Haemophilia 2010; an Exploration of the Lived Experience of Boys with Haemophilia

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DECLARATION

I certify that this work has not been accepted in substance for any degree, and is not concurrently being submitted for any degree other than that of Doctor of Philosophy being studied at the University of Greenwich. I also declare that this work is the result of my own investigations except where otherwise identified by references and that I have not plagiarised the work of others.

Kate Khair 01/05/2013

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ABSTRACT

There is a paucity of psychosocial research into living with haemophilia from a child's perspective. Published works focus predominantly on the impact of HIV infection which occurred in the mid 1980's. Since then treatment has been improved and intensified so that most children with severe haemophilia are treated with intra-venous infusions every other day to prevent bleeding. This means that children can lead near normal lifestyles, yet it is not normal to be expected to learn to self-infuse and self-manage a long term condition from a young age.

This thesis provides a cohesive body of work examining life with haemophilia from the child and young person's perspective. The aims of the study were to capture the views of children across a wide age range using a variety of age appropriate research methods, which play to the strength of individual children. The voices of children as young as four years of age are included and shed light on very young children's views of living with long term health issues.

Twelve peer-reviewed published papers form the main body of the thesis. These represent a significant contribution to haemophilia psycho-social research of the last decade and are already impacting on future studies and outcomes monitoring measures for people with haemophilia and related bleeding disorders.

CONTENTS

BACKGROUND

1.1 Long term conditions in the United Kingdom

Children and young people in the United Kingdom (UK) have benefited from advances in medical care and technology over the last two decades. Therefore there are now many children surviving into adolescence and adulthood with long term conditions (LTC), who would previously died in childhood. Many of these LTC, e.g. epilepsy, asthma or diabetes, present after a period of time where the child was well. However many, such as cystic fibrosis (CF), muscular dystrophy and haemophilia, which result from genetic abnormalities, present with symptoms in neonates and/or infancy; some of these children may have been known to be 'at risk' of, or diagnosed with, these conditions whilst still in utero.

Due to enhanced therapeutic options there has been an increase in births of children in families affected by some inherited LTC, as these conditions have become more treatable, with care being given at home rather than is hospital as was the case in the past. Consequently there are increasing numbers of children growing up with LTC who need to adapt their lifestyles to include health management, which impacts on their individual long term outcome (Kirk et al 2010).

The majority of research undertaken in LTC has been with adults with late onset conditions such as diabetes, cardiac or lung disease, and in children/ young people with relatively common, well known conditions such as diabetes and cancer survivorship. In a meta-analysis of the research on LTC in children (attention deficit hyperactivity disorder, asthma, CF and diabetes) Kirk et al (2010) identified nearly 2700 papers relating to self-care and support interventions with children with LTC. In

less high profile LTC there are significantly fewer publications relating to how children and young people manage their conditions, the impact the condition has on them as individuals both now and in the future. For those with genetic diseases this includes the impact of reproductive choices for them and their potential carrier siblings in the future.

Although historically there has been a lack of research with children with LTC, this is changing as researchers within the fields of health and education recognise the impact that living with a LTC has on children's lives. Whilst the focus of this research will predominantly remain on the higher profile diseases such as asthma and diabetes where the impact is greater for the population, there remains a need to study the impact of growing up with rare diseases such as haemophilia. It is particularly in these rare conditions where children can feel isolation, from knowing no other affected individuals of their own age who live close by, or who have to come to terms with perfecting relatively technical medical interventions as young children to enable them to live full lives.

The world of paediatric haemophilia in the UK is small when compared to other LTC of childhood. The United Kingdom Haemophilia Centre Doctors Organisation (UKHDCO) national haemophilia database (UKHCDO 2011) records only 655 boys aged <18 with severe haemophilia in the UK (the focus of the body of work presented here). One hundred and twenty boys with severe haemophilia (approximately 20% of the UK population) are registered and treated at the haemophilia centre at Great Ormond Street Hospital for Children NHS Foundation Trust (GOSH): the site of this study.

The relatively small size of the UK haemophilia community, both patients and treaters, impacts on the amount of clinical and psychosocial research undertaken with this group of patients when compared to children with other LTC such as cystic fibrosis, asthma and diabetes. However the global haemophilia community is vast with many patients, particularly in developing countries, only recently being afforded a diagnosis or access to treatment. Thus the experiential learning of researchers in haemophilia care in the UK impacts on a global community through shared learning and publication of research results and outcomes, expanding the community of practice (Wenger & Snyder 2000) globally.

Researchers who are health care professionals, working with these groups of children and young people, are in the privileged position of being able to impact both on immediate care and on long term support. By asking pertinent questions of the children/young people we teach and to whom we deliver care, and researching the impacts of care, we are able to direct research into clinical practice, promoting an evidence base which influences the care that these children receive now as well as in the future.

1.2 Introduction to Haemophilia

Haemophilia is an X linked, usually inherited disorder of coagulation factors VIII (haemophilia A) or IX (haemophilia B) which occurs in approximately 1;10,000 live births in the United Kingdom (UK). As an X linked condition it only affects boys, though is carried by females, and results in joint bleeding which can be either spontaneous or due to trauma, which is painful and reduces mobility. Between two-

thirds and one half of newly diagnosed boys are born into families where there is no previous history of haemophilia in the maternal line. Severe haemophilia, with factor levels of <1iu/dl (normal range 50-150iu/dl) has the worst clinical outcome with frequent, spontaneous, bleeding from an early age which precedes young age arthritic joint damage.

Historically boys with severe haemophilia spent many weeks in hospital, on bed rest receiving plasma infusions for bleed treatment. This resulted in extended periods of school non-attendance and the creation of a residential hospital school specifically for boys with haemophilia in Hampshire in the United Kingdom. These boys spent prolonged periods of time in the school hospital and had poor educational achievement (Jones 1995)**.** They are now in their 40's 50's, and 60's and despite the separation from home, and the bleeding, pain and disability that they experienced they describe the school as a place where 'they were all in it together' and where there was 'great camaraderie and support' (personal communication with ex school residents). This is particularly the case for those boys who were infected with blood borne viruses who formed support groups who now lobby the UK Government for compensation for those affected as well as for safer treatments for all.

Treatment of children and adults with haemophilia in the UK recently became more standardised with the introduction of national guidelines for treatment developed by the UKHCDO. Early guidelines on the introduction of prophylaxis following the first few joint bleeds, with the aim of bleed reduction/elimination and the use of recombinant products where available to eliminated the risk of blood borne viral infection (UKHCDO 2003), have delivered haemophilia care to patients in the UK

that is infrequently seen elsewhere in the world (Stonebraker et al 2012). Modern day boys receive prophylactic therapy at home (Khair 2006) from a very early age; this has been proven to reduced bleeding and disability even in children with prior joint damage (Liesner at al 1996), and to prevent joint bleeds in those treated with early prophylaxis compared to children treated only after a bleed has occurred (Manco-Johnson et al 2007). From a medical viewpoint boys who have haemophilia can, for the first time ever, lead a near normal life, including participation in contact sport, and are close to their peers in physical and psychological development.

Care is now given predominantly at home by families/carers and later by the boys themselves, individually tailored around activity and risk. Therefore boys may miss direct contact and shared experience with others who also have haemophilia. Unlike the boys who were historically treated as in-patients in hospital, many contemporary boys have little or no contact with others with haemophilia; this is an unforeseen consequence of improved treatment which has led to a lack of support and peer-to-peer experiential learning.

1.3 Haemophilia care in the UK:

In the 1990's haemophilia care in the UK was re-configured to be delivered through specialist 'comprehensive' care centres (CCC's) which were audited against national standards which included provision of specialist clinical services required by patients with bleeding disorders. To be recognised as a CCC the centre must treat at least 40 severely affected patients, offer twenty-four hour access to care, have clinical nurse specialists (CNS) in haemophilia and participate in research studies. Smaller centres

that could not provide this level of care (Haemophilia centres) joined in networks with CCC's to provide comprehensive care for all patients.

A pivotal role within CCC's is the nurse specialist whose role promotes clinical practice, advocacy, advice, education and research (Manley 1997). The UK Haemophilia Nurses Association publication (HNA 1994) on the role of the haemophilia CNS recognised that the CNS should be aware of research in clinical practice, participate in data collection and seek opportunities to initiate nursing research. Nurse Consultant roles were introduced to haemophilia care in the UK in 2000. These roles have four key components: expert practice, leadership and consultancy, education and training and research and evaluation for which some have protected research time.

Despite these two groups of senior nurses having research as a key component of their role, and the recognition that nurses are best placed to deliver the agreed European Principles of Haemophilia Care (Colvin et al 2008), there remains a paucity of research undertaken by nurses within haemophilia care. Colvin et al (2008) state that haemophilia nurses are key components of the care team, who have excellent relationships with patients and their families and are best placed to undertake qualitative research and studies with patients and families evaluating health care outcomes and quality of life impacts of new treatment options. Despite these recommendations, the work that is the focus of this exposition is unique. Despite there being nine children's hospitals with haemophilia centres run by haemophilia nurse consultants or CNS's in the UK, there remains a scarcity of research practice and a paucity of publications related to our work. The research collated here represents a

significant proportion of the publications related to the psychosocial impact of having haemophilia as a child/young person published in the last decade.

In publication 1**,** a study of thirty two experienced haemophilia nurses within the UK, nurses report a lack of time and the necessary skills to undertake and publish research. These issues are reported by nurses worldwide (Breimaier et al 2011, Morna-Casbas et al 2011), however these same haemophilia nurses recognise the importance of nursing research and are able to identify key aspects of haemophilia nursing that require research including: the role of the nurse and their impact on patient care, issues in females with bleeding disorders, concordance with therapy, reproductive choices for carriers and the impact of genetic disease on families.

Several of these areas of research activity are starting to be addressed within my work, most of which is presented within this exposition. An on-line community of practice (Wenger & Snyder 2000) for the haemophilia nursing community has been established (www.haemnet.com) with interested parties sharing ideas and information, or seeking answers to questions which raise more research questions, I am the lead nurse collaborator for this site; currently we have three research/audit projects in progress with further planned work and publications.

1.4 Background of the researcher

I was appointed as a nurse consultant in haemophilia in 2003 having been a nurse specialist in haemophilia since 1991. The institution in which I am employed recognised the research component of each of these roles as key and protected academic time was provided to undertake doctoral research as a nurse consultant.

Between 1995 and 2013, I have produced 65 peer reviewed papers, 6 book chapters and numerous publications for patients and parents examining disorders of coagulation, therapeutic options, care delivery and outcomes of treatment. The paucity of data on contemporary treatment for modern day children encouraged me to embark on a research programme to discover the views of children and young people with severe haemophilia treated at my own hospital. In selecting 12 of my 65 publications I seek to illustrate the coherence of my work showing progression from the early quality of life papers discussed in 3.1, to later work about children's views of living with haemophilia expressed in their own words. This exposition focuses on twelve papers which are listed in part 6 where each is given a publication number e.g. (see publication 1) or just (1). Other papers, either mine or those of others used to reference the body of this exposition are referenced fully in part 8.

The body of work examines how medical advances in haemophilia treatment impact on the lives of children, giving insight into what it is like to live with a condition that was previously life limiting/threatening and which is now treatable and from a medical perspective allows, for the first time ever, a near normal life style to be led (Liesner et al 1996). Discovering the impact of these medical advances on life with haemophilia has required a multi-method research programme of theory and practice.

Empirical work has included an array of research techniques tailored to the ability of children across a wide age range to elicit their views. This has included draw and write techniques and the use of digital photography (both used as an aide-memoir for face-to-face semi-structured interviews with young children), focus groups, and

individual interviews with older children and young people. The research programme was informed by theory of haemophilia care which draws on international literature from health, nursing, medicine, sociology and psychology.

The papers included in this exposition are shown diagrammatically in figure one (page 10). Here it can be seen how the papers interlink providing a cohesive body of evidence of life with haemophilia from the child or young person's perspective.

2. METHODOLOGY

The 'Haemophilia 2010: an exploration of the lived experience of boys with haemophilia' (Haemophilia 2010) study was designed to allow children to tell their life stories in their own words through narrative accounts. A description of the methods used as well as an analysis of the methodology can be found in paper 2. The research undertaken within this study used multiple methods which although not innovative in themselves are used here in collaboration and have allowed data to be collected with children and young people, across a wide age span (4-18 years) with haemophilia. From this study and my previous published research work, a body of evidence is included in this exposition which relates to the experiences described by the study participants.

The Haemophilia 2010 study included the views of boys with haemophilia from its inception; a patient co-researcher was involved in the study design, suggesting various methods of engaging boys with haemophilia in research stating: '*we are still living in the shadows of HIV and my generation is one that now needs to have a voice'* he also co-facilitated the focus group providing views of an older teenager to the younger

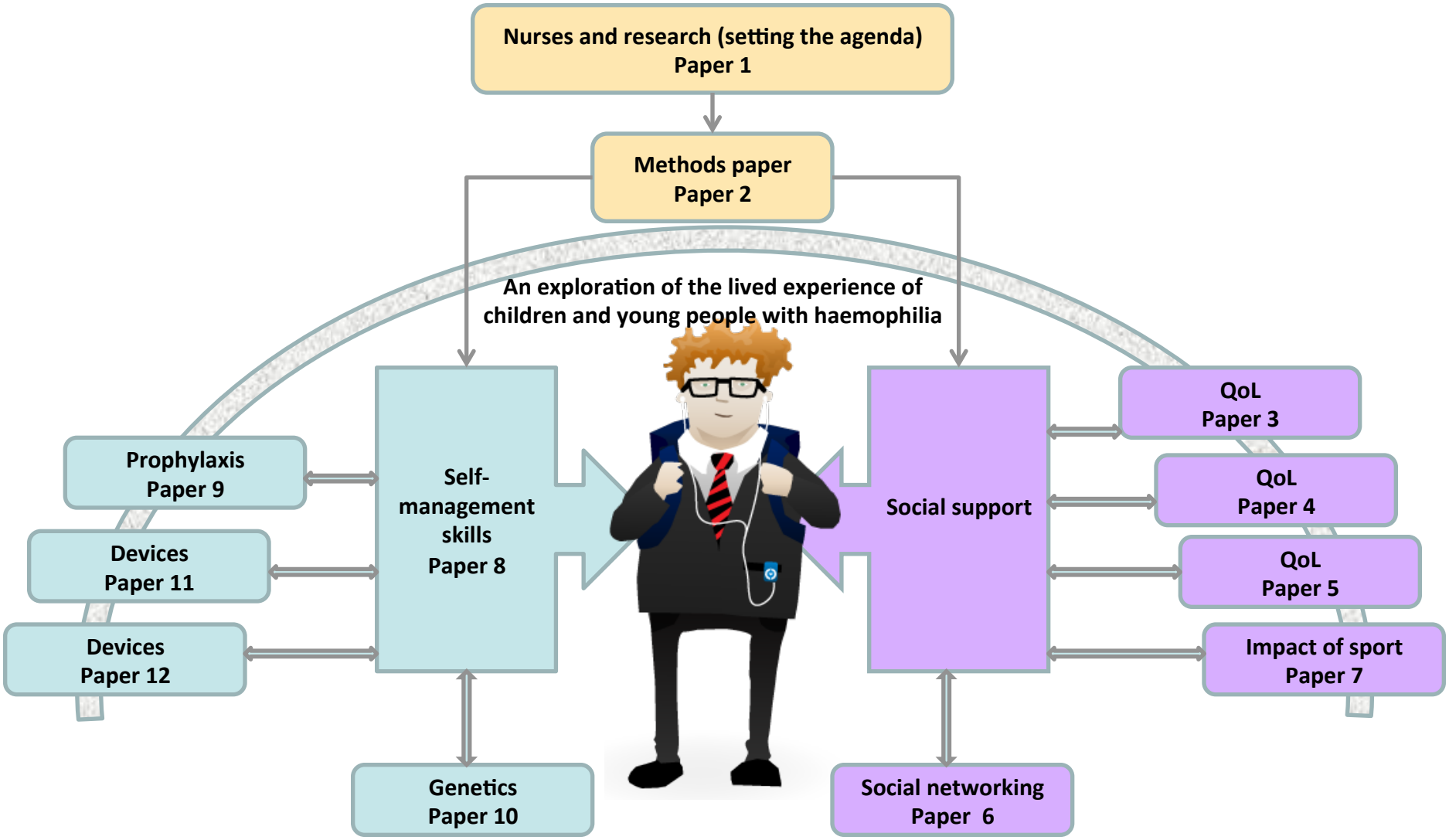

Figure 1: A diagrammatic representation of the papers included in this exposition, showing how the body of work interlinks to report life for children and young people with haemophilia in the twenty-first century.

boys for discussion. In later work (paper 6) focus groups of expert patients were established to shape the research, analyse findings and direct implementation of new and novel ways of supporting other affected individuals.

The UK Haemophilia Society (the national patient forum) reviewed the proposal. They made constructive points about the study which were included before submission for ethical approval which, after considerable debate over the use of this mixed and previously unreported methodology, was granted by South East Research Ethics Committee in 2008. Patient involvement in study design identifies appropriate research questions with an understanding of youth issues and meaningful involvement of co-researchers for whom research experience promotes opportunities to contribute to their communities and services (health and education) (INVOLVE 2004).

Throughout the research presented here (papers 6, 9 -12) focus groups of children and young adults have been used to gain views about living with haemophilia. In paper 6 the focus of work on establishing an educational on line and social network community forum for children with bleeding disorders was shaped by comments made by boys with haemophilia. Their view that a haemophilia specific site was not needed and that information was needed for boys and girls with any manner of bleeding disorder had a significant impact on the way the site has been, and is being, developed. This has led to further competitively funded work (not submitted within this body of work) on issues experience by girls and young women with bleeding disorders. This work is currently being analysed and will result in papers being submitted for publication in early 2013.

3. CHILDREN AND YOUNG PEOPLE AS SOCIAL ACTORS

James and Prout (1995 p78) suggest that focussing on children as competent social actors enables us to learn more about the ways in which 'society' and 'social structure' shape social experiences which can in itself be 'refashioned through the action of members'. Children's understanding of their position within society and how this is affected by familial and societal support impacts upon the child's quality of life.

Paediatrics sees children as individuals who are different from adults, with diseases '**of** rather than **in** children' focusing on the 'social context of the body, disease and impact on children' (James et al 2006 p151) rather than on the disease only. Variation from the 'norm' can be significant, with children wishing to be like every one else whilst, at the same time, wanting to be seen as individuals who are different from their peers. These differences are usually chosen such as clothes, hairstyle, piercings or jewellery, and not imposed by visible ill health or disability.

Figure one (page 10) shows two domains of life with haemophilia, these are the social support domains which will be described here and the development of self-care skills domain which is discussed later in section 4. These papers demonstrate how boys with haemophilia are accepted by family, peers and others with and without haemophilia as part of their own individual societies. This is achieved by family/peer support, sporting participation and prowess, the internet and by using the self-care skills described in this body of work see pages 18-20, which promote good QoL enabling participation in normal daily activities. On pages 22-23 I discuss how Staying Positive courses can be instrumental in improving social support for children and young people with LTC. The opportunity to attend these courses has been declined by

the boys who participated in the Haemophilia 2010 study as they do not define themselves as young people with an illness (which they perceived others at Staying Positive courses would have), but rather as individuals who have an easily managed condition which allows them to have a good QoL and to live near normal lives (personal communication, data not shown).

3.1 Quality of life in children and young people with haemophilia

There is a paucity of psychosocial research with those with haemophilia. The publications that are available focus on the impact of blood borne viruses such as HIV and Hepatitis C, genetics, pain management and disability. Little of this research comes from the UK where haemophilia care has progressed incrementally since the 1980's, when these patients were infected with blood borne viruses in plasma derived coagulation factors. Historically patients were treated 'on-demand' only after bleeding had occurred. In the mid 1990's the haemophilia centre in which I work had changed it's treatment programme to a prophylactic treatment schedule with all children with severe haemophilia being treated three times per week, with intravenous infusions of clotting factors in an attempt to prevent bleeds occurring. This treatment has intensified with some, usually sporty boys, now being treated daily to facilitate sporting activity.

In the mid 1990's there was interest within the haemophilia medical community to explore the impact that the improvements in care were having on the lifestyles of children with haemophilia. A European working party was established to develop tools which could be used to assess and measure quality of life (QoL) in children with haemophilia; I was the representative from the United Kingdom on this working party

which produced a new haemophilia quality of life assessment tool (Haemo-QoL) which could be utilised in children as young as four years of age. My role in this research was to develop pertinent questions relating to QoL for children across an age range using language that could be understood by even very young children. I then field tested the English version of the QoL questionnaires with a small group of boys with haemophilia, and their parents in my own practice. This included scoring how easy the questions were to understand and their relevance to day-to-day life with haemophilia, on a visual analogue scale. The working party then further refined the questions based on ease of answer, percentage of questions that were not answered, and how well questions correlated across the age groups. The final Haemo-QoL questionnaire, the first disease specific QoL assessment for haemophilia, was designed and implemented in a multi-centre (n=20) European study of QoL in 339 children with haemophilia. I recruited the UK cohort to this study and was involved in content validity, data analysis and publication writing.

Three seminal papers were derived from this work, (publications 3-5) which have had a major impact on haemophilia treatment assessment globally with the Haemo-QoL questionnaires now being available in 54 languages including common western European languages, Arabic, Bulgarian, Farsi (Persian), Russian and Thai. Thus comparisons of QoL in children with haemophilia can be made across continents, where therapeutic interventions differ, to the show benefits of one treatment over another, or changes in treatment practices (see impacts 7).

Disease specific QoL assessment is important as generic QoL assessments are not able to provide clear patterns of symptoms or impairments related to specific diseases,

nor sensitive enough to evaluate treatment outcomes (von Mackensen and Gringeri 2005). The Haemo-QoL assessment tool is now used in routine clinical practice as well as in the assessment of new therapeutic treatments to 'prove' the benefit of treatment on QoL. There is an increasing focus on QoL assessment in haemophilia care as patient related outcome measures are now being collected as evidence of effective (and cost-effective) care (Aledort et al 2012).

Over the last ten years however, I have come to perceive the Haemo-QoL tool to be insensitive as it does not address issues that are important for boys living with haemophilia. In part this is because publications 3 and 5 show little difference in QoL across six European countries regardless of treatment availability. Bowling (2005a p149) describes self-reported QoL as good or excellent dependent upon 'finding a balance between body, mind and the self' even when QoL would be described as undesirable by external observers, thus boys in some European countries with limited access to treatment still describe good overall QoL. Boys treated at my own hospital have excellent QoL scores (publications 3 and 5) yet describe a lack of peer-to-peer education and support (see paper 6) in part because treatment is community based and hospital visits are a rarity.

The Haemo-QoL questionnaires demand a four week recall of assessment; this is difficult for children who often can't remember last week, let alone last month, unless something 'different' (such as a birthday or Christmas) occurred in their lives. Recall bias (or memory testing) can occur when asking details about the past and can impact on findings. Bowling (2011 p332) recommends recall time periods being selected by the respondent's age; thus a shorter recall period should have been used for the 4-7

year old boys. The questionnaires demanded answers to questions, on a five point scale: ‘never’, ‘seldom’ ‘sometimes’ ‘often’ ‘always’, there was no ‘not applicable’. In order to complete the questionnaire children had to tick the box that was closest to, or most like, how they felt. This may have changed overall QoL scores.

A visual-analogue score (VAS) for pain is also included in the questionnaires. The boys in the study put a cross on this roughly where they felt their pain was yet these were measured to the nearest millimetre when analysed and significance was given to these results. Although VAS scores are regarded highly for reliability, validity and sensitivity (Bowling 2005b) I do not believe that these were used appropriately by my participants as all boys put a cross somewhere on the scale even if their recall of pain was nil at routine clinical review.

Another concern about Haemo-QoL is that children were not involved in the initial questionnaire design, only in validation of the questionnaires that were designed by haemophilia and quality of life experts; we therefore do not know that these are the questions that children would have asked. Shortly after the initial Haemo-QoL publications the Canadians designed a questionnaire of their own, CHO-KLAT (Young et al 2004) which involved children from its inception (Young et al 2006). In a review of QoL assessment in children (Solans et al 2008 p758) state that children should be involved ‘at critical stages in instrument development though focus groups, interviews and the phases of item reduction and validation’. Bevans et al (2010 p5) concur with this stating that children’s voices ‘should be heard and respected in the instrument development and modifications of content’ Thus the development of

Haemo-QoL is now thought to be flawed with CHO-KLAT having more 'emphasis on children throughout measure development' (Bradley et al 2006).

My concerns about the sensitivity of the Haemo-QoL instrument led me to re-evaluate what living with haemophilia is like from the child's own perspective. A literature review revealed very few publications about living with haemophilia in the post HIV era in either the nursing/medical or psycho-social literature. In the 1980's and early 1990's when it was becoming apparent that HIV was a blood borne viral infection that had infected the haemophilia community, there was significant interest on the impact that this new disease was having, from children (Logan et al 1990) through to adults (Goldman et al 1992, 1993a, 1993b) who were themselves infected and/or worried about infecting their partners and children.

3.2 Social support for boys with haemophilia

The content of the narratives from the Haemophilia 2010 (see section 2 page 9) study described a component of social support that was important for the boys and allowed them to lead the lives that they did; they felt supported and loved, despite the issues that haemophilia caused for them and their families. The boys described their parents, siblings, other family members and friends as key members of their individual support networks and how these relationships enabled them to lead 'normal' lives. Emiliani et al (2011) describes a process of 'normalization' within haemophilia families, led by parents which develops into extended family and peers.

As the boys in this study develop they move from a family driven support network to a more individual network participating in peer-to-peer activities such as sport (see

paper 7) which itself has health benefits by building up strong muscle mass which reduces the risk of spontaneous joint bleeds. Friendships which are made at school, via the internet such as Facebook or haemophilia specific web sites, provide on-going and potentially life-long support with peers and others affected individuals. (See paper 6)

3.3 Social networking for adolescents with haemophilia

During the interviews for the Haemophilia 2010 study, several boys with older affected family members commented, that having an affected older family member was beneficial for support from those who 'really know'. They described how others with haemophilia have knowledge and expertise that is shared, and that this was very supportive all of the time but particularly at times of crisis, for example a bleeding episode. It was apparent that boys who had no older affected family members were missing out on this support and the concept of an internet based support system, which could be accessed from wherever they were, was born.

Paper 6 describes how boys from two haemophilia centres in London, described as 'haemophilia experts' by the nurses at their centres, agreed that a 'Facebook-for Haemophilia' programme would be a good idea, where there could be shared communication about complex health issues related to but not only about haemophilia. Following an anonymous UK wide on-line survey SixVibe (www.sixvibe.com), a social network for boys with haemophilia, was designed. It was launched in Beta testing in for a small cohort of boys in London in 2011 and globally on World Haemophilia Day (17th April) 2012, where the aim of the year was to 'Close-the-Gap', (where better access to treatment and support was being advocated).

SixVibe now has ~150 members from 10 countries, and has evolved: all bleeding disorders are now covered and there are two sites, one for girls only where more intimate issues of women's bleeding can be discussed. The site hosts educational materials as well as chat rooms; there are future plans to analyse the educational aspects of the site as e-health has a recognised role in LTC management in adults with haemophilia (Mulders et al 2012).

The girls' site has raised interesting questions about being a haemophilia carrier, the impact this may have in some 'closed' communities such as the Orthodox Jewish or Muslim communities where being a carrier may impact on marriage choices, and how bleeding disorders in these communities are kept 'secret'. These issues give rise to more research questions such as how to access these young women with appropriate health advice and support, which are being addressed through a second Bayer Caregivers award, awarded jointly to myself and a clinical nurse specialist in another London haemophilia centre who has a particular interest in women with bleeding disorders. This work is currently being analysed and should result in papers being submitted for publication in early 2013.

3.4 The impact of sport on children and young people with haemophilia

Paper 7 presents the UK children's data from a larger, multi-site UK study, Evaluating the Impact of Sport in haemophilia (EIS) in 400 children (aged 6-17) and adults with haemophilia. The study was undertaken in four haemophilia centres in the UK; two paediatric and two combined paediatric/adult centres.

The data from the children differs from that of adults (data not yet published) where historical pressures of bleed avoidance due to lack of treatment meant that sporting activity was not encouraged. This is born out by Park (2000) in his description of sport as a masculinising activity, where non-participation caused psychosocial disruption to patients. Younger adults (those <30) who had access to prophylaxis in late childhood and adolescence show a trend to more sporting activity than the older haemophilia population. However some of these young men limit high impact sports as they have arthritic ankles which become painful and/or bleed following these activities (data from adult participants in EIS study).

This is the first time that sporting participation and QoL has been investigated in a multi site study using QoL assessment tools. The tools used in this study were developed from the Haemo-QoL questionnaires by the EIS study working group. We have shown them to be reliable tools, demonstrating better QoL in boys who do sport than those who do not.

There are two further planned publications from the EIS study; the data from the adults and data from parents of boys with haemophilia, on their views of sport for their children. These papers should be published in early 2013 . They are not included in this body of work as they do not relate to the views of children with haemophilia.

4. DEVELOPING SELF-CARE AND MANAGEMENT SKILLS

The papers in this section focus on self-care and the positive impacts that this has on life. Kirk et al (2013) describe a self-care support model for children and young people with LTC with four key dimensions: a sense of community, independence and

confidence, knowledge and skills and engaging children and young people. Kirk et al (2013) used semi-structured interviews of young people with LTC, their parents and self-care support providers, to evaluate the perceptions of effectiveness of self-care support models. The results presented in this exposition are surprisingly similar to those presented by Kirk et al: they show that 'children and young people with LTC regard medicines and their management as part of 'normal' life, they know their own bodies, articulate symptoms and decide on actions needed' (p1978) 'recognise their own ability, manage social relationships and health'(p1984).

The four key dimension of the Kirk et al model (2013) include promoting a sense of community through social networks, in this exposition this is demonstrated through papers 6-7; promoting independence and confidence (papers 8 and 9); developing knowledge and skills (papers 8-12) and engaging children and young people (papers 6-12). Future work comparing the Kirk et al model (2013) with the body of work presented in this exposition is planned.

There are considerable differences between the LTC that Kirk et al describe (diabetes, asthma, CF, and ADHD) and haemophilia. Haemophilia is controllable with minimal personal intervention or limitation of normal behaviour, for example when compared to blood sugar and dietary monitoring of diabetes. Symptoms are usually prevented and are therefore invisible; affected boys look and sound well unlike perhaps those with CF or asthma and do not demonstrate behavioural problems like those with ADHD. The controllability of a LTC may affect the impact that the LTC has on the child or young persons life and is worthy of further study. The publications included in this exposition will add to the published work around self-care for children with

LTC, where in comparison to adults there remains a paucity of data on impact and outcome of formal and informal self-care support programmes (Kirk et al 2010).

4.1 Self-management and skills acquisition in boys with haemophilia

Paper 8 describes how boys with haemophilia develop expertise in self-care and management becoming 'expert' patients over time, through education and experience. Contemporary haemophilia care consists of prophylaxis initiated at an early age with parents and boys becoming proficient at bleed recognition, treatment administration and management of haemophilia.

The acquisition of these skills in boys with haemophilia has not been described previously. Education and support for these boys starts in early childhood with health care professionals and family members supporting boys encouraging them to; learn about their own bodies, manage risk, understand complex medical concepts and become skilled at self-infusion.

This self-care or 'expert patient role', further described here, is one which is supported by UK policy with the government wanting children/young people to have more opportunities to be involved in the design and evaluation of services that affect them or which they might use through the expert patient programme (Department of Health 2009). Programmes aimed at developing expert patient programmes (EPP) specifically for children/young people with non-specific LTC were developed. Later changes added disease specific (HIV/AIDS, Sickle Cell Disease) information and support. The lack of uptake of EPP courses by children/young people is well recognised (Hawley 2005) and was thought, in part, to be due to programme design,

lack of promotion, lack of trained EPP leaders. An EPP (Staying Positive www.staying-positive.co.uk) aimed specifically at children/ young people aged 11-19 with a range of LTC was developed and piloted in 2006-07. Staying positive courses have been offered at GOSH for the last three years to teenagers with haemophilia, but to date there as been no uptake.

4.2 The benefits of prophylaxis

Treatment adherence in adolescents with LTC is a much debated area of healthcare, where inevitably there is a consensus, usually within the medical professions, that adolescents with haemophilia do not comply with treatment (Lindvall et al 2006, De Moerloose et al 2008). In paper 9 I dispute this, showing how boys this study describe the impact of not undertaking treatment, resulting in rapid onset of symptoms requiring the same treatment, causing significant pain and interruption to mobility and significant disruption to their lives.

Fifteen of the thirty boys in the Haemophilia 2010 study voiced thoughts about the benefits of prophylaxis to them as individuals: how they felt 'protected' and 'safe' so that they could 'forget about haemophilia' day-to-day. The boys were engaged in their own health and well being, tailoring treatment around 'risk' activities (such as sport and nights out) and focused on their own 'wellness' rather than 'sickness'. This allowed many of these boys to participate in sporting activity with their healthy peers and siblings. The importance of sport for feelings of normality and masculinity in boys with haemophilia is well recognised (Park 2000, Petersen 2006) and led to the initiation of a larger study of the impact of sport on children with haemophilia (paper 7) undertaken in four haemophilia centres across the UK.

Understanding the complexity of treatment, including factor VIII half life studies, knowing when they are in a 'safe' period to undertake sport or other 'risky' activities by predicting factor levels post treatment, when they should treat, and with which dose/frequency, shows that these boys are developing self-care skills which make them experts in their own haemophilia management.

4.3 Understanding haemophilia genetics and inheritance

This paper, (paper 10) the first published that we can identify relating to genetic knowledge in children with haemophilia, demonstrates how children with haemophilia gain and understand genetic knowledge. The data in this paper was gained from 24 (of 30) boys who participated in the Haemophilia 2010 study. Whilst some of these boys will have participated in the National Curriculum (science) teaching programme, they demonstrated complex and detailed knowledge about haemophilia inheritance for themselves, their siblings, other current family members and their own offspring that was beyond that predicted by their key stage educational levels.

