Commissioning Board, 2013). However, these are still limited due to their lack of detail to inform decision-making. When there is an absence of NICE guidance other sources of evidence such as clinical evidence, best practice and national policies/guidelines are used to inform decisions about service provision. In the current context, this thesis contributes to this gap in knowledge, but does not provide all the answers. At present there is no official guidance regarding which outcome measures are appropriate for assessing wheelchair interventions and services for children or adults. A specific NHS satisfaction or experience measure for children has yet to be developed, and thus there is a real gap in knowledge about how to assess outcomes in this setting.

In order to appropriately and precisely develop wheelchair services there must be development and utilisation of appropriate outcome measures. During this PhD I sought to examine whether standard methods of economic evaluation are appropriate for this specific setting. Evidence of cost-effectiveness is essential to promote evidence-based decision-making, and requires robust estimates of effect, or specifically utility for the purpose of QALY calculations. The importance of the QALY has grown in recent years, partly due to the importance that NICE place on such calculations and their role in guiding NHS funding allocation (Dakin et al, 2014). As the QALY provides an aggregate score of quantity and quality

of life, it is particularly versatile for use across almost all healthcare settings and therefore facilitates universal comparisons. NICE specifically recommends the use of QALYs as a primary measure of effectiveness (NICE, 2013), setting an acceptable cost per-QALY limit of between £20,000 to £30,000. The arbitrary nature of this limit has raised public concerns. The restriction of any form of health care due to cost will always be a sensitive and emotive issue in countries where public taxation is used to fund health services. It is therefore imperative that such decisions are based on the best quality evidence.

In some respects it may be correct to assume that NHS wheelchair services ration wheelchairs based on price. The use of strict eligibility criteria has the potential to limit provision unfairly, for instance young children are often rejected PWCs due to assumed issues of safety, even though evidence suggests that the benefits would likely outweigh the potential problems (WK, 2011). The variance in assessment procedures and equipment contracts throughout NHS wheelchair services creates a form of postcode lottery, as some families will have access to wheelchairs that others are not able to obtain. The restructuring and re-commissioning of wheelchair services is beyond the remit of this PhD, however I am able to offer advice on the effective application of health economics within this field. If uniform cost-effectiveness data was available issues with eligibility criteria would perhaps be less important, as there would be a clear evidence base to guide provision. It is with this in mind that I have presented the findings from the PhD thesis. From the perspective of a health economist there are three key issues to be addressed:

A. How to cost wheelchair interventions
B. How to measure HRQoL outcomes for the purpose of cost-effectiveness analysis
C. How to prioritise different attributes of wheelchair services

The intention of this PhD research was to address these key issues and to examine the appropriateness of standard health economics methods of evaluation in this specific setting. The results presented in this thesis are intended to inform future economic evaluations and thus do not present generalisable estimates of cost-effectiveness which could inform policy or funding allocation. It is my hope that this thesis will be the starting point for embedding health economics in wheelchair services. As stated previously, wheelchairs for disabled children can offer a wide range of benefits, it is therefore imperative that services are able to provide the most appropriate equipment to each child. With appropriate cost-effectiveness evidence wheelchair provision could be based on both effectiveness and

cost saving principles. Without this evidence NICE cannot make clear judgements and guidance on particular interventions or services.

Future research must focus on developing the capability approach for children or developing alternative methods to elicit utility in disabled child populations, such as child or disability specific preference-based measures.

This thesis is one of the first attempts to re-conceptualise health economics from the perspective of the SMD. The principles of capability and extra-welfarism can indeed be aligned with the SMD and thus provide a good theoretical basis for developing economic evaluation methods which do not discriminate against people with disabilities. I conclude that standard methods of economic evaluation have some relevance in the assessment of wheelchair interventions for disabled children. However, it may be detrimental to assume that health state preference can be utilised in a disabled population using tariffs derived from general populations. Furthermore, the descriptive systems of outcome measures may be too focussed on 'normal' functioning to capture the nuances of HRQoL for disabled children. Future research must focus on additional methods of eliciting health state preferences in disabled populations; value sets which are reflective of how disabled people define HRQoL; the impact of adaptations on HRQoL; and the role of alternative methods such as capability in disabled populations.

8.7. Conclusion

The measurement of wheelchair intervention outcomes must consider more than just health; independence, self-worth and social interaction are key outcomes for disabled children and their parents. Therefore, according to the findings of this thesis and previous research, wheelchair provision should aim to promote health, development, mobility, social inclusion, emotional wellbeing and independence. This can be achieved by NHS and third sector services adopting a holistic approach to wheelchair assessment and provision. The DCE results presented in this thesis demonstrate that comprehensiveness of wheelchair assessment has the biggest impact on the wheelchair service preferences of disabled children and their parents, demonstrating the importance of considering the wider needs of disabled children when supplying a wheelchair. Evidence presented in this thesis and previous literature demonstrates that the key areas for service development are reduced waiting times for assessment and supply of a wheelchair; holistic assessments of wheelchair needs; improved information provision and maintenance procedures; and reduced restriction of provision based on strict eligibility criteria.

NICE recommends the use of the QALY as a primary outcome measure in cost-effectiveness analysis. Robust evidence of cost-effectiveness could help to develop wheelchair services which are both efficient and meeting the holistic needs of disabled children. However, this first requires an understanding of how best to measure HRQoL in this population. The findings presented in chapters six and seven indicate that standardised utility measures such as the EQ-5D-Y do not fully represent how disabled children and their parents define HRQoL in relation to wheelchair use, and thus lack reliability and validity. Generic measures have limited relevance in this group due to their focus on normal functioning, which neglects to consider the positive impacts of assistive technologies and adaptations. This is particularly relevant in children under the age of five. Additional child-specific HRQoL measures should be trialled. Furthermore, the capability approach should be considered. Additional research is needed to develop measures and value sets which are relevant for disabled children and suitable for economic evaluation purposes.

The role of proxy data should also be considered, particularly for young children. Parents of disabled children rate their child's HRQoL significantly lower than the child does by self-report. This has real implications on the use of proxy measures to elicit utility data for disabled children. Clinicians and researchers should look carefully at differences between self-reported and proxy HRQoL data.

A number of methodological conclusions were elicited from this thesis. I found that applying a mixed-method approach to systematic reviewing in health economics provides a richer context for evidence, allowing a deeper understanding of a given topic. I found that when costing wheelchair interventions a number of factors must be taken into account, including the expected life of each wheelchair, the cost of customisation, staff time/overheads, maintenance costs and opportunities for refurbishment/recycling. The use of DCE methods is appropriate and useful in mobility impaired children, and can be used effectively to compare child and parent responses. Finally, I found qualitative health economics to be beneficial for verifying quantitative data and for further examining important issues which affect the way in which economic evidence and utility data is collected and analysed.

During the process of researching and writing this thesis a number of novel contributions were made to this field of research. Chapter three presents the first use of a mixed-methods systematic review to explore the impact of wheelchair interventions on disabled children. Furthermore, the conceptual framework developed from the systematic review findings is the first of its kind designed to guide wheelchair service development from the perspective of a health economist. Chapter four presents a novel costing case-study of wheelchair interventions for disabled children.

Chapter five presents the first robust attempt to elicit the preferences of disabled children and their parents for different attributes of wheelchair services, and secondly to compare these preferences between children and parents. The DCE method has had limited use in children generally, and to my knowledge this is the first attempt to use DCE methods in physically disabled children. The results provide positive encouragement for the use of similar methods in disabled populations and as a means to compare service preferences of children and parents.

Chapter six presents the first attempt to examine the relevance of the EQ-5D-Y and HUI measures for disabled children who use wheelchairs. Furthermore, it provides novel data on the applicability of these measures in disabled children. The evidence in this chapter demonstrates the issues of using correlation alone to compare utility measures and self vs. proxy reporting. This is the first attempt to examine the relevance of standard health economic methods of economic evaluation in a population of disabled children who use wheelchairs.

Chapter seven presents the first attempt to understand how disabled children and their parents define QoL in relation to wheelchair use. I qualitatively assessed the relevance of standard utility measures in this specific population. Furthermore this chapter contains a novel definition of HRQoL from the perspective of disabled children and a new mapping of the domains of health and QoL on to the central tenets of child and disability specific capability definitions.

The evidence base for this setting has developed greatly as a result of the work carried out as part of this thesis. Although further research is still needed, the findings presented in this thesis create a solid evidence base for the future economic analysis of wheelchair interventions for disabled children. Before I carried out this work, the application of health economics and methods of economic evaluation in this field was almost non-existent. I have been able to make clear recommendations for future research and to examine the applicability of standard methods in this specific setting. Without the evidence in the thesis I believe that there would still be a significant gap in knowledge regarding how best to evaluate wheelchair interventions for disabled children.

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Appendices

Appendix A: Participant information sheets

Appendix A.1: Participant information sheet for children and young people

BACK COVER

Contact us if you want to know more!

Where can I get more information?

If you want to know more about the project you can phone us, write to us or e-mail us. We are more than happy to answer any questions you have! You can also visit our Facebook page: www.facebook.com/theWheelsProject

Address:
Mr Nathan Bray
Wheels Project,
Centre for Health Economics
and Medicines Evaluation,
Dean Street Building,
Bangor University,
Bangor, Gwynedd
LL57 1UT

Telephone: 01248 38 2477
Mobile: 07792670053
Email: n.bray@bangor.ac.uk
Website: cheme.bangor.ac.uk

PRIFYSGOL BANGOR UNIVERSITY

If you have any concerns about this project and would like to speak to someone outside of the research team, please contact Professor Bob Woods-
Address: DSDC Wales, Bangor University, 45 College Road, Bangor, LL57 2AS
Email: b.woods@bangor.ac.uk / Phone: 01248 383719

The Wheels Project is in partnership with:

NISCHR — Sefydliad Cenedlaethol er gyfer Ymchwil Gofal Cymdeithasol ac Iechyd | National Institute for Social Care and Health Research

BIME — Bath Institute of Medical Engineering

Whizz-kidz — move a life forward

NHS

FRONT COVER

wheels

The Wheels Project wants to know what **you** think about your wheelchair!

Information leaflet for children and young people
Version 1, 30.01.13

INSIDE LEFT

The Wheels Project wants your help!

What is the Wheels Project?

The Wheels Project is about finding out what young people think about wheelchairs. We want to find out what you like and don't like about your wheelchair and how it has changed your life.

How can I help the Wheels Project?

You can help the Wheels Project by telling us about:

- What you like and don't like about your wheelchair
- Your thoughts on what your wheelchair is worth
- What you think makes a good wheelchair
- Your views and experiences of wheelchair services
- How your wheelchair improves your life

Why have I been invited to take part?

We want to talk to young people who use wheelchairs. We think your opinions will be really useful to our project.

Do I have to take part?

No, it's your choice! It's OK if you don't want to take part and you can stop taking part at any time.

INSIDE RIGHT

Read this leaflet to find out more!

What will I have to do if I take part?

If you would like to help the Wheels Project please complete the questionnaire sent with this leaflet and post it back to us. Nathan is one of the project team and will then arrange to meet with you and listen to your views about wheelchairs and the questionnaire you completed. This will take about an hour. Nathan's picture is on the back of this leaflet. If you say it is OK he will record what you say. You can choose if you want to talk to Nathan by yourself or have someone with you (like a parent or partner). We would like to speak to you but it is also ok if you just want to do the questionnaire.

What will happen to the information I give?

You can choose what you tell us and we will keep it private. We won't tell anyone else unless you tell us something that makes us worry about your safety or someone else's safety.

I would like to help! What's next?

Please fill in the questionnaire (you can ask another person to do this if you need help) and send it back to us using the envelope we have provided. We will then contact you to arrange a meeting. You will receive a £10 voucher as a thank you if you take part.

Appendix A.2: Participant information sheet for parents

BACK COVER

Contact us if you want to know more!

Where can we get more information?

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Address:
Mr Nathan Bray
Wheels Project,
Centre for Health Economics and Medicines Evaluation,
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Email: b.woods@bangor.ac.uk / Phone: 01248 383719

The Wheels Project is funded by NISCHR. It has been approved by an NHS ethics committee. It is sponsored by Bangor University in partnership with:

NISCHR — Sefydliad Cenedlaethol ar gyfer Ymchwil Gofal Cymdeithasol ac Iechyd | National Institute for Social Care and Health Research
BIME — Bath Institute of Medical Engineering
Whizz-Kidz — move a life forward
NHS

FRONT COVER

wheels

The Wheels Project wants to talk to **you** and **your child** about their wheelchair!

Information leaflet for parents and guardians
Version 1, 30.01.13

INSIDE LEFT

The Wheels Project wants your help!

What is the Wheels Project?

The aim of the Wheels Project is to understand the costs and benefits of wheelchairs for children and young people with mobility impairments. In order to do this we want to understand how wheelchairs improve the lives of children and young people, and then place a value on these improvements. We also want to see how parents rank different aspects of wheelchair services, such as waiting times, maintenance services and who assesses your child's needs.

How can we help the Wheels Project?

We would like to talk you and your child (if they are able). You can help the Wheels Project by telling us about:

- What you like and don't like about your child's wheelchair
- Your thoughts on what a wheelchair is worth
- What you think makes a good wheelchair
- Your views and experiences of wheelchair services
- How your child's wheelchair improves their life

Why have we been invited to take part?

We have contacted you because your son or daughter has a mobility impairment and requires a wheelchair to help them get around. We aim to recruit 40 young people with mobility impairments and their parent(s) from across the UK. Even if you are waiting for your child's first wheelchair your views are still important to us.

Do we have to take part?

No, it's your choice. It's OK if you don't want to take part and you can stop taking part at any time. It won't affect the treatment your child receives. If you don't think your child should take part but you would like to take part that is fine, we can interview you on your own.

INSIDE RIGHT

Read this leaflet to find out more!

What will we have to do if we take part?

We have sent **two** questionnaires with this leaflet: one for you to complete and one for your child to complete. The questionnaires ask about your child's quality of life. If you and your child would like to take part in the Wheels Project please complete these questionnaires and send them back to us. If your child isn't able to complete their questionnaire please just complete yours. Once we receive your completed questionnaire(s) Nathan (one of the project team) would like to interview you and your child (if they're able). He will listen to your views about your child's wheelchair and the questionnaire(s) you completed. His picture is on the back of this leaflet. The interview will last about an hour, and with your permission it will be recorded. We would like to interview everyone involved in the Wheels Project, but it is ok if you would just like to complete the questionnaires. If you are waiting for your child's first wheelchair please complete the questionnaire(s) as requested and we will arrange an interview for 3months after their first wheelchair is delivered.

What will happen to the information we give?

You can choose what you tell us. Any information you provide will be kept private and confidential, and your names will not be used in any reports. We will not tell anyone else unless you tell us something that makes us seriously concerned about the safety of a child or vulnerable adult. With your help we hope to improve NHS wheelchair services.

We would like to help! What's next?

Please complete the questionnaire(s) and return using the stamped addressed envelope. We will then contact you to arrange a date for the interview. Every child that takes parts will receive a **£10** highstreet voucher

Appendix B: Study approvals for quantitative and qualitative data collection

Appendix B.1: Study approval letter from sponsor ethics committee (Bangor University)

COLEG IECHYD A GWYDDORAU YMDDYGIADOL
COLLEGE OF HEALTH AND BEHAVIOURAL SCIENCES

YSGOL GWYDDORAU GOFAL IECHYD
SCHOOL OF HEALTHCARE SCIENCES

PRIFYSGOL BANGOR UNIVERSITY

6th March 2013

Mr Nathan Bray
Centre for Health Economics and Medicines Evaluation
Bangor University
Dean Street Building

Dear Nathan

Re: Healthcare and Medical Sciences Academic Ethics Committee (HCMS AEC) Review:
Proposal number: 2013-02-02
Project title: The Wheels Project: Exploring the economic, methodological and service commissioning implications of assistive mobility technology for disabled children and young people

Thank you for your application to the AEC which was subject to an expedited review as requested. All of the necessary documentation was provided and appropriately completed.

I am therefore able to give approval for your study on behalf of the AEC and this letter constitutes evidence of that approval should it be necessary for any applications to other RECs.

Should you need to make any substantial amendments to your study protocol during the lifetime of the research, you are required to submit notice of these to the AEC for further approval, prior to making any changes to the conduct of the study.

Please note that approval from this AEC does not convey automatic authority to proceed with your study. You are formally advised that it is essential to confirm with the relevant administrators whether you are required to submit your proposal to any other Ethics Committee(s) such as Local NHS Research Ethics Committee and NHS Research Governance Departments – prior to commencing your study.

You are required to notify this AEC of any amendments to your proposal that you are required to make by any external body.

Once you have received approval from an external REC, you must provide a copy of your letter of approval for this AEC.

Please do not hesitate to contact me should you require any clarification.

Yours sincerely

Dr Siôn Williams
Chair, HCMS AEC.

CC: Rhiannon Tudor Edwards

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WWW.BANGOR.AC.UK

Appendix B.2: Study approval letter from NHS research and development internal review panel

GIG CYMRU NHS WALES | Bwrdd Iechyd Prifysgol Betsi Cadwaladr University Health Board

Panel Arolygu Mewnol Y&D - Y Gorllewin
R&D Internal Review Panel - West

Betsi Cadwaladr University Health Board
Ysbyty Gwynedd
Clinical Academic Office
Bangor, Gwynedd
LL57 2PW

Mr Nathan Bray
Centre for Health Economics & Medicines Evaluation
IMSCaR
College of Health & Behavioural Sciences
Bangor University
LL57 1UT n.bray@bangor.ac.uk

Chairman/Cadeirydd - Dr. Mike C Jackson, CPsychol, DClinPsych, DPhil
Email: rossela.roberts@wales.nhs.uk
wendy.scrase2@wales.nhs.uk
sion.lewis@wales.nhs.uk
Tel/Fax: 01248 384 877

20 May 2013

Dear Mr Bray

Re: Confirmation that R&D governance checks are complete / R&D approval granted

Study Title The Wheels Project: Exploring the economic, methodological and service commissioning implications of assistive mobility technology for disabled children & young people

IRAS reference 117611

The above research project was reviewed at the meeting of the BCUHB R&D Internal Review Panel

The Committee is satisfied with the scientific validity of the project, the risk assessment, the review of the NHS cost and resource implications and all other research management issues pertaining to the revised application.

Thank you for responding to the Committee's request for further information.
The R&D office considered the response on behalf of the Committee and is satisfied with the scientific validity of the project, the risk assessment, the review of the NHS cost and resource implications and all other research management issues pertaining to the revised application.

The Internal Review Panel is pleased to confirm that all governance checks are now complete and to grant approval to proceed at Betsi Cadwaladr University Health Board sites as described in the application.

The documents reviewed and approved are listed below:

Document:	Version	Date
R&D Checklist	-	-
R&D Form – 117611/440681/14/732	-	18/04/2013
SSI Checklist	-	-
SSI Form – 117611/440757/6/462/178401/270228	-	18/04/2013
Protocol	1.1	30/03/2013
Poster	1	09/04/2013
Invitation Letter – Parent (Child aged 6-15)	1.1	17/05/2013
Invitation Letter – Parent (Child aged 5 or under)	1.1	17/05/2013
Invitation Letter – Young Person (Aged 16 or over)	1.1	17/05/2013
Information Sheet (Children aged under 5)	1.1	17/05/2013
Information Sheet (Consultee)	1.1	17/05/2013
Information Leaflet (Parents/Guardians)	1	30/01/2013
Information Leaflet (Children & Young People with wheelchairs)	1	30/01/2013
Information Leaflet (Children & Young People waiting for a wheelchair)	1	30/01/2013
Consent Form (Parent/Guardian Proxy Consent for Child)	1	30/01/2013
Consent Form (Parent/Guardian without Proxy Consent for Child)	1	30/01/2013
Consent Form (Young person aged 16 or over)	1	30/01/2013
Assent Form (Children under 16)	1	30/01/2013
Consent Form (Consultee)	1	31/01/2013

Questionnaire – Parents/Guardians	1.1	18/04/2013
Questionnaire – Young People aged 16 or over	1.1	18/04/2013
Questionnaire – Young People aged 6-15	1.1	18/04/2013
Questionnaire – Discreet Choice Experiment	1	31/01/2013
NISCHR PhD Studentships (Funding Letter)	-	15/12/2010
Healthcare & Medical Sciences Academic Ethics Committee Letter	-	06/03/2013
Bangor University Professional Indemnity Insurance Certificate	-	-
REC Favourable Opinion Letter	-	18/05/2013
CV of CI/Student (N Bray)	-	09/04/2013
CV of PI (C McCudden)	-	16/04/2013
CV of Academic Supervisor (R Edwards)	-	-
CV of Academic Supervisor (J Noyes)	-	09/04/2013
CV of Academic Supervisor (N Harris)	-	-

All research conducted at the Betsi Cadwaladr University Health Board sites must comply with the Research Governance Framework for Health and Social Care in Wales (2009).
An electronic link to this document is provided on the BCUHB R&D WebPages.
Alternatively, you may obtain a paper copy of this document via the R&D Office.

Attached you will find a set of approval conditions outlining your responsibilities during the course of this research. Failure to comply with the approval conditions will result in the withdrawal of the approval to conduct this research in the Betsi Cadwaladr University Health Board.

If your study is adopted onto the NISCHR Clinical Research Portfolio (CRP), it will be a condition of this NHS research permission, that the Chief Investigator will be required to regularly upload recruitment data onto the portfolio database.
To apply for adoption onto the NISCHR CRP, please go to:
http://www.wales.nhs.uk/sites3/page.cfm?orgid=580&pid=31979.

Once adopted, NISCHR CRP studies may be eligible for additional support through the NISCHR Clinical Research Centre. Further information can be found at:http://www.wales.nhs.uk/sites3/page.cfm?orgid=580&pid=28571 and/or from your NHS R&D office colleagues.

To upload recruitment data, please follow this link:
http://www.crncc.nihr.ac.uk/about_us/processes/portfolio/p_recruitment.
Uploading recruitment data will enable NISCHR to monitor research activity within NHS organizations, leading to NHS R&D allocations which are activity driven. Uploading of recruitment data will be monitored by your colleagues in the R&D office.
If you need any support in uploading this data, please contact wendy.scrase2@wales.nhs.uk or sion.lewis@wales.nhs.uk

If you would like further information on any other points covered by this letter please do not hesitate to contact me.

On behalf of the Committee, may I take this opportunity to wish you every success with your research.

Kind regards

Rossela Roberts

Dr. Mike C Jackson
Associate Director of R&D
Chairman IRP-West

Copy to:

Principal Investigator:	Ms Carol McCudden Clinical Lead Posture & Mobility Directorate Artificial Limb & Appliance Centre Croesnewydd Road Wrexham LL13 7NT	carol.mccudden@wales.nhs.uk
Sponsor:	Professor Bob Woods DSDC Wales 45 College Road Bangor University Gwynedd LL57 2AS	b.woods@bangor.ac.uk
Academic Supervisor:	Professor Rhiannon Tudor Edwards CHEME, IMSCaR Bangor University Gwynedd LL57 1UT	r.t.edwards@bangor.ac.uk
Academic Supervisor:	Professor Jane Noyes Centre for Health Related Research Fron Heulog Bangor University Gwynedd LL57 2EF	jane.noyes@bangor.ac.uk
Academic Supervisor:	Dr Nigel Harris Bath Institute of Medical Engineering The Wolfson Centre Royal United Hospital Bath BA1 3NG	n.harris@bath.ac.uk

Appendix B.3: Study approval letters from NHS research ethic committee

Part of the research infrastructure for Wales funded by the National Institute for Social Care and Health Research, Welsh Government.
Yn rhan o seilwaith ymchwil Cymru a ariannir gan y Sefydliad Cenedlaethol ar gyfer Ymchwil Gofal Cymdeithasol ac Iechyd, Llywodraeth Cymru

NISCHR
Gwasanaeth Moeseg Ymchwil | RES | Research Ethics Service

Pwyllgor Moeseg Ymchwil Gogledd Cymru - Y Orllewin
North Wales Research Ethics Committee - West

Betsi Cadwaladr University Health Board
Ysbyty Gwynedd
Clinical Academic Office
Bangor, Gwynedd
LL57 2PW

Telephone/ Facsimile: 01248 - 384.877
Email: Rossela.Roberts@wales.nhs.uk
Website : www.nres.nhs.uk

Mr Nathan Bray
PhD Student
CHEME, IMSCaR,
Bangor University
Dean Street Building,
Bangor, Gwynedd
LL57 1UT

n.bray@bangor.ac.uk; nathan.bray@hotmail.com

17 May 2013

Dear Mr Bray,

Study title: **The Wheels Project: Exploring the economic, methodological and service commissioning implications of assistive mobility technology for disabled children and young people.**
REC reference: **13/WA/0143**
IRAS project ID: **117611**

The Research Ethics Committee reviewed the above application at the meeting held on 16 May 2013. Thank you for attending to discuss the application.