These boys also showed altruistic feelings toward their unaffected male siblings *'its good he didn't have it cause he is only little ..... I'm better off having it myself'* through to being glad that their sisters are not carriers so they will not have affected babies in the future '*that's good if she has some boys then it [sic] won't have haemophilia'*

They would be supportive of new affected family members *'it would be an advantage ....they would know all the stuff I did when I was young'.*

This is the first paper that we can identify which demonstrates how children acquire genetic knowledge from many sources: their families, their health care providers, teachers and the media through developments in genetics, such as genetically modified foods, that are publicised widely. This knowledge further supports the evidence that these boys are becoming experts in haemophilia in themselves and their current and future family.

4.4 Use of transfer devices

This body of work includes two publications about the introduction of transfer devices for reconstitution of coagulation factors. Historically factor infusions consisted of a vial of freeze dried factor, a vial of diluent and a large bore filter needle for mixing the two vials together. In 1998 new EU health and safety guidelines were implemented within the NHS to minimise the risk of needle stick injury to health care workers (DH 1998). The pharmaceutical industry reacted quickly to these new guidelines and introduced needle-less transfer devices following market research with health care workers, adult patients and caregivers of children with haemophilia. Children aged <18 were not, and still are not, routinely asked about the ease of use of these devices. For children there are additional aspects to reconstitution that need to be assessed, these include: ease and method of reconstitution, likelihood of process failure, size of vials/devices, size of pre-filled syringes relative to a child's hand span and ease of learning the process.

Paper 11 shows the results of a small study of 10 boys (aged 5-16) with haemophilia A who were treated with one brand of factor VIII, where a new device, which

incorporated a pre-filled syringe and a needle-less device was introduced into their routine care. This study revealed that children as young as five years had opinions about their health care and treatment and how changes impacted on their self-care. This study led me to think about whether children's voices are heard in healthcare and how changes that are acceptable to adults are often not discussed with children before product changes are introduced which impact upon them.

At this time, there were five different factor VIII concentrates being used in the UK, each with a different transfer device. I thought it would be interesting to see how these devices compared from a child's perspective. This led to development of a study, supported by four pharmaceutical companies.

Paper 12 describes the process and results of this study. Fifteen boys participated in the study. Two devices, BAXJECT, which used a three-way tap which had to be turned twice in the reconstitution process and BIOSET, which had an activation step, were ranked worst as children struggled with knowing when and which way to turn the tap or when to activate the vial. Subsequently these devices have been redesigned: the three-way tap has been removed in the BAXJECT-2 device www.baxject2.com and the activation step has been removed and the transfer devices changed in the new BIOSET device www.kogenate.co.uk . Although I do not claim that these studies were instrumental in initiating these changes, this paper provides published evidence of the impact these devices have on children which have been deemed important by the pharmaceutical companies.

Following on from this work I have become a chair of an international nurse advisory panel for one of the companies above, looking specifically at improvements in transfer devices from the patient perspective. In 2011 I was involved in designing a market research study of 107 patients, 106 parent/caregivers and 195 health care workers across six countries worldwide evaluating an innovative new device which will be introduced to the market within the next year. This work has generated two further publications. The first, a synopsis of the whole study, will be submitted to *Haemophilia* a subsequent publication of healthcare worker results is planned for submission to the *Journal of Intra-venous Therapy* in early 2013.

5 CONCLUSION

The introduction to this exposition described the limited psychosocial research publications of contemporary haemophilia care and the impact this has on children and young people. The papers submitted in this exposition represent the first cohesive body of work on the lived experience of boys, across a wide age range, with severe haemophilia, treated with prophylactic therapy since diagnosis. Multiple age-appropriate research methods were used to elucidate the opinions of these children and young people. A range of papers not usually published together, including QoL and children's views of health and living with a long term condition are brought together attempting to analyse and influence haemophilia care in the UK and globally. The influence that this body of work has had can be seen in section 7 where the impacts of the publications are listed.

In this conclusion I will seek to briefly summarise some of the main findings of my work and the impact that this has had on clinical care and knowledge, as well as the future directions that are evolving from this body of work so far.

This body of work starts from a background of QoL research which was novel, and at the time I believed the best way to assess the impact of the intensive treatment that was being established in haemophilia care in the UK. The Haemo-QoL tools were validated by healthcare professionals and piloted in haemophilia centres where children and young people were asked to comment on the appropriateness of the questions asked. I now recognise that the children weren't involved early enough in the process and only commented upon the questionnaires – we didn't ask them if we were asking the right questions. Thus QoL was assessed as good or excellent for children across six EU countries regardless of treatment intensity. Whilst frequent infusions of factor are reportedly better for QoL (Liesner et al 1996) no-one questioned the boys about how it felt to inject themselves so often. This is an issue for some boys and formed the basis of the rest of this body of work trying to establish what it is like to live with haemophilia from a child's perspective.

Little work had been published in the nursing/psychosocial/medical journals about life with haemophilia from the patient's perspective. The papers presented in this exposition represent the largest cohesive study of life with haemophilia as a child or young person since early work published in the era of HIV infection. Some of the papers presented in this exposition have shown considerable impacts on haemophilia care; for example the Haemo-QoL questionnaires are now used in routine practice as well as in clinical trials of new coagulation factors and are being adapted for smaller

studies such as the impact of sport study (paper 7) and a current EU caregiver QoL study. Other more recently published papers have yet to have demonstrable publication impact, but are stimulating conversation at international levels. Paper 1, *Why don't haemophilia nurses do research*? was widely cited in oral presentations at the recent World Federation of Haemophilia meeting (Paris July 2012). Paper 9 – The benefits of prophylaxis; views of adolescents, was also debated in one of the plenary sessions at this meeting, by an eminent haemophilia doctor. It is unusual for doctors to cite nursing research at international meetings and it was refreshing to see that this caused a ripple of comment amongst fellow nurses who expressed an interest in establishing an international research forum.

Other papers in this exposition demonstrate the normality of life of the boys in this study. Having been born with a LTC they have no recognition of life before becoming ill, as perhaps a child or young person with asthma or diabetes would. They do not define themselves as children who are sick, but rather as normal boys who have haemophilia which can be easily managed. The management of their haemophilia demands self-care skills, which are learnt from an early age, and greater knowledge about their bodies, genetics, and risk management, than might be expected in boys of their age without a LTC.

My work has brought together a body of evidence which represents the beginning of a research programme which I plan to continue, both on my own and within a community of practice through the newly established Haemnet Haemophilia Nursing Research Group www.haemnet.com. This group has several research proposals that are being 'worked-up' for both funding and ethical review. I would like to argue that

this work would not be feasible if it were not for my previous research and publications, making me an internationally recognised haemophilia nurse researcher. This can be illustrated by the collaborative work in the early QoL papers (papers 3-5) where I am both the UK representative and the only nurse, the soon to be published devices papers where I am the lead nurse in an international working group and the commissioned book chapter on social networking in haemophilia due for publication in 2013. Additionally I have received over £50,000 of competitively sought research grants (see 7.2) enabling me to undertake the work presented in this exposition.

6 PAPERS CITED IN THIS EXPOSITION

1) Khair K, Holland M, Vidler V, Loran C, Harrington C (2012) Why don't haemophilia nurses do research? *Haemophilia;* 18; 4; 540-43

2 Khair K, Collier C, Meerabeau M. Gibson F (2013) Multi-methodology research with boys with severe haemophilia *Nurse Researcher (in press)*

3) Bullinger m, Von Mackensen S, Fischer K, Khair K, Petersen C, Ravens-Sieberer U, Rocino A, Saginer P, Tusell JM, Van Den Berg M, Vicariot M (2002) Pilot testing of the 'Haemo-QoL' quality of life questionnaire for haemophilia children in six European countries. *Haemophilia,* **8**, (supp 2), 47-54

4) Von Mackensen S, Bullinger M and the HAEMO-QOL GROUP (2004) Development and testing of an instrument to assess the Quality of Life of children with haemophilia in Europe (Haemo-QoL). *Haemophilia*, 10**,** 17-25

5) Gringeri A, Von Mackensen S, Auerswald G, Bullinger M, Perez Garrido R, Kellerman E, Khair K, Lenk H, Vicariot M, Villar A, Wermes C (2004) Health status and health-related quality of life od children with haemophilia from six west European countries. *Haemophilia*, 10, 26-33

6) Khair K, Holland M, Carrington S (2011) Social networking for adolescents with severe haemophilia. *Haemophilia;* 18; e290-6

7) Khair K, Littley A, Will A, von Mackensen S (2012) The impact of sport on children with haemophilia. *Haemophilia* 18 (6) 898-905

8) Khair K, Gibson F, Meerabeau L (2013) Self-management and skills acquisition in boys with haemophilia. *Health Expectations(in press)*

9) Khair K, Gibson F, Meerabeau L (2011) The benefits of prophylaxis: views of adolescents with severe haemophilia. *Haemophilia;* 18; e286-9

10) Khair K, Gibson F, Meerabeau L (2011) 'Just an unfortunate coincidence': children's understanding of haemophilia genetics. *Haemophilia*; 17; 470-75

11) Khair K (2006) Evaluating a self infusion device for children with haemophilia. *Paediatric Nursing*, 18, 10, 19-20

12) Khair K (2009) Children's preferences of transfer devices for reconstitution of factors VIII and IX for the treatment of haemophilia. *Haemophilia*, 15, 159-167

7 IMPACTS

The journal *Haemophilia* is a specialist journal (impact factor 2.634) which publishes multi-disciplinary research and outcomes papers promoting scientific knowledge about the management and treatment of patients with bleeding disorders. Haemophilia is published quarterly, has recently added a quarterly e-journal to its repertoire and lso publishes papers 'early-on-line' soon after editorial review and acceptance. Therefore papers published in *Haemophilia* may have a rapid impact on haemophilia treaters and thus impact on care. Papers 8, 9 and 12 were published early- on-line within three to four months of first submission, this timely publication offers rapid access by the haemophilia community and therefore impacts on patient care quickly. For this reason I have published nine of the papers which form this exposition in this journal.

7.1 Citations of my papers

Listed below are examples of where my work has been cited

Paper 1; Khair K, Holland M, Vidler V, Loran C, Harrington C (2012) Why don't haemophilia nurses do research? *Haemophilia*; 18; 4; 540-43
Cited by two:

Skinner M (2012) Closing the global gap – achieving optimal care. *Haemophilia*; 18; 4; 1-12

Khair K, Holland M, Pollard D (2103) The experience of girls and young women with inherited bleeding disorders, *Haemophilia*, 19

Paper 3: Bullinger M, Von Mackensen S, Fischer K, Khair K, Petersen C, Ravens-Sieberer U, Rocino A, Saginer P, Tusell JM, Van Den Berg M, Vicariot M (2002) Pilot testing of the 'Haemo-QoL' quality of life questionnaire for haemophilia children in six European countries. *Haemophilia,* 8; (s2); 47-54
Cited by 71 including:

Solans M, Pane S, Estrada MD, Serra-Sutton V, Berra S, Herdman M, Alonso J, Rajmil L, (2008) Health-Related Quality of Life Measurement in Children and Adolescents: A Systematic Review of Generic and Disease-Specific Instruments *Value In Health;* 11; (4); 742-64

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Paper 4; Von Mackensen S, Bullinger M and the HAEMO-QOL GROUP (2004) Development and testing of an instrument to assess the Quality of Life of children with haemophilia in Europe (Haemo-QoL). *Haemophilia*, 10, 17-25
Cited by 61 including:

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Paper 5; Gringeri A, Von Mackensen S, Auerswald G, Bullinger M, Perez Garrido R, Kellerman E, Khair K, Lenk H, Vicariot M, Villar A, Wermes C (2004) Health status and health-related quality of life of children with haemophilia from six west European countries. *Haemophilia*, 10, (supp 1) 26-33
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Paper 6; Khair K, Holland M, Carrington S (2011) Social networking for Adolescents with severe haemophilia. *Haemophilia* 18; 3; e290-e296

No cites but Commissioned book chapter in: Social Networking: Recent Trends, Emerging Issues and Future Outlook. Nova Publishing expected publishing date autumn 2013.

Paper 7; Khair K, Littley A, Will A, von Mackensen S (2012) The impact of sport on children with haemophilia. *Haemophilia;* 18 (6) 898-905
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Czepa D, von Mackensen S, Hilberg T (2013) Haemophilia & Exercise Project (HEP): The impact of 1-year sports therapy programme on physical performance in adult haemophilia patients. *Haemophilia;* 19; (2); 194-199

Khair K, Holland M, Pollard D (2103) The experience of girls and young women with inherited bleeding disorders, *Haemophilia*, 19

Paper 9; Khair K, Gibson F, Meerabeau L (2011) The benefits of prophylaxis: views of adolescents with severe haemophilia. *Haemophilia*; 18; 3; e286-e289
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Khair K, Littley A, Will A, von Mackensen S (2012) The impact of sport on children with haemophilia. *Haemophilia*; 18; 3

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Paper 11; Khair K (2006) Evaluating a self infusion device for children with haemophilia. *Paediatric Nursing*, 18, 10, 19-20
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Vidovic N, Musso R, Klamroth R, Enriquez MM, Achilles K (2010) Postmarketing surveillance study of KOGENATE® Bayer with Bio-Set® in patients with haemophilia A: evaluation of patients' satisfaction after switch to the new reconstitution system. *Haemophilia*. 16 (1) 66-71

Khair K (2010) Minimizing Joint Damage: The Role of Nurses in Promoting Adherence to Hemophilia Treatment *Orthopaedic Nursing*; 29; 3; 193-200

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Paper 12; Khair K (2009) Children's preferences of transfer devices for reconstitution of factors VIII and IX for the treatment of haemophilia. *Haemophilia*, 15, 159-167
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7.2 Funded research

I have been successful in obtaining three competitively sought grants to undertake the body of work presented here.

7.2.1 Haemophilia 2010 – an exploration of the lived experience of children with haemophilia.

Funded through Bayer Haemophilia Care Givers award 2008 (US$ 17,500).

The Bayer Care Givers awards are funded annually to 'promote the essential role of caregivers and allied health professionals by supporting continuing educational activities'. Expressions of interest are assessed by an international panel of twelve haemophilia health care professionals; if the application is judged as suitable a full application is then made. In 2008 there were seven awards, two within the European Union (EU) and five in developing countries. The two EU awards had direct patient impact, with the other awards supporting training and education roles or developing local laboratory and clinical services.

Basis of publications 2, 9-11

7.2.2 The Stellar Project.

Funded by a grant from The Burdett Fund for Nursing and the Roald Dahl Marvellous Children's Charity 2010, (£7,500).

The Burdett Fund for Nursing/Roald Dahl Marvellous Children's Charity awards were introduced in 2010 for UK nurses working in the fields of non-malignant haematology or neurology. As well as assessing the research proposal there is a component of this award that assesses nursing leadership and education. Assessed by a UK multi-professional awards panel, only six (of a possible ten) awards were granted in 2010. The Stellar project was the only award for non-malignant haematology of six applications received.

Basis of publication 6

7.2.3 The EIS study.

Funded by Wyeth (Pfizer) Haemophilia UK 2010 (£10,000).

This award was through the Wyeth (Pfizer) Investigator Initiated Observation (IIO) process where applications for funding for specific projects can be made annually. The global awards committee assesses the credibility and value of the application and award funding if applications are successful.

Basis of publication 7

7.2.4 An expert patient programme for girls and young women with inherited bleeding disorders.

Funded through Bayer Haemophilia Care Givers award 2011 (£ 15,000).

This award funded jointly with another haemophilia nurse in London has demonstrated that girls are more socially isolated than boys and less likely to discuss intimate bleeding problems. There are religious and cultural aspects that impact on the discussion of having a bleeding disorder for these girls. These first of these issues has been published (Khair et al 2013 EOL) and further papers are planned for publication during 2013 in adolescent, gynaecology and general practice journals.

7.3 Future research activity

In the conclusion of this exposition I stated the body of work presented in this exposition was the beginning of a research career. I outline here how these plans are already underway with a brief description of six current research studies assessing the impact of haemophilia and other bleeding disorders on QoL of children and their families.

7.3.1 The Caregiver Study

This is a European study of the impact of haemophilia on QoL on parents and children with mild, moderate or severe haemophilia A or B treated with on-demand treatment once bleeds have occurred, or with prophylaxis. 100 family pairs from five European countries (Germany, Italy, Sweden, Turkey and the UK) will be recruited. Differences in QoL and coping are expected to be demonstrated between those with mild/moderate haemophilia treated only on demand and. those with severe haemophilia treated prophylactically. GOSH is the only UK haemophilia centre participating on this study for which I am primary investigator (PI).

7.3.2 The AHEAD (Advate HaEmophilia A outcome Database study)

This is prospective four year international study of outcome measures in patients treated with ADVATE. I am the UK PI, thirty patients (aged 4-65) are expected to be recruited from four haemophilia centres. The first patients were recruited in August 2012. Annual assessment of QoL including joint scores, number of bleeds, pain assessment, time missed from school/work and Haemophilia Activities of Living adult/paediatric assessment (HAL/PedsHAL) will be undertaken. The study is funded an a cost-per-case by Baxter Ltd

7.3.3 The WIL-20 Study

This is an international post marketing study of a newly licensed coagulation factor for treatment of von-Willebrands disease. I am the PI for the UK, where ten patients across two sites are expected to be recruited. The study will record bleeds, their treatment, prophylactic therapy, impact on life style, through assessment of time lost from school, range limiting joint bleeds and QoL. The study is funded as a cost-per-case by Octapharma AG Ltd.

7.3.4 The ADVATE Pass Study

This is another international post marketing study assessing patient/parent acceptability of a new smaller volume of diluent for reconstitution of ADVATE. I am the UK PI for this study, planned to recruit children and caregivers from the UK children's haemophilia centres. Assessment of acceptability is by questionnaire and QoL assessment over a one year period. Ethical approval has been granted and patients should be recruited in early 2013. The study is funded as a cost-per-case by Baxter Ltd

7.3.5 The Autism Surveillance Study

The boys with haemophilia treated at Great Ormond Street following intra-cranial haemorrhage appear to be at increased risk of subtle behavioural and physical problems (Bladen et al 2009). Many of these boys are diagnosed with autistic spectrum disorders. This study, funded by the Haemophilia centre at GOSH, will assess all children with haemophilia aged >5 years for early signs of difficulty. The study will be run by me and the haemophilia physiotherapy team with input from the specialist autism service to analyse results and see children who are identified as being at risk.

7.3.6 The GAITRite Study in Children Aged 2-7 years

This study will look at gait assessment in children aged two to seven with haemophilia. The GAITRite walkway is a validated computerised walking mat which records 'swing' and 'stand' time. Previous work has demonstrated its validity in children with haemophilia aged over seven (Bladen et al 2007) this new study is the first in the world looking at younger children. I have been awarded £20,000 from an investigator initiated award scheme by Pfizer Ltd, to undertake this study with a physiotherapy colleague who I will be supervising in the research, data analysis and publication.

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9 Appendices

Appendix 1 – 12 papers submitted in this thesis CV

Appendix 13 – CV for Kate Khair

Appendix 14-15 – Quality of Life Questionnaires

Haemophilia

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ORIGINAL ARTICLE *Clinical haemophilia*

Why don't haemophilia nurses do research?

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Summary. Clinical research should form a core component of the role of haemophilia nurse specialists. The UK Haemophilia Nurses Association sought to determine the barriers that prevent nurse specialists from engaging in research and to seek ways to promote clinical research by haemophilia nurses in the UK. Web-based survey with subsequent workshop discussion was conducted. Responses were received from 32 nurses (a 50% response rate), all of whom agreed that haemophilia nurses should be actively involved in nursing research although only 21 had actually participated in research specifically related to haemophilia practice. Of these, most research had been related to educational programmes or (less commonly) was limited to data collection as part of multidisciplinary studies. Involvement in research rarely resulted in publication. Some barriers to involvement in nursing research and subsequent publication were suggested by survey respondents. They also indentified key practice areas that warranted nurse-based research including carriership and antenatal decision-making, along with the role and impact on care of the specialist haemophilia nurse, education and empowerment. To overcome the barriers to engaging in research and publishing, nurses require dedicated research time, mentorship and collaboration with more experienced haemophilia nurse researchers.

Keywords: haemophilia, nurses, research

Introduction

The care of patients with haemophilia and inherited bleeding disorders has advanced greatly in recent years, as a result of wider access to modern treatments and comprehensive care, resulting in improved treatment outcomes and quality of life for people with haemophilia [1]. In the UK, the provision of haemophilia care is clearly defined and provided in either comprehensive care centres or haemophilia treatment centres, usually delivered by a diverse multidisciplinary team that includes specialist nurses.

Most UK haemophilia nurses develop extensive clinical knowledge and expertize in this specialist field and practice at clinical nurse specialist or in a few cases, at nurse consultant level. The four principal functions of the nurse specialist role are: clinical practice, advocacy, advice, education and research [2,3]. The nurse consultant role also has four key components: expert practice, leadership and consultancy, education and training and research and evaluation for which some have protected research time. Despite research featuring as a key component in both roles anecdotal evidence suggests that the research component of the role is the most complex to establish through lack of time and other challenges such as experience and funding.

The UK Haemophilia Nurses' Association (HNA) is the specialist group for nurses in haemophilia care. The core aim of the HNA is to nurture haemophilia research and publication by nurses. This paper describes a project established by the HNA to promote clinical research by haemophilia nurses in the UK.

Methods

Study design

The HNA established a core group of four nurses experienced in haemophilia nursing research to explore the existing experience, attitudes and barriers towards haemophilia nursing research in the UK. This was undertaken by means of a web-based survey (http://www.surveymonkey.com), which ensures anonymous data collection. Invitations to the survey (including an electronic link) were sent by email to 65 haemophilia nurse specialists and nurse consultants listed on the

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HNA database. Recipients were asked to respond within 25 days, and were sent one follow-up reminder. The questionnaire sought to determine the existing experience, interest in, attitudes towards, as well as barriers to nursing research, and to identify key areas of practice that warrant research, either to improve current care practice or to establish evidence based care for patients and families.

Subsequently, the survey results were discussed in workshops held at the HNA annual general meeting in 2008. Here, the participants were presented with a listing of the topics identified via the online survey, and were asked to discuss these in small groups and to rank them in order of priority for future research.

Statistical analysis

For responses to the questionnaire survey, mean ± SD and median values are reported as appropriate.

Results

In all, responses were received from 32 nurses, a 50% response rate. Respondents represented an established and experienced group of haemophilia nurses, the mean length of time working in haemophilia being 8.7 years (range from 3 months to 23 years).

Although all nurses (32/32) agreed that haemophilia nurses should be actively involved in nursing research, only 21/32 (65%) had ever actually participated in any research at any stage of their career (Table 1). Only 15/32 (49%) had participated in research specifically related to haemophilia practice. Twelve nurses reported that their research experience had been related to educational programmes, most commonly a nursing degree (BSc or MSc). For the remaining nine nurses their involvement in research had been limited to data collection and analysis as part of multidisciplinary studies such as drug trials. However, there was an overwhelming desire among nurses to become actively involved in nursing research with 93.3% (28/30) of nurses stating an interest in becoming involved in nursing research personally.

Even where nurses identified that they had been involved in nursing research, it was rare for this to result in publication. Overall, only five of the nurses who had participated in nursing research indicated that this work had been published; indeed, only five nurses who responded to the survey had ever published any work at all. There are many reasons why research, particularly educational course content, may not be published. Table 2 summarizes the responses of respondents regarding the barriers to publishing and how these could be overcome. Table 3 identifies strategies of overcoming some of these systematic, personal and professional barriers including dedicated research time, mentorship and collaboration with more experienced haemophilia nurse researchers.

Through the online survey, nurses identified a wide range of practice areas that they thought warranted research. When presented to haemophilia nurses at the

Table 1. Attitudes towards research.

Question (response)	Yes	No
Do you think that haemophilia nurses should be actively involved with nursing research? (n = 32)	32 (100%)	0 (0%)
Have you ever participated in nursing research? (n = 32)	21 (65.6%)	11 (34.4%)
As part of a multidisciplinary study	9 (42.9%)	
As part of an educational programme? (n = 21)	12 (57.1%)	
BA	0 (0%)	
BSc	7 (53.8%)	
MA	2 (15.4%)	
MSc	5 (38.5%)	
PhD	1 (7.7%)	
Other	1 (7.7%)	
Was this work published? (n = 18)	5 (27.8%)	13 (72.2%)
Have you ever published any work? (n = 29)	5 (17.2%)	24 (82.8%)
Would you like to become actively involved in nursing research? (n = 30)	28 (93.3%)	2 (6.7%)

Table 2. Barriers to publishing nurse research and potential strategies for overcoming these.

	Barriers to publishing nurse research (n = 24)	What would help you to publish (n = 23)
Systemic	Time	Designated/protected time
Professional	Lack of knowledge	Guidance on process
	Identifying areas for research	Help with identifying subjects
	Lack of support and guidance	Support from managers
Personal	Lack of confidence	Education on writing for publication
	Motivation	Help with writing
	Sense that 'not the opportunities within my job to do this'	Mentorship
		Encouragement from peers

Table 3. Perceived barriers to nursing research (n = 26).

Barriers to nursing research	What would help to overcome these barriers?
Systemic	'Specific time within my job to do research'
Dedicated time	'Time and sounding board inside and outside the trust'
Support from managers	'Time and support to undertake it'
Professional	'Working as part of a small group of other interested parties'
Collaboration with others	'Being part of a larger study rather than undertaking research on my own'
Education	'A team to brainstorm and be supported by'
Personal	'Help and mentorship from someone who has undertaken research'
Mentorship	
Working as part of small group	
Support network	

2008 HNA workshop, this listing was scored to derive a listing based on priority for research topics. Carriership and antenatal decision-making and the role of nurse and impact on patient care, education and empowerment were perceived as key areas for research. Further topics identified included: venous access, concordance with therapy, family dynamics, the need for annual reviews, inhibitor and factor recovery monitoring, women's issues :teenagers with menorrhagia, non-haemophilia bleeding disorders in women, identification of potential carriers and families new to haemophilia compared with those with family history.

Discussion

In its 1994 role description, the HNA recognized that clinical nurse specialists should:

1. Be aware of research in clinical practice
2. Participate in approved data collection, clinical studies and trials
3. Seek opportunities to initiate and undertake nursing research projects within this speciality.

In this context, it is surprising that only two of three HNA members, most of whom had been practicing for more than 8 years, had ever engaged in nursing research. Among those who had, research activities were dictated principally by the need to acquire a professional qualification.

The degree to which nurses engage in nursing research varies according to personal characteristics such as confidence, motivation or creativity, but may also be limited by professional and educational issues, and by barriers that may arise from organizational cultures, and concepts and expectations of roles. To overcome such barriers and engage in research, nurses who responded to our survey identified the need for dedicated time and support from both managers and peers. In addition, our survey identified a distinct need for help and mentorship from those who had successfully undertaken research. These findings are not haemophilia specific, Breimaier *et al.* report that Austrian nurses cite lack of time, lack of information/knowledge and lack of interest as reasons for failing to undertake nursing research leading to a knowledge gap between theory and clinical practice [4]. In a study of >1000 Spanish nurses barriers to research are cited as belief about the value of nursing research, confidence in research skills and a lack of dedicated research time [5].

Similar issues and themes emerged with respect to publishing. Only a minority of nurses had ever published any work, and most often this was in the nursing press. Although there were some systemic and structural reasons given for this, principally lack of time, the survey responses clearly identified a lack of confidence and motivation for publishing and a lack of understanding of how to get work published. This is particularly pertinent for nurses who have completed degree courses with work worthy of publication but where the process of re-formatting course papers and submission was identified as a major hurdle. Several nurses identified protected research/publishing time and managerial support as being a key factor that hindered (or supported) publication, others identified a need for guidance on the process of getting published together with practical help and encouragement from peers.

Where are the legitimate areas for haemophilia nursing research, what methodologies should be used, and what could and should the contribution of nurses be to haemophilia research? The European Principles of Haemophilia Care identified that the priority areas for further research into haemophilia were for modified factor VIII and IX agents with longer half-life and reduced immunogenicity, new administration techniques, better understanding and prevention of the development of inhibitors and gene therapy [6]. Many nurses who engage in research perform a 'supportive role', for instance collecting data for physician-led research on patient demographics, treatment records, bleeding history and so on. Although this work is clearly important, nurses can and indeed should do more as part of their professional development. The focus of funding bodies and high profile journals on research based, randomized controlled trials fosters the impression that other forms of research are of less value. However, in the real world, and perhaps particularly for rare diseases such as haemophilia, experiential or qualitative research is just as important as the evidence produced in randomized controlled trials. Indeed, the authors of the European Principles of Haemophilia Care noted that other areas of research were required to further the development of care for patients and their families [6]. These included examination of different service delivery models, outcomes and quality of life measures. Nurses are key in this qualitative research and are able to utilize numerous research methods to promote measurement of the 'softer' aspects of haemophilia care. Stevenson *et al.* [7] show how nurses and allied health care professionals are best placed to improve patient care through research and service evaluation including, re-designing patient journeys/pathways, improving patient satisfaction, implementing national guidelines and targets and having a positive effect on quality of life scores.

As the funding environment becomes ever tighter, one of the frequently heard criticisms within haemophilia is the lack of demonstrable evidence for many aspects of care, particularly around how services are delivered. Nurses engaged in the 'sharp end' of clinical practice will frequently be the first to identify areas of practice in which new approaches are needed and in which questions need to be answered to improve evidence based care for patients and their families. Our survey identified a wide variety of areas in which nurses perceived a need for further research and evidence. It is

interesting that the proposed research topics were in areas of unmet patient/family need and related to evidence to support the role of the nurse and its impact on care and patient education and empowerment.

Yet, as our survey results clearly highlight, for nurses to engage in research they need greater support and encouragement than they currently receive. This is born out in the Stevenson's [7] study describes the challenges of undertaking research as: a lack of acceptance by medical colleagues, research isolation, negative organizational culture and lack of administration support. It may be that this encouragement/support can be provided through innovative methods such as social networking rather than the 'usual' face to face taught sessions. Social networking offers a powerful tool for promoting health care, giving individuals the ability to share information and learn from the experiences of others [6,8,9], and the potential role of social networking as a tool to facilitate nursing research has recently been described [10].

It is gratifying that the nurses surveyed showed an overwhelming desire to become more actively involved in nursing research, the challenge is now for the members of the HNA to promote nursing research to enable nurses to meet the aspirations of the CNS role and to enhance professional development. With the Haemnet social-network based website (http://www.haemnet.com) which launched in April 2011, an HNA-endorsed initiative to support haemophilia nurses, the HNA now has an online forum in which nurses can come together to share their experiences of research and to discuss, collaborate and promote their own research initiatives. Throughout the coming months, Haemnet will facilitate a series of online educational programmes designed to:

1. identify factors that could encourage greater uptake of research among nurses
2. generate discussion on research topics of interest to nurses
3. encourage formation of research networks
4. support nurses who wish to engage in research through shared expertize of those who already undertake research and publication activity.

It is hoped that this will foster the implementation of research into everyday practice, through collaborative study and support with and of new researchers.

Conclusion

Integrating research into clinical practice is problematic across nursing regardless of seniority, role, educational level or nursing culture. Despite this haemophilia nurses in the UK demonstrate an acceptance that care can be improved for patients and their families through implementing research into clinical care, this includes undertaking small scale multi-site collaborative nursing research supported by more experienced research colleagues, publishing case series and promoting an evidence base for current care.

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Disclosures

The authors stated that they had no interests which might be perceived as posing a conflict or bias.

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Multimethodology research with boys with severe haemophilia

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Abstract

Aim To describe the use of an innovative, multimethodological approach to exploring the day-to-day experiences of boys across a wide age range to better understand the effects of modern haemophilia treatment on their lives.

Background Children and young people with severe haemophilia can now be treated with prophylaxis and potentially have a lifestyle close to that of those without haemophilia. However, boys frequently describe living with haemophilia as burdensome.

Data sources The study, based on a grounded theory approach, was conducted with boys aged four to 16 years old, using research methods that included photo-elicitation, 'draw and write' techniques, focus groups run by participant co-researchers and individual interviews.

Review methods Grounded theory was used to enable rich data capture, through reshaping of research questions as theory developed.

Discussion The effectiveness of the methods used is discussed, along with consideration of the issues raised.

Conclusion These methods are effective for use with children. They can result in robust data and are also fun for child participants.

Implications for research/practice Understanding life with chronic disease from a child's perspective can improve clinical care through a better understanding of health behaviour and lifestyle implications.

Keywords Children and young people, haemophilia, photo elicitation, draw and write, focus groups

Introduction

HAEMOPHILIA IS an inherited disorder of coagulation that largely affects males and results in spontaneous and trauma-related bleeding that may lead to limb-threatening arthritic damage in joints. Prophylactic treatment administered intravenously at least twice a week is now recommended for severe haemophilia to prevent bleeding episodes and minimise disability (Liesner *et al* 1996, Manco-Johnson *et al* 2007). With parental education and support, children can be managed at home (Vidler 1999), resulting in a lifestyle near to that of children without haemophilia, with reports of good quality of life when assessed using haemophilia-specific instruments (Gringeri *et al* 2004).

Nevertheless, boys frequently describe living with haemophilia as burdensome. This study explored these feelings in boys aged four to 16 years old, using research methods that included photo-elicitation, 'draw and write' techniques, focus groups run by participant co-researchers, and individual, tape-recorded interviews.

Use of age-appropriate, multimethod research methods that 'play to children's strengths rather than weaknesses' (Clark 2004) enables children to express their thoughts and feelings, and is creative and interesting for children. Boys with haemophilia are initially recipients of care but develop skills and learn to care for themselves (Khair 2006). This 'expert participant role' is supported by UK policy (Expert Patients Programme 2013).

A participant co-researcher in this study proposed methods for engaging boys in research. He noted that: 'We are still living in the shadows of HIV and my generation is one that now needs to have a voice.' He also co-ran the focus group, offering his views to the younger boys for discussion.

These methods have not previously been discussed as a combined research methodology in a single study. Nevertheless, the approach allowed data to be collected from children and young people across a wide age range.

Methodology

The study was designed to allow English-speaking boys aged four to 16 years old to tell their life stories through narrative accounts. A variety of age and developmentally appropriate research methods were used. Boys aged four to seven years old were asked to take digital photographs to use as discussion prompts; eight to 12 year olds were asked to draw or write about their experiences of haemophilia; while those aged 13 to 16 years were invited to a focus group. Those unable to attend the focus group were interviewed in their homes. Age-appropriate information sheets for younger participants were developed using pictures, and the study process was described in words and writing styles that the boys could understand (Gibson and Twycross 2007).

Grounded theory was chosen as the research method for this study, as it would enable rich data capture across a large age range using multiple methods of engagement, and research questions to be re-shaped as evolving themes and new concepts emerged. As an experienced practitioner familiar with haemophilia, the researcher (KK) had preconceived ideas and theories that could have biased data capture. Using grounded theory, with each interview analysed after it occurred, enabled data comparison and the recognition of emerging themes that could be further investigated in later interviews (Charmaz 2006).

The national patient forum, the UK Haemophilia Society, reviewed the study proposal and offered constructive methodological insights. The South East Research Ethics Committee granted ethical approval in 2008. The study began in May 2009, with 30 boys approached for interview. Information for parents and children was given to the parents, who passed the information for children to their sons.

Three families did not want their children to be included in the study and 12 boys did not participate: four were not interested in the study, two failed to respond to the invitation and follow-up letters, two lived too far away to attend the focus group but participated in home interviews, two boys refused to use the camera, one was too shy to talk about himself and one declined.

Photo-elicitation

The use of photography in education and health research to enable children to reflect on their lives is a recent phenomenon. Photographs may be taken by researchers for discussion with individuals or groups of participants, or may be taken by participants of things that are of interest to them (Close 2007). The photographs are then used to assist discussion. Using researcher-led questioning of the content and importance of photographs through interviews is known as 'photo-elicitation' and is routinely used in research with children (Rose 2007). However, although digital photography enables children to see what they have photographed immediately, as the children can delete photographs with which they are unhappy, some data may be lost.

In this study, ten boys used digital cameras over two weeks to record 'being me' and 'life with haemophilia'. Initially, photographs were to be printed during the home interview using a portable printer. This method had not previously been described so seven-year-old Jimmy (pseudonyms are used for all names in this paper) undertook a pilot for us. He took 214 photographs, of which his mother deleted five, feeling they were 'inappropriate'. It took six hours to print them all, each photo taking 1.75 minutes to print. Despite initially being interested in the printing, Jimmy quickly became bored; a compromise was made, and his 40 favourite photographs were printed and used for the interview.

For subsequent interviews, the parents, who were later reimbursed, printed the children's photographs at local photo-shops: copies were made for the child and for the researcher, and the photographs uploaded onto a computer. The boys were then asked to choose their favourite photographs to talk about during the photo-elicitation interview (PEI) (Clark-Ibáñez 2004). The researcher also selected photographs to use as 'props' to shape the narratives.

Clark (1999) suggested that children 'forget or deny the harshest aspects of illness experiences'. PEI allows them to interpret experiences, it is a method increasingly used for research with children that 'gives detailed information about how informants see their world and allows interviewees to reflect on things they do not usually think about' (Blinn and Harrist 1991).

Children live in a world of 'right' and 'wrong', with questions usually having a 'correct' answer. Children may believe they should provide the answer that they think the researcher expects. Matt, aged ten, has autism and used PEI rather than 'draw and write' along with his younger brother. He took 43 photos of his friends, school, playing and toys. One photograph that stood out from the others as 'different' was a tray with his infusion kit set out

Box 1 Semi-structured interview schedule

Some of the boys have told me... has that happened to you?

Can you tell me about...?

1) Haemophilia:

What is it?
- Bleeds
- Treatment

How did you get it?

Who else has got it?

Who will you give it to?
- Genetics

How is it treated?
- What medicine?
- Who does it?
  - Port/peripheral
  - Parent/self

2) You:

Who are your friends?

What school do you go to?

Do you play games/sport?
- Which can you play?
- Do you need treatment first?
- Why?

3) Your family:

Mum and dad

Brothers/sisters

Aunts/uncles – any haemophilia?

Your house

Any pets – dogs can have haemophilia, too!

4) Your favourite things

5) Things you hate

Modified questions for use after data sampling:

Tell me what a good day/bad day is like.

Do you think about having haemophilia every day?

ready for treatment. When asked why he had chosen to take that photograph he said: 'Because I thought that maybe it would help with your research because it's, like, stuff that we use to take our injections and... erm, that it would be good because of, like, how we use it and what we do with them.'

This 'photo showcase', described by Clark (1999) as a way of children demonstrating their prowess at caring for themselves medically, demonstrates Matt's perception of what he believed was necessary for haemophilia research, knowing that this would be disseminated wider than the interview alone, giving credibility to his haemophilia as well as his life story.

Draw and write

Children are invited to draw a picture or to write a story about a particular subject. This method was introduced in health education (Williams *et al* 1989). It has become a well recognised and widely used research method that enables even very young children to express their views about their health and medical care. Drawings, which require skill and knowledge, can be used to aid communication with adults, giving young children a voice when their views are often overlooked (Balen *et al* 2000, Sartain *et al* 2000).

Horstman *et al* (2008) described draw and write as a research method that is child-centred and can be used to share experiences. Using it as an icebreaker in semi-structured interviews with children provides richer data than interviews alone (Pridmore and Lansdown 1997), allowing issues not reflected in the drawings, such as health, to be drawn into conversation (Backett-Milburn and McKie 1999). Children largely see drawing as fun and non-threatening, making them 'experts', able to communicate their thoughts, beliefs and knowledge as they describe their drawings (Coad *et al* 2009).

The boys in this study were given coloured paper, pens and pencils to use in a 'pre-interview' period. Each was asked to either draw a picture of himself or write something about himself and having haemophilia. They were then interviewed at home at least two weeks later, and the drawings or stories were used as discussion prompts.

Focus group

Qualitative research routinely involves using focus groups (Gibson 2007). They are widely used in paediatric research for gaining children's views and perspectives. Such group interviews encourage in-depth discussion and produce data that reflect the views of each participant as well as the group. Predetermined questions (Box 1) were used to initiate conversations and the participant co-researcher was present to assist.

The focus group took place in the school holidays, following discussion with the participants and the participant co-researcher. The participant co-researcher bridged the gap between young boys with haemophilia and the adult female researcher (Kirby 2004). The study design also benefited from gaining knowledge from young people about themselves (Jones 2004), engaging them as accountable service users with a role in creating real change in research (Kirby 2004). Ten boys were scheduled to participate but last-minute drop-outs – a well recognised problem of focus groups (Morgan 1995) – reduced this to four.

The group still yielded rich data (Gibson 2007). Participants' narratives shared experiences valued by the tellers, the gatherers and the listeners, and offered insight into how people cope with illness

(Hardy *et al* 2009). The six boys unable to attend the focus group consented to semi-structured individual interviews at home that used the same, pre-determined questions as the focus group.

Recording interviews

All the interviews were recorded so full attention could be paid to the children, without the need to write down verbatim comments (Balen *et al* 2000). Each child was allowed to record and listen to his own voice before the interview; this served as an icebreaker, as many of the children were amused by how they sounded. All but one agreed to tape over their 'practice' recordings.

Each child was invited at the end of the interview to ask or say anything off-tape and to question the researcher, a practice described by Cullingford (1997) as allowing the child to feel that an interview is a two-way process. Some boys asked questions off-tape about other children they knew at the hospital, haemophilia, whether they 'got the interview right' and what would happen to their tapes. These questions were addressed honestly, while maintaining other children's confidentiality. One teenager listened to the whole tape twice before agreeing he was 'happy with it'. A transcriber was then paid to transcribe the tape recordings for later analysis.

Results

The lead author repeatedly read all the transcripts and listened to all the interviews, including comments from the field notes written at the time of the interviews for clarification as necessary. The interviews were coded into themes, representing the good and bad aspects of life with haemophilia. The photographs, drawings and stories were used to support these data.

The triangulation of data – photographs, drawings, stories and narratives – collected in this study allowed the researcher to compare views of life with haemophilia across a wide age group of children and young people with haemophilia. The narratives represent a true sharing of experiences valued by the tellers, listeners and gatherers (Hardy *et al* 2009), and offer insight into how these boys cope with haemophilia.

The study was performed using grounded theory to identify and describe the broad spectrum of life with haemophilia from the children's perspectives. Grounded theory enables deep consideration of data, and produces a theory that is saturated in data and observation (Morse 2009). Initial data sampling enables theories to be developed and data further analysed as the study continues.

In his drawings and description, 'Tom', aged 12, described living with haemophilia as a dichotomy: 'On my picture, I have got two sides, I have got a bad side and a good side. I have got a bad side with a person on a chair and he is getting an injection done... and he's crying... because it is hurting... it hurts sometimes and that is bad.'

Conversely, he describes a 'good side' of having haemophilia: 'I have drawn a haemophilia camp: it's good because you have something bad and then you have a treat for it – it's good to get a treat for something you don't really like.'

This description of there being two sides to haemophilia led to a 'mini-analysis' of the first 15 transcripts. This process, described by Glaser (1978) as theoretical sampling to assist with field enquiry into theoretical questions, resulted in the formulation of additional questions about 'good days' and 'bad days'. The semi-structured interview questionnaire was amended to reflect this emerging theory and the final 15 participants were asked two questions: 'Tell me what a good day/bad day is like?' and 'Do you think about having haemophilia every day?'

Aspects of haemophilia described as either good or bad were analysed and discussed with the participant co-researcher, who concurred with the theory but suggested that good and bad would be different for every child and that this should be borne in mind when analysing the data.

The narrative content was identified and coded into recurring themes seen as important aspects of life with haemophilia. These themes were: genetics and inheritance (Khair *et al* 2011), family and siblings, school, sport, career, haemophilia bleeds and treatment (Khair *et al* 2012), and the good and bad aspects of living with haemophilia. With the exception of genetics and inheritance, these themes described the daily experiences of having haemophilia and will be presented in other papers.

Discussion

The research methods described in this paper are well established. This study demonstrated how they can be adapted and incorporated into a single study with a group of children and young people across a wide age range and who have a single, long-term health condition. This has provided rich data that demonstrated through triangulation the experiences of these children.

This study had some limitations. The focus group gathered rich data, but only from a small number of boys due to drop-outs; this required an amendment to the protocols to allow individual interviews to be undertaken with boys at home.

The use of portable printers, not previously reported in visual research methods, proved time-consuming and necessitated a further change so that photographs could be printed before the interview. This meant that the boys had seen their photographs before the researcher had, allowing them to choose which photographs they discussed, which may have affected the data gathered.

The boys who were asked to draw or write about themselves did not always do this; eight boys drew pictures but only two boys wrote about themselves, so the written accounts are sparse. However, these boys were happy to remain in the study, and provided rich narrative accounts of what they had drawn or written. Better guidelines or further support while they were writing and drawing may have produced more data.

Despite these issues and the necessary alterations to the study design, the study methods are cohesive research instruments and reliable individually. The concerns of the ethics committee that these methods had not previously been reported in a single study and that the data generated might not be cohesive have been disproved. This study has given children of different ages the opportunity to describe living with haemophilia while taking contemporary treatment that allows them to live lives less affected by bleeding than in past reports.

Conclusion

This paper describes the use of multimethodology, qualitative research to support extensive data collection among children and young people with haemophilia across a wide age range. This is the first qualitative assessment of the experiences of boys with haemophilia that has been undertaken since the era of HIV infection in the 1980s. The inclusion of a participant co-researcher has enabled the participants to have a direct say in formulating research related to living with haemophilia. This further informs how health care is experienced by children and young people, and may add to future developments in haemophilia care and research.

The results of this study show that children as young as four years are able to voice their personal views of health and wellbeing. Nurses and other healthcare workers should be encouraged to use methods other than verbal questioning when communicating with children. We should communicate with service users to evaluate care and to enable their participation in ways that are comfortable for them.

These encouraging findings invite further research into the experiences of children and young people with haemophilia. They are experts who have valuable stories to tell about living with a long-term health condition and the effects it has on their lives.

Conflict of interest
None declared

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Pilot testing of the 'Haemo-QoL' quality of life questionnaire for haemophiliac children in six European countries

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Summary. In a multinational working group, an instrument (Haemo-QoL) to assess quality of life in children/adolescents with haemophilia and their parents has been developed. In co-operation with haemophilia treatment centres in six European countries, approximately 10 children/adolescents with haemophilia per country and their parents were asked to participate in the pilot-testing. Both self-reported and parent-reported questionnaires were provided for two age-groups of children (4–16 years). Medical data was collected from physicians from patient files. Answers to open questions from participants (58 children and 57 parents) confirmed the content of 116 of the preliminary items. Cognitive debriefing revealed that the majority of the Haemo-QoL was rated favourably, but 29 questions were recommended to be omitted and several items to be reformulated. Preliminary psychometric testing of the revised 77 item questionnaire in the same sample showed acceptable reliability and validity, which will be examined in a subsequent study with a larger patient sample.

Keywords: quality of life, questionnaire, haemophilia, children/adolescents, assessment

Introduction

Health-related quality of life is increasingly considered a relevant health outcome parameter in medicine. In adults, concepts on quality of life, assessment methods and applications of respective instruments in various types of studies have been published within the past 20 years [1]. As concerns the concept, consensus has been reached with regard to the main components of the operationally defined term for quality of life, namely well-being and function in physical, social and emotional domains [2]. Self-reporting by respondents is important to capture the individual perception of health conditions and treatment regimes. Standardized, psychometrically tested and internationally available measurement instruments include the short form SF-36 Health Survey [3], the Nottingham Health Profile [4] or the EuroQol [5] questionnaire amongst others. While generic instruments measure quality of life across health conditions, condition-specific measures do so with regard to a specified disease, treatment or symptom.

In comparison to adults, children's quality of life assessment is a more recent area of research. A Medline literature search involving papers published from 1995 to 2000 identified a total of 319 publications on quality of life research in children [6], few publications introducing or testing instruments to assess quality of life in children.

Generic measures to assess quality of life in children are existent, examples are the Child Health Questionnaire [7], the TAQOL questionnaire [8] or the KINDL questionnaire [9]. Disease-specific instruments, however, have not been developed for children with haemophilia.

Generally, only few studies referring to quality of life in children with haemophilia are published and these

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rarely used standardized and psychometrically tested quality of life questionnaires. Available data on quality of life in haemophilia evaluate the effectiveness of prophylaxis and home treatment. Prophylactic treatment is associated with higher direct treatment costs, but is expected to lower costs in the course of lifetime of a person with haemophilia by reducing adverse consequences of the disease such as immobility, pain and therefore disability, handicap and impairment (e.g. [10]). Studies have shown that prophylactic treatment improved quality of life in terms of less hospitalization, fewer joint bleeds and less time off school or work (e.g. [11]). In children where both types of treatment, prophylactic and on-demand, are administered, health condition and treatment may affect well-being and function of patients and families.

The question of how haemophilia and its care may impact on children's health-related quality of life, motivated the development of a haemophilia-specific quality of life questionnaire with the goal to have at hand an instrument with which different treatment modalities maybe evaluated for their effect on children and families in clinical studies. In this paper the development of such an instrument and results from its pilot test are presented. The work presents a European effort to (a) develop a disease-specific self-report instrument (Haemo-QoL) for children and adolescents with haemophilia and their parents available in different languages; (b) to cognitively debrief it to understand how children respond to the questions; and (c) to preliminarily test it for basic psychometric properties in terms of reliability and validity.

Materials and methods

Within a European collaborative working group of haematological and psychosocial experts from six countries a condition-specific questionnaire for children with haemophilia and their parents was developed. In the development of QoL instruments care was taken to comply with guidelines pertaining to quality-controlled translations (including forward/backward translations and comparison of the retranslated versions with the original) as well as focus group work (ensuring the comprehensibility, relevance and acceptance of the instrument). In addition, testing of psychometric properties (including indicators of reliability, validity and sensitivity) and obtaining reference data for the study population is recommended [12]. These guidelines are especially important in multinational, cross-cultural work to ascertain the equivalence of items and scales across countries. Within the international instrument development, the cognitive debriefing of questionnaires is increasingly considered important. It involves an evaluation of each question of the instrument (item per item) and feedback on comprehensibility, relevance and modification needs [13]. Preliminary psychometric testing helps to select items that best represent the constructs to be measured.

Instrument development

The preliminary version of the Haemo-QoL questionnaire was derived from an expert consensus meeting in which clinicians and social scientists convened to identify relevant dimensions and items of quality of life from the literature, clinical experience and available questionnaires. This resulted in an instrument with 116 items pertaining to 10 domains of quality of life. Item examples of each subscale are shown in Table 1. The questionnaire was professionally translated into all project languages using the forward-backward method.

Pilot-testing

The preliminary version of the Haemo-Qol was given to children/adolescents and their parents in the collaborating haemophilia centres in six different countries (England, France, Germany, Italy, the Netherlands and Spain) together with generic questionnaires. For the children/adolescents these included the Child Health Questionnaire [7], and the KINDL questionnaire [9]. As potential determinants of quality of life, the KID-Cope Questionnaire [14], a treatment motivation questionnaire (M. Bullinger, U.A. Ravens-Sieberer, unpublished data) as well as questionnaires to assess life satisfaction [15], social support [16] and health related locus of control [17] were included. For the parents the parental version of the Child Health Questionnaire [7] and KINDL questionnaire [9] was included in addition to the impact on family scale [18], the SF-12 Health Survey as well as subscales from the SF-36 Health Survey [19].

The testing was performed with the help of staff and trained students at the haemophilia centres. Children included were of two age-groups: younger children (4–7 years) were interviewed by a specially trained student, older children (8–16 years) filled in the questionnaire by themselves as did all parents of the children. A second set of 'take home questionnaires' included the afore mentioned measures of psychosocial predictors of quality of life. All data were checked, plausibility controlled and data inputted.

Cognitive debriefing

The questionnaire was evaluated by children and parents with respect to acceptance, comprehensibility, difficulty and completion time using a standardized feedback evaluation form with visual analogue scales. Feedback was given by children after filling in the

Table 1. Examples of items for each subscale of the original Haemo-Qol questionnaire ($n = 116$ items)

Scale	No. items	Subscales	Example
Physical Health	16	pain	I had pain in my joints
		mobility	My joint felt stiff
		anxiety	I was afraid of bleeds
Feeling	12	mood	I was moody
		emotional consequences	I felt under stress because of my haemophilia
		action	I was upset
Attitude	10	relationship to others	I was envious of healthy kids my age
		relationship to own person	I was happy with myself
Family	13	position in the family	I had a special position within our family because of my haemophilia
		restrictions	My parents forbade me doing certain things because of my haemophilia
		problems	There were problems at home because of my haemophilia
		activities of parents	My parents had to limit their activities because they had to look after me
		own feelings in family	I was happy in my family
Friends	13	relationship	I could talk to my friends about my haemophilia
		activities	I was able to participate in the activities I liked
		anxiety	I was afraid of being an outsider
Other Persons	13	social support	Others were understanding towards me because of my haemophilia
		estrangement	I felt different from others
		isolation	I felt left out when others did things together
Sport and School	13	sports & games	Because of haemophilia I had to refrain from sports that I like
		school	I was afraid of fights in the school playground
Coping	10	control	I felt well informed about haemophilia
		emotional acceptance	I felt healthy as anybody else
Treatment	12	quality	I was satisfied with my haemophilia centre
		side-effects	The injections annoyed me
Future	4		I have worried about my health

questionnaires and a break of 15–30 min. For the older children from age 8 onward, open questions were posed in addition to the in-depth cognitive interview, for younger children only an overall evaluation using smileys was obtained.

The cognitive debriefing (as answered by older children) was conducted in the language of the child. The children and adolescents were asked for each item about difficulty to understand and about its relevance with respect to their haemophilia. If an item was not understandable, they were asked to give a suggestion for rewording the item to improve it.

Data analysis

Analysis involved content analysis for open questions and descriptive analysis on the item feedback during cognitive debriefing, as well as a multitrait analysis program 'MAP:(20)' to identify preliminarily reliability and scale structure of the 116 item original questionnaire. The MAP-analysis program gives information about ceiling and floor effects (dispersion of scores: optimum is a low percentile on the bottom or top of the scale), the scalefit (correlation of items with their own rather than another scale, optimum is 100%) and reliability (Cronbach's alpha, optimum is $\alpha > 0.70$). Together with the cognitive debriefing information the preliminary psychometric analysis results were used to decide on retention, modification or rejection of items using the following criteria:

1. missing values: the item was to be deleted if more than 5% of persons had failed to respond to it;
2. item difficulty: items which did not discriminate between persons, i.e. that were endorsed only by 20% of the respondents or were agreed to by more than 80% of the respondents were omitted;

3. item total correlation: items were omitted if the correlation of the item with the scale was below $r = 0.30$, meaning that the item does not contribute substantially to the quality of scale;
4. changes in alpha: the coefficient Cronbach's alpha is an indicator of the reliability of a scale and should be at least $\alpha = 0.70$. Since deleting a poorly performing item may lead to an increase in α-coefficients, this criterion was applied to omit such items;
5. cognitive debriefing: judgements in terms of comprehensibility and relevance from the cognitive debriefing were used. If these judgements were negative in more than 25% of respondents, the item was rejected.

If at least one of the above criteria applied, the item was omitted from the questionnaire. For the revised and item reduced questionnaire reliability was assessed via the internal consistency coefficient Cronbach's alpha, which gives information about the correlation of the items belonging to one scale. Convergent validity was inferred from correlations of the Haemo-QoL with other instruments measuring similar concepts, such as the KINDL.

Results

The questionnaire was responded to by a total of 58 children (57 male and one female) and 57 parents. Of these 51 children had haemophilia A, five haemophilia B and two were diagnosed with von Willebrand disease. As concerns health status, 56.4% of the children had had 1–3 bleedings in the last 6 months, six children developed an inhibitor and three children had hepatitis C. Of the older children, two children did not participate in the cognitive debriefing because of language problems and/or acute bleeding. Three younger children stopped because of tiredness, problems of cognitive ability or maternal intervention.

Open questions

With regard to open questions, 36 children gave information about problems and restrictions due to haemophilia. In the youngest age group (4–7 years, $n = 10$) this involved four main issues concerning restrictions in sports and play, injections, pain and visits to the hospital. In the older age group (8–16 years, $n = 26$) these issues were also described in addition to three more issues, namely bleeds, comments of other persons and overprotection by the family.

The parents ($n = 44$) also described problems with haemophilia for the child as well as for themselves. For the child restrictions and injections as well as relationship to others were viewed as most problematic ranging between 16 and 36% of the responses. For the parents themselves burden, stress and anxiety with 32%, but also to be within reach and acceptance of the disease and treatment (each 16%) were mentioned. However, 20% of the parents saw no problems for themselves and 16% saw no problems for their child.

Cognitive debriefing

Cognitive debriefing showed that several items in the subscales 'Feeling', 'Attitude', 'Friends', 'Other Persons' and 'Sport & School' obtained up to 30% of negative responses in terms of incomprehensibility or were rated as not relevant by more than 20% of the children. Interestingly, there were country differences with regard to the numbers of suggestions that were made for rewording items. In Germany and England only a few suggestions were made, in France and the Netherlands the children made some suggestions and in Italy and Spain many suggestions were given, which were related to translation problems. Also, country differences were noticed with regard to type and number of items found relevant and with regard to the number of children on prophylactic vs. on-demand treatment.

Overall feedback

The feedback on the overall questionnaire for older children was rated with a visual analogue scale from 0 (lowest rating) to 100 (highest rating). With regard to acceptance the children rated the questionnaire as 'rather good' ($M = 71.49$, $SD = 26.62$), found it 'rather simple' ($M = 26.08$, $SD = 26.62$), thought that the questionnaire relates to haemophilia ($M = 78.29$, $SD = 23.24$), understood the questions or knew, what was meant ($M = 65.80$, $SD = 27.41$) and could cope well with the answer possibilities ($M = 71.20$, $SD = 29.05$). The parents rated the questionnaire 'good' to 'acceptable', but stated that it is difficult to subsume both treatment options in the same questionnaire, feeling that haemophilia is different with or without prophylactic treatment and stating that haemophilia is not a disease but a health condition. The parents found some questions difficult for very young ages and recommended to change these questions.

Item reduction

In addition to the analysis of cognitive debriefing, data quality and preliminary psychometric testing of the 116 items was used to describe about retaining or omitting items. Taking into account the selection criteria described in the method section, 29 items were omitted from the questionnaire and several items were identified as candidates for rephrasing. With the omissions, the

Table 2. Rejection of items from the original Haemo-QoL-Questionnaire for the children aged 8–16 years

Scale	Item	Missing values above 5%	Item difficulty index (%)	Item total correlation below $r = 0.30$	Changes in α if item is omitted	Cognitive debriefing	
						Comprehensiveness: not clear (%)	Relevance not relevant (%)
Physical health	Had to stay lying down		86.5	*			27
	Did not care whether...			*	⇑		
	Afraid of the injections		86.5	*			
Feeling	Cranky and irritable	7%					65
	In a good mood						57
	Moody			*	⇑	22	51
	Didn't mind about...			*	⇑		
	Tried to test my limits	10%					22
	Upset						60
Atitude	Too vulnerable for...	7%		*	⇑	26	41
	In harmony with my body	10%				29	
	Dissatisfied with myself						24
Family	Not let me do sports...			*	⇑		
	Had to stay off work		81.1				
	Felt left alone...		100				32
Friends	Spend my time indoors					24	
	Not go to other children						32
	Able to participate			*	⇑		
	Being an outsider	7%	88.6	*	⇑	24	
	Afraid of being rejected		83.8				
Other persons	In touch with other...						
	Embarrassed						27
	Difficult to get on...		89.2	*	⇑		22
Sports and school	Active in school			*	⇑	24	
	Careful at school			*	⇑		
Coping	Didn't make a secret			*	⇑		
treatment	Afraid of hospital			*	⇑		
	Had to lie in bed			*	⇑		22
Future	Any profession			*	⇑		

If one of the criteria of rejection is fulfilled = omission of item.

questionnaire could be reduced to 77 items reflecting 10 scales of quality of life with haemophilia. Table 2 gives an overview over deleted items and the respective exclusion criteria across all countries.

Psychometric analysis

Results showed that the scale structure of the reduced Haemo-Qol questionnaire could be confirmed with reliability ranging from $\alpha = 0.67$ for the subscale 'Feeling' to $\alpha = 0.89$ for the 'Haemo-QoL total score'. The confirmatory testing results suggest scaling successes in the majority of the scales approaching 100% (scalefit) and reaching few ceiling and floor effects (see Table 3).

In terms of convergent validity correlations of the scales of the Haemo-QoL and scales of the KINDL and SF-36 subscales were calculated. The correlations of the subscales of the Haemo-QoL with the corresponding subscales of the KINDL and the SF-36 showed to be acceptable and ranged between $r = -0.33$ ('School') and $r = -0.63$ ('Physical Health').

Discussion

Only recently effects of haemophilia and its treatment on quality of life have been addressed in the literature. Rosendaal *et al.* [21] obtained information from 935 Dutch adult haemophiliacs. Data from this study revealed that the patients were less often married, 22% were not employed and home treatment had a positive influence on quality of life. Royal *et al.* [22] found that patients? treated prophylactically report a higher quality of life in the SF-36 scales 'Pain', 'General Health' and 'Mental Health' in comparison to a group with on-demand treatment. Most of the studies assessed quality of life

Table 3. Scale structure and internal consistency for the reduced Haemo-Qol questionnaire ($n = 77$ items/children aged 8–16 years)

Scale	No. Items	Min. Value	Max. Value	Mean	SD	Floor Effects	Ceiling Effects	Scalefit	Cronbach's α
Physical health	10	10	30	16.22	6.1	16.7%	0%	93.0%	0.85
Feeling	6	6	16	9.31	3.0	13.9%	0%	88.3%	0.67
Attitude	7	6	24	11.97	4.4	19.4%	0%	88.6%	76
Family	10	11	35	18.22	5.4	0%	0%	70.0%	0.71
Friends	7	7	26	14.89	4.9	0%	0%	45.0%	0.69
Other persons	7	5	20	10.00	3.8	33.3%	0%	91.4%	0.71
Sports and school	11	10	38	19.46	6.1	8.3%	0%	53.3%	0.71
Coping	9	9	35	19.92	6.7	0%	0%	53.3%	0.76
Treatment	7	7	21	11.35	4.1	0%	0%	41.1%	0.70
Future	3	3	11	5.65	2.7	11.1%	2.8%	33.3%	0.75
Haemo-QoL Total Score	77	87	207	136.41	28.0	0%	0%	65.9%	0.89

Min., max., mean scores and SD based are raw data.
Floor-/Ceiling-effects = percentage of respondents at lowest/highest scale level (optimum is 0%).
Scalefit = percentage of items correlating with own scale (optimum is 100%).
Cronbach's α = reliability indicator (expected is $\alpha = 0.70$ or higher).
All results obtained via MAP analysis.

with the Short Form SF-36 Health Survey (SF-36). Tusell *et al.* [23] found impairments in the SF-36 scales 'Pain', 'General Health' and 'Physical Role Functioning' in a sample of 190 haemophilia patients. The SF-36 was also used by Szucs *et al.* [10] in a study evaluating the socio-economic impact of haemophilia care. Djulbegovic *et al.* [24] assessed quality of life with the Quality of Well-Being Scale (QWB) and the SF-36 and Miners *et al.* [25] used the SF-36 and the EuroQol (EQ-5D). The orthopaedic status of severe haemophilia A and B was assessed by Molho *et al.* [26] using the SF-36 in a sample of 116 patients. Impairments in the scales 'Vitality', 'Pain' and 'General Health' were identified. A major consideration in haemophilia research was infection with hepatitis viruses or HIV from infected blood products. Brown [27] investigating coping strategies of 297 HIV infected adolescents, states that distress about reminders of HIV was associated with ineffective coping strategies (e.g. blaming others).

Bussing *et al.* [28] pointed out that HIV-positive children with haemophilia have not received sufficient attention. Psychosocial sequelae to haemophilia in children and their families have been described from the clinical–apsychological viewpoint referring, for example, to functional problems in everyday life, stigmatization or social isolation. However, studies into the quality of life of patients or families as a function of haemophilia treatment are still rare. In a study on prophylactic treatment of children with haemophilia, Liesner *et al.* [29] examined 27 children (age: 1.3–15.9 years) and found that prophylaxis improved the quality of life of the families. Carnelli *et al.* [30] compared three different prophylactic treatment programmes in haemophiliac children ($n = 52$) and concluded that the high costs of the treatment can be justified by the better quality of life of the patients. Pabinger *et al.* [31] examined 88 children and adults with haemophilia and found that home treatment is widely accepted by the patients and improved quality of life [32].

Within the emerging area of quality of life research in children with haemophilia, the question of how to assess quality of life in children is yet to be resolved. The current pilot study attempted to develop and gather information about a condition-specific quality of life instrument for young haemophiliacs in different age-groups, using self-report as well as a proxy report by parents. The questionnaire items were conceptualized, collected and pretested internationally in six European countries with younger (4–7 years) and older (8–16 years) children and their parents. In terms of cognitive debriefing, three quarters of the questions were regarded appropriate and understandable by both parents and children. Surprising, however, was the rejection of almost all items suggesting problems with regard to self-esteem, social acceptance or emotional well-being. This could either be due to the positively viewed health of the respondents as expressed in statements such as 'Haemophilia is a health condition but not a disease' or by the feelings of patients and their family that management is possible so that the condition is not major problem. However, social desirability bias in answers cannot be excluded. Comparing answers to open questions with standard questions of the Haemo-QoL showed that problems with haemophilia are experienced by participants, but are not rated highly in their importance. In general, the newly developed questionnaire was largely considered acceptable.

Psychometric testing and cognitive debriefing results were used to screen items for potential omission. Inspection of all items according to psychometric and cognitive debriefing criteria resulted in a reduced questionnaire version with sufficient reliability coefficients for the scales, adequate convergent correlation with corresponding generic quality of life measures and indication of discriminant validity according to clinical scores. The preliminary work with children's haemophilia questionnaire thus yielded in two age-related versions for children's self-report and parents report on quality of life of children with haemophilia in six languages (German, English, French, Italian, Spanish and Dutch). These versions will be psychometrically tested in the main study involving 50 children per country, i.e. a total of 300 children. In this upcoming study, cross-cultural differences in addition to psychometric criteria and content-related aspects with regard to perceptions of haemophilia will be evaluated further. The pilot work, presented in this paper, is unique in that to the authors knowledge no paper has been published in which cognitive debriefing and psychometrical pretesting to develop a health-related quality of life measure have been conducted simultaneously in six countries in haemophilic children. Although due to sample size, psychometric data has to be carefully interpreted, the results are encouraging. It is hoped that the condition-specific Haemo-QoL questionnaire is reliable, valid and sensitive assessment of quality of life in paediatric haemophilia is available for future clinical studies.

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Development and testing of an instrument to assess the Quality of Life of Children with Haemophilia in Europe (Haemo-QoL)

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Summary. In spite of an increased interest in the assessment of quality of life (QoL) in children, so far no instrument for children with haemophilia is available. Because of the low prevalence of the condition, such an instrument should also be cross-culturally applicable. In the study presented, a (QoL) assessment instrument for children with haemophilia (the Haemo-QoL questionnaire) was developed and tested in six countries (France, Germany, Italy, the Netherlands, Spain and the United Kingdom) for psychometric properties in 339 children with haemophilia and their parents. The Haemo-QoL is a self-reported questionnaire for children in the age ranges 4–7 (I: 21 items), 8–12 (II: 64 items), 13–16 years (III: 77 items) as well as for parent rating containing 9–11 subscales (depending on age-group versions). Psychometric testing involved the examination of reliability and validity. The three age-group versions of the Haemo-QoL had acceptable internal consistency and retest reliability values, as well as possessing sufficient discriminant and convergent validity. However, in young children when compared to older children, these indicators were less satisfactory. The Haemo-QoL full version is now available for children of three age groups and their parents and is ready for use in clinical research (http://www.haemoqol.org).