We plan to publish your research summary wording for the above study on the NRES website, together with your contact details, unless you expressly withhold permission to do so. Publication will be no earlier than three months from the date of this favourable opinion letter. Should you wish to provide a substitute contact point, require further information, or wish to withhold permission to publish, please contact the Coordinator, Dr Rossela Roberts, rossela.roberts@wales.nhs.uk

Ethical opinion

Ethical issues raised by the Committee in private discussion, together with responses given by the researcher when invited into the meeting

Social or scientific value; purpose and need; scientific design and conduct of the study; patient /public representative involvement in study design
The Committee considered whether the objectives, design, methodology, and the conduct of the study are appropriately described in the protocol. The project is described as a low risk service development but the way aims are described varies throughout the project. You clarified that the aim of the project is to provide generalisable guidance to inform a future economic evaluation of assistive technologies for disabled children, and may provide an evidence base for the cost-effectiveness of powered mobility devices for children under the age of 5. The Committee noted that due to small sample size the study lacks power and therefore lacks reliability and has limited generalisation power. Professor Tudor Edwards clarified that this is a small scale feasibility study to inform how best to use the tools of heath economics in this group. The Committee expressed concern in relation to the answer provided to question A62 of the application form, that for an essentially qualitative study it is planned to conduct ad-hoc comparisons, further comparisons, etc.

The Committee suggested that the protocol may be revised to detail the planned analyses, and the corrections intended to use for multiple comparisons need to be explicit. The Committee concluded that the research design and the proposed analysis were deemed suitable for answering the research question. No further ethical issues were raised in relation to the scientific value and conduct of the study.

Independent review
The Committee discussed whether the study has been independently peer reviewed and whether the review is in scale of the research and risks involved. The Committee concluded that the review of the project by Healthcare and Medical Sciences Academic Ethics Committee and by the funder (NISCHR/Welsh Government) is sufficient evidence of peer-review for this type of project. The Committee noted that the application form does not state that a review was conducted by the company that manufactures the 'Wizzybug'. You clarified that the company is a Bath University spin-off and is the only manufacturer of powered assistive technologies for under 5 year olds. The collaboration is purely academic and there are no financial incentives. No further ethical issues were raised regarding the peer-review.

Recruitment arrangements; fair participant selection
The Committee was satisfied that the selection of potential participants has taken into account their clinical condition and sufficient details are provided in the protocol and the application form regarding the inclusion and exclusion criteria. The Committee noted that the study will utilise a purposive sampling and services will be asked to send out the information pack to potential participants; a query was raised on how the assessment of capacity will be carried out. You clarified that the recruiters are asked to consider the eligibility of potential participants against the inclusion/exclusion criteria. Each information pack returned to the research team is followed by an interview. The Committee raised no further issues regarding the participant selection.

Favourable risk benefit ratio; anticipated benefits/risks for research participants
The Committee discussed the anticipated benefits and potential risks to participants and were satisfied that the applicant has suitably identified the risks and lack of direct benefits and highlighted them in the information given to potential participants. The Committee requested a confirmation that participation in the study will not delay young peoples' access to equipment. You confirmed that this is indeed the case. No further ethical issues were raised in relation to the risk/benefit for research participant.

Care and protection of research participants; respect for participants' welfare and dignity; data protection and confidentiality
The Committee discussed the information governance aspects of the study. The Committee discussed where and for how long will data be stored, and clarified who will have access to the data. A query was raised in relation to the level of detail in the demographic questionnaire: Professor Tudor Edwards clarified that the health economic evaluation requires this stratification based on population norms. The Committee requested a clarification on the proposed interviewing of children: the application form states that you want to conduct this interview in the absence of adult supervision. You clarified that it is essential that participants have the ability to speak their mind without feeling that they are being constrained by their parents' presence, but that you will not in fact be alone with the child: the interview will take place with an open door and an adult present in the adjacent room; also the interviews are being recorded from start to finish; in cases when it is not practical to interview children at hoe the interviews will be conducted in public places, such as the school. The Committee queried how the incidental disclosures during the telephone interviews will be dealt with. You clarified that the conversation will be recorded and all the methods described for the face-to-face interviews will apply. No further ethical issues were raised in relation to data protection and confidentiality.

Informed Consent process; adequacy and completeness of Participant Information
The Committee noted that written informed consent is taken as part of a process - with participants having adequate time to consider the information, and opportunity to ask questions. The information is clear as to what the participant consents and there is no inducement or coercion. The Committee agreed that the procedures described in the protocol have been addressed in the Information Sheets but felt that some corrections are required. The Committee noted that the Participant Information Leaflets for various service

user groups, parents and partners lack consistency in describing the voluntary nature of participation and in the information provided about breaking confidentiality in case of incidental disclosures. Also, the Letter of Invitation to Participant states that evidence from this project will be used to develop wheelchair service in the UK but this is not the aim of the study described in the protocol, which states that the research will help the team to develop practical more generalisable guidance for the future economic evaluation of assistive technologies for disabled children, and may provide an evidence base for the cost-effectiveness of powered mobility devices for children under the age of 5. You agreed to rectify.

Compliance with sections 30-33 of the Mental Capacity Act (England and Wales) 2005
The Committee considered the following issues:

i) Relevance of the research to impairing condition The Committee discussed whether the research is connected with an impairing condition affecting persons lacking capacity, or with the treatment of the condition. Professor Noyes clarified that the inclusion of young people aged 16 to 18 who lack capacity is of direct relevance with the impairing condition and any study conducted in this population needs to be as inclusive as possible; excluding young people who lack capacity would be missing really important information that could inform the design of a pragmatic trial / economic evaluation, as there is a possibility that the issues identified by these individuals would be different from the ones identified by young people who do not have a cognitive impairment. The Committee agreed that this is not an intrusive intervention and the study is relevant for the treatment of the condition as the mobility issues faced by the young people are a result of the clinical condition causing the impairment.

ii) Justification for including adults lacking capacity to meet the research objectives The Committee agreed that the research could not be carried out as effectively if it was confined to participants able to give consent.

iii) Balance between benefit and risk, burden and intrusion In discussion the Committee agreed that the risk to participants is negligible, the research will not significantly interfere with their freedom of action or privacy, and it will not be unduly invasive or restrictive. The Committee noted that while the research may not benefit participants directly, it is not imposing a disproportionate burden and is intended to provide knowledge on the treatment or care of the condition affecting participants lacking capacity.

iv) Arrangements for appointing Consultees The Committee considered the arrangements described in the application for appointing Consultees under section 32 of the Mental Capacity Act to advise on whether participants lacking capacity should take part and on what their wishes and feelings would be likely to be if they had capacity. The Committee agreed that reasonable arrangements were in place for identifying personal Consultees or nominated Consultees independent of the project where no person can be identified to act as a personal Consultee.

v) Information for Consultees The Committee reviewed the information to be provided to Consultees about the proposed research and their role and responsibilities as a Consultee. The Committee was satisfied that the information was adequate to enable Consultees to give informed advice about the participants of persons lacking capacity.

vi) Additional safeguards The Committee was satisfied that reasonable arrangements would be in place to comply with the additional safeguards set out in section 33 of the Mental Capacity Act.

vii) Other ethical issues As the project involves adults lacking capacity the Committee is satisfied that the team have the competencies required and an understanding of the relevant aspects of the MCA and code of Practice, including the core principles of the Act, the assessment of capacity and the safeguards relating to research.

The Committee is satisfied that the requirements of sections 30-33 of the Act will be met in relation to research carried out as part of this project on, or in relation to, a person who lacks capacity to consent to taking part in the project – and approved this research project for the purposes of the Mental Capacity Act 2005.

Suitability of the applicant and facilities; requirement for Site Specific Assessment
The Committee discussed the suitability of the applicant and concluded that you are sufficiently qualified and adequately supervised to carry out this research.
The Committee discussed the requirement for Site-Specific Assessment for the non-NHS sites involved and concluded that the study can be considered SSA exempt, in accordance to the provisions of the Standard Operating Procedures for Research Ethics Committees (version 5.1 March 2012) section 4. The study involves no clinical interventions and all study procedures at this site will be undertaken by the Chief Investigator's team. The REC was satisfied that the risk to participants is likely to be negligible and the study procedures will not significantly interfere with participant's freedom of action or privacy or be unduly invasive or restrictive.

General comments/ missing information/ typographical errors/ application errors/ suitability of the study summary
The summary of the study as it appears in section A6-1 of the REC application form was deemed to be an accurate description of the study and suitable for publication on the NRES website.

The Chairman thanked you and the co-applicants for speaking to this submission and gave you an opportunity to ask questions. You did not raise any issues.
The Chairman confirmed that the Committee will deliberate and will be in touch shortly.

The Committee considered your responses.

On the basis of the information provided, the Committee was satisfied with the following aspects of the research:

- Social or scientific value; purpose and need
- Scientific design and conduct of the study
- Independent review
- Recruitment arrangements; fair participant selection
- Favourable risk benefit ratio; anticipated benefits/risks for research participants
- Care and protection of research participants; respect for participants' welfare and dignity; data protection and confidentiality
- Informed Consent process
- Compliance with sections 30-33 of the Mental Capacity Act (England and Wales) 2005
- Suitability of the applicant and facilitiess; no requirement for SSA
- Suitability of the study summary

The Committee identified issues with the following aspects of the research:

- Adequacy and completeness of Participant Information

The members of the Committee present gave a favourable ethical opinion of the above research on the basis described in the application form, protocol and supporting documentation, subject to the conditions specified below.

Mental Capacity Act 2005

I confirm that the Committee has approved this research project for the purposes of the Mental Capacity Act 2005. The committee is satisfied that the requirements of section 31 of the Act will be met in relation to research carried out as part of this project on, or in relation to, a person who lacks capacity to consent to taking part in the project.

Ethical review of research sites

NHS Sites:
The favourable opinion applies to all NHS sites taking part in the study, subject t management permission being obtained from the NHS/HSC R&D office prior to the start of the study (see "Conditions of the favourable opinion" below).

Non-NHS Sites:
I confirm that the Committee has concluded that the study is to be considered Site Specific Assessment exempt in accordance to the provisions of the Standard Operating Procedures for Research Ethics Committees (version 5.1 March 2012) section 4.

Conditions of the favourable opinion

The favourable opinion is subject to the following conditions being met prior to the start of the study.

The Committee requested that the following amendments are to be made to the Letters of Invitation to Participant and Participant Information Sheets:

1. The Letters of Invitation to Participant need to be revised to clarify that the results of this study will inform a future economic evaluation of assistive technologies for disabled children.

2. The Participant Information Leaflets need to have consistency in describing the voluntary nature of participation and the action to be taken in case of incidental disclosures.

3. The amended Letters of Invitation to Participant, Participant Information Sheets and Consent Forms need translating and the Welsh language version made available to participants.

You should notify the REC in writing once all conditions have been met (except for site approvals from host organisations) and provide copies of any revised documentation with updated version numbers. The REC will acknowledge receipt and provide a final list of the approved documentation for the study, which can be made available to host organisations to facilitate their permission for the study.
Failure to provide the final versions to the REC may cause delay in obtaining permissions.

Management permission or approval must be obtained from each host organisation prior to the start of the study at the site concerned.

Management permission ("R&D approval") should be sought from all NHS organisations involved in the study in accordance with NHS research governance arrangements. Guidance on applying for NHS permission for research is available in the Integrated Research Application System or at http://www.rdforum.nhs.uk.
Where a NHS organisation's role in the study is limited to identifying and referring potential participants to research sites ("participant identification centre"), guidance should be sought from the R&D office on the information it requires to give permission for this activity.

For non-NHS sites, site management permission should be obtained in accordance with the procedures of the relevant host organisation.

Sponsors are not required to notify the Committee of approvals from host organisations
It is responsibility of the sponsor to ensure that all the conditions are complied with before the start of the study or its initiation at a particular site (as applicable).

Approved documents

The documents reviewed and approved at the meeting were:

Document	*Version*	*Date*
Covering Letter		18 April 2013
REC application (submission 117611/440686/1/785)		22 April 2013
Protocol	1.1	30 March 2013
Advertisement	1	09 April 2013
Letter of invitation to participant Parent of child aged 5 or under	1	30 January 2013
Letter of invitation to participant Parent of child aged 6 to 15	1	30 January 2013
Letter of invitation to participant Young People aged 16 to 18	1	30 January 2013
Participant Information Sheet: Child aged 5 or under	1	30 January 2013
Participant Information Sheet: Parents and Guardians	1	30 January 2013
Participant Information Sheet: Children and young people who use a wheel chair	1	30 January 2013
Participant Information Sheet: Children and young people on a waiting list for a wheel chair	1	30 January 2013
Participant Information Sheet: Information for Consultees	1	30 March 2013
Participant Consent Form: Parent / Guardian	1	30 January 2013
Participant Consent Form: Parent / Guardian taking part without a child	1	30 January 2013
Participant Consent Form: Partner of young people aged 16 or over	1	30 January 2013
Participant Consent Form: Consultee	1	31 March 2013
Participant Consent Form: Assent form for children and young people aged 15 or under	1	30 January 2013
Participant Consent Form: Young person aged 16 or over	1	30 January 2013
Questionnaire: Parents and Guardians	1.1	18 April 2013
Questionnaire: Yuoung People aged 16 or over	1.1	18 April 2013
Questionnaire: Discrete Choice Experiment	1	31 January 2013
Other: Letter from Funder		15 December 2010
Letter from Sponsor		06 March 2013
Other: Academic Supervisor CV (Prof Jane Noyes)		09 April 2013
Other: Academic Supervisor CV (Prof Rhiannon Tudor-Edwards)		
Other: Academic Supervisor CV (Dr Nigel Harris)		
Investigator CV		09 April 2013
Evidence of insurance or indemnity		09 July 2012

Membership of the Committee

The members of the Ethics Committee who were present at the meeting are listed on the attached sheet.
No declarations of interest were made in relation to this application.

Statement of compliance

The Committee is constituted in accordance with the Governance Arrangements for Research Ethics Committees and complies fully with the Standard Operating Procedures for Research Ethics Committees in the UK.

After ethical review

Reporting requirements

The attached document "After ethical review – guidance for researchers" gives detailed guidance on reporting requirements for studies with a favourable opinion, including:

- Notifying substantial amendments
- Adding new sites and investigators
- Notification of serious breaches of the protocol
- Progress and safety reports
- Notifying the end of the study

The NRES website also provides guidance on these topics, which is updated in the light of changes in reporting requirements or procedures.

Feedback

You are invited to give your view of the service that you have received from the National Research Ethics Service and the application procedure. If you wish to make your views known please use the feedback form available on the website.

Further information is available at National Research Ethics Service website > After Review

13/WA/0143	**Please quote this number on all correspondence**

We are pleased to welcome researchers and R & D staff at our NRES committee members' training days – see details at http://www.hra.nhs.uk/hra-training/

With the Committee's best wishes for the success of this project.

Yours sincerely

Rossela Roberts

Mr Derek James Crawford, MBChB, FRCS
Chair

E-mail: rossela.roberts@wales.nhs.uk

Enclosure: *List of names and professions of members who were present at the meeting and those who submitted written comments.*

"After ethical review – guidance for researchers"

Copy: Sponsor: Prof Robert Woods
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Holywell Community Hospital
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North Wales Research Ethics Committee West

Attendance at Committee meeting on 16 May 2013

Committee Members

Name	*Profession*	*Capacity*	*Present*
Dr. Karen Addy	Clinical Psychologist	Expert	No
Dr. Swapna Alexander	Consultant Physician	Expert	Yes
Ms Valerie Barcoft	Volunteer Worker	Lay +	Yes
Mrs. Kathryn Chester	Research Nurse	Expert	Yes
Dr. Christine Clark	Consultant Obstetrician & Gynaecologist	Expert	Yes
Mr. Derek James Crawford	Consultant Surgeon (Chairman)	Expert	Yes
Mrs. Gwen Dale-Jones	Retired Personal Assistant	Lay +	Yes
Mr. Hywel Lloyd Davies	Solicitor (Alternate Vice-Chairman)	Lay +	No
Ms. Gillian Jones	Student	Lay +	Yes
Dr. Mark Lord	Consultant Pathologist	Expert	No
Dr. Neil McKenzie	Retired Physicist	Lay +	Yes
Dr. Jason Walker	Consultant Anaesthetist	Expert	No
Dr. Philip Wayman White	General Practitioner (Vice-Chairman)	Expert	Yes

Deputy Members

Name	*Profession*	*Capacity*	*Present*
Dr. Michael Cronin	Consultant Paediatrician (deputy to Dr. Clark)	Expert	Yes

Written comments received from

Name	*Profession*	*Capacity*	*Present*
Dr. Jason Walker	Consultant Anaesthetist	Expert	No

In attendance

Name	*Position (or reason for attending)*
Dr. Rossela Roberts	Committee Coordinator

Part of the research infrastructure for Wales funded by the National Institute for Social Care and Health Research, Welsh Government.
Yn rhan o seilwaith ymchwil Cymru a ariannir gan y Sefydliad Cenedlaethol ar gyfer Ymchwil Gofal Cymdeithasol ac Iechyd, Llywodraeth Cymru

NISCHR
Gwasanaeth Moeseg Ymchwil | RES | Research Ethics Service

Pwyllgor Moeseg Ymchwil Gogledd Cymru - Y Orllewin
North Wales Research Ethics Committee - West

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Bangor, Gwynedd
LL57 1UT

n.bray@bangor.ac.uk; nathan.bray@hotmail.com

18 May 2013

Dear Mr Bray,

Study title:	**The Wheels Project: Exploring the economic, methodological and service commissioning implications of assistive mobility technology for disabled children and young people.**
REC reference:	**13/WA/0143**
IRAS project ID:	**117611**

Thank you for your letter of 17 May 2013.

I can confirm the REC has received the documents listed below and that these comply with the approval conditions detailed in our letter dated 17 May 2013

Documents received

The documents received were as follows:

Document	*Version*	*Date*
Cover Letter: documents submitted in compliance with additional conditions		17 May 2013
Letter of invitation to participant Parent of child aged 5 or under	1.1	17 May 2013
Letter of invitation to participant Parent of child aged 6 to 15	1.1	17 May 2013
Letter of invitation to participant Young People aged 16 to 18	1.1	17 May 2013
Participant Information Sheet: Child aged 5 or under	1.1	17 May 2013
Participant Information Sheet: Consultee	1.1	17 May 2013

Approved documents

The final list of approved documentation for the study is therefore as follows:

Document	*Version*	*Date*
Covering Letter		18 April 2013
Cover Letter: documents submitted in compliance with additional conditions		17 May 2013
REC application (submission 117611/440686/1/785)		22 April 2013
Protocol	1.1	30 March 2013
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Participant Information Sheet: Parents and Guardians	1	30 January 2013
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Participant Information Sheet: Children and young people on a waiting list for a wheel chair	1	30 January 2013
Participant Information Sheet: Information for Consultees	1.1	17 May 2013
Participant Consent Form: Parent / Guardian	1	30 January 2013
Participant Consent Form: Parent / Guardian taking part without a child	1	30 January 2013
Participant Consent Form: Partner of young people aged 16 or over	1	30 January 2013
Participant Consent Form: Consultee	1	31 March 2013
Participant Consent Form: Assent form for children and young people aged 15 or under	1	30 January 2013
Participant Consent Form: Young person aged 16 or over	1	30 January 2013
Questionnaire: Parents and Guardians	1.1	18 April 2013
Questionnaire: Yuoung People aged 16 or over	1.1	18 April 2013
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Letter from Sponsor		06 March 2013
Other: Academic Supervisor CV (Prof Jane Noyes)		09 April 2013
Other: Academic Supervisor CV (Prof Rhiannon Tudor-Edwards)		
Other: Academic Supervisor CV (Dr Nigel Harris)		
Investigator CV		09 April 2013
Evidence of insurance or indemnity		09 July 2012

You should ensure that the sponsor has a copy of the final documentation for the study.
It is the sponsor's responsibility to ensure that the documentation is made available to R&D offices at all participating sites.

13/WA/0143	**Please quote this number on all correspondence**

Yours sincerely

Rossela Roberts

Dr Rossela Roberts
Committee Co-ordinator

E-mail: rossela.roberts@wales.nhs.uk

Copy: Sponsor: Prof Robert Woods
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Appendix C: Consent and assent forms

Appendix C.1: Parent consent form for interview

The Wheels project

Consent form for parent/guardian

Short title of project: The Wheels Project

(Please INITIAL each box)

I confirm that I have read and understood the information sheet dated..... version ..... for the above study. I have had the opportunity to consider the information and ask questions. I have had my questions answered satisfactorily. ☐

I understand that my participation and my child's participation is voluntary and that we are free to withdraw at any time without giving a reason and without my child's medical care being affected. ☐

I am happy for the interview to be recorded with an audio recording device. ☐

I understand that you will write a report about the study findings. ☐

I understand that information about me and my child (including names and address) will be held by the research team and that this information will be kept strictly confidential and that no personal information will be used in the study report or other publications. ☐

I agree to allow the information that my child and I give to be used for educational purposes in the future. ☐

I agree that my child and I can take part in the above study. ☐

Name of child	Child's date of birth	
Name of parent/guardian	Date	Signature of parent/guardian
Name of researcher	Date	Signature of researcher

1 copy for the responder
1 copy for the researcher

Version 1: 30/01/13

Appendix C.2: Child (aged 16 or over) consent form for interview

The Wheels Project

Consent form for young people aged 16 or over

(Please put a TICK in each box to say 'yes')

I have read the information sheet about the Wheels Project	☐
I have talked to.....(name of person) about the project	☐
I understand what I am being asked to do.	☐
I know that I can leave the project whenever I want to, and without giving a reason.	☐
I am happy for my words to be recorded.	☐
I know that anything I tell you will be kept private.	☐
I know you will write a report about the project. This will include what I have told you.	☐
I know you will not use my name when you tell people what you found out in the project.	☐
I agree to allow the information I give to be used for educational purposes in the future.	☐
I agree to take part in the above study	☐

Name of young person _____ Young person's date of birth _____

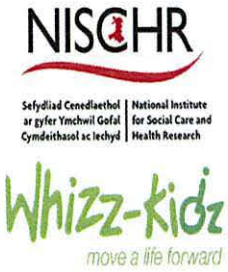
Date _____ Signature of young person _____

Name of Researcher _____ Date _____ Signature of Researcher _____

1 copy for the responder
1 copy for the researcher

Version 1: 30/01/13

Appendix C.3: Child (15 or under) assent form for interview



The Wheels Project

Assent form for children and young people

Child / young person (or if unable, parent on their behalf) to **circle** yes or no on each question:

Have you read (or had read to you) about this project?	Yes / No
Has somebody explained this project to you?	Yes / No
Do you understand what this project is about?	Yes / No
Have you asked all the questions you want?	Yes / No
Have you had your questions answered in a way you understand?	Yes / No
Are you happy to have your words recorded?	Yes / No
Do you understand it's OK to stop taking part at any time?	Yes / No
Are you happy to take part?	Yes / No

If you do want to take part, please write your name below (or ask your parent to write your name)

_____ _____
Your name Date

The person who explained this project to you needs to sign too

_____ _____ _____
Name of Researcher Date Signature of Researcher

Thank you for your help

1 copy for the responder
1 copy for the researcher

Version 1: 30/01/13

Appendix D: Systematic review documentation and additional tables

Appendix D.1: Example database search strategies

Database	Search strategy
CINAHL and MEDLINE	Abstract only, 1997-2012 AB (child* OR adolescen* OR young* OR teen*) AND AB (disab* OR physically impair* OR physical impair* OR handicap* OR dystroph* OR cerebral palsy OR spina bifida OR wheelchair* OR special needs OR amputee OR complex needs OR brain injury OR brain damage*) AND AB (wheelchair OR buggy OR mobility technolog* OR mobility aid OR powered wheelchair OR mobility equipment OR motorised OR mobility training OR wheelchair service OR electric scooter OR pushchair OR mobility NOT crutch* NOT prosthe*)
ASSIA	1997-2012 all(child* OR adolescent* OR young* OR teen*) AND all(disab* OR physically impair* OR physical impair* OR handicap* OR dystrophy* OR cerebral palsy OR spina bifida OR wheelchair* OR special needs OR amputee OR complex needs OR brain injury OR brain damage*) AND all(wheelchair OR buggy OR mobility technology* OR mobility aid OR powered wheelchair OR mobility equipment OR motorised OR mobility training OR wheelchair service OR electric scooter OR pushchair OR mobility) AND all(cost benefit OR cost utility OR cost effective* OR qaly OR quality-adjusted life year OR quality adjusted life year OR health economic* OR economic analyst* OR cost minimisation OR health care cost* OR healthcare cost* OR social economic* OR social care economic*)