Keywords: children, haemophilia, quality of life, questionnaire validation

Introduction

The term 'health-related quality of life' was coined to represent the subjective perception of health. Health indicators refer traditionally to clinical signs and symptoms, morbidity and mortality. The perception of health by patient self-report has only recently been the focus of medical research. While in anthropology and social sciences quality of life (QoL) is a long-standing concept, representing structural indicators of living conditions or the evaluation thereof in terms of life satisfaction, the focus on quality of life in medicine is relatively recent. However, within the last 30 years, after an initial phase of philosophically orientated theoretical debates, QoL measures have been developed and applied in a variety of research contexts. In relation to the QoL literature in adult patients, QoL research in children is even more

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recent. According to a literature research only about 13% of approximately 20 000 publications in QoL-focused on children [1]. This is due certainly to several problems posed by assessment of QoL in children, which not only include the adequate representation of relevant domains or dimensions of QoL, but also the age dependency of the possible self-report, in terms of cognitive capacity, consistency of answers and the role of external parental reports. While in adult QoL literature relevant dimensions and domains have been consensually identified [2], their application in children's QoL concepts has been a challenge [3]. Serious doubts have been raised about whether children below the age of 8 years can reflect and report on their perceived health. The value of parental report has been stressed therefore as an approximation (proxy) for children's feelings and behaviours. Discussion has been extensive, especially concerning so-called generic assessments of QoL in children. Such assessment is applicable to a wide range of health states and has led to the development of generic instruments. These are available as self-report or proxy ratings. Disease-specific or targeted measures for children have been developed in several clinical areas, such as asthma, atopic dermatitis or diabetes. Haemophilia is a congenital coagulation disorder due to the genetically transmitted defect of clotting factor (factor VIII in haemophilia A and factor IX in haemophilia B). These haemostatic defects lead to spontaneous and post-traumatic internal bleeding events, particularly frequent in joints and muscles, but also possible in any tissue and organ, including the central nervous system. Recurrence of bleeding events in joints is the cause of the progressive deterioration of joint function with the development of arthropathy. The treatment is based on substitutive therapy with intravenous clotting factor concentrates to administer 'on-demand', i.e. when a bleeding event occurs, or prophylactically, to prevent the bleeding, two to three times a week. The treatment of haemophilia as well as related complications might influence the QoL of children and their families [4–6].

Targeted QoL measures for children with haemophilia have not been identified so far. For example, in over 300 publications on QoL research in children available between 1995 and 2003, many of them introducing or testing instruments assessing generic QoL in children, only some were disease-specific but none of them related to children with haemophilia. The few studies on QoL in children with haemophilia have not used standardized or psychometrically tested instruments, but rather *ad hoc* questions, or have inferred QoL information from clinical data [7–10].

QoL assessment in children with haemophilia will profit from the availability of quality of life measures for epidemiological reasons (describing the quality of life of this patient group with reference to children with other chronic conditions), for clinical trials (to evaluate the potential benefits of new treatments for haemophilia with regard to QoL), for quality assurance (identifying the quality of care given to children with haemophilia, for example, in haemophilia centres), for health-economic studies (assessing costs and benefits of haemophilia treatment with regard to economic indicators) and for routine treatment (identifying individual treatment options from which specific patients might benefit). Some data are available on QoL in haemophilia with regard to effectiveness of prophylactic home treatment which suggested that prophylactic treatment is associated with higher treatment costs, but is expected to lower costs in the course of the lifetime of a person, i.e. by reducing adverse consequences such as immobility and therefore preventing disability, handicap and impairment. Studies have shown that prophylactic treatment may improve QoL because it leads to reduced hospitalization rates, fewer joint bleeds and less time off school or work [11–13].

The question of how haemophilia and its care may impact on children's health-related QoL stimulated the development of a haemophilia-specific QoL questionnaire. A specific feature of the work was the simultaneous development of such a questionnaire starting from clinical expertise and patients' experience, not only in one country but simultaneously in several European countries. Such an international and cross-cultural perspective is necessary because haemophilia is a relatively rare condition affecting one in 10 000 children in the general population. The present paper describes the development and testing of the QoL questionnaire for children with haemophilia (the Haemo-QoL questionnaire) in a cross-cultural sample of children from six European countries. The aim of this paper is to describe the psychometric properties of the questionnaire with regard to reliability and validity, to understand factors influencing scale scores and to evaluate critically the potential and limitations of the newly developed questionnaire.

Methods

Study design and patient population

In this cross-sectional study, children with haemophilia and their families were included from one to six haemophilia centres in each of the six countries

(France, Germany, the Netherlands, Italy, United Kingdom and Spain). Inclusion criteria were severe (< 1% or between 1 and 2%, but clinically severe) haemophilia A or B, absence of inhibitors and informed consent from both parents and children. With regard to treatment, no restrictions were made. The only exclusion criterion was lack of ability to speak and understand the language of the respective country.

The Haemo-QoL questionnaire

The Haemo-QoL questionnaire is a modular instrument that was developed in expert discussions and from literature review. It has been tested in a pilot study within three age groups (children aged 4–7 years: interviews with the children and their parents' self-report; children aged 8–12 years: self-report by children and by parents; and adolescents aged 13–16 years: self-report by adolescents and parents). The pilot testing of the Haemo-QoL questionnaire was carried out with 58 children from six European countries and has been described recently [14]. The field-test version of the questionnaire was produced after modification of the pilot version and consists of 29 items for the younger children conducted as an interview. The version for the children aged 8–12 consisted of 84 items, and the version for adolescents consisted of 91 items. The difference of the number of items between the older age groups is due to the additional inclusion of two scales to assess the dimensions of specific relevance to adolescents, namely 'relationships' and 'future'.

Variables in addition to the Haemo-QoL questionnaires

In addition to the questionnaire described above, clinical variables as well as psychosocial and sociodemographic information were collected. Specifically for clinical documentation, an effort was made to standardize reporting relevant indicators across countries, using a specific clinical documentation sheet. Sociodemographic information was obtained primarily from parents and included a wide range of information also useable to identify indicators of social class. Psychosocial variables related to instruments assessing coping (KID-Cope [15]), social support (SSS adapted for children [16]), health locus of control (adapted for children [17]), as well as life satisfaction (FLZ adapted for children [18]). Care was taken to include not only the newly developed Haemo-QoL questionnaire [14], but also generic measures of QoL that can be used for convergent psychometric validation, namely the KINDL [19] for child self-report and the Child Health Questionnaire (CHQ) [20], which assesses QoL in children from the parents' perspective, and the SF-12 [21], which related to the perceived health status. An overview over the instruments is given in Table 1.

Conduct of the study

The preparation of the questionnaires in six different languages included the Haemo-QoL questionnaire together with a generic quality of life questionnaire and other instruments, which included psychosocial information, to be administered in the centres and in a take-home questionnaire for retest reliability testing. Clinical data were collected at the haemophilia centre. All data collection was carried out by a nurse or student helping in the project, either by specific appointments with participants or by routine visits through the haemophilia centre. Following information about the study and a signed consent form, the children in the two older age groups sat in a quiet room to fill in the questionnaire; however, they could

Table 1. Domains of the Haemo-QoL and additional questionnaires with number of items per age group (I: 4–7, II: 8–12, III: 13–16 years).

	Age groups				Age groups		
Haemo-QoL	I	II	III	Additional questionnaires	I	II	III
Dimension	4–7	8–12	13–16	Questionnaire	4–7	8–12	13–16
Physical health	5	9	9	KINDL	18	30	30
Feeling	3	6	6	CHQ	1	1	1
Attitude	3	7	7	FLZ	–	11	11
Family	5	11	11	SSS	–	9	9
Friends	2	8	8	Kids-Cope	3	9	9
Other people	5	13	13	KKG	–	7	7
Sport and school	4	11	11	Open questions	5	6	6
Coping	–	10	10	Total	27	73	73
Treatment	2	9	9				
Future	–	–	4				
Relationship	–	–	3				
Total	29	84	91				

ask if they had questions. In parallel, parents, if available, were asked to fill in the respective questionnaire. Young children (4–7 years) were interviewed by trained staff. All children received a small present for participation. Take-home questionnaires – to be filled in separately by children and parents only in age groups II (ages 8–12) and III (ages 13–16) – were provided with a free stamped return envelope so that returning the follow-up questionnaire after 1 week was made as easy as possible. In each centre one responsible person was identified who could be addressed for logistical matters. All centres were visited to introduce and train for the study and were monitored continuously by the project staff.

Data analysis

Upon central data input and plausibility checking, descriptive statistics were performed using the SPSS program (SPSS/PC version 10.0) followed by psychometric testing with the so-called 'multi trait analysis' program (MAP) [22]. The MAP program allows identification of the item characteristics in terms of item distributions and the confirmatory analyses of the scale fit, which gives an indicator of the percentage with which items correlate with the scales to which they are supposed to belong, in comparison to another scale. The MAP program also provides information on floor/ceiling effects of scales, reliability indicators (Cronbach's α) and scale intercorrelations. Other statistical analyses, including comparisons between subgroups using *t*-test or variance analytical methods or correlational analyses, were performed using the SPSS/PC program.

Results

A total of 24 centres in the six countries had expressed interest in participation and contributed patients to the study. Due to organizational reasons, four centres could not participate so that a total of 20 centres were active. Table 2 shows the distribution of centres and sample size per centre across countries. Within these centres, a total of 339 children and their parents were recruited into the study. With regard to an expected number of 476 children, this represents a 70% response rate.

Sociodemographic data

The children's mean age was 10.00 (SD = 3.7) years ranging from 5.56 years in the youngest age group I (4–7) over 10.00 years in the age group II (8–12) to 14.09 in the age group III (13–16). Half of the children had one sibling, 19.0% had no siblings and 17.7% had two siblings (see Table 3).

Table 2. Participating centres (number of children).

Country	Centres	No. of patients
Spain	Valencia	17
	Zaragoza	10
	Sevilla	14
	Madrid	35
France	Nantes	12
	Caen	10
	Tours	15
	Paris	29
	Marseille	15
	Brest	5
Germany	Bremen	26
	Leipzig	14
	Hannover	12
	Munich	8
Italy	Firenze	11
	Milano	41
	Torino	18
the Netherlands	Utrecht	19
	Amsterdam	6
UK	London	22
Total no. of patients		339

The parents' characteristics (see Table 4) show that mainly mothers responded (77.2%). Most of the parents were married (83.0%) and 90.9% of the parents lived with a partner. In terms of schooling the high school level was predominant in 30.1% of the parents and 66.1% of the parents were employed.

Clinical characteristics

Of the 339 patients enrolled no medical documentation was available from 13 patients and eight patients were excluded because they did not fulfil the inclusion criteria. From the 318 patients included in the analysis, 85.5% had haemophilia A and 11.6% haemophilia B. The factor level as an indicator of the severity of the condition was less than 1% in 86.5% and between 1% and < 2% in 12.9%. Of the children, 25.8% had had no joint bleeds in the previous 12 months, 41.2% of the children had had less than five joint bleeds and 9.1% had five to 10 joint bleeds; the remaining 9.1% had had more than 10 joint bleeds. A total of 66.7% of the children were on prophylactic treatment and 31.8% received on-demand treatment (see Table 5); 11.3% of the patients had functional impairments, mainly in age group III (15.5%). Chronic pain was reported in 3.1% of the patients and 6.6% had undergone orthopaedic surgery.

Table 3. Sociodemographic data of the children per age group (I: 4–7, II: 8–12, III: 13–16 years).

Characteristics	Σ ($n = 320$)	I ($n = 95$)	II ($n = 122$)	III ($n = 103$)
Age [mean (SD)]	10.00 (3.7)	5.56 (1.2)	10.00 (1.7)	14.09 (1.5)
Number of siblings				
0	19.0%	26.7%	12.3%	19.8%
1	50.5%	44.4%	57.0%	48.5%
2	17.7%	22.2%	18.4%	12.9%
3	6.9%	3.3%	5.3%	11.9%
4	4.3%	3.3%	3.5%	5.9%
5	0.3%	–	–	1.0%
> 5	1.3%	–	3.5%	–

Table 4. Sociodemographic data of the parents.

Characteristics	Σ ($n = 309$)	I ($n = 95$)	II ($n = 110$)	III ($n = 104$)
Age [mean (SD)]	39.30 (6.1)	36.27 (5.5)	39.23 (6.0)	42.54 (5.1)
Gender				
Female ($n = 233$)	77.2%	81.9%	72.6%	77.5%
Marital status				
Single ($n = 16$)	5.8%	12.2%	4.1%	1.1%
Married ($n = 230$)	83.0%	83.3%	79.6%	86.5%
Divorced ($n = 30$)	10.8%	4.4%	15.3%	12.4%
Widowed ($n = 1$)	0.4%	–	1.0%	–
Living with partner				
Yes ($n = 241$)	90.9%	92.3%	90.6%	88.9%
No ($n = 24$)	9.1%	6.8%	9.4%	11.1%
School grade				
Some high school ($n = 31$)	11.1%	7.7%	13.6%	11.2%
High school ($n = 84$)	30.1%	34.1%	28.2%	27.0%
Vocational school ($n = 59$)	21.1%	20.9%	20.4%	21.3%
College ($n = 31$)	11.1%	11.0%	16.5%	7.9%
Professional degree ($n = 47$)	16.8%	16.5%	13.6%	21.3%
Other degree ($n = 20$)	7.2%	8.8%	2.9%	10.1%
No degree ($n = 7$)	2.5%	1.1%	4.9%	1.1%
Employment				
Yes, full time ($n = 107$)	38.4%	35.1%	38.4%	39.6%
Yes, half time ($n = 66$)	22.6%	17.0%	25.3%	27.5%
Yes, < half time ($n = 15$)	5.1%	8.5%	4.0%	3.3%
No, house wife ($n = 76$)	27.1%	30.9%	25.3%	24.2%
No, in training ($n = 1$)	0.3%	–	1.0%	–
No, unemployed ($n = 9$)	3.1%	4.3%	4.0%	1.1%
No, others ($n = 10$)	3.4%	4.3%	2.0%	4.4%

Psychometric characteristics

Psychometric testing involved *a priori* inspection of item distribution and correlation with the supposed scale. Following this information items were deleted or regrouped for each of the age groups, which resulted in psychometric testing in three age groups: young children (age 4–7 years: 21 items), older children (age 8–12 years: 64 items) and adolescents (age 13–17 years: 77 items). Confirmatory psychometric testing using the MAP program was employed to describe the mean and standard deviation of the scale scores as well as ceiling and floor effects, minimum and maximum scale values and the two psychometric indices, namely scale fit and Cronbach's α. This psychometric testing procedure was also performed for the parents' questionnaires, but will be described elsewhere (paper in preparation).

Reliability and scale structure (Table 6) shows the scale structure and reliability coefficients of the revised Haemo-QoL questionnaire for the three age-group versions. Within the Haemo-QoL for age group I ($n = 90$), which now consisted of 21 items, the scale fit was acceptable in four of the scales. Cronbach's α was acceptable in two of the scales as well as the total score. In age group II (ages 8–12) ($n = 117$), analysis of the instrument, which consisted of 64 items, revealed better psychometric

Table 5. Clinical data.

Characteristics	Σ	I	II	III
Type of haemophilia				
A ($n = 272$)	85.5%	84.5%	83.1%	89.3%
B ($n = 37$)	11.6%	14.4%	12.7%	7.8%
Missing data ($n = 9$)	2.9%	1.0%	4.2%	2.9%
Level factor				
≤ 1% ($n = 275$)	86.5%	86.6%	85.6%	87.4%
> 1% ($n = 41$)	12.9%	12.4%	14.4%	11.7%
Missing data ($n = 2$)	0.6%	1.0%	–	1.0%
Treatment scheme				
Prophylactic ($n = 212$)	66.7%	68.4%	68.6%	62.9%
On-demand ($n = 101$)	31.8%	30.5%	30.5%	34.3%
Missing data ($n = 5$)	1.6%	1.1%	0.8%	2.9%
Joint bleeds				
0 ($n = 82$)	25.8%	22.7%	28.8%	25.2%
< 5 ($n = 131$)	41.2%	43.3%	39.0%	41.7%
5–10 ($n = 29$)	9.1%	6.2%	10.2%	10.7%
> 10 ($n = 29$)	9.1%	8.2%	6.8%	12.6%
Missing data ($n = 47$)	14.8%	19.6%	15.3%	9.7%
Impairment				
Yes ($n = 36$)	11.3%	4.1%	13.6%	15.5%
No ($n = 275$)	86.5%	95.9%	84.7%	79.6%
Missing data ($n = 7$)	2.2%	–	1.7%	4.9%
Chronic pain				
Yes ($n = 10$)	3.1%	1.0%	5.1%	2.9%
No ($n = 304$)	95.6%	99.0%	94.1%	94.2%
Missing data ($n = 4$)	1.3%	–	0.8%	2.9%
Orthopaedic surgery				
Yes ($n = 21$)	6.6%	2.1%	5.1%	12.6%
No ($n = 287$)	90.3%	96.9%	91.5%	82.5%
Missing data ($n = 10$)	3.1%	1.0%	3.4%	4.9%

properties assessing between four and nine items and 10 dimensions of health-related QoL. The scale fit approached the optimum of 100 in almost all the scales; the coefficient alpha ranged from 0.60 to 0.79 and was 0.85 for the total score. In addition the Haemo-QoL, re-evaluated 1 week later, produced test–retest reliability results which for most of the scales were beyond $r = 0.70$, with only two exceptions for the subscales 'dealing with haemophilia' and 'treatment'. A total of 100 adolescents responded to the questionnaires in age group III (13–16 years). The item number ($n = 77$) was higher because two dimensions were added, namely 'future' and 'relationship'. The scale fit was near optimum in almost all the scales (the lowest value was 84.1%). The coefficient alpha was satisfactory except for the scales 'future', 'treatment' and 'perceived support'. The test–retest reliability was again largely satisfactory, with the exception of lower scores in three scales (perceived support, treatment and future). The Cronbach's α of the total scale was 0.91 and of the test–retest correlation the total scale was 0.92.

Convergent validity

For the convergent validity of the Haemo-QoL for age group I, correlations between the KINDL total score with the general item (GHQ) from the CHQ as well as the chronic generic item module of the KINDL were examined (see Table 7), showing that these correlations were approximately $r = 0.30$ for correlations between the Haemo-QoL and the KINDL. However, higher scores with the chronic generic module of approximately $r = 0.40$ indicated an acceptable correlation with the Haemo-QoL. The highest correlation was found here for the dimension 'others' of the Haemo-QoL and the chronic generic module of the KINDL ($r = 0.532$, $P = 0.0001$). Only two significant correlations were found between the Haemo-QoL and the GHQ. In age group II, the correlations between the dimensions of the Haemo-QoL and the KINDL total score were higher, averaging $r = 0.30$ in all correlations. The convergent validity of the Haemo-QoL in age group III reached $r = 0.45$-$.50$ in almost all scales, again with high correlation in the total score (see Table 7).

Discriminant validity

Discriminant validity was assessed using clinical information differentiating Haemo-QoL scores within each of the age groups with regard to treatment and condition-related information. Differences between on-demand and prophylactic treatment largely failed to reach significance although, in general, prophylactic treatment seemed to be associated with less impairment in QoL. This is also true for the discriminant validity of the revised Haemo-QoL with regard to joint bleeds. Here differences appeared, especially in the older age groups, indicating higher impairment for children with more than five joint bleeds in the previous 12 months in the dimensions 'physical health', 'view', 'perceived support' and especially 'sports' activity, yielding a significant difference in the total score (see Table 8).

Correlations of the Haemo-QoL with psychosocial determinants were assessed in age groups II and III. Significant correlation of the Haemo-QoL with social support and life satisfaction, but also with coping and less with locus of control were found, indicating that effective social support was associated with a higher QoL score and impairment in the Haemo-QoL was associated with life dissatisfaction. Multiple regression analysis with QoL as a criterion (Haemo-QoL total score) was performed, which took into account specific clinical and psychosocial data. QoL was clearly associated with life satisfaction and social

Table 6. Psychometric characteristics of the revised Haemo-QoL (all age groups).

Scale	No. of items	Min	Max	Mean	SD	Floor effects	Ceiling effects	Scale fit	α	Test–retest
Age group										
Physical health	4	4	16	7.03	3.15	34.8%	0%	85.7%	0.55	–
Feeling	3	3	15	5.13	3.13	58.4%	2.2%	90.5%	0.82	–
View	2	2	10	3.53	2.40	61.8%	7.9%	78.6%	0.69	–
Family	4	4	20	9.47	3.91	18.0%	1.1%	82.1%	0.66	–
Friend	1	1	5	1.74	1.21	69.7%	6.7%	–	–	–
Others	2	2	10	3.87	2.20	48.3%	2.2%	64.3%	0.56	–
Sport	3	3	12	5.48	2.64	42.7%	0%	95.2%	0.49	–
Treatment	2	2	10	4.00	2.34	47.2%	5.6%	64.3%	0.45	–
Total	21	21	81	40.25	13.65	4.5%	0%	82.1%	0.85	–
Age group II										
Physical health	7	7.00	27.00	11.64	4.80	19.7%	0%	98.4%	0.78	0.87
Feeling	7	7.00	25.67	9.56	3.36	41.0%	0%	96.8%	0.69	0.87
View	9	9.00	39.00	14.89	5.86	14.5%	0%	96.3%	0.79	0.84
Family	5	5.00	21.00	9.28	4.02	23.9%	0%	82.2%	0.68	0.76
Friend	4	4.00	20.00	11.03	4.47	7.7%	5.1%	97.2%	0.71	0.81
Perceived support	4	4.00	20.00	11.84	4.07	6.8%	4.3%	94.4%	0.66	0.74
Others	6	6.00	25.00	8.50	3.33	38.5%	0%	96.3%	0.74	0.76
Sport	8	8.00	28.00	15.49	5.53	13.7%	0%	94.4%	0.67	0.79
Dealing	7	7.00	35.00	16.32	5.54	4.3%	0.9%	96.8%	0.66	0.61
Treatment	7	7.00	25.00	12.13	4.06	15.4%	0%	93.7%	0.60	0.67
Total	64	79.00	200.67	120.68	22.66	0%	0%	95.0%	0.85	0.90
Age group III										
Physical health	7	7.00	27.00	12.51	4.46	14.0%	0%	100%	0.76	0.79
Feeling	8	8.00	36.00	11.71	5.15	30.0%	0%	96.6%	0.87	0.89
View	10	10.00	43.00	18.00	6.92	12.0%	0%	97.3%	0.86	0.86
Family	8	8.00	26.00	13.62	4.37	12.0%	0%	90.9%	0.69	0.76
Friend	4	4.00	20.00	11.20	3.90	2.0%	2.0%	97.7%	0.69	0.81
Perceived support	4	5.00	20.00	12.40	3.75	0%	5.0%	97.7%	0.63	0.57
Others	6	6.00	22.00	9.07	3.85	33.0%	0%	89.4%	0.75	0.71
Sport	9	9.00	35.00	19.18	7.24	10.0%	0%	94.9%	0.76	0.78
Dealing	7	7.00	35.00	15.94	5.38	2.0%	1.0%	98.7%	0.68	0.71
Treatment	8	8.00	29.00	15.41	5.14	6.0%	0%	90.9%	0.67	0.63
Future	4	4.00	16.00	8.66	3.00	6.0%	0%	84.1%	0.52	0.64
Relationship	2	2.00	9.00	2.75	1.46	74.0%	0%	100%	0.73	0.90
Total	77	92.00	240.00	150.42	30.62	0%	0%	94.8%	0.91	0.92

support as well as with the number of bleeds. These data are presented in another paper [23].

Discussion

This study presents the results of the psychometric testing of the final long version of the first disease-specific QoL questionnaire (Haemo-QoL), indicating that the three age-related versions of the haemophilia-specific questionnaire with different item numbers across age groups are methodologically acceptable. The reliability of the scales reached the critical alpha of 0.70 in the majority of the scales with, however, lower values in younger children. The same is true for the scale fit, which is an indicator of the factorial validity of the Haemo-QoL questionnaire. Convergent validity with the KINDL questionnaire as well as with the general item (GHQ) from the child health questionnaire (CHQ) showed acceptable correlations. With regard to discriminant validity, indicators of clinical severity also showed that the instrument is able to differentiate between clinical subgroups in the oldest age group.

Within this cross-sectional study, testing of the sensitivity or responsiveness of the newly developed measure was not possible; however, this could be undertaken and will be performed in ongoing studies (e.g. ESCHQoL Project [25]) using the Haemo-QoL questionnaires. In addition, the Haemo-QoL questionnaire is supplemented by an age-related parent version which uses identical items. The psychometric quality of these scales, as described in a paper in

Table 7. Convergent validity of the Haemo-QoL (all age groups, r = correlation coefficient, P = P-value).

		Age group I			Age group II			Age group III		
Haemo-QoL		KINDL	Chronic–generic	GHQ	KINDL	Chronic–generic	GHQ	KINDL	Chronic–generic	GHQ
Physical health	*r*	–	− 0.332	− 0.318	–	− 0.330	− 0.275	− 0.335	− 0.452	− 0.299
	P		0.002	0.002		0.000	0.004	0.001	0.000	0.004
Feeling	*r*	− 0.280	− 0.383	− 0.217	–	− 0.287	–	− 0.517	− 0.563	− 0.359
	P	0.010	0.000	0.036		0.002		0.000	0.000	0.000
View	*r*	− 0.250	− 0.419	–	− 0.345	− 0.368	–	− 0.575	− 0.518	− 0.462
	P	0.022	0.000		0.000	0.000		0.000	0.000	0.000
Family	*r*	–	− 0.435	–	–	− 0.336	–	− 0.257	− 0.508	− 0.269
	P		0.000			0.000		0.014	0.000	0.008
Friend	*r*	–	–	–	–	–	–	− 0.230	–	− 0.260
	P							0.030		0.011
Perceived support	*r*	–	–	–	− 0.296	− 0.357	–	− 0.573	− 0.454	− 0.309
	P				0.003	0.000		0.000	0.000	0.002
Others	*r*	− 0.265	− 0.532	–	− 0.204	–	–	–	–	–
	P	0.015	0.000		0.044					
Sport	*r*	–	–	–	–	− 0.284	− 0.229	− 0.301	− 0.374	− 0.329
	P					0.003	0.017	0.004	0.000	0.001
Dealing	*r*	–	–	–	− 0.266	–	–	–	–	–
	P				0.010					
Treatment	*r*	–	–	–	–	–	–	− 0.325	–	− 0.264
	P							0.002		0.012
Future	*r*	–	–	–	–	–	–	− 0.408	− 0.328	− 0.387
	P							0.000	0.001	0.000
Partner	*r*	–	–	–	–	–	–	− 0.232	− 0.244	–
	P							0.030	0.019	

Table 8. Discriminant validity of the revised Haemo-QoL for number of (joint bleeds) (mean and P-values).

	Age group I			Age group II			Age group III		
Haemo-QoL	< 5	≥ 5	*P*	< 5	≥ 5	*P*	< 5	≥ 5	*P*
Physical health	6.82	6.43	n.s.	11.05	12.47	n.s.	10.95	15.63	0.000
Feeling	5.10	4.86	n.s.	9.14	9.67	n.s.	11.06	12.54	n.s.
View	3.44	3.71	n.s.	15.11	13.58	n.s.	16.54	21.79	0.002
Family	9.22	9.69	n.s.	8.83	11.00	0.042	13.22	14.67	n.s.
Friend	5.32	6.29	n.s.	11.31	11.84	n.s.	11.11	11.46	n.s.
Perceived support	–	–	–	8.16	8.53	n.s.	8.14	10.88	0.002
Others	3.84	3.86	n.s.	12.64	10.89	n.s.	12.56	12.79	n.s.
Sport	5.16	6.23	n.s.	17.28	17.95	n.s.	17.92	23.21	0.004
Dealing	–	–	–	16.44	16.44	n.s.	15.95	16.83	n.s.
Treatment	3.71	4.17	n.s.	13.93	13.89	n.s.	15.13	15.27	n.s.

preparation, is also satisfactory. Using psychosocial determinants of QoL such as coping, locus of control, life satisfaction and social support it was apparent that QoL is dependent not only on clinical but also on psychosocial characteristics. Such an influence of coping strategies on the psychosocial wellbeing of haemophiliacs has already been found in other studies [24].

An attempt at shortening the Haemo-QoL questionnaire was made by empirically identifying, from the total item pool, items that are available in all age groups and that comply with psychometric standards. Using this formal item reduction process, a short version consisting of 35 items with a scale structure retained was identified for children in age groups II and III which could also be used with the youngest age group (paper in preparation). In addition, an ultra-short version consisting of 15 items present in all age groups was produced, which yields only a summary score. Both the short and the ultra-short version must be tested in an independent study.

With the development of the Haemo-QoL simultaneously in several European countries an attempt was made to develop and test a documentation system to assess children's QoL in haemophilia. This

makes it possible to examine, in the long self-report version, age-related aspects in quality of life in haemophilia as well as parental reports, thus enabling the researcher to compare parents' and children's ratings, as well as making available a short form which can be used according to specific study design needs [26]. Considering that no disease-specific tool in haemophilia is available for children and adolescents, it is hoped that this approach will contribute to increased research in this area.

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Health status and health-related quality of life of children with haemophilia from six West European countries

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Summary. A multicentre, international, cross-sectional study was carried out in the frame of field testing of the first haemophilia-specific quality-of-life (QoL) questionnaire (Haemo-QoL). The aim of this paper is to describe health status and health care and their impact on QoL in haemophilic children in Western Europe. Children aged 4–16 years with severe haemophilia without inhibitors were enrolled by 20 centres in France, Germany, Italy, the Netherlands, Spain and the United Kingdom. Clinical information was collected by the physicians with a medical documentation form. Health-related QoL (HRQoL) of children was assessed with Haemo-QoL, available for three age groups. Clinical data were available in 318 patients, 85.5% with haemophilia A. The mean age at first bleeding was 11 months, at first joint bleed 25 months. Functional joint impairments were found in 11.3%. Prophylaxis treatment was given to 66.7% of children in whom breakthrough bleeds occurred 0.4 times a month compared to 1.1 bleeds in children receiving on-demand treatment. A significantly higher factor consumption was found only in the two younger age groups of prophylaxis patients compared to on-demand patients. HRQoL was satisfactory in this cohort: young children were impaired mainly in the dimension 'family' and 'treatment', whereas older children had higher impairments in the so-called 'social' dimensions, such as 'perceived support' and 'friends'. Health care of children in Western Europe is progressively improving with a large diffusion of home treatment and prophylaxis. This provides a high level of health status and HRQoL, being better in haemophilic adolescents on prophylaxis.

Keywords: haemophilia, quality of life, health status, prophylaxis, European

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Introduction

Haemophilia is characterized by spontaneous and post-traumatic bleeding; its complications in joints and muscles leads almost inevitably to pain, severe joint damage, disability and a dramatic impairment of health-related quality of life [1]. More than 30 years have been spent from the introduction of modern management of haemophilia: bleeding episodes are now rapidly controlled by plasma-derived and recombinant clotting factor concentrates; haemophilic arthropathy is fought either by preventing it with prophylactic treatment or by repairing the damage with joint replacement surgery [2]. In particular, the availability of safer products and early prophylaxis have greatly improved the management of haemophilic children with a consequent dramatic impact not only on symptoms and survival of these patients but also on their health-related quality of life [3]. However, the healthcare of these patients absorbs a huge amount of economic and human resources [4–6], so that it is essential to frame them in the context of the level of quality of life provided.

Health-related quality of life (HRQoL) is increasingly considered one of the most relevant health outcome measures in medicine [7]. In order to measure HRQoL, generic and disease-specific questionnaires have been developed. While generic instruments measure QoL across health conditions, disease-specific instruments measure quality of life related to a specific disease, being more sensitive to specific treatment and clinical outcomes.

Available data on quality of life in haemophilia evaluated the effectiveness of prophylaxis and home treatment [8–11]. Prophylactic treatment is associated with higher direct treatment costs, but is expected to lower costs in the course of lifetime of a person with haemophilia by reducing its complications such as pain and impairment, and therefore disability and handicap. Studies have shown that prophylactic treatment improved quality of life in terms of reduced hospitalization rates, fewer joint bleeds and less time off school or work, but these studies were carried out mainly in adults or in single countries [8–11]. However, it is still unclear how QoL and health status are related in children and adolescents in Europe. The aim of the study is to describe health status and healthcare of children with haemophilia and their impact on HRQoL. The current analysis was based on a European study (Haemo-QoL study) carried out in connection with the validation of a QoL questionnaire for haemophilic children/adolescents and their parents [12].

Methods

A multicentre, international, cross-sectional, observational study has been carried out in the frame of the field testing of a haemophilia-specific QoL questionnaire. The aim of this paper is to describe clinical conditions of haemophilic children in Western Europe, modality of treatment and their effects on HRQoL.

Patient recruitment

Twenty-eight centres in six European countries were asked to participate (France, Germany, Italy, the Netherlands, Spain and the United Kingdom). Patients with haemophilia A and B were enrolled by each centre when they met the following criteria: age ranging from 4 to 16 years, severe factor VIII or factor IX defect (factor VIII $\leqslant$ 1% or lower than 2%, if a clinically severe history of bleeding was present), absence of inhibitors, HIV-1 seronegativity, capacity to understand the questions and informed consent signed by parents or by a family carer.

Medical documentation

Investigators were asked to fill in a medical documentation form for each patient collecting information on health status and modalities of treatment.

Concerning the patient's health status, investigators were asked to provide details about type of haemophilia, level of factor defect, previous history of inhibitor development and treatment for immune tolerance induction, number of joint bleeding events that required treatment in the previous year, presence of target joints, defined as those joints with several bleeds irrespectively of the bleeding frequency, presence of joint impairment, chronic pain and concomitant diseases. Finally, patients were evaluated optionally according to the WFH orthopaedic joint score [13].

Information concerning treatment modalities consisted of type of treatment administration (home treatment, hospital treatment, self-administration, administration by parents, physician or nurses), type of product (plasma-derived or recombinant), factor consumption in the previous year and type of treatment. The latter was defined as continuous prophylaxis when factor concentrate was administered at least twice a week for at least 45 weeks a year; intermittent prophylaxis when it lasted for less than 45 weeks a year; and on-demand treatment when clotting factor concentrate was administered mainly when a bleeding occurred. Patients who underwent intermittent prophylaxis or who were

prophylactically treated less than twice a week were considered in the on-demand group. Continuous prophylaxis was subdivided into two groups: primary prophylaxis when it had started within the second joint bleeding event or the second year of age, secondary prophylaxis when it had started later. Moreover, investigators were asked to report the number of breakthrough bleeding events in the previous 12 months.

Haemophilia-specific QoL questionnaire

A first haemophilia-specific QoL questionnaire (Haemo-QoL) for three age groups was developed and tested psychometrically in a pilot study [14]. The Haemo-QoL was then modified after field testing [12] and the revised version was used for the purposes of this paper.