Appendix D.2: Study inclusion and exclusion criteria (by review question)

Review Question	Inclusion	Exclusion
1	*Participants:* Aged 18 or under with a long-term need for mobility equipment for management of a physical disability *Interventions:* Powered (independent or parent controlled) and manual wheelchairs, buggies and pushchairs *Outcomes:* All relevant clinical and non-clinical outcomes, including (but not restricted to) improved cognitive, physical or behavioural development, improved motor skills, independence, educational achievement, social interaction, initiative development, physical and/or emotional wellbeing and health-related quality of life *Evidence:* All effectiveness evidence related to effectiveness of assistive mobility technology including randomised controlled trials, quasi-experimental trials, clinical trials, epidemiological research, cohort studies, non-randomised controlled trials, mixed-method research, systematic reviews and survey data.	*Participants:* aged over 18, short-term need for mobility equipment (e.g. wheelchair after leg fracture) *Interventions:* crutches/sticks, walking frames, adapted shoes, callipers and prostheses, adaptive seating *Outcomes:* All outcomes not stated in inclusion criteria *Paper details:* Not written or translated into English, published over 15 years ago
2	*Participants:* Children/young people aged 18 or under with a long-term need for mobility equipment for management of physical disability, parent/carer of a child or young person aged 18 or under with a long-term need for mobility equipment for management of a physical disability, healthcare professionals treating/rehabilitating children/young people aged 18 or under with a long-term need for mobility equipment for management of a physical disability *Interventions:* Powered (independent or parent controlled) and manual wheelchairs, buggies and pushchairs *Outcomes:* All experiences, views, perspectives, thoughts and feelings of children/young people, parents and healthcare professionals towards mobility equipment and provision	*Participants:* children/young people and parents/carers/healthcare professionals of people aged over 18, short-term need for mobility equipment (e.g. wheelchair after leg fracture) *Interventions:* crutches/sticks, walking frames, adapted shoes, callipers & prostheses *Outcomes:* All outcomes

	Evidence: All studies using qualitative methodologies, including ethnographic research, grounded theory research, case studies, phenomenological research, qualitative systematic reviews, meta-ethnography, mixed-method research and survey data.	unrelated to barriers, facilitators, positives and negatives of mobility equipment provision *Paper details:* Not written or translated into English, published over 15 years ago
3	*Audience:* Children/young people aged 18 or under with a long-term need for mobility equipment for management of physical disability, parent/carer of a child or young person aged 18 or under with a long-term need for mobility equipment for management of a physical disability, healthcare professionals treating/rehabilitating children/young people aged 18 or under with a long-term need for mobility equipment for management of a physical disability, decision and policymakers influencing NHS wheelchair services *Publications:* All policy, guidelines, frameworks and government and third sector publications regarding mobility equipment provision, use, maintenance and funding	*Audience:* children/young people and parents/carers/healthcare professionals of people aged over 18, service users with short-term need for mobility equipment (e.g. wheelchair after leg fracture) *Publications:* Policy and guidelines from outside of United Kingdom, Obsolete or out-of-date policies and guideline, published over 10 years ago
4	*Participants:* Aged 18 or under with a long-term need for mobility equipment for management of a physical disability *Interventions:* Powered (independent or parent controlled) and manual wheelchairs, buggies and pushchairs *Outcomes:* All relevant clinical and non-clinical outcomes, including (but not restricted to) improved cognitive, physical or behavioural development, improved motor skills, independence, educational achievement, social interaction, initiative development, physical and/or emotional well-being and health-related quality of life. Direct and indirect costs,	*Participants:* aged over 18, short-term need for mobility equipment (e.g. wheelchair after leg fracture) *Interventions:* crutches/sticks, walking frames, adapted shoes, callipers and prostheses *Outcomes:* All outcomes not stated in inclusion criteria

	impacts on quality-adjusted life years gained, utility scores, quality of life measures and incremental cost-effectiveness will inform the economic outcomes. *Evidence:* All economic evidence related to assistive mobility technology including cost-benefit, cost-utility and cost-effectiveness analyses. Partial economic evaluations (including cost analyses, cost-description studies and cost-outcome descriptions) will also be included. Economic evaluations conducted alongside RCTs, quasi-experimental trials, clinical trials, epidemiological research, cohort studies and non-randomised controlled trials will all be considered	*Paper details:* Not written or translated into English, published over 15 years ago

Appendix D.3: Data extraction criteria by evidence type

Intervention evidence	Aims, objectives, hypotheses, study type, methodology, randomisation details, number of groups, number in each group, number completed in each group, data collection time points, participant characteristics, participant age range, type of intervention(s), inclusion/exclusion criteria, country/ethnicity, baseline characteristics, content of intervention(s), duration of intervention(s), control intervention(s), follow-up period, outcomes and measures, narrative summary of findings (including statistical significance, confidence intervals and effect size), identified themes/concepts.
Opinion evidence	Aims, objectives, hypotheses, study type, methodology, number of study groups, number in each group, number completed in each group, data collection time points, participant characteristics, participant age range, type of intervention(s), inclusion/exclusion criteria, country/ethnicity, follow-up period, narrative summary of findings, identified themes/concepts.
Policy/NFPO literature	Type of publication, topic, aims, objectives, related conditions and disabilities, age range of affected individuals/target audience, related interventions, narrative summary of recommendations and guidance.
Economic evidence	Perspective, aims, objectives, hypotheses, study type/methodology, price year/currency, randomisation details, number of groups, number in each group, number completed in each group, data collection time points, measure of benefit, participant characteristics, participant age range, type of intervention(s), inclusion/exclusion criteria, country/ethnicity, baseline characteristics, content of intervention(s), duration of intervention(s), control intervention(s), follow-up period, outcomes and measures, narrative summary of findings (including statistical significance, confidence intervals and effect size), identified economic costs and implications, cost per QALY/Incremental cost-effectiveness ratio conclusions, inflated (2012) cost per QALY/Incremental cost-effectiveness ratio conclusions.

Appendix D.4: Data extraction tools

DATA EXTRACTION TOOL 1- Intervention Evidence				
Study ID				
Study Title				
Author(s)				
Reviewer				
Aims/objectives/hypotheses				
METHOD				
Study type				
Randomised?	Yes	No	If yes, allocation type:	
No. of groups				
No. in each group				
No. completed in each group				
Data collection time points				
PARTICIPANTS				
Types of participants				
Age range				
Inclusion criteria				
Exclusion criteria				
Ethnicity/country				
Baseline characteristics				

INTERVENTIONS	
Type of intervention(s)	
Content of intervention(s)	
Duration of intervention(s)	
Control intervention(s)	
Follow-up period	
OUTCOMES	
Outcome and measure(s) 1	
Outcome and measure(s) 2	
Outcome and measure(s) 3	
Outcome and measure(s) 4	
Outcome and measure(s) 5	
Outcome and measure(s) 6	
Outcome and measure(s) 7	
Outcome and measure(s) 8	
Outcome and measure(s) 9	
Outcome and measure(s) 10	
SUMMARY OF RESULTS	
Narrative summary	
Identified themes/concepts	

DATA EXTRACTION TOOL 2- Opinion Evidence	
Study ID	
Study Title	
Author(s)	
Reviewer	
Aims/objectives/hypotheses	
METHOD	
Study type/methodology	
No. of groups	
No. in each group	
No. completed in each group	
Data collection time points	
PARTICIPANTS	
Types of participants (parent, child or professional?)	
Age range	
Type of intervention(s)	
Inclusion criteria	
Exclusion criteria	
Ethnicity/country	
Follow-up period	
SUMMARY OF RESULTS	
Narrative summary	
Identified themes/concepts	

DATA EXTRACTION TOOL 3- Policy Literature	
Policy ID	
Policy/guidance title	
Type of publication	
Author(s)/organisation	
Reviewer	
Aims/objectives of policy/guidance	
AUDIENCE/POPULATION	
Target audience	
Population (condition, age etc.)	
Related intervention(s)	
SUMMARY OF PUBLICATION	
Narrative summary of key points	
Practice/policy recommendations and guidance	

DATA EXTRACTION TOOL 4- Economic Evaluations				
Study ID				
Study Title				
Author(s)				
Reviewer				
Aims/objectives/hypotheses				
Stated perspective				
Price year/currency				
METHOD				
Study type				
Randomised?	Yes	No	If yes, allocation type:	
No. of groups				
No. in each group				
No. completed in each group				
Data collection time points				
Measure of benefit (QALY, life years gained etc)				
PARTICIPANTS				
Types of participants				
Age range				
Inclusion criteria				
Exclusion criteria				
Ethnicity/country				
Baseline characteristics				
INTERVENTIONS				
Type of intervention(s)				

Content of intervention(s)	
Duration of intervention(s)	
Control intervention(s)	
Follow-up period	
OUTCOMES	
Outcome and measure(s) 1	
Outcome and measure(s) 2	
Outcome and measure(s) 3	
Outcome and measure(s) 4	
Outcome and measure(s) 5	
Outcome and measure(s) 6	
Outcome and measure(s) 7	
Outcome and measure(s) 8	
Outcome and measure(s) 9	
Outcome and measure(s) 10	
SUMMARY OF RESULTS	
Narrative summary	
Cost per QALY/ICER conclusions	

DATA EXTRACTION TOOL 5- Other Economic Data	
Study ID	
Study Title	
Author(s)	
Reviewer	
Aims/objectives/hypotheses	
Price year/currency (if applicable)	
METHOD	
Study type/methodology	
No. of groups	
No. in each group	
No. completed in each group	
Data collection time points	
PARTICIPANTS	
Types of participants (parent, child or professional?)	
Age range	
Type of intervention(s)	
Inclusion criteria	
Exclusion criteria	
Ethnicity/country	
Follow-up period	
SUMMARY OF RESULTS	
Narrative summary	
Identified economic costs and implications	

Appendix D.5: Quality appraisal tools and outcomes

Key

Y-Yes

N- No

NC- Not clear

Qualitative Study Appraisal Outcomes [1a]

	Question									
Paper	**1**	**2**	**3**	**4**	**5**	**6**	**7**	**8**	**9**	**10**
Evans et al, 2007 [7]	Y	Y	Y	Y	NC	N	Y	NC	Y	High Value
Lawlor et al, 2006 [38]	Y	Y	Y	NC	Y	N	Y	NC	Y	High Value
Curtin & Clarke, 2005 [40]	Y	Y	Y	NC	Y	N	Y	NC	Y	High Value
Wiart et al, 2004 [31]	Y	Y	Y	NC	Y	N	Y	Y	Y	High Value
Durkin, 2009 [32]	Y	Y	Y	NC	Y	Y	Y	Y	Y	High Value

Questionnaire Survey Appraisal Outcomes [2a]

	Question											
Paper	**1**	**2**	**3**	**4**	**5**	**6**	**7**	**8**	**9**	**10**	**11**	**12**
Guerette et al, 2005 [33]	Y	Y	Y	NC	NC	Y	Y	NC	Y	N	NC	Y
Shahid, 2004 [39]	Y	Y	Y	NC	Y	N	NC	NC	N	N	Y	Y
Home & Ham, 2003 [34]	Y	Y	Y	N	NC	N	N	NC	N	N	Y	Y
Staincliffe, 2003 [35]	Y	Y	Y	N	Y	N	NC	NC	N	N	NC	Y
Wiart et al, 2003 [36]	Y	Y	Y	NC	NC	N	Y	NC	N	N	NC	Y
Tefft et al, 2011 [24]	Y	Y	Y	NC	NC	N	Y	Y	Y	Y	NC	Y
Benedict et al 1999 [30]	Y	Y	Y	N	NC	N	Y	Y	N	N	N	Y

Case Study Appraisal Outcomes [3a]

	Question									
Paper	**1**	**2**	**3**	**4**	**5**	**6**	**7**	**8**	**9**	**10**
Huhn et al, 2007 [27]	Y	Y	NC	N	Y	N	N	N	NC	NC
Jones et al, 2003 [21]	Y	Y	NC	N	Y	N	N	NC	NC	NC

Descriptive / Cross-Sectional Study Appraisal Outcomes [4a]

	Question										
Paper	**1**	**2**	**3**	**4**	**5**	**6**	**7**	**8**	**9**	**10**	**11**
Østensjø et al, 2005 [29]	Y	Y	NC	Y	NC	NC	Y	Y	Y	NC	Moderate Value

Randomised Controlled Trial Appraisal Outcomes [5a]

	Question									
Paper	**1**	**2**	**3**	**4**	**5**	**6**	**7**	**8**	**9**	**10**
Jones et al, 2012 [23]	Y	Y	Y	Y	Y	Y	NC	Appropriate presentation	High accuracy	NC

Quasi-Experimental Study Appraisal Outcomes [6a]

	Question										
Paper	**1a**	**1b**	**2a**	**2b**	**2c**	**3a**	**3b**	**4a**	**4b**	**5a**	**5b**
Bottos et al, 2001 [25]	Y	NC	Y	Y	N	Y	Y	Y	N	NC	NC
Meiser & McEwan, 2007 [28]	Y	Y	Y	Y	N	NC	NC	Y	N	NC	NC
Furumasu et al, 2008 [22]	Y	Y	NC	Y	Y	Y	Y	NC	N	NC	Y

Economic Evaluation Appraisal Outcomes [7a]

	Question											
Paper	**1**	**2**	**3**	**4a**	**4b**	**4c**	**5**	**6**	**7**	**8**	**9**	**10**
Neilson et al, 2000 [48]	Y	Y	NC	Y	Y	NC	Y	NC	NC	N	Y	Y
Frontier Economics, 2010 [52]	Y	NC	NC	N	NC	NC	NC	Y	NC	NC	NC	Y

Supplementary References for appendix B.5

1a. Critical Appraisal Skills Programme (CASP): **Qualitative Study CASP Tool** [http://www.sph.nhs.uk/what-we-do/public-health-workforce/resources/critical-appraisals-skills-programme]

2a. Centre for Evidence Based Management (CEBMa): **Questionnaire Survey CEBMa Tool** [http://www.cebma.org/ebp-tools/]

3a. Centre for Evidence Based Management (CEBMa): **Case Study CEBMa Tool** [http://www.cebma.org/ebp-tools/]

4a. Milton Keynes Primary Care Trust: **11 questions to help you make sense of descriptive/cross-sectional studies** [http://reache.files.wordpress.com/2010/03/cross-sectional-appraisal-tool.pdf]

5a. Critical Appraisal Skills Programme (CASP): **Randomised Controlled Trial CASP Tool** [http://www.sph.nhs.uk/what-we-do/public-health-workforce/resources/critical-appraisals-skills-programme]

6a. Greenhalgh T, Robert G, Bate P, Macfarlane F, Kyriakidou O: *Diffusion of Innovations in Health* Service Organisations: A systematic literature review. Oxford: Blackwell Publishing, BMJ Books [http://onlinelibrary.wiley.com/doi/10.1002/9780470987407.app2/pdf]

7a. Critical Appraisal Skills Programme (CASP): Economic Evaluation CASP tool [http://www.sph.nhs.uk/what-we-do/public-health-workforce/resources/critical-appraisals-skills-programme]

Appendix D.6: Full disclosure of studies and reports included in systematic review, with extracted data

Author(s) *See full paper for references*	Study or document type; methodology*, aims / objectives / hypotheses*; outcomes / measures* **NA for reports*	Participants* / patient group; sample size (SS)*; country **NA for reports*	Main findings (significant results for quantitative studies)
Whizz-Kidz (2011) [47]	Report	Young wheelchair users; UK	Aim to provide the right wheelchair, based on the right assessments, at the right time. Recommend changes to eligibility criteria to allow provision based on need; reducing expenses so cost of wheelchairs is not prohibitive; ensuring wheelchair services are high on the political agenda; clear and appropriate minimum standards; initial investment to kick-start changes; and joint working across local and national government departments.
Audit Commission (2002) [12]	Report	Wheelchair users; UK	Ineffectual service commissioning is a major cause of service issues and commissioners are not considering the vital contribution that wheelchair services provide to independence and reductions in morbidity.
Barnardos and Whizz-Kidz (2006) [43]	Report	Young wheelchair users; UK	Recommendations included reduced waiting times for assessment and delivery; ensure wheelchairs are suitable/have essential accessories, taking into account additional needs of carers/service users; listen to the needs and choices of children.
Benedict, et al (1999) [30]	Descriptive, non-parametric and qualitative procedures were used. Telephone survey and interviews used. To determine the impact of assistive technology device use on child and family function and whether use by young children is related to caregiver satisfaction with a device. **Measures:** Quebec User Evaluation of Satisfaction with assistive Technology- modified (QUEST); Pediatric Evaluation of Disability Inventory (PEDI)	Parents of a child whom a request for assistive technology device had been placed between child's second and fourth birthday; 11 of 13 children diagnosed with cerebral palsy; Child age range: 34 to 54 months SS=13; USA	No statistical analysis conducted. Regular manual wheelchair use in all places improved functional skill and reduced need for caregiver assistance. Evidence that the environment (manoeuvrability in the home) and the user (child's interest in device) made devices difficult to use regularly and effectively. Carer statements indicated that wheelchairs offered increased independence and reduced need for assistance.
Bottos et al (2001) [25]	Quasi-experimental trial To investigate the effects of early PWC provision on objective outcome indices and subjective outcome indexes. To see which individuals could achieve a good-enough driving competence and what conditions would influence the achievement/non-achievement of this competence. **Measures:** Performance Intelligence Quotient (PIQ) score of the Leiter International Performance Scale; Verbal Intelligence Quotient (VIQ) score of the Peabody; Developmental Verbal Scale; Gross Motor Functional Measure (GMFM); Canadian Occupational Performance Measure (COPM); Furumasu's Driving	Children diagnosed with cerebral palsy; Age range: 3 to 8 years SS=25; Italy	PWC provision had significant positive effects to activities of daily life (in the dimension of functional limitation) ($p<0.00001$), satisfaction with performance ($p<0.00001$) and PWC driving competence after 6 to 8 months of use ($p<0.01$). Confidence intervals not reported.

	Test; Parental perceptions; Impact of Childhood Illness Scale (ICIS)		
Care Services Improvement Partnership (2006) [44]	Report	Wheelchair users; UK	Commissioners, in partnership with providers, need to ensure that children and carers are involved in decisions about their care; that wheelchair provision is based on individual need; that transition from child to adult services is planned for; that wheelchairs are provided promptly based on multiagency assessment; that service users are provided with wheelchairs that can 'grow' to meet their changing needs; and that there is a reasonable balance between risk of denying a wheelchair and the safety of a supplied wheelchair.
Curtin and Clarke (2005) [40]	Biographical research To investigate the life stories of a small number of young people with physical disabilities, in particular focusing on their educational experiences. **Measures:** Qualitative interviews	Children using manual or powered wheelchairs; Age range: 10 to 13 years SS=9; UK	Physical disability and wheelchair use had an effect on the choice of school a child attends (mainstream or segregated special school) based on facilities, level of assistance/support and attitudes of other pupils.
Deitz et al (2002) [26]	Single-subject withdrawal design To explore the effects of a powered mobility riding toy on the participation behaviours of young children with complex developmental delays. **Measures:** Child-initiated movement; Initiation of contact with others; Affect (positive, neutral and negative facial expression).	Young children with complex developmental delays; Age range: 4 to 5 years SS=2; USA	No statistical analysis conducted. Use of a powered riding toy had positive impact on initiation of movement occurrences.
DoH (2004) [16]	Report	Disabled children and those with complex healthcare needs; UK	Disabled children should receive coordinated, high-quality, child and family centred services and these services should promote social inclusion. Appropriate housing and assistive mobility technology is essential and should be promoted by facilitating families with disabled children to live in suitable housing and have the appropriate assistive mobility technology to promote the wellbeing of disabled children and their families. Integration across services that supports personalised, child-centred care to every disabled child.
DoH Commissioning Team (2010) [9]	Report	Wheelchair users; UK	Key issues identified included focusing on the needs of the user; achieving timely access; ensuring equity of provision; improving outcomes for service users; adopting a preventative approach to service provision; shifting balance of resources from service management to wheelchair provision; and encouraging innovation.

Durkin (2009) [32]	Qualitative Grounded Theory To explore the question- "How does a child learn to use powered mobility to explore their environment". **Measures:** Qualitative interviews	Children with disabilities using powered wheelchairs and peer professionals; Age range: 5-12 SS= Typically developing children: 11; Powered mobility users: 18; Peer professionals: 22; UK	Wheelchairs can be more than a tool for movement: a source of enjoyment through play and games, as well as a tool for understanding movement and maintaining development. Playing and driving to learn are important. Parental input was best as a 'responsive partner' facilitating learning.
Evans et al (2007) [7]	Qualitative analysis- a priori interviews To examine the experiences of severely physically disabled young people using electric powered indoor/outdoor chairs (EPIOCs). To address the gap in the young EPIOC user literature by undertaking a qualitative analysis of young people's experience using NHS supplied EPIOC. **Measures:** Qualitative interviews	Young people with physical disabilities using manual and powered wheelchairs; Age range: <18 years. SS=18; UK	NHS wheelchair services generally considered satisfactory and helpful, although waiting times were an issue. Use of EPIOC was considered positive and beneficial for development and independence. More training was wanted to help limit accidents. Bulkiness and difficulty of transporting were issues.
Frontier Economics (2011) [52]	An analysis of Whizz-Kidz work with a primary care trust to improve wheelchair provision. To examine the quantitative impacts of Whizz-Kidz' work with the NHS on children accessing these services. **Measures:** Cost per QALY of meeting additional unmet demand. Secondary sources of utility data	Children with physical disabilities using wheelchairs for mobility; Age range: not stated. SS=not stated; UK	Meeting unmet demand by Whizz-Kidz cost an extra £108,000 and provided an additional 10.7 to 14 QALYs. This resulted in a cost per QALY of between £7,700 and £9,800 to meet additional unmet demand.
Furumasu et al (2008) [22]	Case series To provide more objective evidence of the benefits of powered mobility for young children to aid clinicians in justifying a PWC recommendation to families and physicians. **Measures:** Adaptive Social Behaviour Inventory (ASBI); Preschool and Kindergarten Behaviour Scales (PKBS); Peabody Picture Vocabulary Tests (PPVT-IIIA); Preschool Language Scale-3 (PLS-3); Symbolic Play Assessment; Survey of Technology Use.	Children with orthopaedic disabilities (non-cerebral palsy) and children with cerebral palsy; Age range: 18 to 72 months. SS=23; USA	Significant positive effects after PWC provision to pro-social adaptive social behaviour ($F=5.30$, $p<.05$ at 95%CI); interactions with family ($F=3.2$, $p<.05$ at 95%CI); indoor play motor activities ($F=4.53$, $p<.05$ at 95%CI); quality of interactive play ($F=4.24$, $p<.05$ at 95%CI); and developmental level of symbolic play ($F=4.9$, $p<.05$ at 95%CI).
Guerette et al (2005) [33]	Questionnaire Survey To identify the most common reasons for not recommending a powered wheelchair, and the reasons why a child who is recommended a PWC does not receive one. To gather information regarding the current practice used to evaluate a child for a PWC and the typical recommendation for children who are not prescribed a PWC. To obtain objective and subjective	Suppliers and Clinicians; Child age range: 2 to 6 years SS=140; USA	Cognitive factors and behavioural factors were considered (by clinicians and suppliers) the most common cause for non-recommendation of PWC, while funding issues and lack of family support were considered most common reasons why a PWC was not received.