An interview version for smaller children (age group I: 4–7 years) was available with 21 items pertaining to eight dimensions ('physical health', 'feelings', 'view', 'family', 'friends', 'others' 'sport and school/kindergarten', 'treatment'). For the schoolchildren aged 8–12 years (age group II) the self-administered questionnaire consisted of two additional domains ('perceived support', 'dealing') with overall 64 items; for adolescents (age group III: 13–16 years) it was expanded with two further additional domains ('relationships', 'future') and consisted of 77 items.

The 'physical' dimension includes questions concerning pain and bleeding, etc.; in the dimension 'feeling' it was asked how children feel related to their haemophilia; questions about how children perceive themselves are included in the dimension 'view'; the interaction in the family was questioned in the dimension 'family'; the dimension 'friends' contains questions about the interaction with friends; questions about how children perceive the support they receive from others pertain to the dimension 'perceived support'; in the 'sport and school' dimension children were asked about their school/kindergarten life; the dimension 'dealing' contains questions about how children deal with their haemophilia; questions in the dimension 'treatment' concern treatment issues; in the dimension 'others' the interaction with others is of interest. The two additional dimensions 'future' and 'partnership' asked about specific issues, such as the view of the future and the possibility of having a girlfriend.

Because there were different numbers of items between the dimensions and in the different age groups, the values were transformed to a scale from 0 to 100 to allow comparison between the answer patterns across the age groups and to compare the impairments in the different dimensions. High values indicate high impairments in HRQoL.

Results

Of 28 centres who were asked to participate, 20 contributed to the study: six centres in France, four in Germany, three in Italy, two in the Netherlands, four in Spain and one in the United Kingdom. Overall 339 children with haemophilia with an average age of 10 years (range interval: 4–16 years) were enrolled. Eight patients were excluded because they did not meet the inclusion criteria. An additional 13 patients were not included in the analysis because the medical documentation was not available. Of 318 assessable patients, 95 were in age group I (4–7 years), 118 in age group II [8–11,13] and 105 in age group III (13–16 years).

Health status

Of 318 assessable patients, 85.5% had haemophilia A and 11.6% haemophilia B, with a factor level of 1% or less (= 0.01 IU dL^{-1}) in 86.5% of them. A history of inhibitor was present in 52 children (16.4%), without any difference in the three age groups. Of these, 37 children (71.2%) underwent an immune tolerance induction treatment, most of them in age group I (85.0%). The mean peak titre of the inhibitor was 147 BU (range 0.5–2640.0); 60.5% of these patients had been high responders (peak titre > 5 BU).

The mean age at the first bleeding was 11 months (median 9, range 0–88), while the age at the first joint bleed was 25 months (median 21, range 0–96). On average the children had had 7.8 bleeds in the past 12 months (median 2, range 0–120); no difference between age groups was found. Less than five joint bleeds in the previous 12 months were reported in two-thirds of the children (67.0%), being similar in all age groups. Half the children were reported to have a target joint (50.3%): 61.0% of the oldest children (age group III) had a target joint ($n = 64$) in comparison to 37 children (38.9%) in age group I (χ^2 test, $P < 0.01$). The most involved target joint was the ankle ($n = 88$), followed by the elbow ($n = 51$) and the knee ($n = 51$), some patients suffering from recurrent bleeding in more than one joint.

A small proportion of patients (11.3%) was suffering from functional joint impairments ($n = 36$), with a significant increase in the older age groups (χ^2 test, $P < 0.018$). Chronic pain was reported in 3.1% of the children ($n = 10$): no difference was found between the groups.

Table 1. Treatment modalities in children with severe haemophilia (figures in parenthesis indicate the number of patients with that particular feature).

	Σ ($n = 318$)	I ($n = 95$)	II ($n = 118$)	III ($n = 105$)
Home treatment				
No ($n = 63$)	19.8%	29.9%	16.1%	14.6%
Yes ($n = 252$)	79.2%	69.1%	83.1%	84.5%
Missing data ($n = 3$)	0.9%	1.0%	0.8%	1.0%
Treatment administration*				
Self-administration	34.1%	7.7%	29.9%	58.6%
Mother administered	67.1%	75.4%	74.2%	52.9%
Father administered	36.1%	52.3%	36.1%	24.1%
Other relatives administered	4.0%	3.1%	4.1%	4.6%
Treatment/physician				
At centre	11.3%	16.5%	8.2%	10.5%
At home	2.8%	5.2%	1.6%	1.9%
Both	1.8%	5.2%	0.8%	–
Treatment/nurse				
At centre	17.8%	22.7%	20.5%	9.5%
At home	11.7%	10.3%	10.7%	13.3%
Both	8.0%	10.3%	7.4%	6.7%
Catheter implantation				
No ($n = 273$)	85.8%	79.4%	87.3%	90.3%
Yes ($n = 40$)	12.6%	20.6%	11.9%	5.8%
Missing data ($n = 5$)	1.6%	–	0.8%	3.9%
Type of product				
Plasma derived ($n = 75$)	24.2%	16.5%	27.2%	28.3%
Recombinant ($n = 235$)	75.8%	83.5%	72.8%	71.7%
Treatment scheme				
Prophylaxis ($n = 212$)	66.7%	68.4%	68.6%	62.9%
On-demand ($n = 101$)	31.8%	30.5%	30.5%	34.3%
Missing data ($n = 5$)	1.6%	1.1%	0.8%	2.9%

*Multiple answers were possible.

The most frequently reported concomitant disease was HCV infection in 19 children, being present in 18 children of age group III and absent in age group I. Developmental delay was found in four patients and neuromuscular disease in one patient. None of the patients had malignancy.

Table 1 shows the treatment modalities of the enrolled children. The majority of all 318 patients were treated at home (79.2%), with a significant difference between the age groups (χ^2 test, $P < 0.02$). One-third of patients (34.1%) self-administered the product, especially the oldest children (58.6%); mothers were the most involved relatives in their home treatment (67.1%). An indwelling central venous catheter was implanted in 12.6% of the children, significantly more often in young children (χ^2 test, $P < 0.01$). Approximately three-quarters of the children (75.8%) were treated with recombinant products in comparison to plasma-derived products (24.2%). Two-thirds of assessable children ($n = 212$) were on prophylaxis, 31.8% received on-demand treatment ($n = 101$).

In Table 2 the characteristics of prophylactic and on-demand treatment are described. Only 19.8% of children on prophylaxis were on true primary prophylaxis, as defined in the Methods section, whereas the great majority was on secondary prophylaxis. Most of the children (78.7%) were receiving the prophylactic treatment three times a week or more. No differences were found between the age groups. The dosage used per infusion was 34.5 IU kg^{-1}, being significantly higher in younger patients (ANOVA, $P < 0.001$). The average number of breakthrough bleeding events per month was 0.4 (SD = 0.4), being lower in the younger age group.

Data for children who were actually treated on demand are also presented in Table 2. Children on on-demand treatment had on average 1.1 bleeding events per month, ranging from 0.6 in age group I to 1.5 in age group III, this difference being not significant. They received on average 3.2 infusions per month: the difference between the age groups was statistically significant (ANOVA, $P < 0.001$). The average total amount of factor consumption was different between age groups (ANOVA, $P < 0.001$), as expected, as patients' weights and bleeding incidence were higher in the older age group.

Interestingly, the difference in the incidence of breakthrough bleeding events between prophylaxis patients and on-demand patients was statistically

Table 2. Type of treatment, bleeding events and factor consumption in children on prophylactic or on on-demand treatment (figures in parenthesis indicate the number of patients with that particular feature).

	Σ	I	II	III
Patients on prophylaxis				
Type of prophylaxis				
Primary prophylaxis ($n = 42$)	19.8%	26.2%	17.3%	16.7%
Secondary prophylaxis ($n = 167$)	78.8%	70.8%	81.5%	83.3%
Missing data ($n = 3$)	1.4%	3.1%	1.2%	–
Frequency of infusions/week				
Two ($n = 44$)	20.8%	24.6%	22.2%	15.2%
Three ($n = 136$)	64.2%	64.6%	59.3%	69.7%
Every other day ($n = 31$)	14.5%	10.8%	17.3%	15.2%
Missing data ($n = 1$)	0.5%	–	–	–
Dosage IU kg^{-1} ($n = 197$) (mean ± SD)	34.5 ± 18.3	44.6 ± 23.1	33.8 ± 15.5	25.7 ± 9.8
Weeks on prophylaxis ($n = 212$) (mean ± SD)	51.4 ± 2.4	50.8 ± 4.0	51.6 ± 1.2	51.8 ± 0.8
No. of breakthrough bleeding events per month ($n = 51$) (mean ± SD)	0.4 ± 0.4	0.2 ± 0.4	0.4 ± 0.4	0.5 ± 0.4
Factor consumption IU per month ($n = 212$) (mean ± SD)	13 191 ± 9159	12 509 ± 8576	13 479 ± 8.995	13 509 ± 9.983
Patients receiving on-demand treatment				
Number of bleeding events per month ($n = 81$) (mean ± SD)	1.1 ± 1.6	0.6 ± 0.4	1.1 ± 1.2	1.5 ± 2.3
Number of infusions per month ($n = 72$) (mean ± SD)	3.2 ± 2.6	2.0 ± 1.7	2.7 ± 2.2	4.6 ± 2.9
Factor consumption IU per month (mean ± SD)	5815 ± 6753	1568 ± 1271	3926 ± 3.458	10 591 ± 8399

Per month ($n = 90$).

Table 3. WFH score in children with severe haemophilia (figures in parenthesis indicate the number of patients with that particular feature).

Characteristics	Σ	I	II	III
Swellings				
None ($n = 211$)	94.2%	95.5%	95.1%	92.1%
Swellings present ($n = 12$)	5.4%	4.5%	3.7%	7.9%
Chronic synovitis ($n = 1$)	0.4%	–	1.2%	–
Muscle atrophy				
None or minimal ($n = 219$)	97.8%	100%	97.6%	96.0%
Present ($n = 5$)	2.2%	–	2.4%	4.0%
Axial deformity knee				
Normal ($n = 212$)	95.9%	100%	93.9%	94.6%
8–15° varus or 0–5° valgus ($n = 9$)	4.1%	–	6.1%	5.4%
Axial deformity ankle				
No deformity ($n = 213$)	98.6%	100%	98.8%	97.2%
< 10° valgus ($n = 3$)	1.4%	–	1.3%	2.8%
Crepitus on motion				
None ($n = 203$)	92.3%	100%	92.6%	84.9%
Present ($n = 17$)	7.7%	–	7.4%	15.1%
Range of motion				
Loss of 10% ($n = 179$)	93.2%	100%	94.4%	87.0%
Loss of 10–33% ($n = 13$)	6.8%	–	5.6%	13.0%
Flexion contracture				
≤ 15° ($n = 190$)	96.9%	94.4%	100%	95.6%
> 15° ($n = 6$)	3.1%	5.6%	–	4.4%
Instability				
None ($n = 213$)	96.4%	95.5%	98.8%	94.5%
Present, but not interfering ($n = 8$)	3.6%	4.5%	1.2%	5.5%
WFH orthopaedic joint total score (mean ± SD)	2.19 ± 0.77	2.23 ± 0.60	2.10 ± 0.74	2.25 ± 0.91

different in the whole cohort as well as in each age group (ANOVA, $P < 0.001$). Almost all the patients receiving on-demand treatment (97.8%) experienced at least one bleed in the previous 12 months, in comparison to 60.7% of patients on prophylaxis. The incidence of bleeding episodes per month in the prophylaxis group ($n = 51$) were 0.4 (SD = 0.4) and in the on-demand group ($n = 81$) 1.1 (SD = 1.6). The

total factor consumption per month was significantly higher in prophylaxis patients in comparison to on-demand patients in age group I and II ($P < 0.000$), but no differences were shown between on-demand and prophylaxis patients in age group III (see Table 2).

The distribution of the WFH orthopaedic joint score is described in Table 3, where only the percentage of the given answers is reported. Only few children had joint swelling ($n = 12$), muscle atrophy ($n = 5$), axial deformity in knee ($n = 9$) or ankle ($n = 3$). Crepitus of motion was present in 17 children and showed a significant difference between the groups (χ^2 test, $P < 0.01$). A loss of motion of 10–33% was reported in 6.8% of patients ($n = 13$), being more frequent in older children, age group III (χ^2 test, $P < 0.02$). Of the assessed children 3.1% had a flexion contracture > 15 ($n = 6$) and 3.6% had an instability ($n = 8$) that did not interfere with functioning. The WFH score reached a mean of 2.19 (SD = 0.77) and was similar over the three age groups.

HRQoL

The HRQoL of children with haemophilia was assessed with the recently developed disease-specific questionnaire Haemo-QoL [12], consisting of six to 12 dimensions according to the different age groups, with a smaller number of items for younger children.

As shown in Fig. 1, the youngest children were mainly impaired in the dimension 'family' (M = 34.38, SD = 24.8), which can be related to overprotection by the parents. Young children were only partially impaired in the interaction with 'others' (M = 22.28, SD = 27.4) or concerning their 'treatment' (M = 24.45, SD = 28.1). Children in age groups II and III were perceived not to receive sufficient support from others reported in the dimensions 'perceived support' (II: M = 49.41, SD = 25.23; III: M = 53.00, SD = 23.79) and were impaired in the interaction with their 'friends' (II: M = 43.91, SD = 28.05; III: M = 46.12, SD = 25.06). In addition older children had problems in dealing with their haemophilia as reported in the dimension 'dealing' (II: M = 33.21, SD = 20.36; III: M = 31.92, SD = 19.65).

Clinical data such as type of treatment and number of bleeds were chosen to examine whether they were associated with differences in Haemo-QoL scores. Regarding type of treatment, differences were found in young children (age group I): children on prophylactic treatment were more impaired ($P < 0.01$) in the subscale 'feeling' of the Haemo-QoL. Adolescents (age group III) on prophylactic treatment were less impaired in the subscale 'sport and school' than adolescents receiving on-demand treatment ($P < 0.05$) and showed less impairments in the Haemo-QoL total score ($P < 0.01$) (for further details, see also [14]).

The severity of clinical manifestations of haemophilia was investigated by the number of joint bleeds and major bleeding events. Differences in HRQoL regarding incidence of joint bleeds are reported in this issue [12].

Considering the number of major bleeds, significant but small differences in HRQoL (transformed data) were found in age group I: the 39 children with three major bleeds or more in the previous 12 months had less impairments in their quality of life in comparison to the 38 children with less than three bleeds in the total score (Table 4) and in three

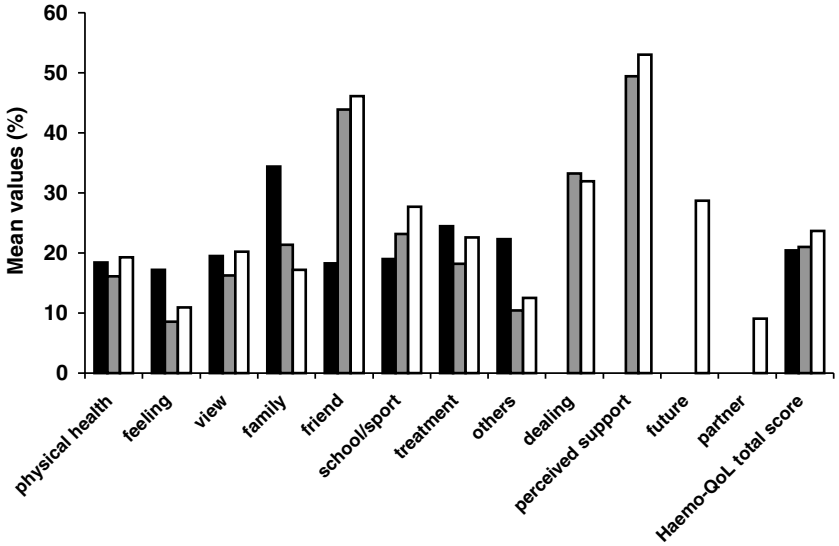

Fig. 1. Mean values (transformed) of the Haemo-QoL dimensions in the three age groups (low values represent low impairments in quality of life and high values high impairments): age group I (black bars), age group II (grey bars) and age group III (white bars).

Table 4. Number of major bleeding events in the previous 12 months and HRQoL (transformed data).

Haemo-QoL	I			II			III		
No. of major bleeds	< 3	≥ 3	*P*	< 3	≥ 3	*P*	< 3	≥ 3	*P*
Physical health	21.71	13.78	n.s.	14.64	16.03	n.s.	13.16	23.91	0.002
Feeling	24.12	11.54	0.041	8.40	8.93	n.s.	9.38	12.37	n.s.
View	10.90	27.08	0.017	16.56	13.62	n.s.	16.69	21.22	n.s.
Family	43.40	27.88	0.004	24.13	21.31	n.s.	13.93	19.02	n.s.
Friend	21.62	15.00	n.s.	38.58	52.03	0.020	44.49	45.48	n.s.
Perceived support	–	–	–	51.72	48.69	n.s.	46.14	58.02	0.028
Others	26.35	16.03	n.s.	11.14	8.33	n.s.	11.31	14.45	n.s.
Sport	18.98	16.67	n.s.	23.69	23.47	n.s.	21.51	32.80	0.014
Dealing	–	–	–	32.66	34.73	n.s.	33.61	30.63	n.s.
Treatment	25.00	21.15	n.s.	18.50	19.69	n.s.	23.07	22.16	n.s.
Future	–	–	–	–	–	–	30.15	28.80	n.s.
Partner	–	–	–	–	–	–	8.46	10.05	n.s.
Total	25.56	16.27	0.006	21.21	21.08	n.s.	21.58	25.74	n.s.

n.s.: Not significant.

dimensions ('feeling', 'view' and 'family'). For age group II a difference was shown in the subscale 'friends': the 43 children with three major bleeds or more were more impaired than the 52 children with less than three major bleeds. The 47 adolescents (age group III) with three major bleeds revealed more impairments in the dimensions 'physical health', 'perceived support' and 'school and sport' than the 35 adolescents with less than three major bleeds.

Discussion

This analysis was carried out in the framework of a study designed to develop an instrument to assess health-related QoL in children with haemophilia [12]. To evaluate better the children's quality of life and their determinants, sociodemographic, clinical and psychosocial characteristics were included in the study protocol, using additional standard measures. The analysis of data obtained by these additional measures provided valuable information about clinical status and healthcare of haemophilic children in Western Europe.

The results of this analysis indicated that most of the patients with haemophilia suffer from bleeding in the first year of life, and the first joint bleeding occurs mainly within the second year of life, providing an authoritative confirmation of what has been reported previously by other authors [15]. Furthermore, this information provides further grounds and confirmation of the recommendation to start prophylaxis as soon as possible within the second year of life [16,17].

Surprisingly, half the children of this cohort were reported to have one or more recurrently bleeding joints (target joints), represented in particular by ankles. These findings can be explained by the weak definition adopted by this study ('several bleeds in the same joint') to indicate a target joint, which was independent of the frequency of bleeding events.

As expected, only a very small proportion of patients showed clinical signs of joint involvement, as shown by the WFH orthopaedic joint score. Home treatment seems to be widely present in Western European children, self-treatment being more frequent in older children, and mothers the most active in their children's treatment. An indwelling central venous catheter was implanted in one-fifth of younger children, due to the need for easy venous access for prophylactic treatment. Recombinant products were the most used in these children, even though plasma-derived concentrates were still largely utilized, confirming the observations provided by the European Paediatric Network for Haemophilia Management [17].

Two-thirds of haemophilic children in Western Europe at the time of the analysis were on prophylaxis, even though the prophylaxis met the criteria for primary prophylaxis in only one-fifth of them [16,17]. The study confirms a significant reduction of bleeding incidence in children on prophylaxis compared to children receiving on-demand treatment. By contrast, the total concentrate consumption appears to be similar in adolescents on prophylaxis and on-demand treatment, indicating that prophylaxis absorbs more economic resources at the beginning in small children, but thereafter it leads to a saving of resources in older patients. These findings demonstrate clearly that cost–effectiveness and cost–benefit ratios of prophylaxis must be evaluated after a relatively long period of observation.

HRQoL seems satisfactory in this cohort, as shown by the newly developed haemophilia-specific questionnaire Haemo-QoL, with scores widely below 50 in a range from 0 to 100, high scores being associated with high impairments. Young children appeared to be impaired in the dimension 'family' and 'treatment' which can be explained, for example, by the overprotection of parents assessed in the dimension 'family' or the burden throughout the treatment. Older children had higher impairments in the so-called 'social' dimensions, such as 'perceived support' and 'friends' and also in the dimension 'dealing', which concerns personal adaptation to the disease.

Prophylaxis affected HRQoL: the youngest children were bothered by it, shown particularly by the dimension 'feeling'. By contrast, adolescents showed a higher HRQoL, revealed particularly by the dimension 'school and sport'. This response pattern is confirmed by a slightly lower HRQoL score in smaller children with less major bleeding events, which are clearly associated with prophylactic treatment, whereas HRQoL tested better in adolescents with less bleeding events. These findings indicate strongly that, first, the initial burden induced by prophylaxis in younger children is highly compensated by improvements in HRQoL in older children; secondly, it is essential to evaluate HRQoL and other outcome measures in patients on prophylaxis in the long term.

In conclusion, healthcare of children in Western Europe is improving progressively with a large diffusion of home treatment and prophylaxis. This provides a high level of health status and HRQoL. The newly developed haemophilia-specific QoL questionnaire is sensitive and versatile and discriminates between clinical subgroups and treatments. Even though the study was not designed to evaluate the impact of prophylaxis on HRQoL, it showed a better HRQoL in haemophilic adolescents on prophylaxis. Further specific studies are required to confirm these findings.

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ORIGINAL ARTICLE *Paediatrics*

The impact of sport on children with haemophilia

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Summary. Sport is nowadays perceived as beneficial for children with haemophilia, as good muscle strength supports joints and may reduce bleed frequency; by contrast psychological benefits are less known. This study introduces the impact of sport on health-related quality of life (HRQoL) and physical performance in children with haemophilia. A cross-sectional, multi-site, study of boys aged 6–17 years with haemophilia A or B of any severity, current or past inhibitor, which assessed physical performance, sporting activity and HRQoL using age appropriate questionnaires including KINDL, Haemo-QoL and HEP-Test-Q. Eighty-four haemophilic boys (23 mild, 19 moderate, 42 severe) with a mean age of 11.52 years (SD = 3.4) were enrolled from two haemophilia centres in the United Kingdom. 28.4% were overweight/obese according to their BMI/age and had a good orthopaedic status (M = 1.55, SD = 3.3). Boys watching < 1–2 h of TV/PC/day had fewer days lost (M = 3, SD = 3.2) than those with a more sedentary lifestyle (M = 9.40, SD = 7.1) ($P < 0.032$). 90.5% participated in regular sporting activity; 79.9% at least twice a week. HRQoL in children was generally good, with highest impairments in boys aged 8–12 years. Boys aged 8–16 years reported good physical performance (M = 80.0, SD = 16.0) with highest impairments in the dimensions 'endurance' and 'mobility'. Boys doing sport had a significant better physical performance and HRQoL than boys not doing sport. Sedentary life styles had a negative impact on the subjective physical performance and number of days lost of children. Encouraging haemophilic boys to participate in sport will have a direct impact on their overall HRQoL.

Keywords: children and adolescents, haemophilia, health-related quality of life, physical performance, sporting activity

Introduction

Haemophilia is an X linked, usually inherited disorder of coagulation factors VIII (haemophilia A) or IX (haemophilia B) which occurs in approximately 1:5000 – 1:10 000 live births in the UK [1]. It affects boys, causing painful, spontaneous or trauma related bleeding predominantly in the weight bearing joints. Patients were sub-categorized in the 1950's by Biggs and Macfarlane [2] as severe (with factor levels of <1 iu dL^{-1}) moderate (levels 2–5 iu dL^{-1}) and mild haemophilia (5–50 iu dL^{-1}) according to factor level (normal range 50–150 iu dL^{-1}). Bleeding mostly correlates with factor level, thus those with severe haemophilia are most clinically affected. Children with haemophilia experience spontaneous or trauma related bleeding from an early age, without treatment early arthritic joint damage occurs [3].

Until the mid 1970's it was usual practice to discourage sporting activity in those with haemophilia because of the bleeding risk [4]. Today this attitude is more flexible, with many clinicians believing that sport is in fact beneficial for physical [5], social [6] and psychological [7] well-being. In part this is due to better treatment with prophylactic therapy being given to those patients with clinically severe haemophilia to prevent bleeds, minimize disability and improve quality of life (QoL) [8]. Many boys are now active sportsmen, participating at local, national and international levels, and have intensive prophylactic treatment tailored around their individualized sporting activity [9]. This enables them to participate fully in any activities they choose. The changes in treatment and resulting physical benefits have been shown to improve health-related quality of life (HRQoL) in these children [10]. There is, however, little data to support the non-physical benefits of sport in these

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boys. Therefore, a multi-centre study into the 'Evaluation of the Impact of Sport Activities on Health-Related Quality of Life of Haemophilia Patients' (EIS Study) was designed.

Study design and methods

The EIS Study aimed to recruit up to 400 children (aged 6–17) and adults with haemophilia (aged 18–65), and parents of children. The data from children are presented in this article.

One hundred and twenty children with haemophilia of any severity or type, with or without inhibitors aged 6–17 years and their parents from two centres in the UK were invited to participate in the study. The children were divided into three age groups: [4–7 years (group I), 8–12 years (group II), 13–16 years (group III)] and were requested to complete age appropriate questionnaires which studied the impact of sport on their lives. The questionnaires were designed specifically for the study using validated questionnaires to collect data on HRQoL and physical performance. In addition, questions concerning sporting activities (e.g. frequency of sport per week and number of hours spent participating in sport) and attitudes towards sports were assessed using specially developed questionnaires, which were completed following parental consent and child assent at routine haemophilia appointments. Clinicians completed medical documentation including information about type and severity of haemophilia, bleeding, inhibitor history, concurrent illness, type and schedule of treatment and frequency of medical visits. The orthopaedic status was evaluated by the physiotherapist at each participating centre. Ethical approval for the study was granted by a local research ethics committee.

Instruments

The HRQoL was assessed using respective age-group versions [4–7 (group I), 8–12 (group II), 13–16 (group III) years] of the generic KINDL [11] and the haemophilia-specific Haemo-QoL instruments [12]. The KINDL questionnaire assesses self-reported HRQoL in six domains (physical function, psychological well-being, self-esteem, family, friends, school) and has an additional chronic-generic module with high values (range 0–100) indicating a good HRQoL. The disease-specific Haemo-QoL assesses self-report HRQoL of children with haemophilia. It consists of 8–12 dimensions dependent upon age (8, 10 and 12 dimensions, respectively) of HRQoL (physical health, feelings, attitudes, family, friends, perceived support, other persons, sports & school, dealing with the disease, treatment, future, relationships) with high values (range 0–100) indicating high impairments in HRQoL.

Physical performance was assessed by a patient-rated outcome using the HEP-Test-Q [13] and by an objective measure assessed by clinicians using the paediatric Petrini Haemophilia Joint Score [14]. The HEP-Test-Q was originally developed for adults with haemophilia and assesses four dimensions (mobility, strength & coordination, endurance and body perception) with high values (range 0–100) indicating better physical performance. In this study a child-adapted version of the HEP-Test-Q was included for children aged over 7 years, which varied only concerning the wording of some items compared with the adult version. The Petrini Joint Score ranks six joints (the elbow, ankle and knee, right and left) on swelling, muscle atrophy, axial alignment, crepitus on motion, flexion and extension loss, instability, joint pain, gait and strength. Scores of 0 indicate no joint problems; a maximum score of 156 would indicate severe joint damage and immobility.

Statistical analysis

All statistical analyses were conducted using the SPSS program version 17 (SPSS Inc. Chicago, IL, USA). Descriptive data are shown as frequency distribution in percent or as mean ± standard division SD (range), median and interquartile ranges (IQR) and were tested for normal distribution using the Kolmogorov-Smirnov test. The comparison of differences between groups was examined by Student's test or Mann–Whitney *U*-test according to distribution; P values < 0.05 were defined as significant.

To investigate the impact of sport on children's well-being we considered the following variables: doing sport (yes vs. no), sedentary life style [<12 h/day in front of television (TV) or computer vs. ≥ 2 h/day, frequency of doing sport (2 times/week vs. ≥ 3 times/week) and hours of sport (<5 h/week vs. <5 h/week)]. To determine the cut-off point for these variables the median split was calculated.

Results

Clinical data

From the 120 children invited to participate 84, with a mean age of 11.52 years (SD = 3.4, range 5.83–17.86), were enrolled into the study (70%). 92.3% had haemophilia A, half were severely affected, 22.6% had moderate and 27.4% had mild haemophilia. Two-thirds of the boys had regular prophylaxis; 16.9% reported targeted prophylaxis before sport. Nine boys had target joints and 28.8% were overweight or obese according to their BMI and age (see Table 1).

Overall bleeds were reported at a median of 0 (range 0–18, IQR = 2) in the 6 months preceding questionnaire completion, of these a median of 1 (range 0–10, IQR = 3) where joint bleeds and 0 bleeds in median (range 0–6, IQR = 0) were attributed to

Table 1. Clinical data of children with haemophilia (n = 84).

Clinical data	*N*	Percentage (%)
Type of haemophilia: A	77	91.7
B	7	8.3
Severity: severe	42	50.0
moderate	19	22.6
mild	23	27.4
Inhibitor: past or current	18	21.4
still present	5	27.8
Type of treatment: on demand	28	33.3
prophylaxis	56	66.7
Prophylaxis prior to sports:	14	16.9
Home treatment:	64	76.2
Presence of target joints:	9	10.8
Blood-borne infections:	0	0
Presence of chronic pain (M = 6.2, range 1–10)	22	27.5
BMI: under weight	27	33.8
normal weight	30	37.5
overweight	12	15.0
obesity	11	13.8

sport. A visual analogue scale was used to record chronic pain, (ranging from 0–10, where a score of 10 indicates maximal pain), 22 boys (26.2%) reported chronic pain in the preceding 6 months with a mean pain score of 6.19 (SD = 2.2 range 1–10). The Petrini score attained a median of 0 (range 0–15, IQR = 1), thus the boys in this study had evidence of good joint function. Nineteen boys missed days at school, 18 due to haemophilia: with a mean of 6.56 (SD = 6.5, range 1–26) days lost in the preceding 6 months. Three missed a mean of 3 days at school due to sporting injury (SD = 2.6, range 1–6). Clinical data across all three age groups are described in Table 2.

No differences in clinical data in terms of orthopaedic status, BMI, number of days lost from school and number of bleeds were found for sedentary lifestyle, doing sport or frequency/hours of sport. The only significant difference was found for number of days lost ($P < 0.032$) for boys watching <1–2 h of TV/computer games per day who had fewer days lost (M = 3, SD = 3.2) than those with a more sedentary lifestyle (M = 9.40, SD = 7.1) and for those boys doing sports more than 5 h week^{-1} (M = 4.3, SD = 3.9) compared with those doing sports less than 5 h/week (M = 11.5, SD = 8.2) ($P < 0.032$).

Sporting activity

Of the 84 participating boys only eight reported not doing any sport. The reasons for this were: that they weren't allowed to do it (n = 5), that they did not like it (n = 4) or that they were afraid of hurting themselves (n = 4) (more than one reason was given by some boys).

Seventy-six boys (90.5%) did sport. They reported participation in an average of four sports each, with the majority of boys doing sport twice weekly (see Fig. 1) They mainly performed sport with friends (80%) and at school (80%), although 40% of children reported participation in team sports at a sports club. In total, boys were doing an average of 4.9 hours sporting activities per week (range 1–13 h). Just over half of the boys (59.2%) participated in sport for 2–5 h/week with 35.5% stating they did 6–9 h, with 2.6% doing as much as 10–13 h/week (see Table 3) An extensive array of sporting activity was reported with the top five sports being: football (77.4%), jogging (76.2%), swimming (59.5%), gymnastics (36.9%) and cycling (25%).

Seventy-seven boys (95.17%) thought that doing sport was good, with arguments that it: 'is healthy and keeps you fit' (n = 36), 'is fun' (n = 21), 'makes you active' (n = 6) and 'is social' (n = 2). Other arguments in favour of sport were that it 'got you outside', 'gives you confidence' and 'is good when eating junk food'. Seventy-one boys (93.4%) reported that they would like to continue sports when they are older. Of these boys, 57 would like to try new sports such as rugby (n = 4), boxing (n = 3), cricket (n = 3), golf (n = 3) and hockey (n = 2).

Eight boys (9.5%) thought that sport was bad or dangerous: four boys considered specific sports such

Table 2. Clinical data according to age groups.

Clinical data	6–7 years (n = 15) M ± SD Median [IQR]	8–12 years (n = 41) M ± SD Median [IQR]	13–17 years (n = 28) M ± SD Median [IQR]	$\sum$ (n = 84) M ± SD Median [IQR]
BMI	17.74 ± 2.4 17.72 [4.1322]	19.04 ± 4.7 17.36 [3.8075]	22.95 ± 4.4 21.72 [5.3375]	20.11 ± 4.7 18.92 [5.7869]
Orthopaedic status (Petrini Score)	0 ± 0 0 [0]	1.32 ± 2.8 0 [1.5]	2.7 ± 4.3 0 [5]	1.53 ± 3.2 0 [1]
No of total bleeds in the past 6 months	2.13 ± 4.5 1 [2]	1.88 ± 4.0 0 [2.5]	1.57 ± 2.5 0.5 [2]	1.82 ± 3.6 0 [2]
No of joint bleeds in the past 6 months	1.60 ± 3.1 0 [2.25]	2.26 ± 3.2 1 [3]	1.84 ± 2.8 1 [3]	1.96 ± 3.0 1 [3]
No of sports-related bleeds in the past 6 months	0 0 [0]	0.72 ± 1.6 0 [0.25]	0.41 ± 0.8 0 [0.5]	0.45 ± 1.2 0 [0]
Number of days lost in the past 6 months:				
haemophilia-related	11.67 ± 12.4 5 [22]	6.22 ± 5.2 6 [9]	4.50 ± 3.9 3.5 [5]	6.56 ± 6.5 4.5 [9.25]
sport related	0 – [–]	3.5 ± 3.5 3.5 [5]	2.00 ± 0 2 [0]	3.0 ± 2.6 2 [5]

IQR, interquartile range.