	baseline data regarding the impact of PWC on young children. **Measures:** Qualitative interviews		
HM Treasury and Department for Education and Skills (2007) [45]	Report	Children and young people with disabilities; UK	Government should take action in three priority areas to improve outcomes for disabled children: access and empowerment; responsive services and timely support; improving quality and capacity.
Home and Ham (2003) [34]	Retrospective survey Exploring the experiences of children issued with powered mobility by the charity Whizz-Kidz. **Measures:** questionnaire survey	Families of children who had been issued with a PWC from Whizz-Kidz between 1990 and 2000; Child age range: <7 years. SS=57; UK	General consensus on a number of positive effects of PWC use including independent mobility and facilitation of integration with other children. Several issues were highlighted, including costs of maintenance and suitability of wheelchairs.
Huhn et al (2007) [27]	Case report/description To describe the physical therapist's clinical decision-making related to power mobility for a child with multiple disabilities. **Measures:** Driving wheelchair through a standard width doorway; Manoeuvring through three cones set three feet apart; Driving wheelchair through a 100-foot hallway with typical school traffic.	Children with spastic quadriplegic cerebral palsy; Age range: 9 to 12 years. SS=1; USA	No statistical analysis conducted. For disabled children with multiple impairments mid-wheel drive PWCs facilitate better control as compared with rear-wheel drive equivalent PWCs. Results indicated that length of time training and learning to use a PWC correlated with achieving independence. Motivation and level of frustration impact length of time taken to achieve independence.
Jones et al (2003) [21]	Case report To show that a child as young as 20 months of age can learn to use a PWC. To describe the procedures used in training a young child to use a PWC and in evaluating the developmental changes made after receiving the PWC. **Measures:** Powered mobility skills- 7 question powered mobility skills indicator; Battelle Developmental Inventory (BDI); PEDI.	Child with spinal muscular atrophy; Age range: 17 to 23 months. SS=1; USA	No statistical analysis conducted. Communicative, personal-social and cognitive development increased by greater than the expected age-equivalent scores (for typically developing peers) after provision of PWC. Positive trends in self-care, mobility and social function after intervention.
Jones et al (2012) [23]	Randomised controlled trial To identify any effects of PWC on the development and function of young children with severe motor impairments. **Measures:** BDI; PEDI; Early Coping Inventory (ECI)	Children with motor impairment preventing functional independent mobility; Age range: 14 to 30 months. SS=14 (control), 14 (intervention); USA	Significant improvements after PWC intervention (compared to control) in receptive communication (p=.03, Effect size=6.1 [0.95-9.2] at 90% CI); mobility functional skill (p=.04, Effect size=6.5 [2-11] at 90%CI); need for caregiver assistance for mobility (p=.01, Effect size=12.35 [6.5-20.5] at 90%CI); need for caregiver assistance for self-care (p=.0007, Effect size=11.95 [7.5-16.15] at 90%CI); and overall development scores (p=.083, Effect size=2.0 [0.0-3.5] at 90%CI).
Lawlor et al (2006) [38]	Qualitative in-depth interviews To ascertain from families of children with cerebral palsy the features of social, attitudinal and physical	Families of children with cerebral palsy; Child age range: 4 to 17 years SS=12; USA	PWC perceived to reduce the level of support required from parents/carers. Lack of space, ramps and adequate paths restricted access and leisure activities. Public buildings, toilets,

	environments which facilitate or restrict participation. **Measures:** Qualitative analysis		transport and shopping aisles restricted participation. Barriers were costs, effects on earnings, negative attitudes of public and lack of knowledge regarding benefits. Wheelchair services act as barriers to participation due to long waiting times for wheelchairs.
Meiser and McEwen (2007) [28]	ABA Single Subject design To compare propulsion in young children with spina bifida when using two styles of wheelchairs: an ultralight titanium rigid-frame wheelchair and a lightweight aluminium folding frame wheelchair. **Measures:** Speed for 50ft in controlled environment; speed in a typical school activity; moving in the hallway as the line-leader of classmate; distance of propulsion in familiar surroundings; energy expenditure; energy expenditure index; perceived exertion.	Children with spina bifida (L2 or above); Age range: <6 years. SS=2; USA	No statistical analysis conducted. Ultralight wheelchairs facilitated better results than lightweight wheelchairs in propulsion. Both parents and children indicated preference for the ultralight chairs over lightweight chairs.
Muscular Dystrophy Campaign (2010) [41]	Report	Wheelchair users; UK	Waiting times for wheelchairs should be a maximum of 18 weeks from initial referral to delivery. A national consensus of a uniform eligibility criterion is required to improve equality in wheelchair provision criterion should be established to improve equity in services. Maintenance of wheelchairs (including privately funded chairs) should be covered by NHS primary care trusts.
Muscular Dystrophy Campaign (2011) [42]	Report	Wheelchair users with muscular dystrophy and other neuromuscular conditions; UK	Key identified issues included supplying wheelchairs that promote independence; support good posture; allow exploration and opportunities for play; promote development; and restrict development of deformities.
National Assembly for Wales (2010) [11]	Report	Wheelchair users; UK	Recommendations included defining a strategic plan to promote better integration with other services and organisations; performance measures focussing on outcomes for users; better provision of information to users; streamlining of referrals process; exploring the possibility of pooling existing budgets; reviewing arrangements for short-term loans of wheelchairs; and ensuring that regular reviews for service users (especially those with changing needs) are maintained.
Neilson et al (2000) [48]	Case series / cost-effectiveness analysis The present exploratory study considered the impact of a wide range of surgical and orthotic interventions on the quality of life of children and adults with profound intellectual and multiple disabilities. **Measures:** Short Form 36 (SF36); QALY gains	Children and adults with profound intellectual and multiple disabilities (22=cerebral palsy); Age range: 2 to 55 years (mean age= 19). SS=1 relevant to review; Scotland	Cost per QALY (compared with a 'do nothing' scenario) for provision of an EPIOC ranged from £734 to £1378 (dependent on time horizon) based on a cost per wheelchair intervention ranging from £1500 to £2000.
NHS Modernisation Agency (2005) [13]	Report	Wheelchair users; UK	Recommendations included agreed eligibility criteria and information/tuition for each chair supplied; efficient use of resources and minimising delay; 100% of standard prescriptions

			delivered within 10days; and all users made aware of mechanism for contact/review.
Østensjø et al (2005) [29]	Cross-sectional To describe all assistive devices and other environmental modifications provided to support everyday activities in young children with cerebral palsy, and the benefits of these modifications for functioning and care giving. **Measures:** PEDI; Classification of Technical Aids for Persons with Disabilities; Questionnaire-sociodemographic factors and associated problems.	Children with cerebral palsy and their parents; Age range: 2 to 7.5 years. SS=95; Norway	Strong correlation between the amount of caregiver assistance and the number of modifications in use for purposes of mobility ($p<0.001$) and level of independence and the demands placed on caregivers ($p=0.002$ to <0.001). Confidence intervals not reported.
Prime Minister's Strategy Unit (2005) [14]	Report	People with disabilities; UK	Government policy should enable young disabled children and their families to live 'ordinary' lives, through effective support in mainstream settings. There should be timely access to wheelchairs and a specific key worker for each family with high needs. Supply based on a multi-agency assessment and should be delivered promptly after assessment. Strategic planning between health, social services and education.
Scottish Executive (2006) [46]	Report	Wheelchair users; Scotland	Recommendations included comprehensive access to multi-disciplinary teams for assessment and review; all services undertaken in child-oriented facilities; clinics providing access to specialist paediatric clinical expertise; access to extended wheelchair loan programmes; and the establishment of properly functioning multi-agency links.
Shahid (2004) [39]	Questionnaire Survey To explore the opinions between health professionals and parents of children with cerebral palsy with regard to factors affecting buggy-to-wheelchair progression. **Measures:** Questionnaire survey	Parents of children with cerebral palsy and healthcare professionals; Child age range: 3 to 14 years. SS= Parents: 28 Professionals: 17; UK	Most common issues preventing buggy-to-wheelchair progression were transport problems (private and public); housing problems inhibiting storage and mobilisation of wheelchair; lack of information regarding positives of progression; and carer difficulty in manoeuvring a wheelchair. There were difference in opinions between professionals and parents.
Staincliffe (2003) [35]	Questionnaire survey To find out the current policy and practice for issuing PWC to children. **Measures:** Questionnaire survey	NHS wheelchair services; Child age range: not stated. SS=69; UK	54% of NHS wheelchair services receive referrals for PWC for children under the age of 5, 10% had an age exclusion policy (children less than 5 years old not eligible for PWC).
Tefft et al (2011) [24]	Questionnaire survey To evaluate the impact of PWC on parental stress, negative emotions, perceived social interactions and parental satisfaction with wheelchair characteristics	Parents of children with orthopaedic disabilities or cerebral palsy; Child age range: 18 to 42 months (children with orthopaedic disabilities), 18 to 72 months (children with cerebral	PWC provision facilitated significant improvement in parents' satisfaction with child's ability to go where they desire ($F[2,21]=11.69$, $p<.05$); interactions with family ($F[2,21]=3.3$, $p<.05$); parental satisfaction with child's social and play skills ($F[2,21]=3.27$, $p<.05$); and parents' belief that the general public

	such as size and durability. **Measures:** Developmental Observation Checklist System Part III Parental Stress and Support Checklist (PSSC); Matching Assistive Technology & Child (MATCH); Survey of Technology Use; QUEST	palsy). SS=23; USA	accepts their child ($F[2,21]=3.65$, $p<.04$).
Welsh Assembly Government (2005) [8]	Report	Children and young people (with and without disabilities); Wales	Disabled children should have access to any assistive mobility technology they require, wherever they require it, and services should be integrated across health, social care and education, to enable multiagency assessments and streamline of provision. Key priorities include prompt services; in depth assessments; family support services; educational support services; clear and accurate information; and emotional support services.
Wiart et al (2003) [36]	Telephone survey/interview To explore environmental (i.e. physical, social and attitudinal) barriers and facilitators to successful powered mobility use with the participants and their families. **Measures:** Questionnaire survey	Young people who had received a PWC at age 18 or under; Age range: 4.5 to 27.5 years (mean= 15.2 years). SS=66; Canada	Reasons why PWC use was discontinued included concerns regarding safety; poor fit between type of PWC/access system and user; change in medical/functional abilities; and lack of success with using PWC effectively. Barriers to PWC use included physical barriers at public and private buildings and transportation issues.
Wiart et al (2004) [31]	Phenomenology To explore parents' experiences and perceptions of their children's experiences with powered mobility. **Measures:** Qualitative interviews	Mothers of children with physical disabilities using powered mobility devices; Child age range: 10 to 18 years. SS=5; Canada	Consensus that PWC use can facilitate a number of benefits including independence; participation in social and peer age-appropriate activities; facilitation meaningful relationships; and development of personal control. Initial attitude to both PWC and manual chairs was of sadness and despair, which after PWC provision developed into positive feelings about the benefits of PWC.

Appendix D.7: Full disclosure of full-texts excluded from the systematic review

Author(s)	Title	Publication Details	Reason for Exclusion
Aldersea, P. (1999)	NHS wheelchairs and seating for disabled children	British Journal of Therapy & Rehabilitation, 6(8), pp. 408-412	Not a research study
Alriksson-Schmidt, A.I., Wallander, J. and Biasini, F. (2007)	Quality of life and resilience in adolescents with a mobility disability	Journal of Pediatric Psychology, 32(3), pp. 370-379	Not directly related to wheelchair outcomes
Bamer, A.M., Connell, F.A., Dudgeon, B.J. and Johnson, K.L. (2010)	Frequency of purchase and associated costs of assistive technology for Washington State Medicaid program enrolees with spina bifida by age	Disability and Health Journal, 3, pp. 155-161	Not an economic evaluation and limited relevance to UK population
Bartlett, D.J., Chiarello, L.A., Mccoy, S.W., Palisano, R.J., Rosenbaum, P.L., Jeffries, L., Fiss, A.L. and Stoskopf, B. (2010)	The Move & PLAY study: an example of comprehensive rehabilitation outcomes research	Physical Therapy, 90(11), pp. 1660-1672	Not directly related to wheelchair outcomes
Beekman, C.E., Miller-Porter, L. and Schoneberger, M. (1999)	Energy cost of propulsion in standard and ultralight wheelchairs in people with spinal cord injuries	Physical Therapy, 79(2), pp. 146-158	Not related to review questions
Birenbaum, A. (2010)	Children, disability, and chronic care	Intellectual and Developmental Disabilities, 48(5), pp. 393-395	Not a research study
Bode, H., Weidner, K. and Storck, M. (2000)	Quality of life in families of children with disabilities	Developmental Medicine & Child Neurology, 42(5), pp. 354-354	Not a research study
Bottos, M. and Gericke, C. (2003)	Ambulatory capacity in cerebral palsy: prognostic criteria and consequences for intervention	Developmental medicine and child neurology, 45(11), pp. 786-790	Not a research study
Brown, J.P. (2001)	Orthopaedic care of children with spina bifida: you've come a long way, baby!	Orthopaedic nursing / National Association of Orthopaedic Nurses, 20(4), pp. 51-58	Not a research study
Chau, T. (2007)	Intelligent systems in pediatric rehabilitation	Assistive Technology, 19(1), pp. 17-20	Not a research study
Chen, C.C., Heinemann, A.W., Bode, R.K., Granger, C.V. and Mallinson, T. (2004)	Impact of pediatric rehabilitation services on children's functional outcomes	American Journal of Occupational Therapy, 58(1), pp. 44-53	Outcomes not specifically related to wheelchair use

Chen, X., Ragonesi, C., Galloway, J.C. and Agrawal, S.K. (2011)	Training toddlers seated on mobile robots to drive indoors amidst obstacles	IEEE Transactions On Neural Systems And Rehabilitation Engineering: A Publication Of The IEEE Engineering In Medicine And Biology Society, 19(3), pp. 271-279	Not related to review questions
Chiarello, L.A., Palisano, R.J., Maggs, J.M., Orlin, M.N., Almasri, N., Lin-Ju Kang and Hui-Ju Chang (2010)	Family priorities for activity and participation of children and youth with cerebral palsy	Physical Therapy, 90(9), pp. 1254-1264	Not related to wheelchair use
Cox, D.L. (2003)	Wheelchair needs for children and young people: a review	British Journal of Occupational Therapy, 66(5), pp. 219-223	Literature review- references unpicked and screened for relevance
Davies, A., De Souza, L.H. and Frank, A.O. (2003)	Changes in the quality of life in severely disabled people following provision of powered indoor/outdoor chairs	Disability and rehabilitation, 25(6), pp. 286-290	Wrong age range (mean age = 52)
De Judicibus, M.A. and McCabe, M.P. (2005)	Economic deprivation and its effects on subjective wellbeing in families of people with multiple sclerosis	Journal of Mental Health, 14(1), pp. 49-59	Not related to wheelchairs
Dicianno, B.E., Bellin, M.H. and Zabel, A.T. (2009)	Spina bifida and mobility in the transition years.	American Journal of Physical Medicine & Rehabilitation / Association of Academic Physiatrists, 88(12), pp. 1002-1006	Wrong age range (mean age = 21)
Fernandes, T. (2006)	Independent mobility for children with disabilities.	International Journal of Therapy & Rehabilitation, 13(7), pp. 329	Not a research study
Flodin, E. (2007)	Interactive design -- the desire for autonomous upright mobility: a longitudinal case study.	Technology & Disability, 19(4), pp. 213-224	Not related to review questions
Frank, A., Neophytou, C., Frank, J. and De Souza, L. (2010)	Electric-powered indoor/outdoor wheelchairs (EPIOCs): users' views of influence on family, friends and carers	*Disability and Rehabilitation: Assistive Technology*, 5(5), pp. 327-38	Wrong age range (mean age = 46 for women, 38 for men)
Fuhrer, M.J. (2007)	Assessing the efficacy, effectiveness, and cost-effectiveness of assistive technology interventions for enhancing mobility	Disability and Rehabilitation: Assistive Technology, 2(3), pp. 149-158	Opinion article- not economic evaluation / primary data

Gamble, D. and Satcher, J. (2002)	Rehabilitation outcomes, expenditures, and the provision of assistive technology for persons with traumatic brain injury	Journal of applied rehabilitation counselling, 33(3), pp. 41-44	Wrong age range (mean age = 35.6)
Hatta, T., Nishimura, S., Inoue, K., Yamanaka, M., Maki, M., Kobayashi, N., Kishigami, H. and Sato, M. (2007)	Evaluating the relationships between the postural adaptation of patients with profound cerebral palsy and the configuration of the Seating Buggy's seating support surface.	Journal of physiological anthropology, 26(2), pp. 217-224	Wrong age range (mean age = 37.6)
Henderson, S., Skelton, H. and Rosenbaum, P. (2008)	Assistive devices for children with functional impairments: Impact on child and caregiver function	Developmental Medicine & Child Neurology, 50(2), pp. 89-98	Systematic review-references unpicked and screened for relevance
Holliday, P.J., Mihailidis, A., Rolfson, R. and Fernie, G. (2005)	Understanding and measuring powered wheelchair mobility and manoeuvrability. Part I. Reach in confined spaces	Disability and Rehabilitation, 27(16), pp. 939-949	Wrong age range (not explicitly stated, although no mention of children)
Isabelle, S., Bessey, S.F., Dragas, K.L., Blease, P., Shepherd, J.T. and Lane, S.J. (2002)	Assistive technology for children with disabilities	Occupational Therapy in Health Care, 16(4), pp. 29-51	Literature review-references unpicked and screened for relevance
Knox, V. (2008)	Do parents of children with cerebral palsy express different concerns in relation to their child's type of cerebral palsy, age and level of disability?	Physiotherapy, 94(1), pp. 56-62	Not related to wheelchair use
Krey, C.H. (2005)	Special seating considerations for the child with a spinal cord injury.	International Journal of Therapy & Rehabilitation, 12(2), pp. 84-86	Not a research study
Lau, H., Tam, E.W.C. and Cheng, J.C.Y. (2008)	An experience on wheelchair bank management	Disability and Rehabilitation: Assistive Technology, 3(6), pp. 302-308	Not relevant to UK population
Livingstone, R. (2011)	Power mobility for infants and preschool children	Evidence for Practice, retrieved from: http://www.childdevelopment.ca/Libraries/Evidence_for_Practice/Power_Mobility_for_Infants_Preschoolers_2012.sflb.ashx	Literature review-references unpicked and screened for relevance

McDonald, R.L., Surtees, R. and Wirz, S. (2007)	A comparative exploration of the thoughts of parents and therapists regarding seating equipment for children with multiple and complex needs.	Disability and Rehabilitation: Assistive Technology, 2(6), pp. 319-325	Not related to review questions
McNamara, L. and Casey, J. (2007)	Seat inclinations affect the function of children with cerebral palsy: a review of the effect of different seat inclines	Disability and Rehabilitation: Assistive Technology, 2(6), pp. 309-318	Not related to review questions
Montesano, L., Diaz, M., Bhaskar, S. and Minguez, J. (2010)	Towards an intelligent wheelchair system for users with cerebral palsy.	IEEE transactions on neural systems and rehabilitation engineering : a publication of the IEEE Engineering in Medicine and Biology Society, 18(2), pp. 193-202	Not related to review questions
Nelson, V.S. (2007)	Durable medical equipment for children with spinal cord dysfunction: implications of age and level of injury.	The journal of spinal cord medicine, 30 Suppl 1, pp. S172-S177	Not a research study
Nilsson, L., Eklund, M., Nyberg, P. and Thulesius, H. (2011)	Driving to Learn in a Powered Wheelchair: The Process of Learning Joystick Use in People With Profound Cognitive Disabilities.	American Journal of Occupational Therapy, 65(6), pp. 652-660	Not related to review questions
Ragonesi, C., B., Chen, X., Agrawal, S. and Galloway, J., Cole (2011)	Power Mobility and Socialization in Preschool: Follow-up Case Study of a Child With Cerebral Palsy	Pediatric Physical Therapy, 23(4), pp. 399-406	Not related to review question
Reid, A., Imrie, H., Brouwer, E., Clutton, S., Evans, J., Russell, D. and Bartlett, D. (2011)	"If I Knew Then What I Know Now": Parents" Reflections on Raising a Child with Cerebral Palsy	Physical and Occupational Therapy in Pediatrics, 31(2), pp. 169-183	Not related to wheelchair interventions
Reid, D., Rigby, P. and Ryan, S. (1999)	Functional impact of a rigid pelvic stabilizer on children with cerebral palsy who use wheelchairs: users' and caregivers' perceptions	Pediatric Rehabilitation, 3(3), pp. 101-118	Not related specifically to wheelchair use-focus on wheelchair adaptation
Rendeli, C., Salvaggio, E., Sciascia Cannizzaro, G., Bianchi, E., Caldarelli, M. and Guzzetta, F. (2002)	Does locomotion improve the cognitive profile of children with meningomyelocele?	Child's Nervous System: Chns: Official Journal Of The International Society For Pediatric Neurosurgery, 18(5), pp. 231-234	Outcomes not related specifically to wheelchair use

Richardson, M. and Frank, A.O. (2009)	Electric powered wheelchairs for those with muscular dystrophy: problems of posture, pain and deformity	Disability and Rehabilitation: Assistive Technology, 4(3), pp. 181-188	Wrong age range (mean age = 25)
Rodby-Bousquet, E. and Hagglund, G. (2010)	Use of manual and powered wheelchair in children with cerebral palsy: a cross-sectional study	BMC Pediatrics, 10, pp. 59.	Not related to effectiveness or service user perspectives
Rosen, L., Arva, J., Furumasu, J., Harris, M., Lange, M.L., Mccarthy, E., Kermoian, R., Pinkerton, H., Plummer, T., Roos, J., Sabet, A., Vander Schaaf, P. and Wonsettler, T. (2009)	RESNA position on the application of power wheelchairs for pediatric users	Assistive Technology : The Official Journal of RESNA, 21(4), pp. 218-225; 228	Not a research study
Sanderson, D., Place, M. and Wright, D. (2000)	Evaluation of the Powered Wheelchair and Voucher Scheme Initiatives	NHS Executive and Department of Health	Not specifically related to children
Shields, M. (2004)	Use of wheelchairs and other mobility support devices	Health reports / Statistics Canada, Canadian Centre for Health Information, 15(3), pp. 37-41	Not a research study
Simpson, R.C. (2005)	Smart wheelchairs: a literature review	Journal of Rehabilitation Research & Development, 42(4), pp. 423-435	Not related to review question outcomes
Sprigle, S., Wootten, M., Sawacha, Z. and Thielman, G. (2003)	Relationships among cushion type, backrest height, seated posture, and reach of wheelchair users with spinal cord injury	Journal of Spinal Cord Medicine, 26(3), pp. 236-243	Wrong age range (age >18)
Telfer, S., Solomonidis, S. and Spence, W. (2010)	An investigation of teaching staff members' and parents' views on the current state of adaptive seating technology and provision	Disability and Rehabilitation: Assistive Technology, 5(1), pp. 14-24	Intervention not related to review questions
Van Den Berg, R., De Groot, S., Swart, K.M. and Van Der Woude, L.H. (2010)	Physical capacity after 7 weeks of low-intensity wheelchair training	Disability and Rehabilitation, 32(21), pp. 1717-21	Wrong age range (age 18 – 30)

Van Der Dussen, D.D., Nieuwstraten, W., Roebroeck, M. and Stam, H.J. (2001)	Functional level of young adults with cerebral palsy	Clinical Rehabilitation, 15(1), pp. 84-91	Wrong age range (age >26)
Vargus-Adams, J.N. and Martin, L.K. (2011)	Domains of importance for parents, medical professionals and youth with cerebral palsy considering treatment outcomes	Child: Care, Health and Development, 37(2), pp. 276-281	Not related to review questions
Voll, R. (2001)	Aspects of the quality of life of chronically ill and handicapped children and adolescents in outpatient and inpatient rehabilitation	International Journal Of Rehabilitation Research, 24(1), pp. 43-49	Not related to wheelchair outcomes
Voll, R., Krumm, B. and Fichtner, H. (1999)	Demand for psychosocial counselling of young wheelchair users	International Journal Of Rehabilitation Research, 22(2), pp. 119-122	Wrong age range (mean age = 20.5)
Wiart, L. (2011)	Exploring Mobility Options for Children With Physical Disabilities: A Focus on Powered Mobility	Physical and Occupational Therapy in Pediatrics, 31(1), pp.16-18	Not a research study
Zeng, Q., Burdet, E. and Teo, C.L. (2009)	Evaluation of a collaborative wheelchair system in cerebral palsy and traumatic brain injury users	Neurorehabilitation and neural repair, 23(5), pp. 494-504	Not related to review question outcomes
Zeng, Q., Teo, C.L. and Burdet, E. (2008)	Is the collaborative wheelchair adapted to cerebral palsy and traumatic brain injury subjects?	Conference proceedings: Annual International Conference of the IEEE Engineering in Medicine and Biology Society. IEEE Engineering in Medicine and Biology Society Conference, 2008, pp. 1965-1968	Not related to review question outcomes

Appendix E: Questionnaires for quantitative data collection

Appendix E.1: Discrete choice experiment example choice task, as presented in questionnaire

Question 1: Which service would you prefer?

Service A	Service B
Your **health needs** will be considered in your wheelchair assessment	Your **health, school and social life needs** will be considered in your wheelchair assessment
The service will be **free**	You will have to **contribute £50** for your wheelchair
FREE	
You will receive **wheelchair skills** training	You will receive **wheelchair and life skills** training
It will take between **1 and 3 months** for your chair to arrive	It will take between **6 and 12 months** for your chair to arrive
Delivery in 1-3 months	Delivery in 6-12 months
Your needs and your wheelchair will be reviewed **every 6 months**	Your needs and your wheelchair will be reviewed **every 12 months**
Review every 6 months	Review every 12 months
☐	☐

OR

Please tick (✓) **only ONE box**

What would be your ideal wheelchair service?

Think about going to a wheelchair service for a new wheelchair and all of the different aspects that would make a good wheelchair service.

In the next section of this questionnaire you will be shown **8 pairs** of wheelchair services, a different pair in each question. For each pair you will be asked to choose between **Service A** or **Service B**. Each service will **always** be described to you using the same five aspects, which are:

- How your wheelchair needs will be assessed
- Cost (£) of the wheelchair to you and/or your family
- The level of training you will receive
- Length of time it takes to receive your wheelchair
- How often your wheelchair and needs will be reviewed

These aspects will be slightly different between **Service A** and **Service B** in each pair and you will be asked to decide which service you think is best.

A detailed description of each aspect can be found in the **Supplementary Notes** section at the end of this questionnaire. Please read through the **Example question** on the following page, which shows you how to answer.

There are no right or wrong answers, we are simply interested in your views about wheelchair services!

Supplementary notes for the questionnaire

Aspects of Wheelchair Services

Each service is described in terms of the following aspects:

- **How your wheelchair needs will be assessed**
  This describes how you will be assessed for a new wheelchair, particularly which of your needs will be assessed. Your needs are defined as health, social life and school needs in relation to using a wheelchair.

- **Cost (£) of the wheelchair to you and/or your family**
  This describes how much you will be asked to contribute to the wheelchair service. This would be a one-off payment for each new wheelchair. If you are still at school your parent or guardian would be asked to pay this.

- **The level of training you will receive**
  This describes what sort of training you would be given by the wheelchair service. Wheelchair skills training will include wheelchair driving techniques, road safety and maintaining your wheelchair. Life skills training will include work placements, learning independence and ambassador groups.

- **Length of time it takes to receive your wheelchair**
  This describes the length of time it takes for your wheelchair to be delivered after your final assessment.

- **How often your wheelchair and needs will be reviewed**
  This describes how often you will receive a full review from the wheelchair service. This will include a reassessment of your needs and a review of your wheelchair for any maintenance or repairs it requires.

Please Turn Over

Describing a wheelchair service

Five aspects of a wheelchair service are presented. Each aspect has more than one outcome. To help you complete the questionnaire, please read the descriptions of the aspects and possible outcomes in the following table.

Aspects	Possible Choices
How your wheelchair needs will be assessed	Assessment of your health needs
	Assessment of your health, school and social life needs
Cost (£) of the wheelchair to you and/or your family	No cost
	£50
	£150
	£300
The level of training you will receive	Wheelchair skills training
	Wheelchair and life skills training
Length of time it takes to receive your wheelchair	Between 1 and 3 months
	Between 6 and 12 months
How often your wheelchair and needs will be reviewed	At least once every 6 months
	At least once every 12 months

Appendix E.3: Discrete choice experiment random effects logit model used to estimate preferences

Δ Utility = $\alpha + \beta 1$Asssess + $\beta 2$CostCon + $\beta 3$LvlTrain + $\beta 4$DelTime + $\beta 5$FreqRev+ $\varepsilon 1 + \varepsilon 2$

Model definitions:

Δ Utility	=	The change in utility in moving from scenario A to scenario B
α	=	Constant term
$\beta 1$ - $\beta 5$	=	The beta coefficients of the model to be estimated
Assess	=	The difference in comprehensiveness of assessment between scenario A and scenario B
CostCon	=	The difference in cost contribution between scenario A and scenario B
LvlTrain	=	The difference in level of training between scenario A and scenario B
DelTime	=	The difference in delivery waiting time between scenario A and scenario B
FreqRev	=	The difference in frequency of review between scenario A and scenario B
$\varepsilon 1$	=	The error term because of differences amongst observations
$\varepsilon 2$	=	The error term because of differences amongst respondents

Appendix E.4: Example HRQoL/demographics questionnaire, containing EQ-5D-Y and HUI measures.

Office use only

Questionnaire for Parents and Guardians

Thank you for taking part in the Wheels Project!

Instructions

Please complete **ALL** sections of this questionnaire and return using the stamped addressed envelope provided. Please tick all boxes that apply to you and your child and when requested write all of your answers in BLOCK CAPITALS.

This questionnaire should be completed independently, you should **not** talk about your answers with your child until after you have both completed your questionnaires. Parts 2 and 3 should be answered **on behalf of your child.**

If your child is able to complete their questionnaire but need help to do so, please feel free to offer any support they may need, including reading questions and writing answers. Please try not to influence their answers and allow them to consider each question independently. If they do not understand a question please repeat it to them and encourage them to answer it according to what they think the question means. If there are any questions that you do not want to answer please leave them blank.

If you have any questions please contact the research team-

Address:
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PRIFYSGOL
BANGOR
UNIVERSITY

Part 1

Please read the following information and put a tick in each box to say 'yes'

(Please put a TICK in each box to say 'yes')

I have read the information leaflet about the Wheels Project. ☐

I agree for my child to complete the Wheels Project questionnaire. ☐

I understand what is being asked of me and my child. ☐

I know that our information and answers in the questionnaire will be kept private. ☐

I know you will not use our names when you tell people what you found out in the project. ☐

I agree to allow the answers we give to be used for educational purposes in the future. ☐

I agree to complete the questionnaire(s) as part of the Wheels Project ☐

Your name	Your date of birth
Your child's name	Your child's date of birth
Date	Your signature

In the future if you would like us to write and speak to you in Welsh please tick here: ☐

Please provide your contact details (these will be kept private and confidential):

Home address _____

_____

_____

Post code _____

Phone number _____

Email address _____

Part 2

Please tell us about your child's health today

The purpose of this part of the questionnaire is to explore how a care-giver or someone who knows the child well (proxy), thinks that the child would rate his/her own health. The proxy should answer as he or she thinks that the child would respond if he or she were filling out the questionnaire for him or herself.

PLEASE ANSWER ON BEHALF OF THE CHILD: Under each heading, mark the ONE box that you think **your child** would mark to describe his/her own health **TODAY** if **he/she** were filling out the questionnaire. Please **do not** tick more than one box in each group.

Mobility *(walking about)*

He/she has no problems walking about ❑
He/she has some problems walking about ❑
He/she has a lot of problems walking about ❑

Looking after myself

He/she has no problems washing or dressing him/herself ❑
He/she has some problems washing or dressing him/herself ❑
He/she has a lot of problems washing or dressing him/herself ❑

Doing usual activities (for example. *going to school, hobbies, sports, playing, doing things with family or friends)*

He/she has no problems doing his/her usual activities ❑
He/she has some problems doing his/her usual activities ❑
He/she has a lot of problems doing his/her usual activities ❑

Having pain or discomfort

He/she has no pain or discomfort ❑
He/she has some pain or discomfort ❑
He/she has a lot of pain or discomfort ❑

Feeling worried, sad or unhappy

He/she is not worried, sad or unhappy ❑
He/she is a bit worried, sad or unhappy ❑
He/she is very worried, sad or unhappy ❑

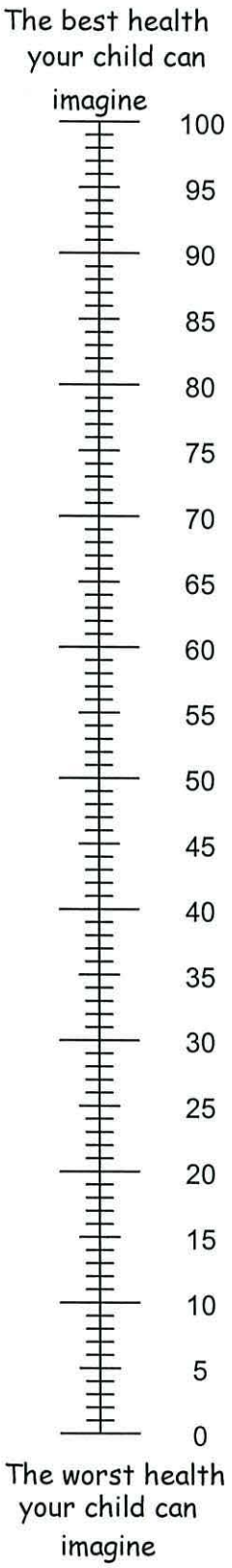
How good is the health of your child <u>today</u>

We would like to know how good or bad you think **your child** would rate **his/her** own health TODAY

This line is numbered from 0 to 100

100 means the <u>best</u> health your child can imagine
0 means the <u>worst</u> health your child can imagine

Please, mark an **X** on the line that shows how good or bad you think **your child** would rate **his/her** health TODAY



Part 3

Please tell us about your child's health over the past 2 weeks

Please note that the word "subject" is used in this part of the questionnaire to refer to the person you are answering for, for example, your daughter or son. This questionnaire has been written for use by a wide variety of respondents and we apologise for having to use the word "subject" in the questions about the health of your relative.

This questionnaire contains a set of questions that ask about various aspects of the subject's health. When answering these questions please think about the subject's health and ability to do things on a day-to-day basis, during the past 2 weeks. To define the past 2 week period, please think about what the date was 2 weeks ago and recall the major events that the subject has experienced during this period. Please focus your answers on the subject's abilities, disabilities and how the subject has felt during the past 2 weeks.

You may feel that some of these questions do not apply to the subject, but it is important that we ask the same questions of everyone. Also, a few questions are similar; please excuse the apparent overlap and answer each question independently.

Please read each question and consider your answers carefully. For each question, please select one answer that best describes the subject's level of ability or disability during the past 2 weeks. Please indicate the selected answer by circling the letter (a, b, c, ...) beside the answer.

All information you provide is confidential. There are no right or wrong answers; what we want is your opinion about the subject's abilities and feelings.

---

1. Which one of the following best describes the subject's ability, during the past 2 weeks, to see well enough to read ordinary newsprint?
    a. Able to see well enough without glasses or contact lenses
    b. Able to see well enough with glasses or contact lenses
    c. Unable to see well enough even with glasses or contact lenses
    d. Unable to see at all

2. Which one of the following best describes the subject's ability, during the past 2 weeks, to see well enough to recognise a friend on the other side of the street?
    a. Able to see well enough without glasses or contact lenses
    b. Able to see well enough with glasses or contact lenses
    c. Unable to see well enough even with glasses or contact lenses
    d. Unable to see at all

3. Which one of the following best describes the subject's ability, during the past 2 weeks, to hear what was said in a group conversation with at least three other people?
    a. Able to hear what was said without a hearing aid
    b. Able to hear what was said with a hearing aid
    c. Unable to hear what was said even with a hearing aid
    d. Unable to hear what was said, but did not wear a hearing aid
    e. Unable to hear at all

4. Which one of the following best describes the subject's ability, during the past 2 weeks, to hear what was said in a conversation with one person in a quiet room?
    a. Able to hear what was said without a hearing aid
    b. Able to hear what was said with a hearing aid
    c. Unable to hear what was said even with a hearing aid
    d. Unable to hear what was said, but did not wear a hearing aid
    e. Unable to hear at all

5. Which one of the following best describes the subject's ability, during the past 2 weeks, to be understood when speaking his/her own language with people who do not know the subject?
    a. Able to be understood completely
    b. Able to be understood partially
    c. Unable to be understood
    d. Unable to speak at all

6. Which one of the following best describes the subject's ability, during the past 2 weeks, to be understood when speaking with people who know the subject well?
    a. Able to be understood completely
    b. Able to be understood partially
    c. Unable to be understood
    d. Unable to speak at all

7. Which one of the following best describes the subject's feelings during the past 2 weeks?
    a. Happy and interested in life
    b. Somewhat happy
    c. Somewhat unhappy
    d. Very unhappy
    e. So unhappy that life was not worthwhile

8. Which one of the following best describes the pain and discomfort the subject has experienced during the past 2 weeks?
    a. Free of pain and discomfort
    b. Mild to moderate pain or discomfort that prevented no activities
    c. Moderate pain or discomfort that prevented some activities
    d. Moderate to severe pain or discomfort that prevented some activities
    e. Severe pain or discomfort that prevented most activities

9. Which one of the following best describes the subject's ability, during the past 2 weeks, to walk? ***NOTE:*** *Walking equipment refers to mechanical supports such as braces, a cane, crutches or a walker.*
    a. Able to walk around the neighbourhood without difficulty, and without walking equipment
    b. Able to walk around the neighbourhood with difficulty, but did not require walking equipment or the help of another person
    c. Able to walk around the neighbourhood with walking equipment but without the help of another person
    d. Able to walk only short distances with walking equipment, and required a wheelchair to get around the neighbourhood
    e. Unable to walk alone, even with walking equipment. Able to walk short distances with help of another person, and required a wheelchair to get around the neighbourhood
    f. Unable to walk at all

10. Which one of the following best describes the subject's ability, during the past 2 weeks, to use his/her hands and fingers? ***NOTE:*** *Special tools refers to hooks for buttoning clothes, gripping devices for opening jars or lifting small items,& other devices to compensate for limitations of hands / fingers.*
    a. Full use of two hands and ten fingers
    b. Limitations in the use of hands or fingers, but did not require special tools or the help of another person
    c. Limitations in the use of hands or fingers, independent with use of special tools (did not require the help of another person)
    d. Limitations in the use of hands or fingers, required the help of another person for some tasks (not independent even with use of special tools)
    e. Limitations in the use of hands or fingers, required the help of another person for most tasks (not independent even with use of special tools)
    f. Limitations in the use of hands or fingers, required the help of another person for all tasks (not independent even with use of special tools)

11. Which one of the following best describes the subject's ability, during the past 2 weeks, to remember things?
    a. Able to remember most things
    b. Somewhat forgetful
    c. Very forgetful
    d. Unable to remember anything at all

12. Which one of the following best describes the subject's ability, during the past 2 weeks, to think and solve day to day problems?
    a. Able to think clearly and solve day to day problems
    b. Had a little difficulty when trying to think and solve day to day problems
    c. Had some difficulty when trying to think and solve day to day problems
    d. Had great difficulty when trying to think and solve day to day problems
    e. Unable to think or solve day to day problems

13. Which one of the following best describes the subject's ability, during the past 2 weeks, to perform basic activities?
    a. Eat, bathe, dress and use the toilet normally
    b. Eat, bathe, dress and use the toilet independently with difficulty
    c. Required mechanical equipment to eat, bathe, dress or use the toilet independently
    d. Required the help of another person to eat, bathe, dress or use the toilet

14. Which one of the following best describes the subject's feelings during the past 2 weeks?
    a. Generally happy and free from worry
    b. Occasionally fretful, angry, irritable, anxious or depressed
    c. Often fretful, angry, irritable, anxious or depressed
    d. Almost always fretful, angry, irritable, anxious or depressed
    e. Extremely fretful, angry, irritable, anxious or depressed; to the point of needing professional help

Part 3 continues on the next page

15. Which one of the following best describes the pain and discomfort the subject has experienced during the past 2 weeks?
    a. Free of pain and discomfort
    b. Occasional pain or discomfort. Discomfort relieved by non-prescription drugs or self-control activity without disruption of normal activities
    c. Frequent pain or discomfort. Discomfort relieved by oral medicines with occasional disruption of normal activities
    d. Frequent pain or discomfort; frequent disruption of normal activities. Discomfort required prescription narcotics for relief
    e. Severe pain or discomfort. Pain not relieved by drugs and constantly disrupted normal activities

16. Overall, how would you rate the subject's health during the past 2 weeks?
    a. Excellent
    b. Very good
    c. Good
    d. Fair
    e. Poor

17. Who provided information used to answer the questions in this part of the questionnaire? (please indicate all that apply?)
    a. Person recording the answers on this form
    b. Subject
    c. Others. Please list the relationships between the subject and each person who provided information:
        1. _____
        2. _____
        3. _____
        4. _____

Please continue to the next page for Part 4

Part 4

Information about your child

Please place a tick in the box that applies to your child and write further information in BLOCK CAPITALS when requested. If you do not know the answer to a question please leave it blank.

Q1 **Child's gender?** Male ☐ Female ☐

Q2 **How old is your child?** Years ☐ Months ☐

Q3 **Is your child in education?** Yes ☐ No ☐

If **yes**, are they in: Infants school ☐ Primary school ☐

High school ☐ Other (*please specify*)_____

Q4a **Does your child currently have a wheelchair?** Yes ☐ No ☐

If **no**, when are they expecting to get a wheelchair? _____ go to question **Q5**

If **yes**, please answer questions **Q4b, Q4c and Q4d**

Q4b **Please provide details of all the wheelchairs (WC) your child currently uses.**

	Powered	Manual	What is the wheelchair used for? (e.g. everything, home, school, exercise/sport, leisure activities)	Supplied by (e.g. NHS, Whizz-Kidz, other charity, bought privately)
WC 1 (main)	☐	☐		
	Please tick one			
WC 2	☐	☐		
	Please tick one			
WC 3	☐	☐		
	Please tick one			
WC 4	☐	☐		
	Please tick one			

Q4c **How long has your child been using a wheelchair?** (*Please specify number of years and months if known*) Years ☐ Months ☐

Q4d **How often does your child use their wheelchair(s)?** (*Please choose only one option*)

A little of the time ☐ **Some** of the time ☐

Most of the time ☐ **All** of the time ☐

Q5 **What is the name of your child's disability/condition?**_____

Q6 **How long has your child had their disability/condition?** (*Please specify number of years and months if known*) Years ☐ Months ☐

Q7 **What is your child's ethnic background?** (Please tick only one box)

White
- ☐ White British
- ☐ White Irish
- ☐ Other white background

Mixed
- ☐ White & Black Caribbean
- ☐ White & Black African
- ☐ White & Asian
- ☐ Other Mixed background

Chinese or other ethnic group
- ☐ Chinese
- ☐ Other ethnic group

Black or Black British
- ☐ Caribbean
- ☐ African
- ☐ Other black background

Asian or Asian British
- ☐ Indian
- ☐ Pakistani
- ☐ Bangladeshi
- ☐ Other Asian background

Not stated
- ☐ Not stated

If 'other' please state _____

Part 5

Information about you

Please place a tick in each box that applies to you and write further information in BLOCK CAPITALS when requested. If you do not know the answer to a question please leave it blank.

Q1 Your gender? Male ☐ Female ☐

Q2 How old are you? Years ☐ Months ☐

Q3 What is your marital status?

Single ☐ Co-habiting ☐ Married ☐

Separated ☐ Divorced ☐ Widowed ☐

Other (*please specify*)_____

Q4 What is your household employment status?

Mother: Work full-time ☐ Work part-time ☐ Unemployed ☐

Father: Work full-time ☐ Work part-time ☐ Unemployed ☐

Q5 What is your highest level of education?

Higher (e.g. University) ☐ Further (e.g. A level) ☐

GCSE/O level ☐ None ☐

Other (*please specify*) _____

Q6 What type of accommodation do you and your child live in?

Owner-occupied ☐ Privately rented ☐

Rented from housing association/local authority ☐ Other (*please specify*) _____

Q7 **What is your annual household income (including any benefits you may receive)?**

Less than £5000	☐	£5000-£15,000	☐		
£16,000-£25,000	☐	£26,000-£35,000	☐		
£36,000-£50,000	☐	£50,000-£75,000	☐	£75,000 or more	☐

Q8 **What is your ethnic background?** (Please tick only one box)

White
- ☐ White British
- ☐ White Irish
- ☐ Other white background

Mixed
- ☐ White & Black Caribbean
- ☐ White & Black African
- ☐ White & Asian
- ☐ Other Mixed background

Chinese or other ethnic group
- ☐ Chinese
- ☐ Other ethnic group

Black or Black British
- ☐ Caribbean
- ☐ African
- ☐ Other black background

Asian or Asian British
- ☐ Indian
- ☐ Pakistani
- ☐ Bangladeshi
- ☐ Other Asian background

Not stated
- ☐ Not stated

If 'other' please state _____

Q8 **What is your relationship to the child?** (e.g. mother, father)_____

Please continue to the next page for Part 6

Part 6

Tell us what you thought of this questionnaire: Did you think that the questions were relevant to your child?:_____

We would also like to invite you and your child to take part in the second part of the Wheels Project, which is an interview. We would like to meet with you (in your home or somewhere else if you prefer) and speak to you about your child's wheelchair and about how it affects their life. The interview will take about an hour and with your permission we will record what you and your child say. Any information you give will be kept private and we will not use your name in any reports we right.

The interview is a very important part of the Wheels Project and we would like to interview everyone that completes this questionnaire. Every child will receive a £10 highstreet voucher for taking part in the interview.

Are you happy for the research team to contact you about taking part in the interview?-

Yes ☐ No ☐

How would you prefer to be contacted?

Phone ☐ In writing ☐ Email ☐

Thank you for completing this questionnaire

Please use the stamped addressed envelope we provided to send the completed questionnaire back to us.

Appendix F: Tests of normality

In order to guide statistical analysis of mean scores between children and parent proxies I tested for normality of the data by respondent type using the Shapiro-Wilk test. The null hypothesis for this test is that data is normally distributed within a specified sample, thus a non-significant result (p>0.05) indicates normal distribution. In order to guide my analysis of correlations between measures and respondent types, I again tested for normality of the data using the Shapiro-Wilk test but based this on outcome measure scores and individual domain scores. The data was not sufficiently normally distributed so non-parametric tests of significance were subsequently used.

Appendix F.1: Statistical analysis of HRQoL scores- Testing for normality

Parent VAS data was not found to be normally distributed (*p*=.011*)* however all other measures/groups were (see table AF.1). As a small proportion of the data was not normally distributed and sample sizes were small I chose to assume that the data was not normally distributed to a sufficient extent. In light of these findings, I used non-parametric tests (Wilcoxon signed rank and Mann-Whitney U) to compare the means of child and parent proxy results.

Table AF.1: Test of normality by respondent type

		Shapiro-Wilk		
		Statistic	**df**	**Sig.**
EQ-5D-Y	Child	.888	13	.093
	Parent	.948	29	.161
VAS	Child	.872	13	.056
	Parent	.902	29	.011
HUI2	Child	.906	13	.160
	Parent	.985	29	.945
HUI3	Child	.933	13	.368
	Parent	.929	29	.051

Appendix F.2: Analysing correlations between measures and respondents: Testing for normailty

I found normality of data to vary depending on the outcome measure and variable (child age, child gender, type of wheelchair used). In light of these findings, I chose to use Spearman's rank-order correlation (a non-parametric test of correlation), as normal distribution was not observed throughout

the data. Below are Shapiro-Wilk results for child self-report measures by age group, where VAS results for children aged 6-15 are not normally distributed (p=0.008), see table AF.2.

Table AF.2: Test of normality by child age group

		Shapiro-Wilk		
		Statistic	**df**	**Sig.**
EQ-5D-Y	6-15	.826	7	.073
	16-18	.855	5	.211
VAS	6-15	.733	7	.008
	16-18	.936	5	.637
HUI2	6-15	.911	7	.400
	16-18	.868	5	.257
HUI3	6-15	.944	7	.676
	16-18	.942	5	.678

Appendix G: Bland-Altman plots and interpretation

Appendix G.1: Agreement between child self-report measures

The mean (± SD) child reported VAS outcome score was 0.79 (±0.15) compared with 0.27 (±0.29) for the child reported ED-5D-Y. The bias (mean difference) of the two measures was -0.52 (95% CI -0.73 to -0.30), with children scoring the EQ-5D-Y lower than the VAS on average (see figure AG.1). Precision (difference standard deviation) was 0.36 (95% confidence limit from -1.21 [95% CI -1.59 to -0.84] to 0.18 [95% CI -0.20 to 0.55]) with an overall confidence limit of 1.39. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -1.21 to 0.18; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 1.39 on a utility scale from 0 to 1. In practice these discrepancies could include variance from perfect state of health to a state considered worse than death. I therefore conclude that child reported data from the EQ-5D-Y and VAS were not in agreement for this cohort.