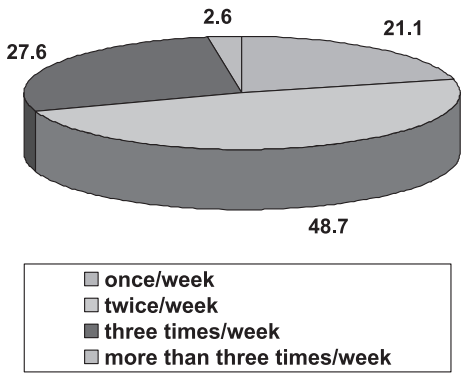

Fig. 1. Frequency of reported sporting activity.

Table 3. Hours of sporting activity across age groups.

Hours	Age 6–7 *N* (%)	Age 8–12 *N* (%)	Age 13–17 *N* (%)	∑ *N* (%)
1 hour	0	2 (5.3%)	0	2 (2.6%)
2–5 h	12 (85.7%)	21 (55.3%)	12 (50%)	45 (59.2%)
6–9 h	2 (14.3%)	13 (34.2%)	12 (50%)	27 (35.5%)
10–13 h	0	2 (5.3%)	0	2 (2.6)
∑	14 (100%)	38 (100%)	24 (100%)	76 (100%)

as boxing, rugby and hockey as dangerous and stated they considered getting hurt ($n = 4$) or falling ($n = 2$) too risky.

Health-related quality of life (HRQoL)

In the generic KINDL questionnaire children reported a quite good overall HRQoL in the total score, being highest in the youngest age group (I: M = 77.61, SD = 14.2; II: M = 70.40, SD = 8.9; III: M = 70.38, SD = 12.3), while for the chronic-generic module best values were reported by the oldest age group (I: M = 82.67, SD = 17.6; II: M = 82.98, SD = 20.9; III: M = 86.88, SD = 11.8). Haemophilic children reported the highest impairments in the dimension 'school' (see Table 4).

The haemophilia-specific HRQoL in children was generally good. Children in age group II (8–12 years) reported the highest overall impairment in the Haemo-QoL (M = 41.43, SD = 10.5). The youngest boys (6–7 years) reported highest impairment in the domains 'sport and school' (M = 44.44, SD = 25.7) followed by the dimensions 'treatment' (M = 32.14, SD 37.2) and 'other' (M = 32.14, SD = 30.1). In contrast, boys in age group II reported highest impairments in the domains 'dealing' (M = 75.74, SD = 16.3) followed by 'treatment' (M = 68.47, SD = 8.0) and 'friends' (M = 64.3, SD = 27.0). Adolescents reported highest impairment in the domains 'perceived support' (M = 50.8, SD = 27.5) 'friends' (M = 44.75, SD = 24.3) and 'future' (M = 33.11, SD = 20.5) (see Table 4).

Sport did not prove to have an impact on the HRQoL of children in age group I. By contrast there was a significant difference in HRQoL between children in age groups II and III grouped together doing sport and those not doing sport. Boys who did not do sport were more impaired in the dimension 'feeling' ($P < 0.014$) and 'family' ($P < 0.13$) than those doing sport (see Fig. 2). Children practising sport ≥ 3 times per week (M = 22.34, SD = 15.5) reported a better 'view of themselves' ($P < 0.017$) than children doing sport twice a week or less (M = 33.83, SD = 18.5). Children doing <5 hours sport per week (M = 44.53,

Table 4. HRQoL scores across age groups (KINDL, Haemo-QoL).

HRQoL questionnaire	Domains	Age 6–7 Mean (SD)	Age 8–12 Mean (SD)	Age 13–17 Mean (SD)
KINDL	Physical	75.00 (21.1)	74.41 (19.0)	74.31 (16.8)
	Emotional	76.67 (24.0)	79.49 (12.8)	77.31 (15.7)
	Self-Esteem	75.00 (23.1)	59.46 (17.1)	63.43 (21.9)
	Family	81.67 (24.0)	73.96 (18.6)	72.99 (18.1)
	Friend	81.67 (24.0)	79.55 (16.9)	75.45 (18.7)
	School	75.00 (31.0)	55.01 (17.2)	57.93 (20.4)
	KINDL TOTAL	77.61(14.2)	70.40 (8.9)	70.38 (12.3)
	Chronic-Generic	82.67 (17.6)	82.98 (20.9)	86.88 (11.8)
Haemo-QoL	Physical Health	22.12 (26.1)	28.39 (26.6)	22.69 (20.0)
	Feeling	14.44 (17.7)	18.38 (24.7)	13.17 (15.1)
	View	14.29 (25.4)	36.1 (15.1)	23.93 (20.1)
	Family	31.39 (32.9)	21.08 (19.4)	25.65 (26.2)
	Friends	23.33 (32.0)	64.3 (27)	44.75 (24.3)
	Support	–	57.94 (27.7)	50.80 (27.5)
	Other	32.14 (30.1)	12.39 (18.8)	17.11 (20.5)
	Sport	44.44 (25.7)	46.48 (10.3)	25.72 (20.8)
	Dealing	–	75.74 (16.3)	19.36 (15.2)
	Treatment	32.14 (37.2)	68.47 (8.0)	21.30 (14.5)
	Future	–	–	33.11 (20.5)
	Relationships	–	–	9.26 (16.5)
	Haemo-QoL TOTAL	28.09 (14.8)	41.43 (10.5)	24.44 (12.7)

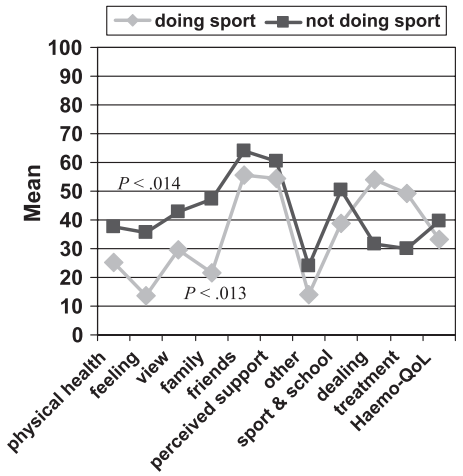

Fig. 2. Comparison of HRQoL (Haemo-QoL) across boys doing sport and those not doing sport.

SD = 23.8) felt less impaired ($P < 0.021$) in their 'perceived support' than children doing ≥ 5 h sport per week (M = 61.58, SD = 28.8).

Physical performance

Psychometric testing of the child-adapted version revealed excellent values in terms of reliability ranging from Cronbach's $\alpha = 0.855$ for 'endurance' to $\alpha = 0.936$ for 'the HEP-Test-Q total score'. The child-adapted HEP-Test-Q version showed as well good values for discriminant validity demonstrating that children who suffered from chronic pain reported in almost all subscales a significant lower subjective physical performance than those without pain.

The 67 boys in groups II and III (aged 8–17 years) completed the HEP-Test-Q. In general they reported good physical performance (M = 80.0, SD = 16.0) with highest impairments in the dimensions 'endurance' (M = 73.70, SD = 19.1) and 'mobility' (M = 75.29, SD = 24.0). There were no differences between age groups II (8–12 years) and III (13–17 years).

There was significant difference between children doing and not doing sport in all domains of physical performance (see Table 5). This demonstrates that children who do not do sport perceive their physical performance worse than those who do. A difference was also seen for sedentary lifestyles of children who watched television or played video games for more than 1–2 h on a weekday, who reported more impairment in 'endurance' ($P < 0.001$), 'body perception' ($P < 0.024$) and 'total physical performance ($P < 0.004$) than children who spent less time in front of the TV/computer (see Fig. 3).

Boys doing sport ≥ 3 times per week (M = 93.43, SD = 7.9) had significantly better 'co-ordination' ($P < 0.009$) than children doing less frequent sport

Table 5. Physical performance in 67 children doing sport and those not doing sport (HEP-Test-Q).

HEP-TEST-Q	Children not doing sport ($n = 5$) Mean (SD)	Children doing sport ($n = 62$) Mean (SD)	*P*-value
Mobility	50.00 (22.1)	77.40 (23.1)	0.013
Endurance	41.07 (23.7)	76.37 (16.2)	0.000
Co-ordination	52.63 (9.3)	88.34 (13.4)	0.000
Body perception	56.00 (16.4)	86.80 (18.6)	0.001
Total	48.58 (7.1)	82.52 (13.7)	0.000

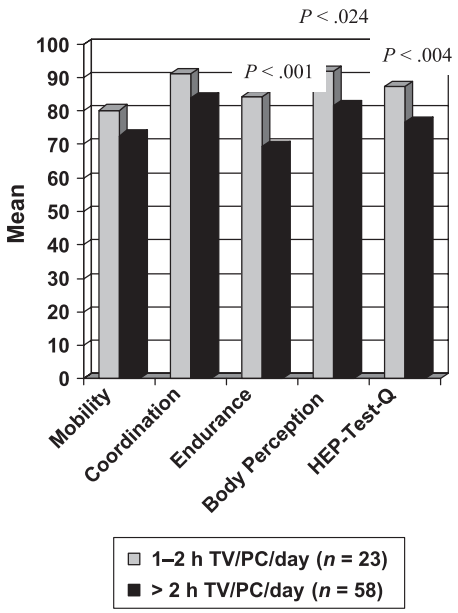

Fig. 3. Physical performance (HEP-Test-Q) based on hours of TV/computer games.

(M = 85.55, SD = 15.0). Those boys who reported doing ≥ 5 h sport per week (M = 81.52, SD = 13.0) reported significantly better 'endurance' ($P < 0.008$) than children practicing less sport (M = 70.69, SD = 17.6).

Discussion

Almost all boys in this study did sport, of which 80% played sport at school and 40% were part of a sports club. It is recognized that this level of sport in young boys will encourage sporting activity in adulthood where additional benefits such as improved personal physical health and individuals being able to play with their own children/grandchildren are cited as reasons to continue sport [15].

The impact of sport on children's health status and well-being was based on; doing sport, sedentary life style, frequency of doing sport and hours of sport. The only impact of sport on the health status was found for the number of days lost from school, where

boys with a more sedentary lifestyle had significantly more days lost from school. Differences in HRQoL were found in some domains of the Haemo-QoL ('feeling', 'family', 'view') for doing sport and frequency of doing sport per week. By contrast, significant differences concerning the subjective physical performance were found in all domains of the HEP-Test-Q between boys doing vs. boys not doing sport; significant differences in their physical performance were found for other aspects such as sedentary life style, frequency of doing sport and hours of sport.

The boys in this study are treated at haemophilia centres where sporting activity is encouraged, where prophylaxis is targeted around sport and where physiotherapy reviews are part of routine haemophilia care. This may have biased the results that we report. Nevertheless, it is encouraging to see a cohort of children who perceive sport to be a part of normal daily life, similar to their non-haemophilic peers, and not something to be scared of because of the risk of trauma induced bleeding.

Initially, we had planned to enrol 400 patients in this study (100 per centre), but this was not possible due to the lack of interest of patients in participating in this observational study and completing the very complex questionnaire. Therefore, our study investigated only a small sample size of children with mild, moderate and severe haemophilia where milder disease is less likely to limit sporting activity. Another limitation is that the data are only from two children's comprehensive care centres and cannot be attributed to all children in the UK/Europe.

According to international recommendations children should participate in moderate-to-vigorous physical activity (MVPA) at least 60 min daily [16,17]. In our study children were asked for how long they were doing sport every time they practised a respective sport; answer categories were '1 h', '2 h' or 'more than 2 h'. To calculate the average time they were doing sport in total we added the hours for each sport they were practicing resulting in 4.9 h/week in average (range 1–13 h). This is higher than might be expected; in the international HBSC REPORT [18] in the UK only 18% of boys aged 15 years (23% of boys aged 13 years, 27% of boys aged 11 years) were doing at least 1 h of MVPA daily [19]. This is probably due to the fact that we did not ask in an open-ended question the exact time for each sport. Even though this is a methodological error related to the answer categories provided in the questionnaire (we assume that children doing a specific sport less than 1 h ticked the answer category '1 h', which results in a higher total amount of hours doing sport), this is a systematic error for all participants which did not lead to incorrect results, but this result should not be compared with other surveys, where an exact time of MVPA is assessed.

Health-related quality of life in children with haemophilia is not only influenced by disease severity and treatment but also by the impact that these have on the ability to participate in routine childhood activities in day-to-day life. For boys, perhaps more than girls, there is a social pressure to conform to peers by participating in sporting activity. This has been shown to enhance masculine identity and is increasingly important in adolescence [20]. Past studies have demonstrated how not being able to play sport or be part of a team, has had a negative impact on masculinity in men with haemophilia [21]. These men described the wish to participate in sport vs. the risk of joint damage as a 'mental battle that you have to overcome as a child' and that this continues into adulthood when they can not do sport with their own children [22].

Young British children have been shown to find exercise enjoyable when it is not only competitive but something that encourages experimentation with different activities that are beneficial to them [15]. Parents play an important role in enabling their children to play sport safely [23] this may be part of an overall health and fitness campaign. Recent British Government recommendations around weight and activity state that 70% of the population should do 30 minutes moderate exercise, five times per week [24]. Only 37% of men in the UK were achieving this benchmark in 2006; one reason for this is negative participation experience at school [20]. We have shown that many of the boys in our study already exceed this target.

In this study 95.17% of boys thought that doing sport was good and 90.5% participated in leisure sports, mainly in football, jogging, swimming, gymnastics and cycling; this is comparable to a German study, where 44 children with any form of haemophilia (aged 4–16 years) were investigated. In the German cohort 88.6% of the adolescents actually performed one or more leisure sports, mainly swimming, tennis and football and 79% considered exercise in daily routine to be important or very important [25].

In the current study no differences were found in clinical data for children doing sport vs. those not doing sport. This is in line with other studies conducted in the US with boys with severe haemophilia, where level of athletic participation was not a significant prognostic factor for joint haemorrhage [26], or in the Netherlands, where no relationship between exposure to risk during sports participation and physical outcome measures was found [27], or in Israel, where level of physical activity in 44 young patients (aged 12–25 years) with severe haemophilia, assessed by the Godin and Shephard questionnaire, did not show a difference in bleeding frequency and pain [28].

Only few data on the impact of sport on HRQoL in children with haemophilia are available [29,30]. In the Australian cohort of haemophilic boys, no correlation was found between quality of life and fitness. In

general their HRQoL was high (measured via the Haemo-QoL) and comparable to that of haemophilic boys from Europe [29]. In a Dutch study [27] no differences for risk exposure factor and HRQoL measured with the Haemo-QoL Index [31] were found.

In our study we found a higher prevalence of heavy and overweight boys (28.4%) compared with a Dutch study of 158 haemophilic boys, where only 16% were classified overweight [32]. In the Dutch cohort a negative association of being overweight and HRQoL evaluated via the Haemo-QoL Index was found. This was not corroborated in the present UK study nor in another Dutch study involving 13 severe haemophilic boys, which demonstrated that there was no significant relationship between the child report of HRQoL, as assessed via the long-form of the Haemo-QoL and physical fitness, as evaluated via both absolute and relative peak oxygen uptake [30].

Haemophilia treaters now recognize that sport is beneficial both physically and psychologically for children with haemophilia [33]. There are currently no internationally recognized sport recommendations for boys with haemophilia, only on national levels [34] and individual children participate in many sports depending on their own interests and abilities. Indeed encouraging participation in different types of physical activity and sport encourages boys to play sport that they are able to do well, as well as those that they enjoy [35].

Better HRQoL is demonstrated in those doing sport more than three times per week than those doing twice a week or less. Therefore, encouraging boys with haemophilia to participate in sport will have a direct impact on their overall HRQoL. Sporting activity should be recorded as part of the haemophilia clinical review and combined into HRQoL assessment. Decisions about the most suitable sport for individual children should be made by haemophilia clinicians, physiotherapists, patients and parents [36,37], including orthopaedic examination, fitness check and motion analysis [38,39]. Treatment regimens should be individualized to support sporting activity [9] whilst minimizing the risk of long-term joint damage [5].

Even though it is widely recognized that physical activity is important for boys with haemophilia as it promotes healthier joints and reduces the risk of bleeding, the selection of an appropriate sport that minimizes the risk of injury and matches the patient's skill and needs is important [38].

Conclusion

Boys participating in sport had a significant better physical performance and HRQoL than boys not doing sport. Sedentary life styles had a negative impact on the subjective physical performance and number of school days lost by children. Therefore encouraging boys with haemophilia to participate in sport will have a direct impact on their overall HRQoL. Sporting activity should be recorded as part of the haemophilia clinical review and combined into HRQoL assessment. Participation in sport did not increase the risk of bleeding or developing target joints. Interventional studies are needed assessing the impact of sports on boys with haemophilia.

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Author contributions

KK and SvM designed the study and wrote the paper. KK coordinated the centres in the UK. KK and AL recruited patients at their HCTC. SvM analysed the data. All authors contributed to the paper and its revision.

Disclosures

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ORIGINAL ARTICLE *Adolescence*

Social networking for adolescents with severe haemophilia

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Summary. Access to modern treatments allows adolescents with haemophilia to manage their haemophilia at home, with improved treatment outcomes and quality of life, but has reduced peer support and the potential for experiential learning from older peers. Social networking, aided by modern communication technologies, may offer health benefits through peer support. We sought to assess whether or not disease-specific social networking could benefit adolescents with severe haemophilia. A total of 150 adolescents (aged 10–18) with severe haemophilia A or B from 11 UK treatment centres or those who had attended focus groups to explore the potential for a social network designed specifically for their use were surveyed. Teenage boys with severe haemophilia in the UK who responded to an online and paper questionnaire (n = 47; 31% response rate) rarely knew of or socialized with others with haemophilia outside their families. Two-thirds of respondents said they would like to meet others. For 70% of boys, parents were the major source of information about haemophilia, yet more than half said they often had trouble finding answers to their questions. These boys frequently used online social networks to chat with friends. Adolescents with severe haemophilia frequently have limited contact with others and many wish to have greater contact. They may benefit from peer support and experiential learning gained through online social networking. The SixVibe restricted access social network is to be launched in 2011. It includes features designed to promote and facilitate the development of peer-to peer disease management skills for adolescents with severe haemophilia.

Keywords: adolescent, haemophilia, self-management, social networking, survey, transition

Introduction

Modern treatments have had an enormous impact on the management of haemophilia, improving both the length and quality of life and reducing long-term disability. For most children born recently with haemophilia, the availability of home therapy has reduced the amount of time spent in hospital compared with those of earlier generations. Whereas this is clearly a benefit, it means that children with severe haemophilia rarely meet. This lack of peer support means that they may grow up feeling isolated both socially and geographically.

As haemophilia is a rare disease, affected adolescents already face relative geographical isolation. The Pan Thames Haemophilia Consortium is the largest purchaser of haemophilia services in the United Kingdom, comprising 10 acute trusts and 48 primary care trusts in London and South-East England. The consortium treated over 1300 patients in 2008–09, of whom almost 500 were children under the age of 18. In its 2009 re-audit of the implementation and monitoring of the clinical guideline for prophylaxis in children with haemophilia A/B, the 10 Haemophilia Centres treated 233 boys with moderate or severe haemophilia A or B with no current inhibitor. These boys lived across a wide geographical area of London and South-East England.

The extent of the social isolation associated with haemophilia is more difficult to quantify. But patients without older affected relatives grow up lacking the potential for experiential learning from older family members. Those who rarely see the consequences of poor adherence with treatment may ultimately find it more difficult to follow health care advice and take prophylaxis.

Second generation web application ('Web 2.0') tools allow individuals to join virtual communities, generate content, comment on and evaluate other users' actions. Such tools include blogs, wikis and online social

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networks such as Facebook, which allow individuals to create user profiles, upload information and establish connections with others [1]. Facebook was launched in 2004 and as of April 2011 claims to have more than 500 million active users, making it the most popular social networking site worldwide [2]. Social networking offers a powerful tool for promoting health care, giving individuals the ability to share information and learn from the experiences of others [3,4]. Indeed, social networks may have the greatest utility to persons with rare diseases who feel isolated as a result of their conditions [5].

We sought to explore, by means of focus groups and an online and paper-based questionnaire, the potential for a social network designed specifically for use by adolescents with severe haemophilia, and to determine which features such a site would need to incorporate.

Methods

Study design

The social networking habits and preferences of adolescents with severe haemophilia were initially discussed in a focus group of seven boys (aged 9–17) from two London centres, funded by the Millennium Commission. This consisted of informal moderated discussion to explore how the boys balanced sport with treatment, reconciled having haemophilia with being a teenager, and how they used online media and information. Focus group discussion was transcribed and interpretive analysis applied to identify recurring themes. This work was subsequently discussed and evaluated by an advisory board consisting of haemophilia health care professionals from a further six centres around the UK. A larger and a more in-depth questionnaire was developed, both on paper and online using the web-based application (http://www.surveymonkey.com), which ensures anonymous data collection. The survey was distributed by haemophilia specialist nurses in 11 centres in the UK to 150 adolescents aged 11–18 years with severe haemophilia A or B. Surveys were returned in prepaid envelopes to the researchers. The survey asked questions about haemophilia knowledge, self-management skills and social network use. Full ethical committee approval was not required as the survey was deemed to be social market research by the chair of the local ethics committee. The results of this work were subsequently tested on six 15–17-year-old boys perceived by their centres as being 'expert' haemophilia patients, recruited from three London centres to help develop an expert patient programme.

Statistical analysis

For continuous variables in the questionnaire survey, mean ± SD and median values are reported as appropriate [6]. For categorical variables, the number of boys and the corresponding percentage are given (the number of respondents to a particular item was not always equal to the total number of respondents to the survey).

Results

The initial focus group work revealed that boys were keen to participate in sport and understood that their treatment allowed them to do so. Indeed, having haemophilia did not prevent them from being 'normal teenagers'. All boys regularly used social networks to chat with friends, and knew how to search online to find information. Even though all the boys attending the focus group attended the same centre, they agreed that they rarely met others with haemophilia but said that they wished to do so. All agreed that a restricted social network offered a safe way to do this.

In all, 47 completed questionnaires were returned by boys with haemophilia in 11 centres (31% response rate). Interestingly, only five questionnaires were completed online. The mean (SD) age of responders was 13.78 ± 2.2 (median 14, range 10–17) years.

With regard to their haemophilia, around half of the boys had a few concerns about who knew about their haemophilia, and only one boy said he did not tell anyone about it. Most boys rarely knew of or socialized with others with haemophilia outside their families, and generally only saw these boys when they attended clinic. Yet three-quarters (72%) of respondents said that they would like to meet others of their own age with haemophilia (Table 1).

Seventy-six per cent of boys stated that their parents were the major providers of information about haemophilia (this may be outdated information gathered at diagnosis), yet more than two-thirds (68%) said that they often, always or sometimes had trouble finding answers to their questions about haemophilia (Table 2).

These boys appeared to be somewhat blasé about their haemophilia treatment (Fig. 1), yet around one-third acknowledged that they sometimes required help with managing bleeds. Bleeds, both in terms of pain and the limitations they impose, represented the worst aspects of living with haemophilia (Fig. 2), although around one-fifth of the boys indicated that it made them feel 'different' or 'alone', suggesting that haemophilia results in some degree of social isolation.

These boys were frequent users of social networks (81%), mainly Facebook (76%), using them for a variety of reasons but mainly chatting with friends (Fig. 3). Most boys (94%) accessed social networks by a home computer or laptop. Interestingly, 63% of respondents believed that social networks for people with haemophilia should be open to those of all ages; only a minority (13%) of respondents wished it to be restricted to teenagers (Fig. 4).

Table 1. Contact with other people with haemophilia.

Question	Answer	Response	
		Count	Percent
Do your friends know you have haemophilia?	I don't tell anyone	1	2.1
	I tell only my closest friends	12	25.5
	I tell people only if it comes up	13	27.7
	I don't care who knows	21	44.7
Do you meet other people with haemophilia?	Only when I attend the Haemophilia Centre	19	40.4
	Only members of my family	13	27.7
	I know some people with haemophilia but we don't really socialize	5	10.6
	Occasionally I socialize with other people with haemophilia	10	21.3
	I am in regular contact with other people with haemophilia	2	4.3
How many people outside your family do you know who are with haemophilia?	0	23	48.9
	1–2	10	21.3
	3–5	8	17.0
	6+	6	12.8
Would you like to meet more people with haemophilia?	Yes	34	72.3
	No	13	27.7

Table 2. Sources of information about haemophilia and use of social networking sites.

Question	Answer	Response	
		Count	Percent
Do you use any of the following sources to get information or advice about haemophilia?	Hospital leaflets	13	27.6
	Parents	36	76.5
	Other family members	8	17.0
	Haemophilia Society website	6	12.7
	Wikipedia	6	12.7
	Books	4	8.5
	Other	3	6.3
Do you ever feel that you cannot find answers to all of your questions about haemophilia?	Never	15	31.9
	Sometimes	29	61.7
	Often	2	4.3
	Always	1	2.1
Do you use any social networking sites (such as Facebook or Habbo)?	Yes	38	80.9
	No	9	19.1
How do you access social networks?	Home computer/laptop	44	93.6
	School computer	4	8.5
	Mobile phone	5	10.6
	Smart phone (iPhone, Blackberry)	5	10.6
	WiFi device (iTouch, iPad)	11	23.4

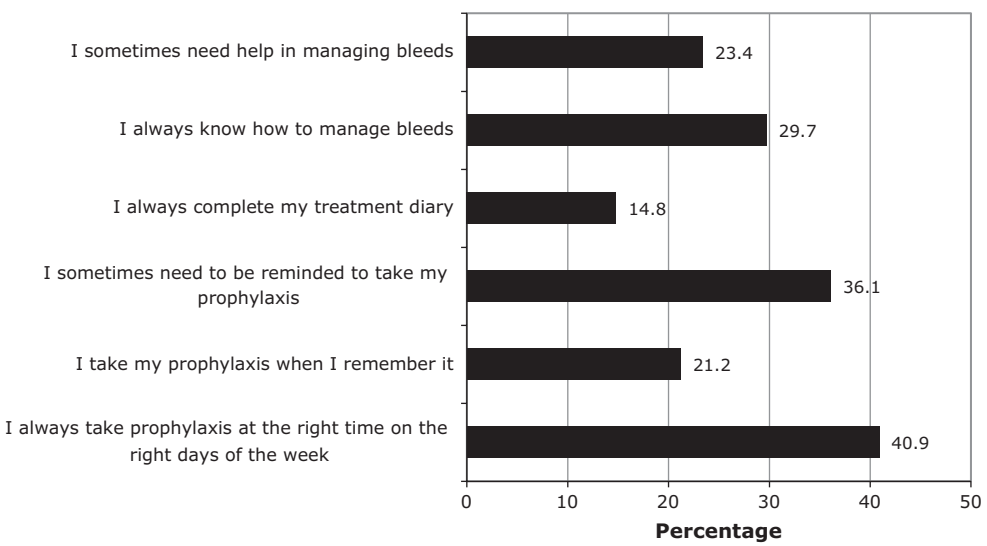

Fig. 1. Which of the following statements are true for you?

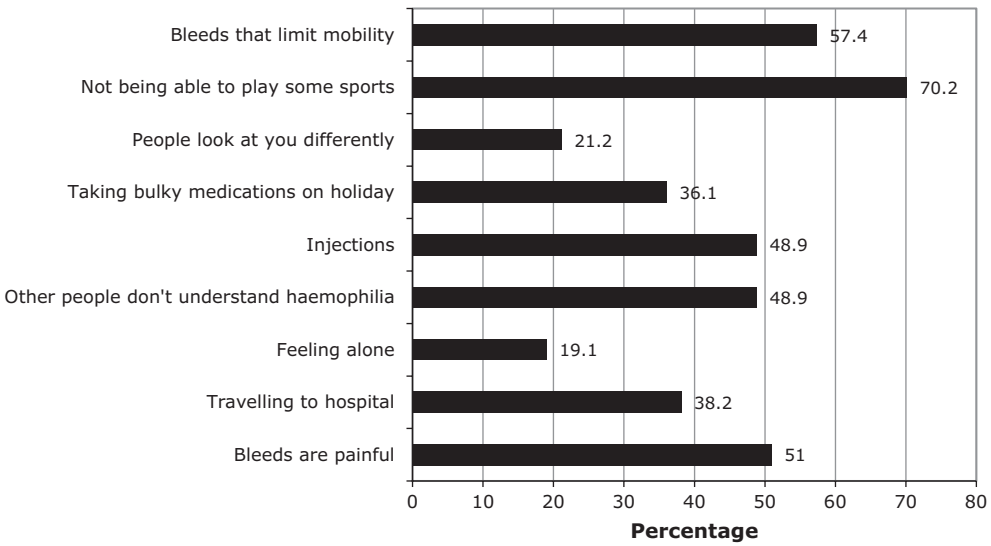

Fig. 2. Which, for you, are the worst aspects of living with haemophilia?

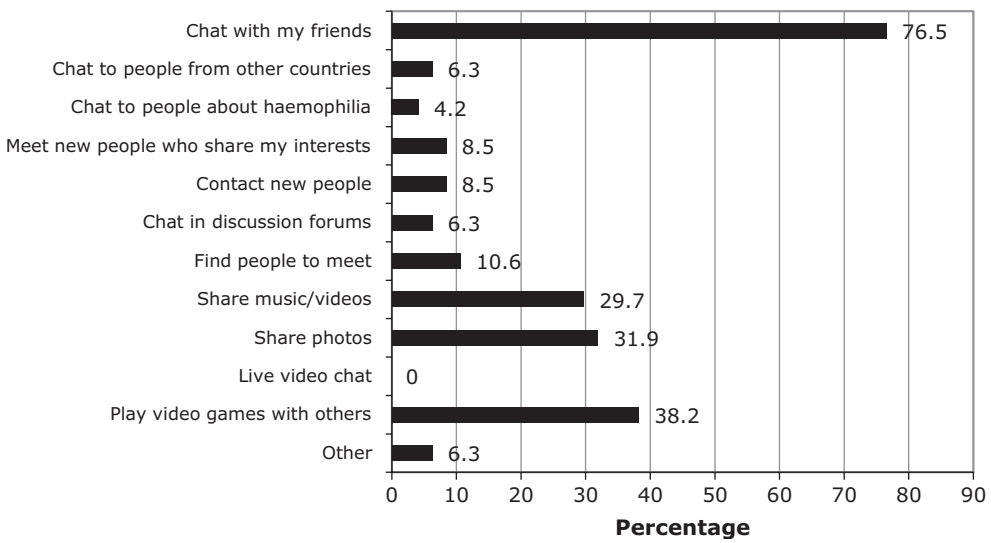

Fig. 3. What do you use social networks for?

All respondents used the telephone to contact their haemophilia nurse when they were outside the hospital. When asked how they would like to be able to contact their haemophilia nurse when outside the hospital, the boys saw potential for use of emails, text messages and even social networking (Fig. 5). In fact, 62% of respondents said they would happily use their mobile phones to send their nurse a picture of their bleed when seeking medical advice (data not shown).

Discusssion

It has long been known that epidemics such as obesity, alcoholism, depression and even suicide spread in a peer-to-peer fashion [7,8]. In the same way, health care interventions have both direct outcomes for individual patients and collateral effects on their social contacts. People generally learn most effectively from those they admire or value, this is why patient advocacy groups and public health doctors frequently engage celebrities to promote public participation in preventive care campaigns [9–11].

Living with a chronic disease can be a source of stress and anxiety, particularly for children, requiring affected individuals to acquire considerable knowledge and management skills [12]. Those who understand their disease and its treatment are less anxious and better able to manage their health both physically and emotionally [13]. In addition to the health expertise provided by

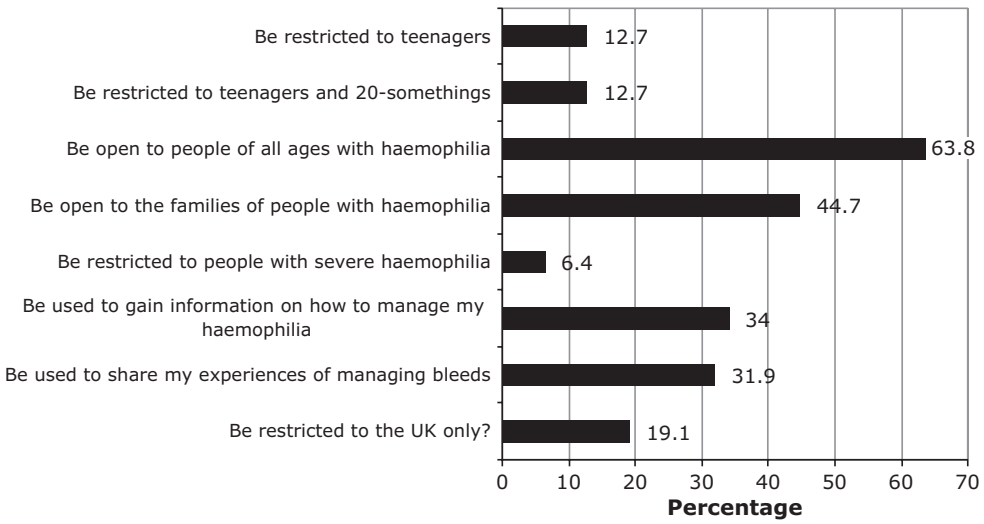

Fig. 4. If there were a social network that ONLY people with haemophilia could join, should it exist?

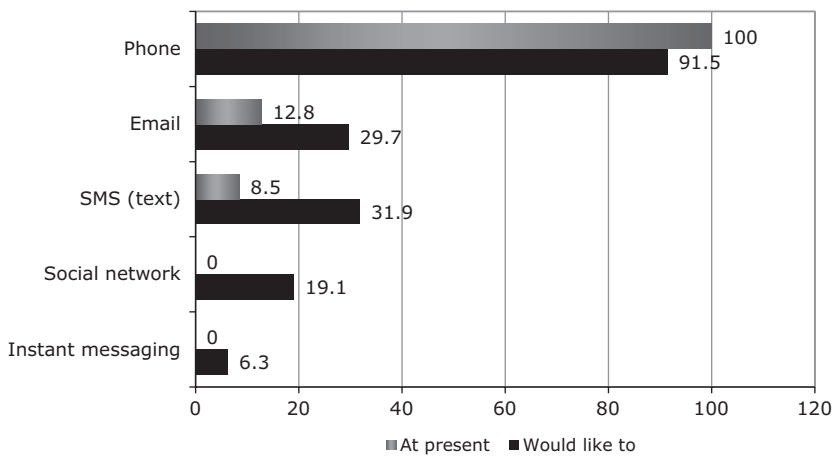

Fig. 5. How do you contact your haemophilia nurse at present, and how would you like to be able to contact him/her?

formal sources, those living with chronic illness will gain a wealth of practical advice from friends, relatives and other patients as well as their own physical and emotional experience of managing similar health situations. This experiential knowledge – or patient expertise – may be lacking in adolescents with severe haemophilia who rarely have contact with others with haemophilia besides their own family members.