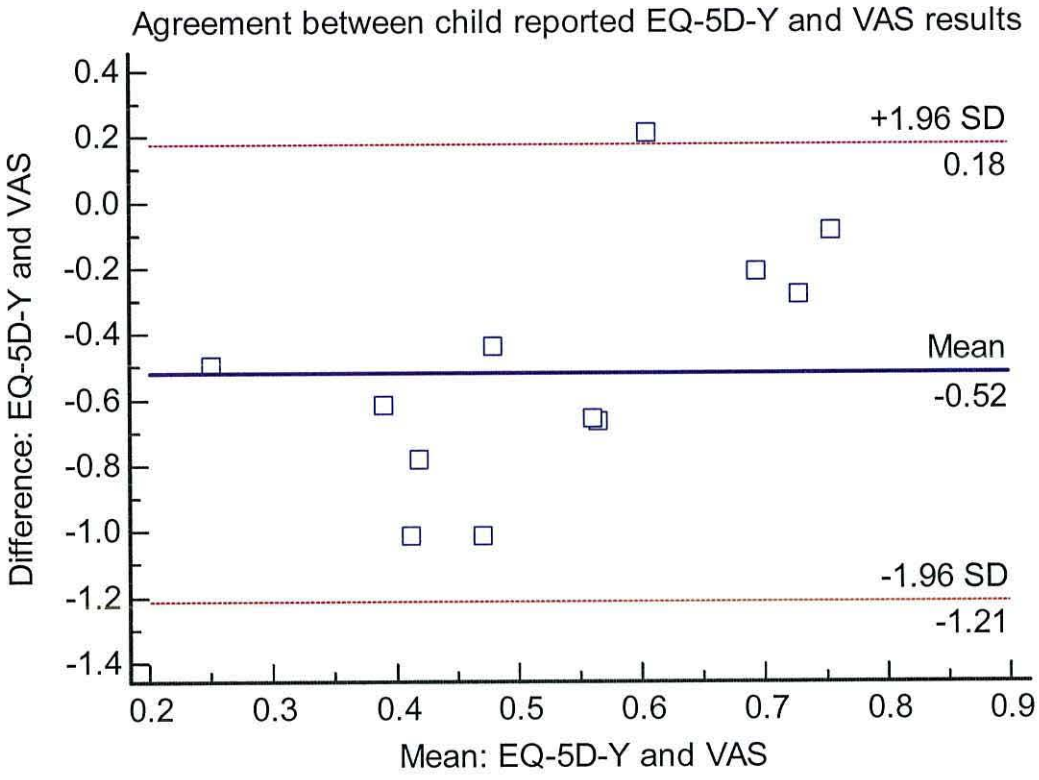


Figure AG.1: Agreement between child self-reported EQ-5D-Y and VAS scores

The mean (± SD) child reported HUI2 outcome score was 0.55 (±0.08) compared with 0.27 (±0.29) for the child reported ED-5D-Y. The bias (mean difference) of the two measures was -0.28 (95% CI -0.44 to -0.12), with children scoring the EQ-5D-Y lower than the HUI2 on average (see figure AG.2). Precision (difference standard deviation) was 0.27 (95% confidence limit from –0.81 [95% CI -1.09 to -0.52] to 0.25 [95% CI -0.04 to 0.54]) with an overall confidence limit of 1.06. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.81 to 0.25; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 1.06 on a utility scale from 0 to 1. In practice these discrepancies could include variance from perfect state of health to death. I therefore conclude that child reported data from the EQ-5D-Y and HUI2 were not in agreement for this cohort. The Bland-Altman plot appears to indicate a proportional error, as difference appears to increase in proportion to mean score increases, although the sample size is too small to make clear precise judgements about error.

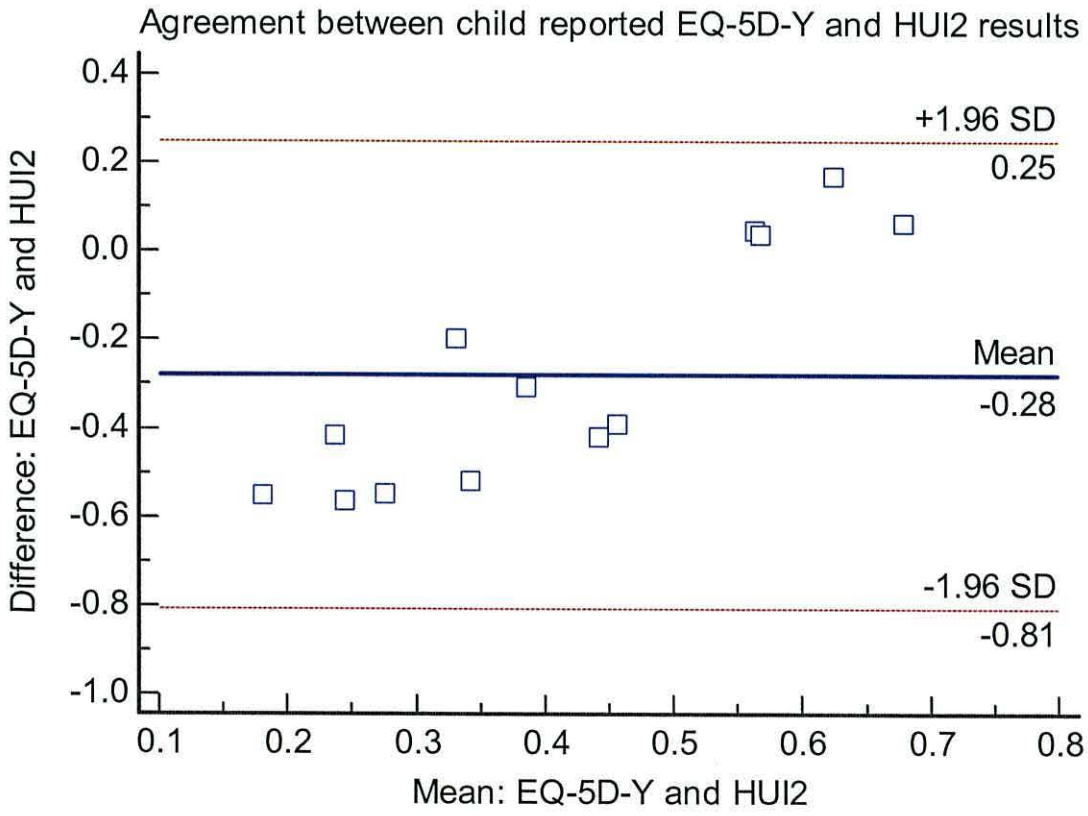


Figure AG.2: Agreement between child self-reported EQ-5D-Y and HUI2 scores

The mean (± SD) child reported HUI3 outcome score was 0.24 (±0.15) compared with 0.27 (±0.29) for the child reported ED-5D-Y. The bias (mean difference) of the two measures was 0.04 (95% CI -0.14 to 0.21), with children scoring the EQ-5D-Y slightly higher than the HUI3 on average (see figure AG.3). Precision (difference standard deviation) was 0.29 (95% confidence limit from –0.53 [95% CI -0.84 to -0.22] to 0.61 [95% CI 0.30 to 0.91]) with an overall confidence limit of 1.14. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.53 to 0.61; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 1.14 on a utility scale from 0 to 1. In practice these discrepancies could include variance from perfect state of health to a state worse than death. I therefore conclude that child reported data from the EQ-5D-Y and HUI3 were not insufficient agreement for this cohort, however they did should some potential in terms of bias and mean scores. The Bland-Altman plot appears to indicate a proportional error, as difference appears to increase in proportion to mean score increases, although the sample size is too small to make clear precise judgements about error.

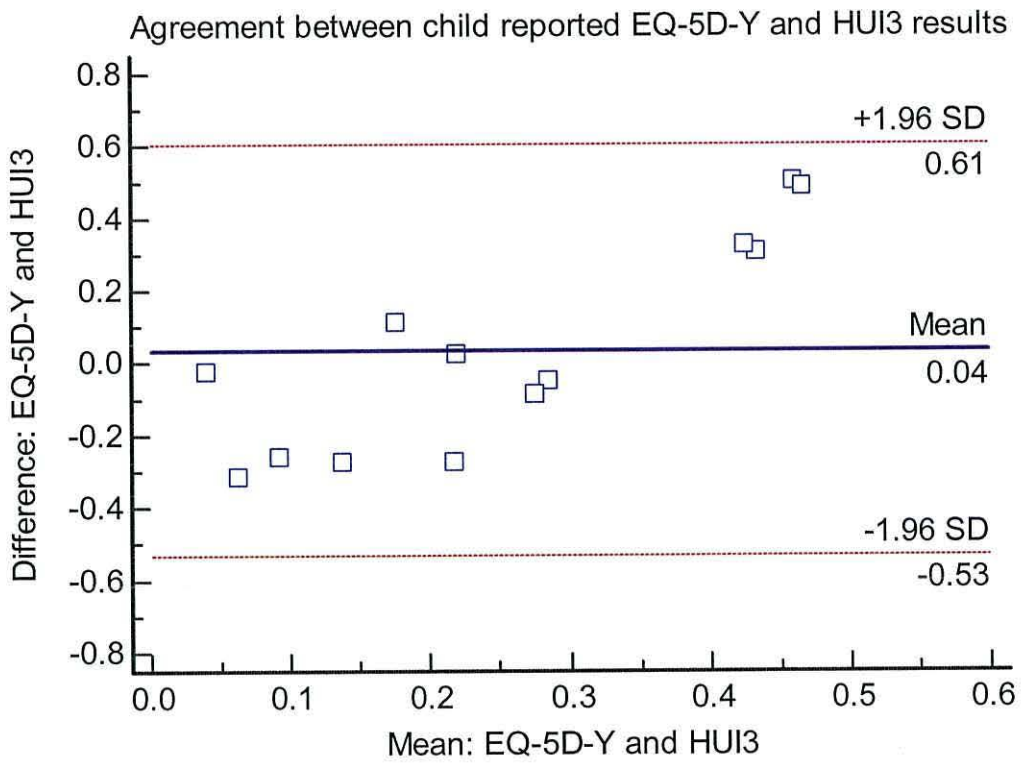


Figure AG.3: Agreement between child self-reported EQ-5D-Y and HUI3 scores

The mean (± SD) child reported HUI2 outcome score was 0.54 (±0.07) compared with 0.79 (±0.15) for the child reported VAS. The bias (mean difference) of the two measures was 0.25 (95% CI 0.14 to 0.36), with children scoring the VAS higher than the HUI2 on average (see figure AG.4). Precision (difference standard deviation) was 0.18 (95% confidence limit from —0.11 [95% CI -0.31 to 0.08] to 0.60 [95% CI 0.41 to 0.80]) with an overall confidence limit of 0.42. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.11 to 0.60; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods show some consistency in outcomes as the level of disagreement may not be clinically important. A confidence limit of 0.42 on a utility scale from 0 to 1 is borderline acceptable. In practice these discrepancies could, for example, include variance from perfect state of health to a moderate state of health. I therefore conclude that child reported data from the VAS and HUI2 may be exhibit sufficient agreement for this cohort, however a larger sample would be needed to verify this.

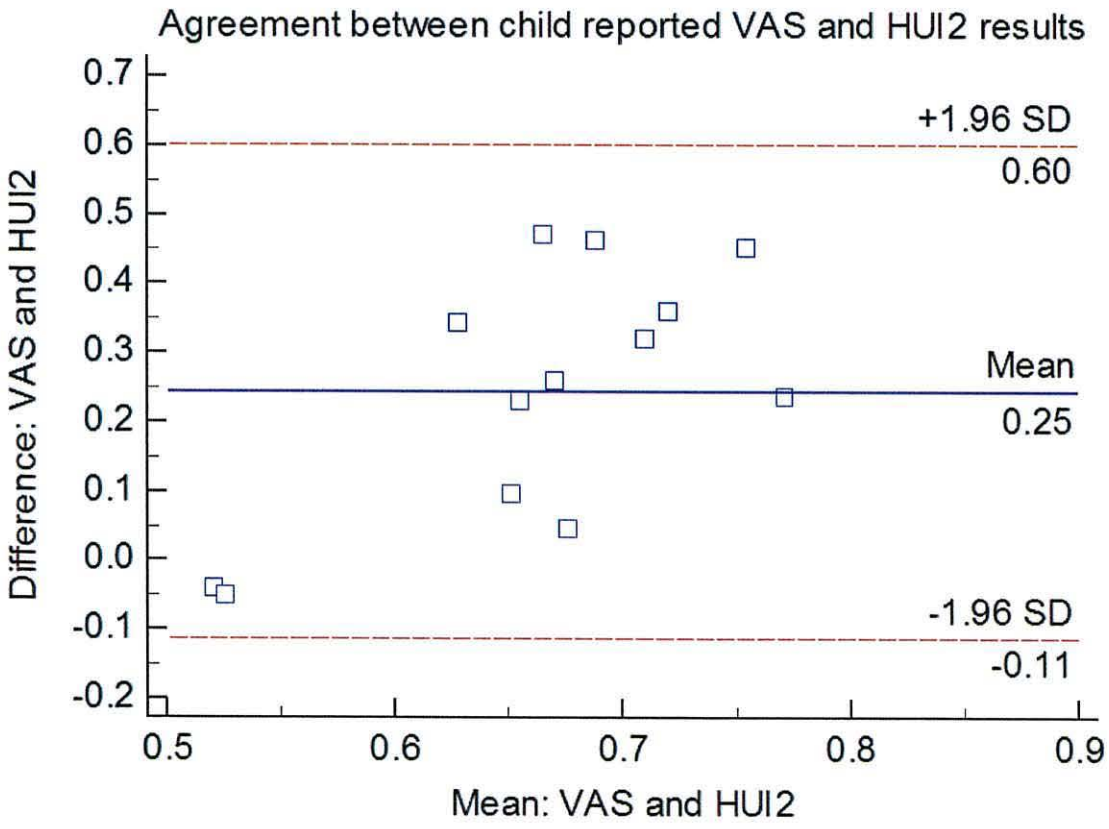


Figure AG.4: Agreement between child self-reported VAS and HUI2 scores

The mean (± SD) child reported HUI3 outcome score was 0.24 (±0.08) compared with 0.79 (±0.15) for the child reported VAS. The bias (mean difference) of the two measures was 0.54 (95% CI 0.43 to 0.66), with children scoring the VAS higher than the HUI3 on average (see figure AG.5). Precision (difference standard deviation) was 0.19 (95% confidence limit from 0.18 [95% CI -0.02 to 0.38] to 0.91 [95% CI 0.71 to 1.11]) with an overall confidence limit of 0.73. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from 0.18 to 0.91; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 0.73 on a utility scale from 0 to 1. In practice these discrepancies could include variance from perfect state of health to a poor state health. I therefore conclude that child reported data from the VAS and HUI3 were not insufficient agreement for this cohort.

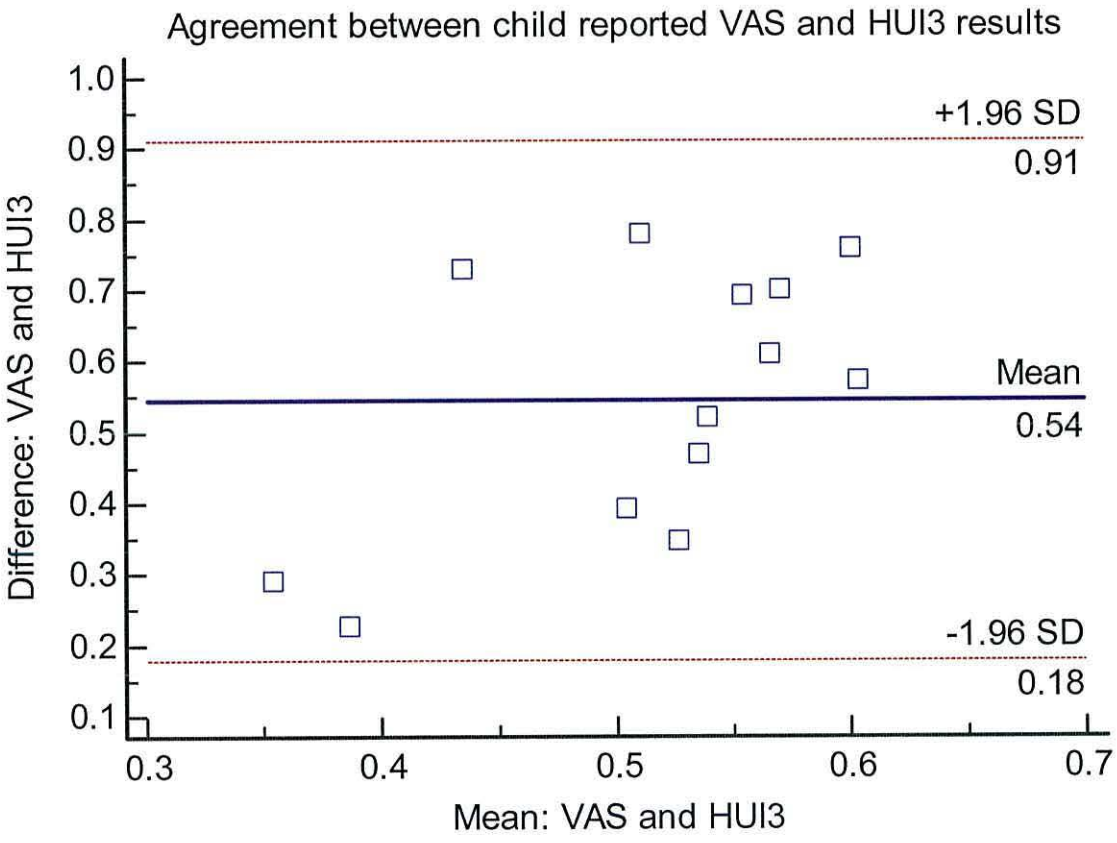


Figure AG.5: Agreement between child self-reported VAS and HUI3 scores

Appendix G.2: Agreement between parent proxy measures

The mean (± SD) proxy VAS outcome score was 0.72 (±0.20) compared with -0.05 (±0.14) for the proxy ED-5D-Y. The bias (mean difference) of the two measures was -0.76 (95% CI -0.84 to -0.68), with parents scoring the EQ-5D-Y lower than the VAS on average (see figure AG.6). Precision (difference standard deviation) was 0.24 (95% confidence limit from -1.22 [95% CI -1.36 to -1.08] to -0.30 [95% CI -0.44 to -0.16]) with an overall confidence limit of 0.92. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -1.22 to -0.30; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 0.92 on a utility scale from 0 to 1. In practice these discrepancies could include variance from an almost perfect state of health to death. I therefore conclude that parent proxy data from the EQ-5D-Y and VAS were not in agreement for this cohort.

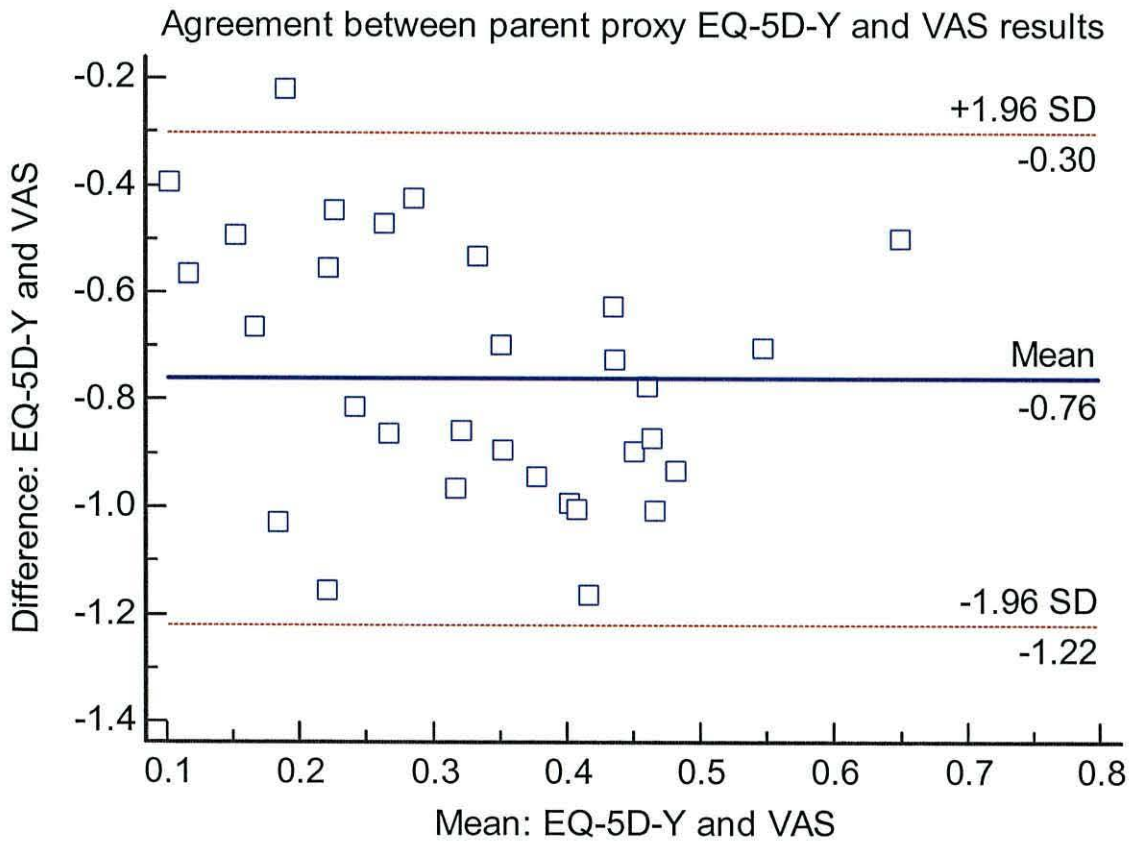


Figure AG.6: Agreement between parent proxy EQ-5D-Y and VAS scores

The mean (± SD) proxy HUI2 outcome score was 0.42 (±0.17) compared with -0.04 (±0.15) for the proxy ED-5D-Y. The bias (mean difference) of the two measures was -0.46 (95% CI -0.52 to -0.38), with parents scoring the EQ-5D-Y lower than the HUI2 on average (see figure AG.7). Precision (difference standard deviation) was 0.16 (95% confidence limit -0.77 [95% CI -0.87 to -0.66] to -0.15 [95% CI -0.25 to -0.04]) with an overall confidence limit of 0.62. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.77 to -0.15; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 0.62 on a utility scale from 0 to 1. In practice this discrepancy could include variance from good to poor states of health. I therefore conclude that parent proxy data from the EQ-5D-Y and HUI2 were not in agreement for this cohort.