It is well known that some boys with haemophilia manage their condition better than others. Our early focus group research indicated a strong willingness on the part of boys with haemophilia to communicate experiences of living with and managing haemophilia with others. This was confirmed in the questionnaire survey. Furthermore, the focus group work also suggested that:

1. Adolescents are more likely to follow advice and information if it comes from other boys of a similar age with the same condition than from health care professionals.
2. Boys are more likely to discuss sensitive topics (such as those related to sexual health, sexual preferences, drugs and lifestyle) with each other than with health care professionals.

Disease-specific social networking has the potential to empower patients and families, which could in turn enhance compliance with treatment and improve quality of life. In addition, a haemophilia-specific social network may help facilitate the spread of information about managing haemophilia from well funded expert treatment centres to those that face greater socioeconomic and cultural challenges in accessing medical information.

Adolescents with chronic conditions such as haemophilia engage in experimental behaviours and place themselves in risky situations as often as or more often

than their peers. It has been suggested that engaging in risky activities, such as not wearing helmets, abusing substances and engaging in unprotected sex, is linked to an unconscious desire to "be alike" [14,15]. Our survey suggests that teenagers attending specialist centres are open with others about their haemophilia. This is confirmed by anecdotal comments made in the expert focus group, where boys indicated that they were unafraid to discuss haemophilia with others, and in fact often welcomed the opportunity to correct people's misperceptions surrounding the disease.

Nearly three-quarters of the boys in the national survey indicated a desire to meet more boys with haemophilia. This desire was echoed by the confident and knowledgeable older boys who attended the expert patient focus group, who said that they would welcome the opportunity to meet and to learn from older boys who had already left home or gone to university.

To date, little has been published on the health benefits of social networking. In a pilot study conducted in a group of children with type 1 diabetes, active message boards and chat rooms were found to enhance mediation of third-party peer-to-peer information [16]. The need for adolescent-centred electronic resources to address educational needs prior to transition for patients with haemophilia has been recently recognized by Breakey *et al.*, who voiced the need for interactive websites with features identified by young people as being desirable, such as games, animations, messaging and chat features [17]. Our survey showed that these are the functions that teenagers with haemophilia access regularly on sites such as Facebook, whereas our group of 'expert' patients confirmed the need for such features on haemophilia-specific internet sites.

Online support groups offer many benefits – they are easy to access, and offer no difficulties to users with mobility, speech or hearing impairment. They are not subject to socio-demographic (age, gender, race or culture) or geographical barriers, while the potential for anonymity promotes both honesty and intimacy. They also offer the potential to reach groups that are traditionally difficult to reach through traditional health education approaches, such as men. However, online social networking has some potential drawbacks: the socially disadvantaged may have limited access, while those with literacy problems may feel excluded. Furthermore, the potential for 'group think' can be misleading (in some cases even dangerous), while the lack of transparency means that networks can be subject to misuse by commercial organizations, particularly where networks encourage patients to share data.

Nevertheless, it is undeniable that social networks and other forms of electronic communication are widely used by adolescents. Haemophilia health care professionals put a great deal of effort into educating adolescents and promoting transition arrangements to ensure continuity of care. It is important that health care professionals reach out to and communicate with patients in ways that they understand and that are relevant to their lives, particularly in late adolescence when adherence with health care advice is known to decline [18,19].

Conclusion

Our study suggests that adolescents with severe haemophilia frequently have limited contact with their peers, and so lack peer support and opportunities for experiential learning. Most boys rarely knew of or socialized with others with haemophilia outside their families, generally only seeing these boys when attending the haemophilia centre. Two-thirds of respondents to our survey indicated that they would like to meet other boys with haemophilia. Such boys may obtain peer support and experiential learning through online social networking. The SixVibe social network site (http://www.sixvibe.com) offers such benefits. Access is restricted to adolescents with haemophilia and other bleeding disorders.

In addition to educational content developed by professional writers in association with health care professionals, the website includes games and other features to promote and facilitate the development of peer-to-peer transmission of disease management skills. This includes a modular training scheme devised with, by and for adolescents with severe haemophilia through which site users are encouraged to become 'experts' in understanding and managing haemophilia. Those who complete the programme and who 'win' sufficient endorsement from other site users for advice and comments made in discussion forums may qualify as expert patients, effectively becoming advisers for younger patients using the site.

Innovative web-based interventions such as the SixVibe website and its associated expert patient programme offer the potential to promote peer-to-peer transmission of disease management skills. Further research and formal evaluation of such approaches is clearly warranted to determine the extent to which this actually happens in practice. It will be interesting to see how boys with haemophilia use the site, particularly as it is opened up to those in other countries, and those with other bleeding disorders. Such research may focus on the extent to which users define themselves by their disease, and at the potential for online interaction between individuals with different bleeding disorders.

Disclosures

The authors stated that they had no interests which might be perceived as posing a conflict or bias.

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Self-management and skills acquisition in boys with haemophilia

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Abstract

Background There is an increasing prevalence of children/young people with long-term conditions (LTC) in the UK due to improvements in health-care management and delivery. These children are often involved, from an early age, in their own care and management; yet, there are little data to support how or when they develop the necessary skills and knowledge to become competent at this care.

Objective This study aimed to understand self-management of haemophilia, from a child's perspective, in the 21st century in the UK where intensive prophylactic therapy is given from early childhood.

Design A qualitative study using grounded theory to evaluate life-experiences of children and young people with haemophilia.

Setting and participants Thirty boys aged 4–16 with severe haemophilia treated at a single paediatric haemophilia care centre were interviewed at home or in a focus group.

Intervention/variables Multimethod qualitative research including age-appropriate research tools (draw and write, photo-elicitation and interviews) to facilitate data collection from children.

Results Boys develop self-management skills over time. They learn from health-care professionals, their parents and other family members with haemophilia.

Discussion Self-management skills (bleed recognition, self-infusion, self and medicines management, pain and risk management and conceptualizing preventative therapy) are developed through experiential learning and individualized education, and not through formalized expert patient programmes.

Conclusion The boys in this study have benefited from early prophylactic factor replacement therapy. They develop skills in haemophilia and self-management at a relatively young age and are experts in their own haemophilia care.

Introduction

Changing patterns of disease include an increase in the prevalence of long-term conditions (LTCs); Dowrick *et al.*[1] consider that part of the response to this increase should be the growth of patient empowerment through self-management. Jordan *et al.*[2] describe self-management as a 'patient's health literacy': their capacity to 'seek, understand and utilize health information to participate in decisions about health'. They cite five components to management: access (to information), knowledge, health education and empowerment, self-management and command (of the health-care system including confidence to use their initiative). Self-management in LTCs refers to 'daily activities that individuals undertake to keep illness under control, minimize its impact on physical health and psychological sequelae of the illness'.[3] Pritchard-Kennedy[4] argues that children's role as partners in care has often been confounded by parental dominance; thus, the 'self' in self-management in children is a combination of children and adult carers.[5]

This paper explores how boys with haemophilia develop expertise in self-management, becoming expert patients over time, through education and experience. Haemophilia reviews occur every 3–6 months during which the boys are encouraged to self-infuse and are asked questions about activity, treatment and genetics. This reflects Kirk *et al.*'s.[5] four-stage model of 'community, independence and confidence, knowledge and skills and engaging children/young people' in self-care. The boys in this study are the first generation to grow up with intense preventative treatment, (rather than treatment only after a bleed had occurred) recommended in the United Kingdom[6] with genetically engineered therapy that is known to be safe from bloodborne viruses,[7] as this has been shown to minimize disability and improve quality of life (QoL).[8]

Children are treated on home therapy programmes from early childhood,[9] We know little of how this experience impacts upon them, other than that this results in a lifestyle that is near to that of a child without haemophilia, where QoL scores, when assessed using haemophilia- and age-specific QoL assessment tools, are high.[10] This contrasts with prior research undertaken in the 1980's when many boys co-infected with bloodborne viruses, reported severe impact on life-experience.[11–13]

The study

The aim of the wider study, a multimethod study using age-appropriate research methods[14] from which this paper is derived, was to understand what living with haemophilia is like for boys in the 21st century. The study was undertaken between March and September 2009 at a paediatric haemophilia comprehensive care centre in the UK; ethical approval was granted by a local National Health Service Research Ethics Committee. Parental consent and patient assent were obtained from all participants at study entry. Forty-six boys aged 4–16 years were invited to participate; 16 refused for a variety of reasons including not being interested in the study or not wanting to talk about having haemophilia.

The methodology included photo-elicitation for boys aged 4–7,[15] draw and write techniques for 8- to 12-year-olds,[16] focus groups for those aged 13–16[17] and individual interviews for those teenagers who were unable to attend the focus groups. The younger cohorts of boys were given digital cameras, or 'art packs' 2 weeks before having individual interviews in their own homes, and were asked to either take photographs of their normal daily activities or to draw a picture or write a story about being themselves. These were used as icebreakers and/or aide memoire to enhance discussion about their lives in the preceding 2 weeks during the interviews, which lasted between 20 and 60 min and were semi-structured, asking questions about the effects of haemophilia on their lives, their friends, sporting activities, school, their family and things they like and do not like. They were tape-recorded, allowing the researcher to give full attention to the interviewee, what he was saying or doing at the time.

Open-ended prompt questions, such as 'can you tell me what it is like to (experience something?)' and 'other boys have said (something) has that ever happened to you?', were used to elicit their views of living with haemophilia. Further details of the methods used in this study are reported.[18]

The study was conducted using grounded theory, where each interview was analysed as it occurred; this enabled modifications and additions to the semi-structured questionnaires and continued until data saturation within an emerging framework occurred.[19] The recordings from the interviews and the focus group were professionally transcribed verbatim and listened to repeatedly by the lead researcher before analysis and manual coding. The content of the narratives could be grouped and coded into seven recurring themes, which were recognized by the research team as important aspects of modern day life with haemophilia. Six of these themes have been published elsewhere[20,21]; the seventh, development of self-management skills, is discussed here.

Results

Thirty boys with severe haemophilia A (n = 27) or B (n = 3) participated in the study.

Half the boys were the first in their family to be affected by haemophilia; the other half had a previous family history of haemophilia with an affected grandfather (n = 5), uncle (n = 3), older brother (n = 4) or multiple family members (n = 3). All of the boys were treated with prophylactic factor therapy from early childhood and were able to participate fully in normal family, school and peer-led activities.

What was striking through the interview analysis was that the boys were developing self-management skills: a concrete knowledge of bleeding mechanisms, risk avoidance and treatment, as well as self-infusion skills. All children with LTCs, particularly those with congenital disease, are involved in their own health care from early childhood. The level of involvement changes as children develop the physical and psychological means to cope with self-care and eventually self-management.[22] These self-management skills are learnt from parents and health-care workers, and often for boys with haemophilia, from other affected family members; there was evidence in our study of increasing self-management skills from a very early age.

Haemophilia management includes an ability to self-monitor, to be aware of subtle bodily fluctuations, using a range of resources, such as previous knowledge and experience[21,23] and knowing when to seek help from adults or health-care professionals. In boys with haemophilia, this includes knowledge of how their genetic mutation is expressed as symptoms[23] and how these should be assessed, treated, monitored and reported.

The major impacts of haemophilia on the life of the boys in this study are described in three themes: recognition of bleeds, self-infusion, and medicine and self-management, with an emphasis on sport. In the accounts below, pseudonyms have been used to protect respondent identities.

Recognition of bleeds

Unlike many LTCs, haemophilia is 'invisible' with few signs of illness unless there is an acute bleed. Even with total adherence to prophylactic treatment regimens, bleeds can occur as a result of trauma. Bleed recognition, described by boys as a bubbly, tingling feeling inside the joint and treatment strategies (when and how to treat and what dose to give) are integral parts of haemophilia self-management. Prophylaxis, which requires frequent infusions but offers the best bleed protection, is the treatment regimen most commonly used in boys with severe haemophilia:

> It's not really a big deal, it's just like as long as you do your injections when you are meant to you just crack on with it – a normal life, it's not like there is anything wrong with you, it's just simple. Haemophilia is something that only happens when you like, get hit or something, you'll bleed a bit more than the average person then you just need a bit more factor (Oliver aged 14)

Without early recognition of bleeding, or with recurrent bleeds into the same joint, boys with haemophilia might, in response to the pain of bleeding, withdraw from regular activities and social contacts; this may affect self-image[24] Paul aged 16 describes how bleeds impact on him:

> Sometimes just when I get a bleed it hurts loads so I take codeine at least twice but only because I think it knocks me out as well and puts me in kinda a place where well …… I just daydream or fall asleep and get rid of it [the pain] with luck. I think it puts you in that better state and if it takes a bit of the pain away then I think you are in a better state of mind, more chance of repairing it [the bleed] quicker

Paul describes the physical and psychological impact of bleeding, showing resilience to the pain and the social isolation that haemophilia can cause, which is more often seen in adults with haemophilia[25] However, he also shows that he is able to make appropriate treatment decisions, demonstrating skilled practice, competence, confidence and control, integrating haemophilia treatment options including analgesia as well as factor therapy into his personal management plan.[26]

Self-infusion

Peripheral venipuncture is a skill that many health-care professionals are unable to undertake, particularly in young children. In haemophilia management, parents learn this skill to facilitate home treatment of their sons,[27] who learn to self-infuse in late childhood or early adolescence.[28] Even though this is a routine treatment for boys, becoming self-sufficient at infusion is complex, most boys can do this at least some of the time by the age of twelve. To infuse into one arm makes the use of that arm for anything other than venipuncture impossible; thus, self-infusion becomes a one-handed technique. They need to apply a tourniquet, find a vein, puncture the vein, release the tourniquet, infuse the factor, and most difficult of all remove the needle and apply pressure to the puncture site at the same time. This sometimes occurs in the face of a bleed in the infusing arm, further reducing mobility. Many boys become ambidextrous at this skill; this gives them more choice of venipuncture sites and more ability to repeatedly infuse.

> At first I felt quite scared, when I done it [sic] I thought to myself 'ok this is it one, two, three' and then I put it in and it did not really hurt that much and then I was really happy. Well I was scared of if I went wrong because it's quite scary sticking a needle into yourself. Before when it went wrong it didn't really hurt but I knew I went wrong because it started to balloon [extravasate] and so we [Tom and his mum] took it out and then I tried again in my right arm with my left hand and that went wrong as well because I am right handed and it was quite hard with my left hand (Tom aged 12).

Starting to become a self-infuser often raises anxiety around what will happen if the infusion goes wrong – in the past, a parent will have been the main infuser and will have had the necessary skills to 'fix' the infusion if a vein is missed or if extravasation occurs as described by Tom above and 10-year-old Jonathan:

> I am finding it a little bit hard, because I am frightened that if I do it wrong then I might die, because if I do it wrong maybe the vein will puff up into a lump and pop. It would hurt. Sometimes I stick the needle in, that feels weird cause one hand is actually pushing down and one hand has actually got it in, and it's like pins and needles [due to tourniquet] while I'm doing it. It just feels really weird.

Tom and Jonathan show how becoming competent at self-infusion, itself a process of skill and expertise developed through trial and error as well as education and support, enables them to start on the path to becoming a patient with expert skills for current haemophilia care. For boys with haemophilia, self-management includes an understanding of not just how, but when to treat themselves.

Medicine and self-management

Treatment with replacement therapy, both prophylactically and 'on demand' when bleeds occur, is anchored in everyday life for boys

with haemophilia. Routine, such as having treatment at the same time every day, is essential to remain concordant with treatment.[29] The ability to control symptoms and the severity of bleeding achieve some normality and control in daily life.[30] Ingadottir *et al.*[31] discuss how, in children with diabetes, self-knowledge is key for integrating treatment regimens and achieving good QoL. They describe a concept of 'body-listening' – knowing when to treat and developing self-management strategies which suit lifestyle, belief patterns and personal priorities. However, in haemophilia, 'body-listening' (taking a 'wait and see' approach) or missing treatment will result in the rapid onset of bleeds which are painful and ultimately result in joint damage[32] and increased pain as shown by 10-year-old Will:

> ankle bleeds I can always tell when they are going to hurt and when they are not …… it feels a bit stiff and then later can be oh it's just really really hurting.

Understanding how to manage treatment, including storage, reconstitution and infusion[33] how to integrate this into daily life and how to develop sustainable routines, is a key part of medicine management.[29] Motivated adolescents are more likely to demonstrate good adherence[34] as they then have less restriction placed on lifestyle choices. Oliver aged 14, a keen golfer, showed a good understanding of the rationale for a daily treatment regimen, including the concept of factor half life, where factor degenerates and does not accumulate in the body, thus requiring regular infusion:

> Because then it [the factor level] will be on one consistent level because at the moment it's like if you had a graph it would be going up down, up down, up down so if you have it every day it would be pretty straight. Because like if you are on a low, and you had an injury, you would be even more smashed up but if, because it's all on one level its like you won't get [hurt] as much.

Adolescents handle illness and related situations, by 'focusing on disease and its treatment for self protective reasons',[35, p. 283]. Hinds,[35, p. 283] shows that adolescents shift from 'wellness-in-the-foreground to illness-in-the-foreground' when they are in situations of 'threat, transition, suffering, pain or coping with physical or psychological limitations of disease or treatment'. For boys with haemophilia, life without it is unimaginable[25,36] and it is integral to who they are as individuals:

> [I] suppose it just becomes part of you doesn't it; it's what you think about. I find it's just life so I kind of just make it part of my life so just do certain things to ensure that I keep myself safe and kind of take it in your [sic] stride. (Peter aged 14)

The concept of 'this is it/normal for me' is described by Atkin & Ahmad[37] who show that adolescents with haemoglobinopathies emphasize the importance of 'just getting on with life' and 'limiting the consequences of their illness on their day-to-day lives' by using normalization strategies to maintain 'normal' lives and identities. For a majority of boys, 'normalization' includes the ability to participate in sport. Koiter *et al.*[38] state that choices around specific sporting activity, often considered 'dangerous' for boys with haemophilia, must be based on adaptation to the haemophilia diagnosis and treatment regimen. Adolescent boys are aware of potential risks that sporting activity may pose and may participate with caution. They may adjust their normal medication routine, gaining control over their haemophilia, to be able to participate in sport, further demonstrating medicine management skills[39] as well as risk-taking (or avoiding) strategies.

Christensen & Mikkelsen[40] suggest that children strategize risk management in everyday life. They judge and balance the chances and risks they encounter, learning to control and steer through these, distinguishing between negative risk-taking and positive risk management. Risk-taking and pushing at boundaries is an activity especially valued amongst boys, who generally engage in more physical activity, such as contact sports, where injury is an accepted risk of participation, as they 'prove themselves'.[41] For boys with haemophilia, the choice of sporting activity may be affected by the diagnosis[39] and the treatment as well as by child/family expectations.

Contact sports are undertaken with extreme caution[32]; many younger boys participate but choose to adapt their activity as they (and their opponents) become bigger. Mark (aged 16) described how he chose to manage the risk of sporting participation by adapting his sporting choice to one with less contact:

> I used to do football and golf, but not now – no sport like that for me now or I'll be in bed for a week with a bad back – so just swimming or cycling.

Mark treats himself with prophylaxis punctiliously but has decided that this positive risk management is still outweighed by the negative risk-taking of participating in contact sport now that he and his opponents are bigger. Haemophilia demands adjustments to avoid risk, lifestyle disruption and uncertainty associated with trauma-related bleeding.[30] For the majority of boys, the risk is manageable and causes no loss or biographical disruption to themselves. However, global limitations in haemophilia care can cause frustration; George, a 16-year-old polo player, expresses feelings of biographical disruption and loss[42] because of haemophilia:

> It [haemophilia] doesn't really stop me from doing anything except living in a load of other countries or spending a long time like a gap [year] in Argentina, which is a bit annoying.

The risks for George of a gap year as a gaucho, so far from home, are deemed to outweigh the benefits, something with which he has, with reluctance, come to terms.

Discussion

Like other adolescents with LTCs, boys with haemophilia are responsible for their own health, making daily changes in lifestyle and treatment choices, although their mothers act as 'alert assistants'.[43] These include healthy eating, participation in exercise, getting enough sleep and making other health choices such as not smoking. Taylor *et al.*[44] argues that children and young people with LTCs strive for 'normalcy'; the focus of their care should therefore be on making and taking healthy choices which result in wellness rather than illness. In haemophilia, wellness is achieved through individualized treatment, self-care and an understanding of medicine management which reduces bleed frequency.

Unlike many other LTCs, haemophilia, if untreated, leads to a rapid onset of bleeding which requires the same treatment. This rapid feedback of poor self-management resulting in rapid symptoms is unlike most other LTCs where missing or stopping treatment rarely results in visible changes to wellness. Usually in boys with haemophilia, wellness is in the foreground of their daily experience. Paterson[45] shows that illness moves to the foreground of people's worlds when they have an acute crisis, which forces them to attend to the illness. In haemophilia, the event would be a joint bleed, with its associated decreased range of movement, pain and the need for further factor therapy. The paradox of this model is that to keep wellness in the foreground, disease management must also be foremost; 'the illness requires attention in order not to pay attention to it',[45, p. 24], and the body becomes something to which things are done, in this instance by regular self-infusions. The routine of this treatment becomes immersed in the everyday normality of haemophilia care such that haemophilia *per se* can be forgotten.[29] There is therefore little impact on day-to-day activity as long as treatment is adhered to.[21]

The core category of this study is how young people with haemophilia have developed self-management skills. Young people with haemophilia are an 'untapped resource of expertise, that often know more about their particular condition and its management than their doctors, and this knowledge may assist treatment',[36, p32]. However, adolescents with LTCs need to demonstrate more 'refined social skills than their healthy peers, since they have to be able to accommodate treatment requirements and social demands',[40, p. 26] into their everyday lives. Daily life needs to be planned to reduce the risk of bleeding, so that haemophilia does not come to the foreground, removing wellness from its prime position. This may encompass

adapting the medical treatment regimen to fit within other priorities of daily life, such as school and sport so that daily routines are contextualized within both illness sequelae and personal experience that is reflective of (and sometimes conflicting with) lived events, experiences and perspectives.[22]

An expert patient is one who has the necessary skills to be a competent individual. These include knowledge, clinical judgement, self-efficacy, physical skills, persistence, paying attention to tasks and integrating these into daily management.[26] The boys in this study identify with others on the same 'illness trajectory'[23] where they recognize the consequences of haemophilia in other family members. Mark tells how his older brother who also has haemophilia '*keeps an eye out for me too'* by recognizing subtle signs of bleeding which their non-haemophilic brother would not necessarily notice:

> we kind of ….you know we have this kinda relation thing where …….. you know you walk along and you are hobbling a bit perhaps and he [his brother with haemophilia] goes 'oh you've got a bad ankle'. He would always notice.. and tell me to get a needle [factor infusion]

These boys have access to treatment which their older haemophilic family members did not have and are able to tailor their own treatment around sporting or other risk activities. All children make mistakes in their judgements; when they do this, they often find solutions to the mistake in their own ways.[40] This is demonstrated by 16-year-old Mark who had adapted his sporting activities to those which involve less contact as he recognizes that contact sports now have more risk than benefit for him as an individual. The independence gained by young people with LTCs as they grow older allows them more autonomy over their treatment, making it easier for them to appear 'normal' to others.[36] For Mark being able to infuse before cycle races means that several people in his cycling club are unaware that he has haemophilia (data not shown); thus, he is not defined by haemophilia but as a fellow cyclist.

Strengths and limitations

The strengths of this study include the use of age-appropriate research methods and the provision of insights into children's views of living with haemophilia where prophylaxis enables near normal lifestyles. The study is limited as it was undertaken in a single paediatric haemophilia comprehensive care centre in the UK; thus, the results of this study cannot be extrapolated to children and young people with haemophilia in other centres or those treated with different therapeutic regimens. Further work is necessary to confirm these findings with others who self-manage haemophilia through home treatment programmes. However, this study confirms the need to further discover the experiences and impact of haemophilia on children and young people, to continue to shape how we support them in developing self-management strategies.

Conclusion

The boys in this study are amongst the first generation in the UK to be offered safe and effective treatment, enabling them to lead more 'normal' lives than any person with haemophilia before them. They received prophylactic therapy from an early age and consequently do not have joint damage which has historically, limited mobility and sporting activity. Thus, they have grown up describing themselves as they see their peers, as normal young men who participate fully in day-to-day life. However, if they fail to treat themselves, they develop bleeds which are painful and debilitating, reminding them that they have haemophilia.

Haemophilia knowledge and education begins in early childhood, with basic recognition of bleeding and infusion skills being taught to young boys by health-care professionals. Other education and learning occurs on an 'ad-hoc', individualized basis with most boys learning through personal experience, this is described by Kirk *et al*.[5] as 'individualized self-care projects' which 'maintain involvement'. Some boys have other, usually older,

affected family members, from whom knowledge and skills can also be gained. All boys with haemophilia are experts, and as Petersen[30] states, they are probably the best haemophilia experts that there are.

There are no haemophilia-specific training courses for children or adolescents with haemophilia, and many boys have declined the opportunity to participate in programmes aimed specifically at adolescents with LTCs. For some, this is because they do not want to be defined by haemophilia. The boys in this study have developed expert patient skills. They are without doubt expert patients, from whom younger boys with haemophilia may gain peer support in relation to performing self-care (self-infusion techniques); these boys do not have the benefit of a generation before them who have had access to the same care and treatment; they are the first generation of experts with haemophilia who perceive themselves to have normal lives and are a great resource for haemophilia care in the future.

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Conflict of interest

None.

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Haemophilia

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ORIGINAL ARTICLE *Adolescence*

The benefits of prophylaxis: views of adolescents with severe haemophilia

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Summary. It is well known and often reported that patients with long-term health conditions have problems adhering to treatment regimens. This is often reportedly worst in adolescents who struggle with the physical and psychological impact of adolescence as well as with the limitations that treatment regimens impose on their day-to-day activities. This article presents results from a larger study that aimed to discover what living with haemophilia in the 21st century was like for boys with severe haemophilia. The overall study was a multi-method, cross-sectional interview based study of 30 boys with severe haemophilia, treated with prophylaxis at a single site in the UK. Although not specifically asked in the interview schedule, opinions about treatment (prophylaxis) were given by 66% of the boys. These boys recognized that prophylaxis offered them protection from bleeding, the older and more sporty boys understood the need for tailored prophylaxis around 'risk' activities such as sport or events away from home. For some boys this meant low dose daily prophylaxis, and this further enhanced treatment adherence, as it became firmly embedded in their daily ritual of health care. This study shows that adolescent boys are in fact adherent with treatment, possibly at a schedule decided upon by them rather than one directed by the haemophilia centre. They are able to comprehend complex treatment decisions and make treatment plans that offer them maximum protection with minimal interference in their day-to-day activities.

Keywords: adherence, adolescence, children and young people, haemophilia treatment, prophylaxis

Introduction

Treatment adherence, in patients with long-term illness, is a much debated area of health care. It is estimated by the World Health Organisation that only 50% of patients are adherent [1]. Adherence in children with long-term illness is reportedly higher [2] perhaps because of parental influence [3,4] that may wane in adolescents and young adults as they become more responsible for their own disease management [5,6]. For many with long-term conditions, the impact of non-adherence lacks immediate consequence, for example 'non-compliance' with chelation therapy in thalassaemia has seemingly little impact (that can been seen or felt by the patient), but causes long-term complications of iron overload that may result in premature death; a complication that patients are well aware of [7]. In boys with severe haemophilia, non-adherence with prophylactic therapy leads to almost immediate symptoms where the necessary treatment is that with which they were non-adherent in the first instance [8]. Education about the need for treatment and the impact that non-adherence may have, is part of good haemophilia care. This article presents the converse view of boys with severe haemophilia who recognize the benefits of prophylaxis, and are adherent to treatment. These boys are part of a new, and perhaps unique, cohort of children and young men with haemophilia, who have been treated with intensive, often primary prophylaxis, for the duration of their childhood.

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Methods

The data in this article comes from a single site, qualitative study of living with haemophilia in boys, aged 4–16 years with severe haemophilia A (n = 27) or B (n = 3). Sixty percent have been treated with primary prophylaxis started at ≤2 years of age, with the

remaining 40% starting late prophylaxis between the ages of 3 and 6 years. The mean age at start of prophylaxis for the whole cohort was 28.8 months (range 0.5–72 months). Over the life time of these boys, the prophylactic regimen used at this centre has changed from three times per week for those with severe haemophilia A, and twice weekly for those with haemophilia B, to individualized targeted dosing schedules, administered at least every 48 h in haemophilia A and where in some, particularly sporty adolescents, low dose daily prophylaxis is now the 'norm'.

English-speaking boys were asked to participate in the study, as they needed to be able to participate in tape recorded interviews. Due to this wide age range a multi-method, age and development appropriate, study using four different research tools was designed. The boys were divided into three age groups, 4–7 year olds who took digital photographs to use as interview discussion prompts; 8–12 year olds used draw or write techniques to describe their experiences of having haemophilia; and 13–16 year olds who were invited to a focus group. Three families did not want their children to be included in the study. Twelve boys chose not to participate in the study; four stated they were not interested in the study, two failed to respond to invitation and follow-up letters, two lived too far away to attend the focus group, two boys refused to use the camera, one said he was too shy to do talk about himself and one had only just been diagnosed with haemophilia on moving to the UK. The socio-demographic data between the study group and those boys who refused to participate were similar.

Ethical approval for the study was granted by the South East Research Ethics Committee. The boys were invited to participate in a larger study evaluating the lived experience of haemophilia, where semi-structured individual or group interviews were undertaken to better understand haemophilia, its treatment and its limitations, from the child/young persons' perspective.

The tape recorded interviews were listened to several times, the transcripts were analysed and coded by hand. Narrative content was identified and coded into recurring 'themes' that were identified as important aspects of modern day life with haemophilia. Many of the boys, particularly the adolescents, talked about the significant issues of bleed management and prophylaxis in relation to the impact that haemophilia had on their lives this data is presented in this article.

Results

Boys as young as five showed a rudimentary knowledge of the benefits of prophylaxis, recognizing that this made them safe and stopped them from having bleeds (data not shown as this article addresses adolescents views). Fifteen of the adolescent boys,(aged 10–16) who were independent with regard to haemophilia management as they were self reliant and able to self infuse, vocalized their thoughts and feelings about prophylaxis and how it offered them 'protection' and made them feel 'safe', so that they could 'forget about haemophilia' and concentrate on being like their normal peers:

> Yeah I feel more confident.. you know if I've done it [treatment] that morning then I feel like I would be able to go in for it [sport] more… actually work a lot harder than if I hadn't done it [prophylaxis] then I would feel more cautious (participant 08, aged 16)

> I try to keep on top of the prophylaxis because I think that it keeps the bleed at bay and that keeps me in a better state of mind …. so I do it [prophylaxis] personally for the fact that I want to carry on living my life and I don't want it [haemophilia] to affect me (participant 09, aged 14)

On non-treatment days boys felt unlike their usual selves:

> I find that if I haven't done my injections then I feel vaguely weird and I feel like I really can't be bothered to do much. When I've just done my injection I feel sort of more alive and decent (participant 14, aged 14)

These boys recognize that prophylaxis offers protection from unpredictable bleeding, allowing them to engage in a more 'normal' lifestyle. The limitations caused by bleeding: reduced mobility, the need to use crutches, pain etc. are alleviated by continued primary or tailored prophylaxis programmes. On non-treatment days, the boys describe non-haemophilia-related physical impacts such as those described by participant 14 above, as well as psychological impacts of perceiving themselves to be at risk of bleeds as described by participants 8 and 9.

Having a bleed reminds boys, who are usually bleed free due to good prophylaxis, that they have haemophilia which causes pain, reduced mobility and imposes on the way in which they usually live their life:

> Well I only have them [bleeds] very rarely and depending on where they are, if its in my leg or something it means like a tight pain if I try and walk or something [then I] hobble or limp and I can't play sport or be like me (participant 19, aged 12)

> Yeah I hate it when I get a bleed and taking thousands of units to try to get rid of the bleed and it doesn't work and that's when it [haemophilia] really gets me down, makes me miserable (participant 29 aged 16)

Although prophylaxis offers protection from bleeds, bleeding reminds boys that they have haemophilia and

that they are different from their peers. Participant 29 described how bleeding (and not haemophilia *per se*) made him miserable because of reduced mobility, pain and the impact that bleeding (and not haemophilia) had on his life. Bleeding has a variable impact on the lives of adolescent boys with haemophilia, but is most obvious in those who play sport and who have to reduced or limit their participation because of bleeding. Several of the boys in this study were active sportsmen participating at local, regional and national level as footballers, golfers, rowers, runners and swimmers. They recognized that to continue participation in sport at these levels, they must tailor their treatment around activity, taking personal responsibility for treatment demonstrating expert thinking when planning their own treatment:

> [About low dose daily prophylaxis] 'Because then it [factor level] will be on one consistent level because at the moment its like if you had a graph it would be going up down up down up down so if you have it every day it would be pretty straight. If you are on a low and you had an injury you would be even more smashed up but if, because its all on one level it's like you won't get it [hurt] as much' (participant 27 aged 14, a golfer on daily prophylaxis)

> I think I prefer that [daily prophylaxis] because then I have kind of a routine. I get up in the morning, have my needle then feel ready for school (participant 9, aged 12)

Making prophylaxis part of the routine of everyday life as described by participants 9 and 27, is similar to the ritual of brushing your teeth [9], and improves treatment concordance as having to remember if each day is a treatment day or not is eliminated. This sustainable routine relieves 'adherence stress,' reduces the risk of forgetting to take medicines, encourages normalcy [10] as well as mastery of disease [11] and eliminates bleeding risk, and at the same time ensures that haemophilia does not become the total focus of an individuals' life.