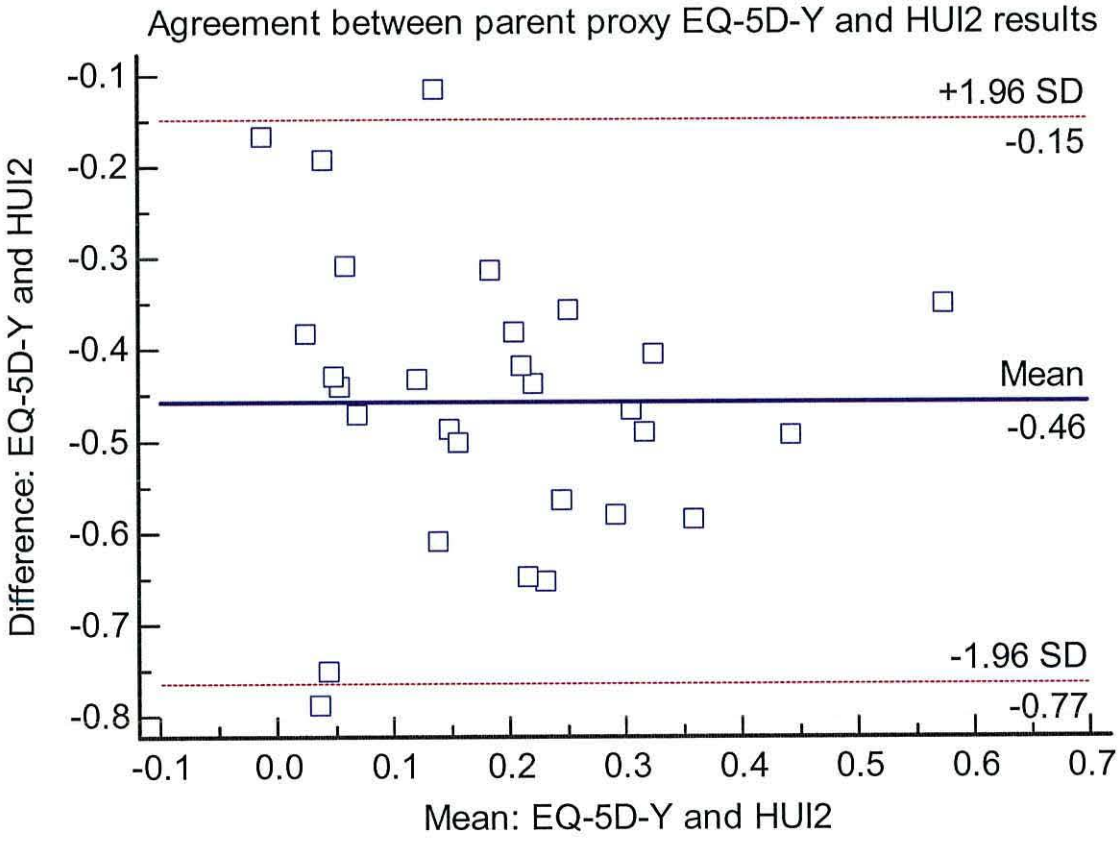


Figure AG.7: Agreement between parent proxy EQ-5D-Y and HUI2 scores

The mean (± SD) proxy HUI3 outcome score was 0.10 (±0.24) compared with -0.04 (±0.15) for the proxy ED-5D-Y. The bias (mean difference) of the two measures was -0.14 (95% CI -0.21 to -0.06), with parents scoring the EQ-5D-Y slightly lower than the HUI3 on average (see figure AG.8). Precision (difference standard deviation) was 0.20 (95% confidence limit -0.53 [95% CI -0.67 to -0.40] to 0.26 [95% CI 0.13 to 0.40]) with an overall confidence limit of 0.80. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.53 to 0.26; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 0.80 on a utility scale from 0 to 1. In practice this discrepancy could include variance from good to poor states of health. I therefore conclude that parent proxy data from the EQ-5D-Y and HUI3 were not in agreement for this cohort.

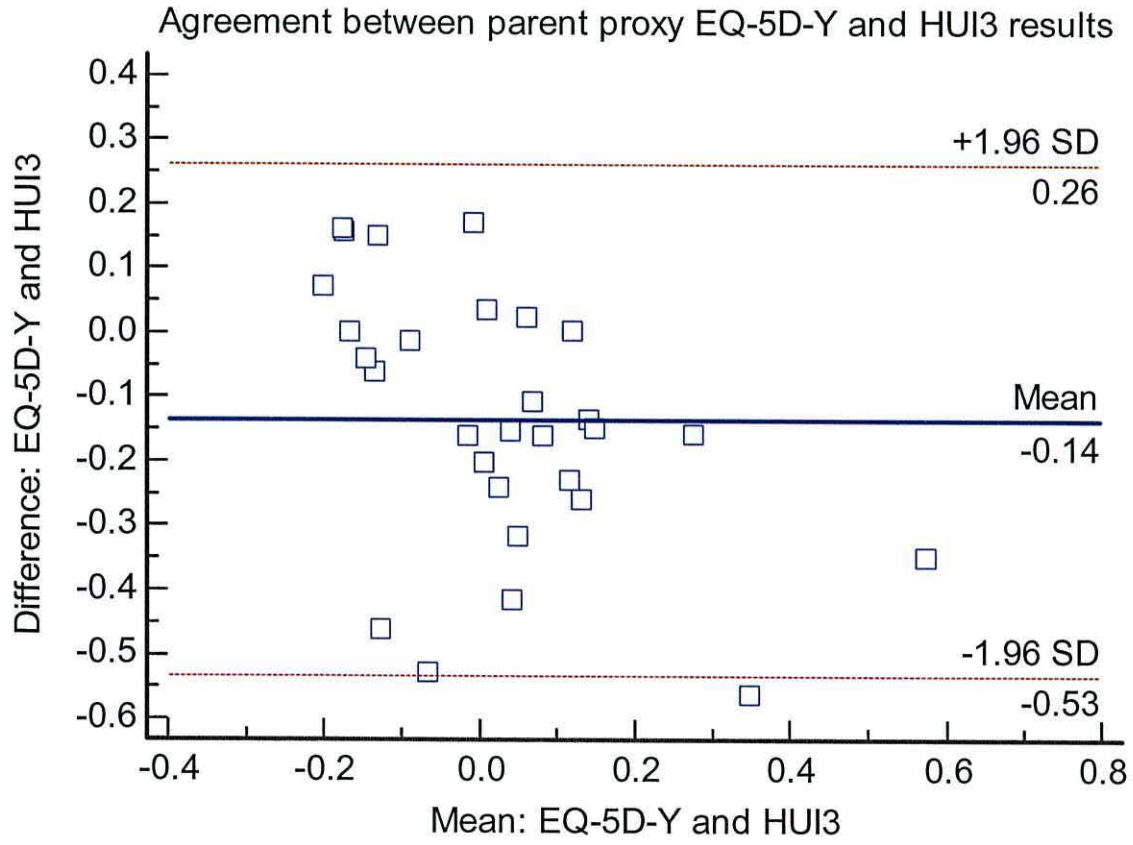


Figure AG.8: Agreement between parent proxy EQ-5D-Y and HUI3 scores

The mean (± SD) proxy HUI2 outcome score was 0.42 (±0.16) compared with 0.71 (±0.20) for the proxy VAS. The bias (mean difference) of the two measures was 0.29 (95% CI 0.22 to 0.36), with parents scoring the VAS higher than the HUI2 on average (see figure AG.9). Precision (difference standard deviation) was 0.19 (95% confidence limit –0.09 [95% CI -0.21 to 0.03] to 0.66 [95% CI 0.54 to 0.78]) with an overall confidence limit of 0.75. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.09 to 0.66; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 0.75 on a utility scale from 0 to 1. In practice this discrepancy could include variance from good to poor states of health. I therefore conclude that parent proxy data from the VAS and HUI2 were not in agreement for this cohort.

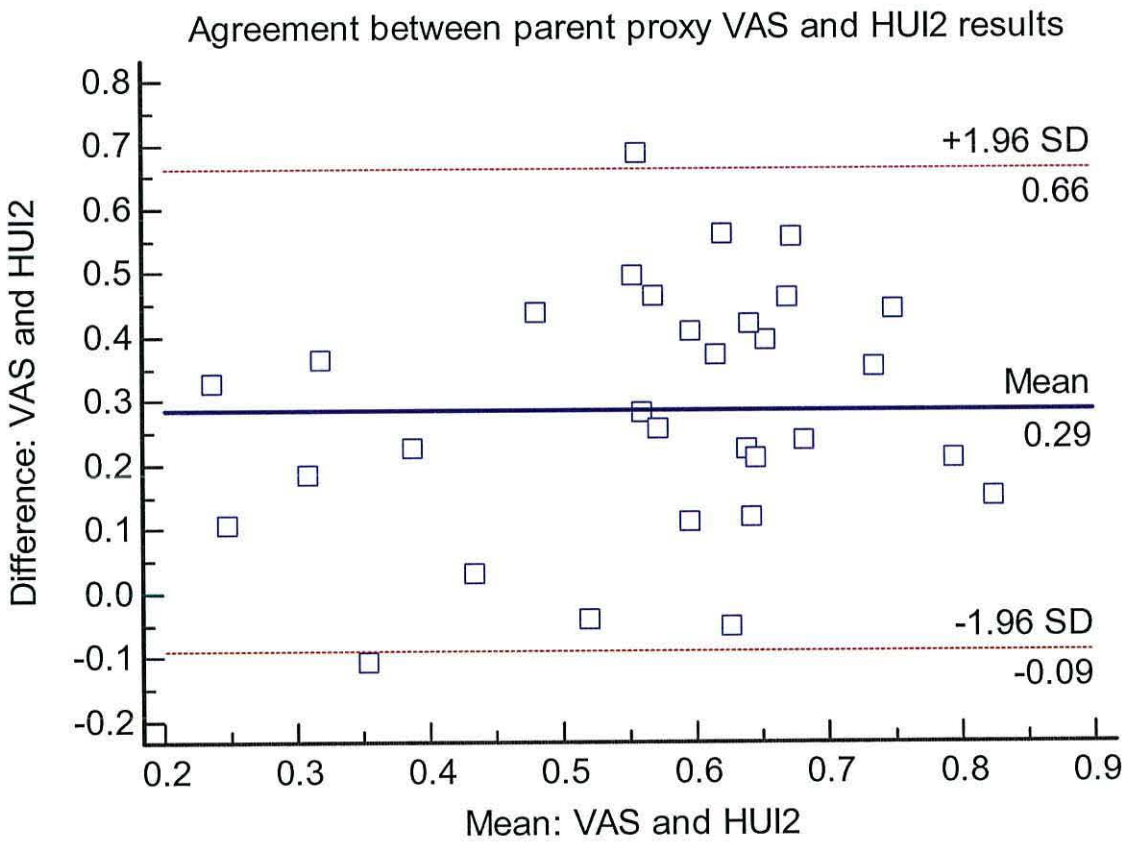


Figure AG.9: Agreement between parent proxy VAS and HUI2 scores

The mean (± SD) proxy HUI3 outcome score was 0.10 (±0.23) compared with 0.71 (±0.20) for the proxy VAS. The bias (mean difference) of the two measures was 0.61 (95% CI 0.51 to 0.71) with parents scoring the VAS higher than the HUI3 on average (see figure AG.10). Precision (difference standard deviation) was 0.27 (95% confidence limit 0.09 [95% CI -0.08 to 0.26] to 1.13 [95% CI 0.96 to 1.30]) with an overall confidence limit of 1.04. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from 0.09 and 1.13; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two methods do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 1.04 on a utility scale from 0 to 1. In practice this discrepancy could include variance from perfect health to death. I therefore conclude that parent proxy data from the VAS and HUI3 were not in agreement for this cohort.

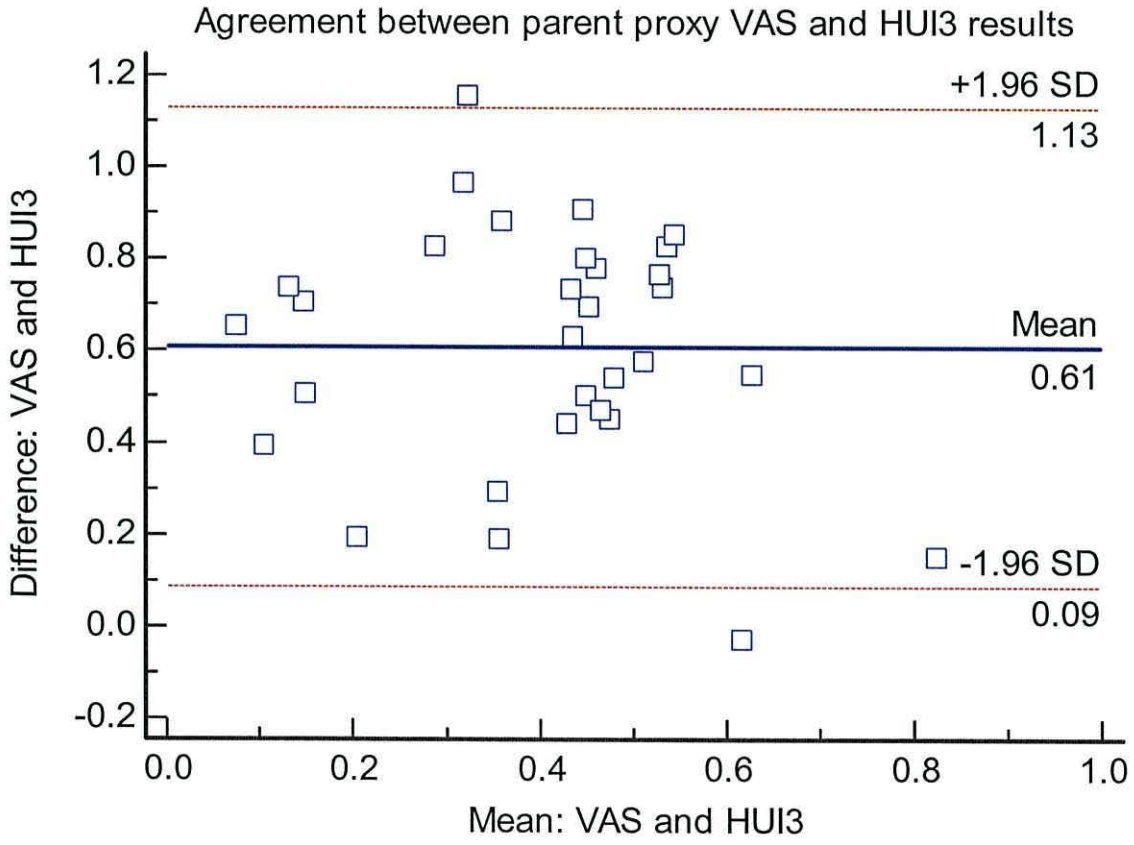


Figure AG.10: Agreement between parent proxy VAS and HUI3 scores

Appendix G.3: Agreement between child reported and parent proxy measures

The mean (± SD) child EQ-5D-Y score was 0.24 (±0.0.30) compared with 0.01 (±0.14) for the parent proxy version. The bias (mean difference) of the two measures was 0.23 (95% CI -0.06 to 0.41), with children scoring the EQ-5D-Y higher than their parents on average (see figure AG.11). Precision (difference standard deviation) was 0.27 (95% confidence limit from -0.29 [95% CI -0.60 to 0.028] to 0.75 [95% CI 0.44 to 1.07]) with an overall confidence limit of 1.04. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from –0.29 to 0.75; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two respondents do not consistently provide similar outcomes as the level of disagreement includes clinically important discrepancies and a confidence limit of 1.04 on a utility scale from 0 to 1. In practice these discrepancies could include variance from perfect state of health to death. I therefore conclude that child reported and parent proxy EQ-5D-Y data were not in agreement for this cohort.

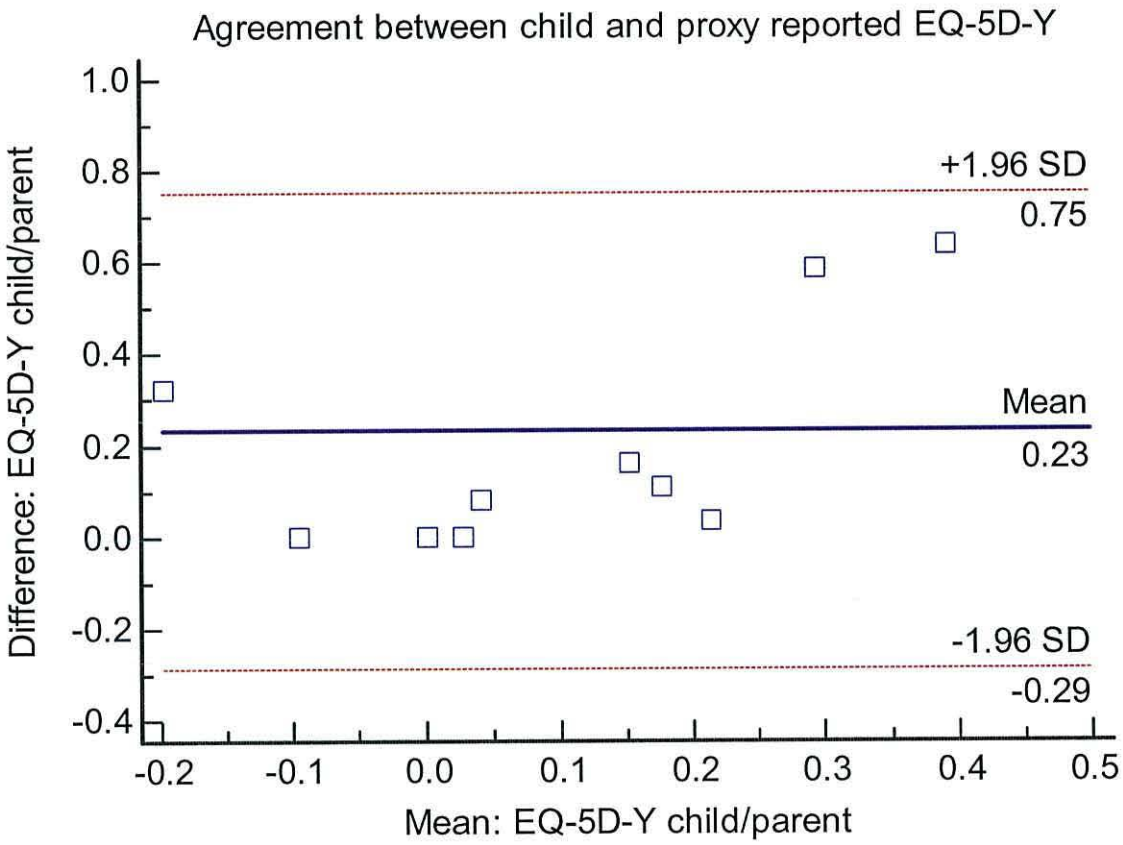


Figure AG.11: Agreement between child self-report and parent proxy EQ-5D-Y scores

The mean (± SD) child VAS score was 0.80 (±0.150) compared with 0.76 (±0.15) for the parent proxy version. The bias (mean difference) of the two measures was 0.04 (95% CI -0.01 to 0.09), with children scoring the VAS slightly higher than their parents on average (see figure AG.12). Precision (difference standard deviation) was 0.08 (95% confidence limit from -0.12 [95% CI -0.21 to -0.04] to 0.20 [95% CI 0.11 to 0.29]) with an overall confidence limit of 0.32. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.12 to 0.20; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two respondent groups show consistency in outcomes as the level of disagreement is unlikely to be clinically important; a confidence limit of 0.32 on a utility scale from 0 to 1 would be acceptable. In practice these discrepancies could, for example, include variance from perfect state of health to a good/moderate state of health. I therefore conclude that child and parent proxy VAS data exhibits sufficient agreement for this cohort to allow respondents to be used interchangeably.

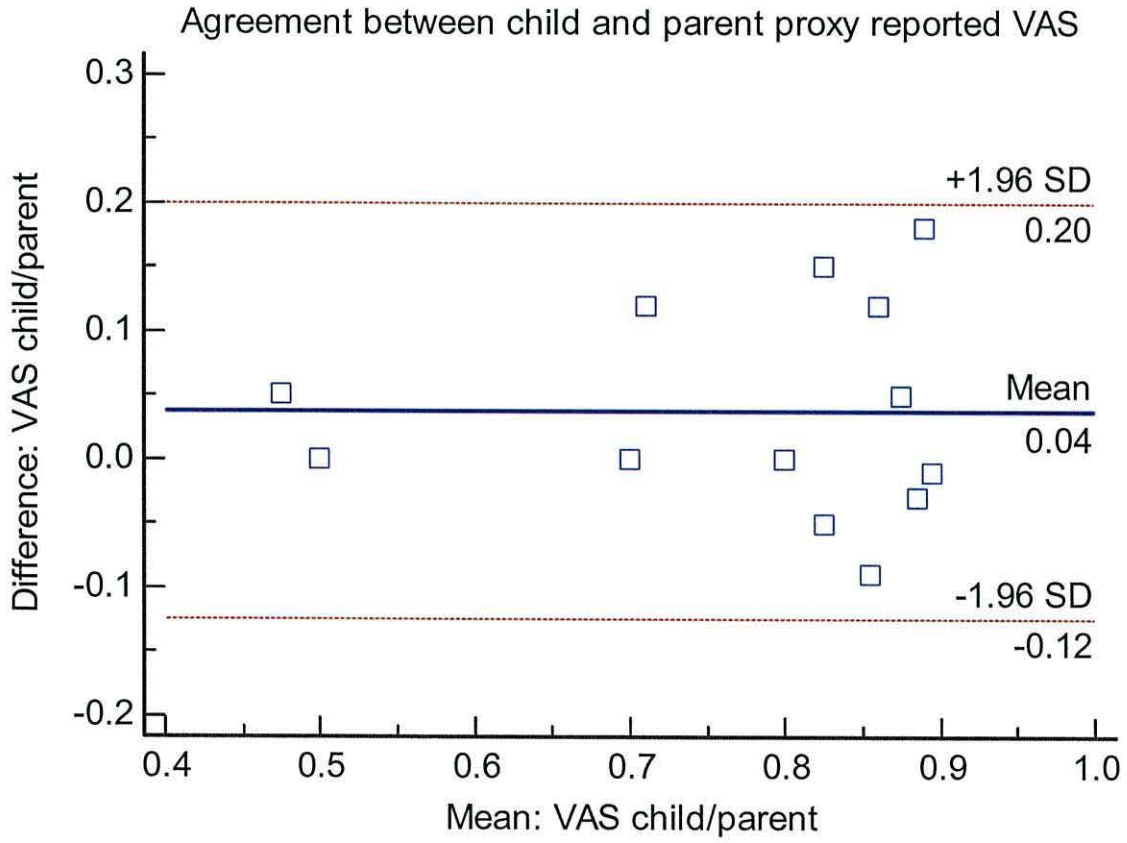


Figure AG.12: Agreement between child self-report and parent proxy VAS scores

The mean (± SD) Child HUI2 score was 0.53 (±0.073) compared with 0.49 (±0.10) for the parent proxy version. The bias (mean difference) of the two measures was 0.05 (95% CI -0.01 to 0.08), with children scoring the HUI2 slightly higher than their parents on average (see figure AG.13). Precision (difference standard deviation) was 0.06 (95% confidence limit from –0.06 [95% CI -0.12 to 0.01] to 0.15 [95% CI 0.10 to 0.21]) with an overall confidence limit of 0.22. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.06 to 0.15; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two respondent groups show consistency in outcomes as the level of disagreement is unlikely to be clinically important; A confidence limit of 0.22 on a utility scale from 0 to 1 would be acceptable. In practice these discrepancies could, for example, include variance from perfect state of health to a good state of health. I therefore conclude that child and parent proxy HUI2 data exhibits sufficient agreement for this cohort to allow respondents to be used interchangeably.

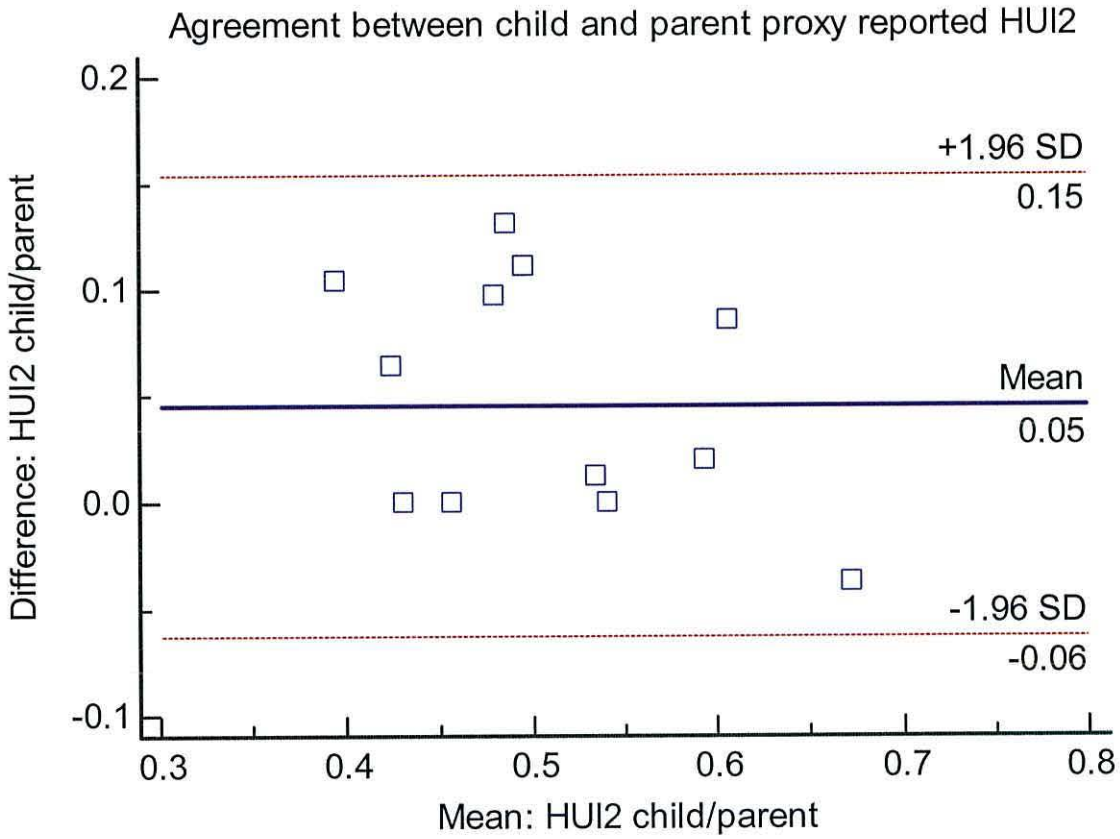


Figure AG.13: Agreement between child self-report and parent proxy HUI2 scores

The mean (± SD) Child HUI3 score was 0.22 (±0.09) compared with 0.16 (±0.10) for the parent proxy version. The bias (mean difference) of the two measures was 0.06 (95% CI 0.024 to 0.10), with children scoring the HUI3 higher than their parents on average (see figure AG.14). Precision (difference standard deviation) was 0.06 (95% confidence limit from -0.06 [95% CI -0.12 to 0.01] to 0.18 [95% CI 0.11 to 0.24]) with an overall confidence limit of 0.22. The Bland-Altman analysis indicates that the 95% limits of agreement between the two methods ranged from -0.06 to 0.18; if differences between methods are normally distributed 95% of the differences from the bias would fall between these figures. In this cohort, the two respondent groups show consistency in outcomes as the level of disagreement is unlikely to be clinically important; a confidence limit of 0.22 on a utility scale from 0 to 1 would be acceptable. In practice these discrepancies could, for example, include variance from perfect state of health to a good state of health. I therefore conclude that child and parent proxy HUI3data exhibits sufficient agreement for this cohort to allow respondents to be used interchangeably.

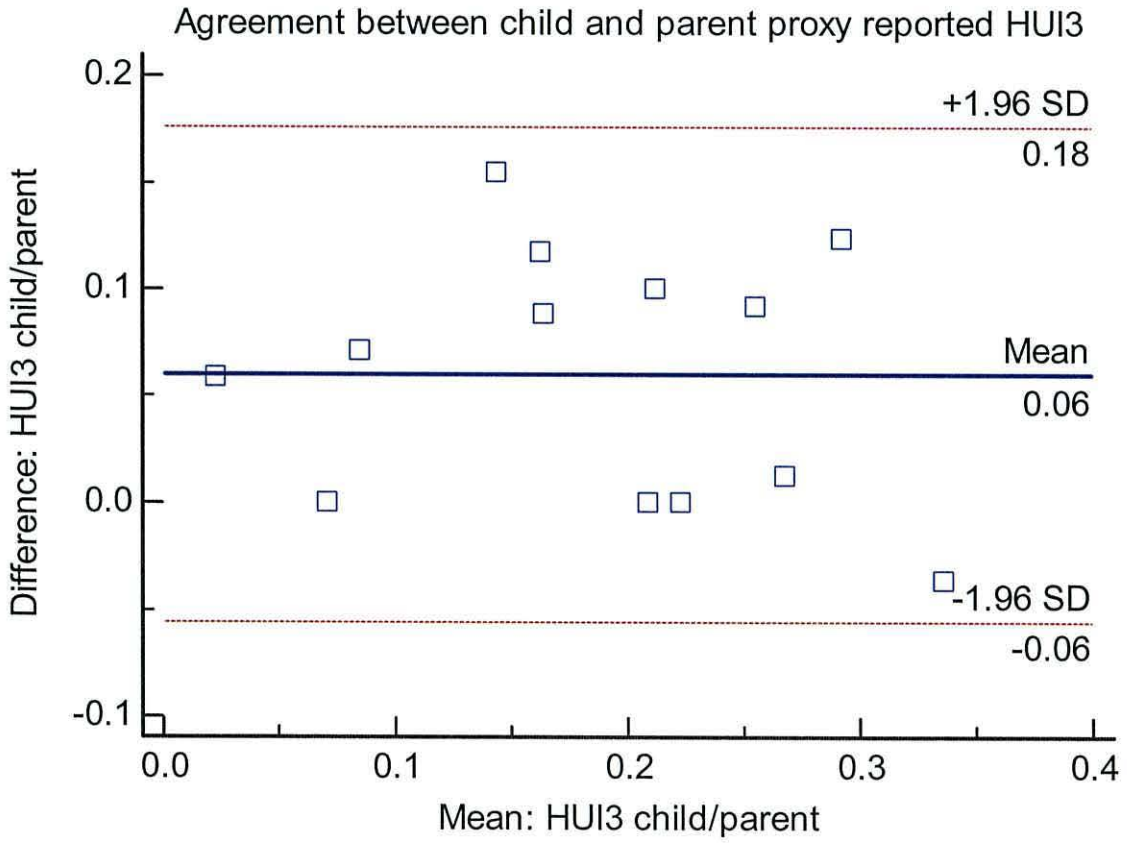


Figure AG.14: Agreement between child self-report and parent proxy HUI3 scores

Appendix H: Qualitative interview schedule

Questions are not restrictive, discussion around topics encouraged.

(amend wording for parents e.g. your = your child's)

Overall topics

Quality of life – impacts from wheelchair use, defining quality of life, outcome measure suitability

Quality of life questions

1. What does the term 'quality of life' mean to you?
2. Please describe your quality of life
    a. Which aspects of your life have the biggest impact on your quality of life (e.g. ability to get around, socialising with friends or managing pain and discomfort)
3. How does your wheelchair affect your quality of life?
4. What changes to your wheelchair would improve your quality of life?
5. Please review these questionnaires [health-related quality of life measures]
    a. How relevant are the questions to you?
    b. The EQ-5D defines quality of life in 5 ways- mobility, looking after yourself, doing usual activities, pain/discomfort and feeling worried, sad or unhappy. If you had to define quality of life what would be the 5 most important aspects of quality of life?
    c. The HUI defines quality of life in 8 ways- senses, communication, emotions, pain/discomfort, mobility/ambulation, dexterity/using hands, cognition (memory and problem solving), usual activities,
    d. To what extent do these questionnaires represent your understanding of the term quality of life?
6. Do you think your quality of life would be worse if you didn't have a wheelchair / didn't have a wheelchair you could control yourself?
7. What activities does your wheelchair help you to do?
8. To what extent has your wheelchair improved the quality of life of your family?

Appendix I: Dissemination of PhD work

As part of the process of this PhD five empirical chapters were written (chapters 3-7). Thus far one has been published and a further two will be submitted for publication shortly. The systematic review chapter was published as a shortened paper in BMC Health Services Research in July 2014. The DCE and systematic review results were presented as posters at the annual RESNA conference in Indianapolis in June 2014. I will disseminate relevant findings regarding service delivery to local and national wheelchair services and social services on the completion of this PhD studentship.

Published papers

Bray, N., Noyes, J., Edwards, R.T., Harris, N. (2014) *Wheelchair interventions, services and provision for disabled children: a mixed-method systematic review and conceptual framework*. BMC Health Services Research, 14:309.

Oral presentations

The value of a wheelchair: Applying health economics to wheelchair provision for children with disability. Institute of Medical and Social Care Research Winter Symposium; December 2011, Deganwy.

Effectiveness, cost-effectiveness and service-user perspectives of assistive mobility technology for disabled children: An ongoing mixed-methods systematic review. Welsh Health Economists Group Meeting; June 2012, Swansea.

Effectiveness, cost-effectiveness and service-user perspectives of assistive mobility technology for disabled children: A mixed-methods systematic review. Children, Young People and their Families Health and Well Being Seminar Series; January 2014, Bangor.

Poster presentations

Economic evaluation of mobility technology for disabled children. Health Economists' Study Group Meeting; June 2011, Bangor University

Assistive mobility technologies for children and young people with disabilities: A mixed-method systematic review. Children and Young People's Research Network Conference; October 2012, Cardiff.

Pilot discrete choice experiment to understand the wheelchair service priorities of young wheelchair users and their parents. Rehabilitation Engineering and Assistive Technology Society of North America Conference; June 2014, Indianapolis

More than mobility: a mixed-method systematic review of wheelchair interventions for children with disabilities. Rehabilitation Engineering and Assistive Technology Society of North America Conference; June 2014, Indianapolis

Appendix J: Revisiting research objectives with key findings from the thesis

In this section I will review each research objective outlined in chapter one and present evidence from relevant chapters to address these objectives. The evidence will be mapped on to the conceptual framework to illustrate how this thesis has created new knowledge to guide the development of cost-effective wheelchair services in the UK. All findings and conclusions are drawn from the stated chapter.

Chapter three: Relevant thesis aim: Through a mixed-method systematic review explore effectiveness evidence, service user perspectives, current policy and economic evidence relating to wheelchairs for disabled children, in order to develop a conceptual framework to inform future research and wheelchair service development.

1. *To establish what evidence currently exists regarding the effectiveness of wheelchairs in terms of clinical, social, educational and developmental benefits for disabled children.*

Wheelchair interventions for disabled children offer a range of clinical, social and developmental benefits, including improved social and play skills; improved functional movement and mobility; developmental benefits including communication, social behaviour, occupational performance and activities of daily life; and improved wheelchair driving skill/competence. Furthermore, wheelchairs for disabled children can also provide better outcomes for caregivers through children's reduced need for caregiver assistance and reduced caregiver stress. Specific educational improvements were not measured or found in the literature. Most literature was in reference to PWC interventions, and thus these results have specific relevance to PWCs.

2. *To establish what evidence currently exists regarding the perceived barriers and facilitators of providing and using wheelchairs for disabled children, taking into account the different perspectives of disabled children, parents/carers, and healthcare professionals.*

Barriers to provision of wheelchairs include long wheelchair service waiting times, poor maintenance procedures and restrictive eligibility criteria. Parents described a process of coming to terms with their child's wheelchair use, which can affect their acceptance of certain forms of mobility equipment prior to provision. Environmental and structural factors, such as access to public spaces and ability to transport wheelchair equipment can be a barrier to wheelchair use. However, wheelchairs were perceived to offer a new lifestyle to children, allowing increased independence and socialisation. These benefits facilitate improved QoL and better health/wellbeing.

3. *To gather current policy, not-for-profit organisation publications and clinical guidelines regarding wheelchair provision for disabled children.*

A number of recommendations were found in the policy/NFPO literature relating to wheelchair provision and wheelchair services. The key issues were reducing waiting times for assessment and delivery of wheelchairs; improving joined-up working between health, social care, education and charitable organisations; adopting a holistic approach to measuring effectiveness and outcomes relating to wheelchair interventions; improving funding and procurement strategies, particularly for PWCs; improving aftercare and information provision; reducing the restrictiveness of eligibility criteria to improve access; and giving service users more involvement in how services are structured. Many of these recommendations reflect the opinions of service users.

4. *To establish what evidence currently exists regarding the costs, economic implications and incremental benefits of wheelchair interventions for disabled children.*

There is a distinct lack of high quality economic evidence in this field. At present there are no appropriate economic evaluations regarding wheelchair interventions for disabled children. Only two relevant studies were found, both of which lacked sufficient quality to make accurate judgements about the cost-effectiveness of wheelchairs for disabled children.

5. *To understand the extent to which intervention study outcomes and policy recommendations reflect the barriers and facilitators of wheelchair use (expressed in opinion evidence).*

In general policy and NFPO recommendations did reflect the perspectives of disabled children and their families, but due to a lack of effective knowledge translation current practice is unable to fulfil all of these recommendations. The barriers to effective provision and use of wheelchairs are still present in NHS services, although steps have been made to reduce waiting times and eligibility criteria issues. Both the intervention evidence and opinion evidence demonstrate the need for holistic assessment of wheelchair intervention effectiveness, with particular focus on children's psychosocial needs. Policy could do more to improve the accessibility of public spaces for people with disabilities.

6. *To build a conceptual framework mapping areas for future research and service development to facilitate cost-effective wheelchair services for disabled children.*

The conceptual framework developed as part of chapter three represents one of the key novel contributions from this thesis. It details areas for future wheelchair service development and the

relevant steps needed to address current service issues. The aim of this conceptual framework is to guide the development of wheelchair services for disabled children using high quality effectiveness and cost-effectiveness evidence.

Summary of conclusions: Chapter three

A. A mixed-method approach to systematic reviewing in health economics allows a greater contextual understanding of the intervention(s) and population(s) of interest.
B. Wheelchairs offer a range of clinical, developmental and social benefits to disabled children. The most important outcomes for service users and their parents were related to socialising and independence.
C. Wheelchair services can act as a barrier to wheelchair provision due to strict eligibility criteria, waiting times and lack of information. Policy and NFPO guidance reflects these issues, however subsequent recommendations have not been fully implemented into practice due to knowledge translation issues.
D. There are important gaps in current knowledge regarding health economic methods and available outcome measures in wheelchair provision for disabled children.
E. The lack of economic evidence in this field highlights the lack of appropriate methods to evaluate cost-effectiveness. Establishing the cost-effectiveness of interventions is a key priority to promote efficient services and appropriate allocation of funding.

Chapter four: Relevant thesis aim: To examine the costs associated with the supply of a wheelchair to a disabled child.

1. *To compare the relative wheelchair and customisation costs for different types of wheelchairs.*

The mean cost of a wheelchair was £3294.15 per child, with customisation costing an average of £769.31 per person and making up 18.9% of capital costs. PWCs had the highest average wheelchair costs (£4,421.80) and total costs (£5871.81). Adult active MWCs had the highest average customisation costs (£1985.81). The lowest cost wheelchairs were child standard MWCs (£300), which also had the lowest customisation costs (£55.82). Adult active MWCs had the highest proportion of customisation costs compared to wheelchair costs, with an average of 60.3% of adult active MWC capital costs associated with customisation. On average the BIME Wizzybug (Under 5's PWC) had the lowest customisation cost to wheelchair cost ratio, with 5.7% of capital costs associated with customisation.

The annual and total cost of supplying a wheelchair was greater for charity wheelchair services than for state (NHS) services. This may reflect the level of provision provided by each service, and the amount of customisation provided to each recipient. On average, WK supplied PWCs had the highest annual and total cost per recipient while NHS supplied standard MWCs had the lowest total cost per recipient. For almost all equivalent types of equipment, WKs annual and total costs were greater than those of the NHS, although comparable individual wheelchair models could not be analysed between services.

2. *To estimate staff time and costs associated with the provision of a wheelchair.*

There was variance in staff time and costs between the different wheelchair services due to differences in salaries and time spent on each stage of wheelchair assessment/provision. BIME staff costs were lowest per wheelchair provision (£259.74) while NHS staff costs were highest (£306.61). This was mainly due to differences in length of time spent on work/consultations directly associated with provision of a single wheelchair. In general staff costs were relatively similar between the three services. In order to accurately estimate staff costs, four factors must be taken into account: salary, staff time per stage of assessment/provision, follow-up procedures (e.g. annual reviews and maintenance) and overheads.

3. *To examine theoretical cost savings associated with recycling wheelchairs.*

By recycling wheelchairs theoretical NHS total cost savings of between 9% and 14% could be achieved, compared to a 'no-recycle' scenario. This provides impetus for improving the maintenance procedures of wheelchair services so that stock is fit for re-use where possible.

Summary of conclusions: Chapter four

A. Costing of wheelchair interventions requires consideration of wheelchair costs, customisation costs, staff time (and associated overheads), repair/maintenance costs and refurbishment costs.
B. PWCs appear to be the most expensive wheelchair interventions, while standard MWCs are the least expensive. Charity services have higher capital costs than NHS services, potentially due to higher quality of wheelchairs supplied and additional customisation.
C. The cost of wheelchair customisation accounted for almost a fifth of capital costs associated with the supply of a wheelchair. It is therefore paramount that customisation costs are factored into all costing exercises.

D. Refurbishment and recycling of wheelchairs was estimated to provide cost savings of between 9% and 14% to NHS wheelchair services. Repair and maintenance resources could therefore be increased to maintain the condition of NHS fleet stock and to reduce overall expenditure.
E. The BIME Wizzybug is a relatively low cost powered mobility device specifically designed for children under the age of 5. Additional research is needed to understand the cost-effectiveness of the Wizzybug and similar equipment.

Chapter five: Relevant thesis aim: To undertake a pilot Discrete Choice Experiment (DCE) to explore families' views on the most important attributes of wheelchair services.

1. *To compare the preferences of disabled children and their parents for different attributes of wheelchair services.*

Child and parent samples showed wheelchair service preferences for comprehensive wheelchair assessments and shorter wheelchair delivery times (in that order). The cost contribution attribute was only significant for the child sample, who showed preference for lower cost contribution. The disabled child and parent samples differed in direction of coefficient preference for level of training provided by the service and the frequency of wheelchair reviews. Everything being equal, parents preferred basic wheelchair skills training and more frequent wheelchair reviews, while disabled children preferred wheelchair and life skills training but less frequent wheelchair reviews. Sub-group analysis of matched-pairs of children and parents revealed that only comprehensiveness of wheelchair assessment was significant.

2. *To calculate hypothetical marginal rate of substitution values for different configurations of wheelchair services using cost-contribution as the denominator.*

MRS values were higher for parents than for disabled children, suggesting that the parent sample were willing to contribute more for preferable service attributes. However, as the cost contribution attribute was not significant for parents, it is difficult to make direct comparisons between the samples, as parental service preferences were not significantly impacted by cost contribution. MRS values for the child sample were £152.61 for wheelchair assessment and £98.77 for delivery waiting time.

3. *To evaluate the use of DCE methods in disabled children in relation to wheelchair services.*

Chapter five presents a key novel contribution from this thesis, as it documents the first attempt to use DCE methods to elicit wheelchair service preferences. Furthermore, the incorporation of disabled child

preferences and comparisons between children and parents provides another novel application of the DCE method in this population. One of the most important outcomes from this chapter is that the sampled children had the cognitive ability to understand the process and methodology, thus supporting the use of DCE methods in mobility impaired children from age 11.

Summary of conclusions: Chapter five

A. Discrete choice experiments can be used effectively in young disabled populations (from age 11) to elicit preferences for health care services.
B. The key wheelchair service priorities for sampled parents and children were holistic assessment of wheelchair needs and wheelchair delivery in a timely manner.
C. Theoretically, disabled children and parents would have been willing to contribute financially to receive preferred attributes of wheelchair services, although cost contribution was not shown to significantly impact parental service preferences.
D. Children and parents differed in preferences for frequency of wheelchair reviews and level of training provided by the wheelchair service, however these attributes did not significantly impact service preferences.

Chapter six: Relevant thesis aim: Assess the appropriateness of the EQ-5D-Y and HUI health-related quality of life (HRQoL) outcome measures for eliciting accurate HRQoL estimates from disabled children (and their parents by proxy)

1. *To compare the HRQoL results of disabled children and their parents by proxy.*

For all outcome measures the child sample scored their HRQoL higher than parents by proxy, indicating that parents underestimated their child's HRQoL. The VAS had the highest overall mean score for children and parent proxies (78.93 and 71.75 respectively), followed by the HUI2 (0.54 and 0.42 respectively). Children scored the EQ-5D-Y higher than the HUI3 (0.37and 0.23 respectively), while parent proxies scored the EQ-5D-Y lower than the HUI3 (-0.04 and 0.10 respectively). Differences between child and parent scores were statistically significantly different for all measures besides the VAS.

2. *To assess correlation between the EQ-5D-Y and HUI measures, and respondent type (child and parent proxies).*

Significant large correlations were found between child and parent proxy results for the EQ-5D-Y, HUI2 and HUI3. Strong but non-significant correlation was found between the child and parent proxy VAS results. The HUI2 and HUI3 measures showed the greatest convergence between child and parent proxy results, while the parent VAS showed the least convergence, with all measures other than the child VAS having absent or weak negative correlations. Generally speaking, convergence between equivalent child and parent proxy measures was good, with only the parent HUI3 and child HUI2 exhibiting stronger correlations with non-equivalent measures. Therefore, child self-report and parent proxy results were relatively well correlated for all equivalent measures.

3. *To assess the construct validity of the EQ-5D-Y and HUI measures, with consideration of validity between measures and respondent type (child and parent proxies).*

Construct validity was sufficient between the EQ-5D-Y and HUI2 for both children and parents (and the HUI3 for just parents). Construct validity between the child reported EQ-5D-Y and HUI3 was relatively limited. In general there was sufficient convergence between measures for equivalent HRQoL domains to conclude that they were assessing similar constructs in both samples. Construct validity between children and parents on equivalent measures was satisfactory, although there was some unexpected divergence. The results indicate satisfactory construct validity between equivalent child and proxy measures and thus they can be assumed to be measuring the same HRQoL constructs/domains.

4. *To assess the agreement between the EQ-5D-Y and HUI measures, and respondent type (child or parent proxy).*

All measures showed (to some extent) acceptable agreement between child and proxy measures, besides the EQ-5D-Y. Therefore the results indicate that child and parent VAS and HUI measures could be used interchangeably, but with caution. Although equivalent HUI measures were significantly different between children and parents, agreement and correlation results indicate sufficient association in the matched-pair sample. Conversely, within groups there was insufficient agreement between all parent proxy measures to allow them to be used interchangeably. Similarly, only the child reported VAS and HUI2 measures showed sufficient agreement. Therefore, there appears to be little agreement between measures within both cohorts. This indicates that the measures are not wholly comparable within groups.

Overall, although measures were relatively well correlated they exhibited low agreement within groups and thus were not particularly comparable in terms of HRQoL measurement within groups.

Summary of conclusions: Chapter six

A. On average parents reported their child's HRQoL to be significantly lower than the self-report of the child.
B. The VAS elicited the highest health status scores overall, while the EQ-5D-Y and HUI3 had the lowest.
C. Although outcome measures were relatively well correlated within groups and between groups, they exhibited low agreement within groups and thus were not particularly comparable in terms of HRQoL measurement.
D. Bland-Altman plots offer a robust approach to assessing agreement between measures and respondents, and should be used alongside tests of significance and correlation when assessing association and agreement of outcome measures.
E. The child self-report HUI2 appeared to be the most accurate measure for children with disabilities in this cohort. However there still remains uncertainty as to the validity of both the child and proxy versions of each measure and their applicability in this specific setting. The EQ-5D-Y has limited applicability in this specific setting.

Chapter seven: Relevant thesis aim: To examine how HRQoL is defined by disabled children and their parents, and how it can best be measured for the purpose of economic evaluation.

1. *To understand the key domains of QoL defined by disabled children and their parents in relation to wheelchair use and mobility impairment.*

15 categories of QoL were identified by participants. Through qualitative framework analysis these categories were used to develop three analytical themes which reflected how participants defined QoL in relation to health and wheelchair use, these were: participation and positive experiences; self-worth and feeling fulfilled; health and functioning.

2. *To examine differences in how disabled children and parents define QoL in relation to wheelchair use and mobility impairment.*

The greatest difference between children and parents was the theme of health and functioning. Parents, particularly those of younger children, indicated that health and functioning significantly impacts QoL. Health and functioning became a less common theme as child age increased, and was of little relevance to older children's definitions of QoL. Self-worth and feeling fulfilled was exclusively important for

parents of children under the age of 5 and older children, indicating different QoL perspectives associated with age as well as perspective (child or parent).

3. *To explore the extent to which generic preference-based HRQoL measures, such as the EQ-5D-Y and HUI2/3, reflect how disabled children and their parents define HRQoL in relation to wheelchair use.*

In general participants indicated that the domains of HRQoL in the EQ-5D-Y and HUI measures were to some extent relevant to disabled children, although the available options for each question were insufficient and question wording had generally limited applicability to people with disabilities. Specifically, the mobility/ambulation domains had little relevance to wheelchair users due to positivist assumptions about walking and mobility. All participants indicated that although mobility does impact HRQoL, mobility is more than just walking. In conclusion, the HRQoL domains used in these measures are generally appropriate, but the available answers and question wording lack applicability to disabled children.

Summary of conclusions: Chapter seven

A. The EQ-5D-Y in particular needs to be updated in order to improve applicability to children with disabilities. Rewording or restructuring of levels is required. Furthermore, child value sets are required to fully understand the HRQoL of children using the EQ-5D-Y.
B. Disabled children and their parents defined QoL through three distinct but interrelated concepts: participation and positive experiences; self-worth and feeling fulfilled; and health and functioning.
C. It is important to consider differences in the way children and parents define health and QoL, as this may affect the applicability of self-report and proxy measures.
D. For the purpose of economic evaluation it appears that the EQ-5D-Y in particular is not appropriate to elicit reliable utility scores for disabled children, as the HRQoL domains do not match with the perspectives of disabled children and their parents.
E. Capability or child-specific HRQOL measures may be a more reliable and valid source of outcomes in this population, although additional evidence and measures are needed.