Discussion

During childhood, when prophylaxis is most commonly administered by parents, treatment compliance is good [3,4,6,8]. This correlates with a good quality of life, in part due to a reduction of bleeding, pain and reduced mobility [12] and an ability to participate in normal childhood activities. It is recognized that adolescents are likely to reduce or stop prophylaxis [4–6]; this is because they are primarily oriented in the present and are less influenced about long-term health risks than their parents or affected adults [13]. However, at the same time, these boys are striving for 'normalcy', they may forget about their haemophilia and see themselves primarily as being like their healthy peers and siblings [14] able to participate fully at all routine activities of early adulthood. We should focus on the 'wellness' of these boys, through individualized treatment and education programmes/reviews rather than focusing solely on adherence at clinic reviews. Self-management of haemophilia involves complex skills: knowledge of haemophilia including management and treatment, being vigilant to subtle bodily messages, noticing symptoms [15] and mastering self infusion techniques, sometimes in the face of restricted mobility due to upper limb bleeds.

As children grow, they gain haemophilia specific knowledge and skills, as well as attitudes and life skills that enable them to understand lifestyle issues which impact on their health. Woodgate and Leach [16] describe how healthy adolescents recognize health as a 'state of doing' and not a 'state of being'. They show how adolescents understand that there are healthy and unhealthy choices in their lives. These include watching what they eat, avoiding alcohol and smoking and participating in exercise and other health promotion activities. For boys with haemophilia, this includes understanding complex bodily functions: why bleeding occurs, when it occurs, when why and how to treat and when to seek advice and help [5].

Having to think about health and well-being everyday is a burden which in some instances leads to a cessation of treatment [7]. However, boys with haemophilia, who are involved in the design of their own, tailored prophylactic regimen around 'risk periods' in their day-to-day lives (sport, evenings out etc.), and are more likely to continue with treatment, especially as missing treatment results in rapid bleed onset which necessitates the treatment that was missed in the first place. Prophylactic regimens designed by health care professionals, which fail to recognize the individual needs of these boys, are less likely to be complied with labelling boys as 'non-adherent'. This is unjust, as it fails to recognize the developing role of 'self medicator' that is inherent in patients with long-term conditions. We should talk to boys about how they manage their treatment regimen, how they integrate this into everyday life and how to develop sustainable routines to facilitate this [10], rather than only asking at what time of the day and which day they treat themselves.

The boys in this study bring insightful, meaningful commentary to haemophilia health care. Their expertise in persevering with life, amidst illness and personal challenge adds to their sense of self and of becoming an expert in the haemophilia community [17]. These boys, who have grown up being treated with primary prophylaxis with limited restrictions on life-style choices, are more likely to continue with tailored treatment into adulthood as they see themselves as they

see their peers: able to participate fully in all activities and to live a 'normal' (bleed free) life.

Conclusions

This article describes, perhaps for the first time, the day-to-day implications of life for adolescents with haemophilia, treated with intensive prophylaxis for all of their lives. Despite what the literature says about adolescents being the group who are the least adherent to therapy, the boys in this cohort incorporate prophylaxis as part of normal daily, health promotion activity. This is as 'normal' an activity for these boys as cleaning their teeth. Health care workers should recognize these self-management skills during clinic reviews, and promote these expert patients in supporting others who are younger than them, in developing their skills to become future expert patients.

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Disclosures

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Haemophilia

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ORIGINAL ARTICLE *Paediatrics*

'Just an unfortunate coincidence': children's understanding of haemophilia genetics and inheritance

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Summary. This paper presents the results of a study talking to children and young people affected with severe haemophilia A and/or haemophilia B about their knowledge and understanding of genetics and inheritance. These data were gathered in a qualitative study using semi-structured interviews with thirty boys aged four to sixteen discussing the impact of haemophilia on their lives. Responses were tape recorded, transcribed and analysed, using thematic analysis; one of the themes identified was genetic knowledge which is presented in this paper. Genetic knowledge was formed within the context of normal day-to-day lives within families affected by haemophilia, with parents and haemophilia centre staff being sources of information about individual inheritance patterns as well as providers of information about the future genetic impact of having haemophilia.

Keywords: carriers, children, genetics, haemophilia, inheritance, young people

Introduction

Haemophilia A and haemophilia B are X linked inherited disorders of coagulation, which are carried by females and affect males. Healthcare workers readily draw family trees of affected individuals and easily identify 'obligate' and 'at risk' females within the family. The focus of genetic counselling and support is weighed heavily in favour of these women [1] with little thought being given to the current and future genetic consequences of the affected male proband.

There is limited research describing communication of genetic information within haemophilia families [2] or about how parents share genetic information within families [3,4]. Spitzer [5] wrote, in 1992, that children (aged 6–13 years) with haemophilia, knew 'very little about their disease and especially about the way a person contracted it'. Since then genes and genetics have become more widely discussed and debated, and at this haemophilia comprehensive care centre forms part of routine clinical review of all children, by the haemophilia team [6,7].

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Children and young people with haemophilia in the twenty-first century, are the first to grow up in the genetic age with the human genome project, genetic testing, genetic modification of food, recombinant factor VIII/IX technology and gene therapy are talked about in everyday parlance [8]. Genetics and inheritance are taught in English schools as part of the National Curriculum for Science [9]. This is divided into four age appropriate 'key stages' with each 'key stage' being further divided into areas of scientific interest. Human genetics, reproduction and inheritance are taught in key stages 2–4 as part of the 'life process and living things' theme. The content of this teaching is shown in Table 1.

What is yet to be described in any detail is where young people with haemophilia learn about their own genetic make-up and its consequences. This paper presents findings about genetic knowledge and understanding as part of a larger qualitative study exploring the lived experience of boys with severe haemophilia.

Methods

This was a single site, qualitative study of 30 boys aged 4–16 years with severe haemophilia A ($n = 27$) or haemophilia B ($n = 3$) undertaken in 2009 (see Table 2). Prior to commencement of the study ethical approval was granted by the South East Research Ethics Committee. The boys were invited to participate in a

Table 1. Knowledge/Education about genetics/inheritance in National Curriculum in England and (in column 4) haemophilia genetic knowledge demonstrated by this cohort of boys.

National curriculum key stage	Expected age of achievement	National curriculum teaching programme: scientific knowledge about genetics/ inheritance	Quotes which demonstrate advanced genetic knowledge in boys with haemophilia
2	11	Recognizes the roles of drugs as medicine	Children aged 5 (data not shown)
		Are knowledgeable about human life cycles	'it might not run in the family' (Edward aged 9)
3	14	Human reproduction with inherited variations within species	'she didn't get it – she's a girl' (Murray aged 5)
			'it's good he didn't have haemophilia' [about his little brother] (Nick aged 9)
		Selective breeding can lead to new varieties	'I just came out that way' (Jack aged 4)
			'Just a rare unfortunate coincidence' (Edward aged 9)
			'I was born with it … because my mum's a carrier' (James aged 13)
4	16	Composition and function of blood	'part of the gene missing' (Henry aged 9)
			'you don't clot fully' (Matt aged 13)
		The mechanism of inheritance with recessive and dominant genes	'mum's got part missing … but she's got two' (Henry aged 9)
			'it's from my mum … she carrier it' (David aged 13)
		That some diseases are inherited	'the girls will carry it and the girl's boys will be haemophilia' (Matt aged 13)
			'I was born with it … because my mum's a carrier' (James aged 13)
		Genetic engineering is possible	'they had this treatment ……..like IVF …. So she's not a carrier' (Oliver aged 14)

Table 2. Boys who discussed knowledge of genetic information.

Pseudonym	Age	Diagnosis	Previous family history
Jack	4 years 11 months	Severe haemophilia A	Y (brother of Duncan)
Murray	5 years 3 months	Severe haemophilia A	N
Isaac	6 years	Severe haemophilia A	Y (brother of Henry)
Jimmy	7 years 10 months	Mild haemophilia A with high titre inhibitor (severe phenotype)	Y
Neil	8 years 1 months	Severe haemophilia A	Y
Duncan	8 years 9 months	Severe haemophilia A	N
Edward	9 years 2 months	Severe haemophilia A	N
Nick	9 years 2 months	Severe haemophilia A	Y
Robert	9 years 8 months	Severe haemophilia A with high titre inhibitor	N
Henry	9 years 11 months	Severe haemophilia A	N
Will	10 years 2 months	Severe haemophilia A	Y (brother of Paul)
Jonathan	10 y 8 months	Severe haemophilia A	Y (brother of David)
Tom	12 years 3 months	Severe haemophilia A	Y
Michael	12 years 10 months	Severe haemophilia A	N
James	13 years	Severe haemophilia A	Y
Matt	13 years	Severe haemophilia A	Y
David	13 years 3 months	Severe haemophilia A	Y
Peter	14 years 5 months	Severe haemophilia A	N
Oliver	14 years 5 months	Severe haemophilia A	Y
Paul	14 years 10 months	Severe haemophilia A	Y
Ian	15 years 3 months	Severe haemophilia A	Y (brother of Neil)
Simon	15 years 6 months	Severe haemophilia A	N
George	16 years 4 months	Severe haemophilia A	Y
Mark	16 years 10 months	Severe haemophilia A	Y

Y, yes; N, no.

larger study evaluating the lived experience of haemophilia, where individual or group interviews were undertaken to better understand haemophilia, its treatment and its limitations, from the child's perspective.

The interviews were semi-structured and tape recorded, thus allowing the researcher to give full attention to what was being said. The tape recordings were listened to on several occasions, were transcribed by a professional transcriber and then analysed and coded by hand. Narrative content was identified and coded into recurring 'themes' which were identified as important aspects of modern day life with haemophilia. These themes were: family and siblings, school, sport, career, haemophilia – bleeds and treatment, good and bad aspects of living with haemophilia and genetics and inheritance. All of the themes, with the exception of

genetics and inheritance, fit into a core category around the daily life experience of having haemophilia and will be presented in later publications. The narratives relating to genetics and/or inheritance are presented here.

Results

Twenty four boys of the 30, who were interviewed, offered a range of responses in terms of complexity and detail about genetic knowledge and/or understanding of inheritance of haemophilia. Those who did not offer a response were aged seven or younger.

Boys with haemophilia grow up in families affected by haemophilia and develop a gradual understanding of the implications of haemophilia for them and their family members. The youngest boys in this study are at an age where they are beginning to understand 'symptoms, treatments and physical differences from peers' [10], and their parents are starting to reveal genetic information, such as 'you were born with it' as they perceive their child to be developmentally ready for it [3,4]. It is therefore not surprising that the smallest boys in this study have a lack of 'real' knowledge about haemophilia genetics and inheritance as these are complex issues [11,12] probably beyond their developmental ability [5].

A rudimentary understanding of haemophilia genetics is demonstrated by some of the youngest boys through statements such as 'I just came out that way' (Jack aged 4*) 'girls can't get haemophilia can they?' (Nick aged 9) and 'she didn't get it because she is a girl' (Murray aged 5). The data from boys is presented in two sub themes:

1. Genetics, their haemophilia gene and its mutation and
2. Inheritance – how they inherited haemophilia including implications for them and their family in the future.

These themes are discussed in more detail below:

Genetics

Four of the older boys described the gene mutation/genetic component of their haemophilia in a medical/scientific way indicating that they understood the importance of genes and chromosomes in causing both haemophilia and bleeding:

> It's a blood disorder when you don't clot fully …….or nothing at all [I got it] because one of my chromosomes are bent [he has IVS 22 inversion]. Matt aged 13

> It's in part of our genes – our mums got part missing, but she's got two and that's why girls can't get haemophilia … but boys only have one so when part of our gene is missing which means our blood doesn't clot. Henry aged 9

> My mums a carrier ……it means it's in your DNA or whatever, but you haven't actually got it. It just means that if you have a child they are more likely to have it. Will aged 10

> It's in the X chromosome, only males have XY and YY …… or something [questioning] that's a girl so no haemophilia. Mark aged 16

The knowledge demonstrated by these boys, exceeds the background genetic knowledge that is expected for boys of this age [9]. Haemophilia specific genetic knowledge has thus been gained either from their families, their haemophilia care providers or from both. Most parents see themselves as having 'prime responsibility' in discussing genetics with their children yet struggle with 'what and how to tell' at different stages of children's lives [13]. Women (mothers) are often seen as 'genetic housekeepers' and 'kinkeepers' [those who hold the family (or kin) together] [14] for family members, often negotiating genetic testing and coordinating counselling for family members, it is imperative that these women have the correct information and support to inform family members correctly [1].

Inheritance

Approximately half to two-thirds of boys with haemophilia come from 'affected families' i.e. those where there has been haemophilia in previous generations. Spontaneous genetic mutations, either in the mother or the child are responsible for causing haemophilia in the remainder of those affected. This prevalence is reflected in this study where 10 of the 30 boys were the first affected member in their family. Despite being the first, or only, affected individual in their family, the boys demonstrated an awareness of the impact of haemophilia on other family members: their mothers, siblings and their own future children.

Edward, aged 9, the only affected member of his family with haemophilia described its inheritance as 'just a rare unfortunate coincidence', something that his parents confirm they have told him repeatedly, and that his children might not be affected as 'it might not run in the family'. Edward has an older unaffected brother, his mother has chosen not to be carrier tested and there are no other 'at risk' females in the family. If Edward only has sons then he is correct in thinking that haemophilia will not run in the family; however, all of his daughters will be carriers and their children might be affected. This

*Pseudonyms have been used throughout this paper to protect the identity of the interviewees.

notion is better narrated by boys with previously affected family members:

> Well it runs in the family so my granddad had it and he had a girl, my mum, and so she's a carrier and she carried it to me. Oliver aged 14

> I was born with it ……because my mums a carrier …… but she doesn't have it …… my mum's uncle and cousin and a brother do. James aged 13

Two boys came from families where genetic knowledge of ancestors was limited or unknown. David aged 13, whose mother is adopted, said:

> It's from my mum and then I don't know how it passed on [to her]. I don't know from my mum's mum or my mum's dad she carries it but she doesn't have to deal with it as bad as boys [affected carrier]

Sixteen-year-old George, who knew that his maternal grandfather was not his biological relative, described his inheritance of haemophilia from a known affected individual as:

> My mums' birth father had it so that made her a carrier and I got it

These four boys were able to describe complex genetic inheritance patterns both from knowledge of their own families and from learning about genetics and inheritance at school and at the haemophilia centre where frequent discussion about this occurs. Miller [15] describes treatment centre education and counselling protocols and states how affected boys and men are often 'forgotten' in this, assuming that they know the obligatory carrier status of their daughters. Several of the boys in this study showed a good understanding of the impact of haemophilia on their own children and their children's children:

> It means when men go on to have kids the boys will be OK and the girls will be carriers ….. it's a never ending circle really. Mark aged 16

> The girls will have the gene and her children, if she had a boy or a girl there is a 50/50 chance that either of them would have it. David aged 13

> Well the boys will be fine but the girls will carry it and the girl's boys will be half haemophilia. Matt aged 13

> Our [sic] boys will be perfect … but my ……if I get a girl she will be a carrier … she then has a boy and he has haemophilia. Jonathan aged 10

> It's in part of our genes – our mums got part missing, but she's got two and why girls can't get haemophilia … but boys only have one so when part of our gene is missing which means our blood doesn't clot. Henry aged 9 on behalf of himself and Isaac aged 6.

Sixteen-year old George (who had an affected Grandfather whom he never knew) describes how being an affected grandfather himself would benefit his grandchildren:

> It would be a big advantage because they would know …….. all the stuff I did when I was young – so it can only be good

This 'untapped expertise' [16] of the expert patient whose life story is more knowledgeable about the impact of the illness than the expertise of the haemophilia health care professionals, shows how people can be 'successfully ill' [17]. This is enhanced in inherited congenital disease where there is no 'before and after' to compare life with and without the illness, and where older affected individuals can have a significant impact on younger people:

> I've got a friend who is about 42-ish and he's got haemophilia and we chat, I think I look to him for a friend because he is older and he has lived with it longer than me, then we have a discussion about it, and I find it nice because things that kept on going for him he has been through as well, and I might go through exactly the same so that means quite a lot. He is someone I can actually relate to on a level where they know where I am coming from I suppose. Mark aged 16

The sense of belonging to an extended family or a unique 'haemophilia community' [14] through shared genetics (or genetic mutations) makes living with haemophilia an unexceptional everyday reality [18] bound within individual, family, and cultural values. Modern day, openly communicated day-to-day lived experience enables boys to communicate freely about genetic inheritance and haemophilia management within their families. This leads to an understanding of the potential carriership of their sisters, and a relationship that 'becomes stronger as siblings grow up' [14]. Having this knowledge enables the next generation to confront their fate [15], laying down a foundation for their future communication within his family [4].

> She is fine, she is normal, she has been tested. James 13

> She had like a test to see if she was a carrier, like my mum, and she wasn't a carrier ……..that's good because is she has some boys when she is older that means that it won't have haemophilia. Tom 12

> They [his mum and dad] had this special treatment – they had like IVF and then some special treatment

> which had never been done before and it worked out she's not a carrier …….. she can't ever get it again …….. it wont just randomly pop into the boy again. Oliver 14

Genetic testing of female siblings in families with X linked inherited disorders is an area of debate, but many children state that they are glad to have been tested and been able to grow up with knowledge about carriership [1,4,14,15,19]. However, in a paper about Duchenne Muscular Dystrophy (DMD) carrier testing and status was not routinely known by 'at risk' girls as parents felt a need to protect them from the knowledge that they were carriers [20]. The girls in this study felt that they should know everything about the genetics of DMD even if that knowledge was upsetting at the time, as this gave a better 'family view' of inherited disease.

Unaffected siblings are recognized as not being part of the immediate 'haemophilia family' but of being affected by it, and therefore being part of, the global haemophilia community:

> [It's good he] didn't have it, ……. because he does not have haemophilia cause he is only little ……I don't want my little brother to have haemophilia so I am better off having it myself. Nick aged 9

> I think my brother, my older brother who hasn't got haemophilia it affected, I think in a way he feels guilty that we have haemophilia and he doesn't and he goes out of his way to help us out. In fact I know he does because we are only a year apart me and my older brother, me and him have a good relationship so I know for a fact that if certain things he'll do for me which are just little things that mean a lot…… we have had discussions about it, …… and obviously it's not his fault [that he doesn't have it] and he says he finds it hard sometimes I know that he's always stuck close to me cos of that. Mark 16

Family membership and relatedness is a construct of genes and genetics that cause disease as well as those that do not – for example, those that cause haemophilia and those which lead to blue eyes or curly hair and a family resemblance. Genetically unaffected siblings may still be affected by having a sibling with, or who is a carrier of, haemophilia. These siblings experience haemophilia on a day-to-day basis and understand how it affects family life and relationships both today as well as in the past and future.

Discussion

This is the first paper that the authors can identify, which presents the genetic understanding and knowledge of children and young people who have haemophilia. Studies of young people and adults with haemophilia have shown that 69% of patients aged 13–25 years [21] and 96% of patients aged over 25 [22] were aware of heredity i.e. a healthy son and carrier daughter. When asked to rank genetic knowledge about haemophilia, 80% of respondents, patients (aged >18) and parents, ranked knowledge as high or medium high [23]. These data are from studies about disease and treatment and not specifically about genetic knowledge and understanding.

Within this study, the role of the families in supporting and educating boys about haemophilia is apparent. Growing up with a brother with haemophilia leads to strong sibling relationships [14], experiential knowledge [16] and communication of family values about the condition, which may support carriers and boys with haemophilia in future reproductive choices. The boys in this study understand the implications of genetic disease on their own, their siblings and their future children's health. This knowledge develops in an age-appropriate way: where the youngest of the children knew only that girls do not get haemophilia ('she didn't get it because she is a girl' Murray aged 5), in contrast to older boys such as Henry aged 9 who showed a greater understanding of inheritance:

> It's in part of our genes – our mums got part missing, but she's got two and why girls can't get haemophilia … but boys only have one so when part of our gene is missing which means our blood doesn't clot.

The boys in this study will have all had science education at school using the National Curriculum for England (science) teaching programme [9]. This knowledge will be enhanced through parental discussion of genetic knowledge with children. This occurs in a developmental way from basic information for preschoolers, family relationships and genetics for school age children and understanding of the implications of genetic disease on their own reproduction for adolescents [10]. The boys in this study demonstrated genetic and inheritance knowledge that exceeds that expected for their educational age (see Table 1); this demonstrates knowledge acquisition from parents, families and haemophilia care providers.

The oldest boys in this study were able to perform 'verbal drawing of family trees' [24] fully describing the genetic implications and inheritance patterns of haemophilia for them, their ancestors and their descendants including George's biological (and unknown) grandfather:

> My mum's birth father had it so that made her a carrier and I got it

Simon (aged 15, who is the first in his family to be affected) understands impact of haemophilia on future children within his family:

> It will be good for my sisters' kids, my girls and my grandsons… 'cos they will have me to help them through it.. so better for them than for me who had no one who knew

For some this will be following formal education about genetics and inheritance in science at school [9] however in day-to-day life communication about haemophilia within the family is in the domain of the everyday (as treatment is given etc.) and judgement about it as a condition, where individuals can lead fulfilling and worthwhile lives will develop [2,18].

The narratives told by these children are not only about themselves but are also 'partly stories about others in their family' [16] providing self identity, which relates them to 'others': these are family members identified as relatives as well as those belonging to the haemophilia community as described by Mark when talking about his 42-year-old friend with haemophilia.

Conclusion

Genetic knowledge, testing and technology have been major scientific breakthroughs in haemophilia care in the twentieth century, and will continue to improve as further developments occur in genomics and genetic therapeutic options. Comprehensive strategies are needed to ensure that those affected, either directly or indirectly by haemophilia, fully understand their own genetic risk. This paper is a first insight into genetic knowledge of young people affected by haemophilia and should be used as evidence for future education and support of both affected boys and their (potential or actual) carrier sisters who may pass on the haemophilia gene to future generations.

This paper demonstrates that the children and young people in this study have a good awareness of haemophilia genetics and inheritance. Some of this information comes from within the family while the remainder comes from haemophilia care providers whose responsibility it is to inform and correct misinformation. The study is limited in that it only includes children from a single site where knowledge of inheritance and genetics is seen as an important part of haemophilia care and is repeatedly addressed at review clinics. Haemophilia health care workers should provide tailored, age-appropriate information to those with haemophilia as well as to those who do, or may, carry it addressing age-appropriate issues such as reproductive choices, as they occur.

Disclosures

The authors stated that they had no interests which might be perceived as posing a conflict or bias.

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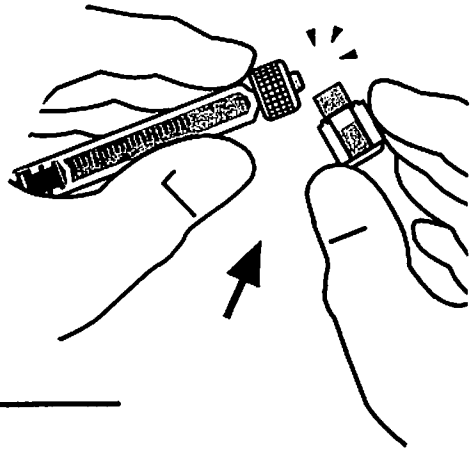
Evaluating a self infusion device for children with haemophilia



Abstract

Although prophylactic treatment of boys with severe haemophilia has progressed significantly, there has been very little change in the way that coagulation factors are reconstituted and administered. Most boys with severe haemophilia can self infuse by the age of eight and most are totally self sufficient by the age of 14. This small study evaluated a product developed to improve the reconstitution and administration process by seeking the views of ten boys who self infuse recombinant coagulation factor. The boys completed a simple questionnaire related to the current method and after the introduction of new method of reconstitution using the ReFacto Rapid Reconstitution™ (R2) device. The R2 device was reported by most boys to be advantageous in terms of time and safety in reconstitution as well as being acceptable to all but one. Even relatively young children can be successfully included in research and their opinions about their health care should be sought before introduction of changes, especially when proposed changes could affect their ability to perform their own treatment.

Haemophilia is an X linked, inherited disorder of coagulation, which results in both spontaneous and trauma related bleeding predominantly into the weight bearing joints. The severest form of haemophilia (with factor VIII or IX levels of < 0.01 IU(international unit)/dl (normal range = 50-150 IU/dl) affects around 950 boys aged under 19 in the UK (United Kingdom Haemophilia Centre Doctors Organisation (UKHDCO) 2004).

Historically, treatment was with plasma-derived products only after a bleed had occurred. In the early 1990s prophylactic treatment was introduced. The coagulation factor was given as an intravenous injection two to three times per week in an attempt to prevent bleeding episodes, minimise disability and improve quality of life (Liesner *et al* 1995). Since then recombinant (or 'genetically engineered') coagulation factors have been introduced and are now the accepted treatment for all children in the UK (UKHCDO 2003). Recombinant products are easier to administer as they require smaller volumes of diluent than plasma derived products, and there is now an expectation, from healthcare providers and funders, that boys will be able to self infuse at an early age. Most boys with severe haemophilia can self infuse by the age of eight and most are totally self sufficient by the age of fourteen. Their ability to perform this task is audited by the Pan Thames Haemophilia Consortium who fund haemophilia care for children and adults with haemophilia within the London strategic health authority (www.croydon.nhs.uk).

The challenge

Despite advances in manufacturing processes, the actual reconstitution and administration of coagulation factors has not changed significantly since the 1980s. Over the last five years new methods of reconstitution have been introduced, in part in line with new NHS and EU guidelines about health and safety in prevention of needle-stick injury. The devices have been shown not to affect the factor VIII/IX molecule or the efficacy of treatment (Prabhu *et al* 2006) and have been introduced following market research with healthcare professionals and occasionally adults with haemophilia. This article reports a study undertaken with ten children with haemophilia, assessing a new method of reconstitution using the ReFacto Rapid Reconstitution™ (R2) device.

Conventional reconstitution of factor VIII uses two vials (one with a diluent (saline or water for injection) and one with lyophilised (freeze dried) factor) and a double-ended needle to transfer the diluent into the factor. A separate filter needle is then required to draw up the reconstituted factor into a syringe. The ReFacto R2™ device comprises a syringe which is pre filled with saline and a plastic transfer device with integral filter which fits onto the factor VIII vial and allows injection of the saline, mixing and withdrawal of the reconstituted factor VIII in a needle-less method (see figure 1 overleaf).

Methods and sample:

All boys with severe or moderate haemophilia A who were treated with recombinant factor VIII ReFacto®

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Key words

- Children: haemophilia
- Self medication

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evaluation

(Moroctocog alpha, Wyeth Maidenhead Berks) and who were able to self infuse were invited to participate in a study of the ReFacto R2 device. Only children being treated with ReFacto were eligible to participate as the ReFacto R2 device was to be introduced for commercial use with this product only.

The study was granted full ethical approval by the Great Ormond Street Hospital for Children NHS Trust/ Institute of Child Health research ethics committee. The children were invited to participate in the study by letter and began their participation at a routine clinic visit, with parental consent.

Ten children aged five to 16 years agreed to participate. They were first asked to complete a simple questionnaire comprising tick boxes with space for comments giving their views on ease of reconstitution using the conventional double-ended needle technique.

The younger children were helped to complete the questionnaire by the researcher who recorded their comments verbatim. The children were then shown how to use the ReFacto R2 device in the haemophilia centre, and were encouraged to practice the techniques necessary using dummy devices. They were given written and pictorial guides (see figure 1) to take home. When they had been assessed as competent, the boys used the ReFacto R2 device at home for at least six infusions and completed a follow up questionnaire assessing: ease of use; time taken to learn the new method; comparison of the ReFacto R2 device against the standard double-ended needle method; and overall satisfaction with the ReFacto R2 device.

Results

Nine (of ten) children reported the R2 device to be fairly or very simple to use with an average time of less than five minutes to learn the technique. They found the R2 device to be satisfying or extremely satisfying to use. Six boys stated that it was fairly or very simple to use the R2 device to mix the factor and to fill the syringe. This figure is lower than expected: two of the older children reported difficulty with leakage from the syringe. This proved to be caused by problems with their technique i.e. inaccurate positioning of the R2 device into the centre of the rubber bung on the factor vial.

Five boys reported that using the R2 device for reconstitution was quicker than the conventional method, with seven boys stating is was more convenient than the double-ended needle method. One boy disliked the device as he had to reconstitute multiple vials for his dosing schedule, this left him either having to swap (pre-filled) syringes as he infused or using an additional larger syringe to draw up his total dose. Conversely, another boy, who had been using a 10ml syringe for his 5ml dose, commented how easily the new syringe fitted into his hand and how this made self-infusion easier for him.

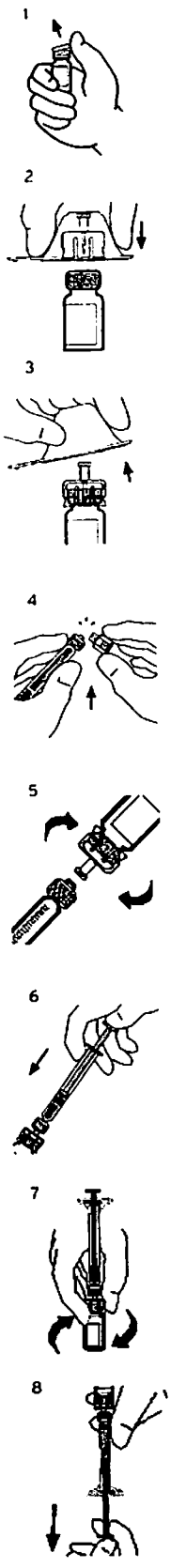
Conclusion

This small study suggests that even relatively young children can be successfully included in evaluation research and that they have opinions about their health care that should be sought before introduction of changes, especially when proposed changes could affect their ability to perform their own treatment.

The R2 device was reported by these boys to be advantageous in terms of time and safety in reconstitution as well as being acceptable to the majority. It is likely that these devices will continue to develop both within haemophilia and other treatment areas. Wherever possible children's opinions should be sought prior to their introduction into mainstream care PN

■ **Support for this study was provided by Wyeth Pharmaceuticals (UK) Ltd**

Figure 1
Pictorial guide to reconstitution



1 Remove the plastic 'pull-top' cap from the ReFacto R2 vial
2 While holding the ReFacto R2 package, place the ReFacto R2 device over the vial and press down firmly on the package until the spike penetrates the vial stopper
3 Lift the package away from the ReFacto R2 device and discard
4 Break off the tamper resistant plastic tip cap from the syringe by snapping the perforation of the cap. Bend up and down until the perforation is broken. Do not touch the inside of the cap or the syringe tip
5 Connect the syringe to the vial by inserting the tip into the adapter while firmly pushing and turning the syringe clockwise until secured. Inject the water.
6 Without removing the syringe, gently rotate the vial until the powder is dissolved
7 The final solution should be inspected, the solution should appear clear and colourless
8 Turn the vial upside down and draw back all the solution into the syringe

Source: Wyeth Pharmaceuticals

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ORIGINAL ARTICLE *Paediatrics*

Children's preferences of transfer devices for reconstitution of factors VIII and IX for the treatment of haemophilia

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Summary. The past decade has seen the introduction of a number of needleless transfer devices for the reconstitution of coagulation factors. This study investigated the use of four commercially available transfer devices by children with bleeding disorders, and assessed their preferences for the devices. Fifteen children with bleeding disorders requiring prophylactic home treatment with coagulation factors were recruited to the study. The participants tested reconstitution with four dummy transfer devices: BAXJECT, BIOSET, ReFacto R2 and Mix2Vial. Participants did not infuse the factors reconstituted using the devices. Each participant then answered a series of questions on each device; answers were based on a five-point Likert scale and the devices were ranked in order of preference. All participants were able to use the devices within 5 min and 78% of participants ranked all devices as 'fairly easy' or 'very easy' to use. The most popular device was Mix2Vial, which was the most preferred by seven participants (46.7%). BAXJECT was the preferred device of four participants, but was also the least, or least but one, favourite of nine participants. Pre-filled syringe transfer devices, BIOSET and ReFacto R2, were preferred by only 26% of participants. This is the first published study comparing children's preferences for transfer devices. All children were able to manage the devices readily, and Mix2Vial was the preferred device of the majority of participants. Newer, advanced transfer devices offer a user-friendly and more convenient and effective way for children to reconstitute factors.

Keywords: bleeding disorders, children, haemophilia, reconstitution, transfer device

Introduction

Haemophilia affects over 7500 patients in the United Kingdom [1], of whom 37% are aged under 18 years [2]. Severe haemophilia results in spontaneous and traumatic bleeding and was historically treated by plasma products after a bleed occurred. Advances in the treatment of haemophilia led to the introduction of prophylactic treatment in the 1990s [3], with the administration of recombinant coagulation factors two to three times per week. Furthermore, recombinant products are easier to administer than plasma-derived products as they require lower infusion volumes [4].

Since its introduction, the uptake of prophylaxis has steadily increased, and in 2005 its overall use in the United Kingdom had risen to 61% [2]. In the United Kingdom, recombinant factor VIII is recommended for boys with haemophilia A and recombinant factor IX is the treatment of choice for haemophilia B [5]. Prophylaxis offers a number of practical benefits to patients. As well as reducing the number of bleeding episodes [6], prophylaxis facilitates home treatment of haemophilia, with recent estimates suggesting that most factor VIII and factor IX infusions are administered in the home [7]. Whilst prophylaxis offers significant improvement in quality of life, it does require substantial commitment from the patient [8], and approaches to simplify treatment are important in ensuring concordance.

ReFacto® R2 is a trademark of Wyeth, Madison, NJy, USA; BIOSET® is a trademark of Bayer Corporation, Leverkusen, Germany; BAXJECT® is a trademark of Baxter, Deerfield, IL, USA and Mix2Vial™ is a trademark of CSL Behring, Marburg, Germany.

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Traditionally, haemophilia treatments required reconstitution by a double-ended needle method. Although relatively easy to use for adults, this complex method may have delayed learning of self-infusion in young children [9]. In 1998, new health and safety guidelines of the European Union and National Health Service (NHS) were introduced to prevent needlestick injury [10]. In line with these changes, a number of new devices were developed for reconstituting coagulation factors, including factors VIII and IX.

Advanced reconstitution devices are often easier to use than double-ended needles and can lead to improved safety and potentially better patient compliance [11]. Given the increasing use of needleless devices in clinical practice, the effect of passing coagulation factors through needleless devices on treatment efficacy was examined by Prabhu and colleagues who demonstrated that neither the structure nor function of the factor concentrates was adversely altered by the use of needleless devices, and that needleless devices do not affect treatment efficacy [12].

Patient preference for a number of individual needleless devices has been assessed [4,13,14]. In a study of 161 participants including patients, caregivers and nurses, BIOSET (Bayer Corporation, Leverkusen, Germany) was preferred over double-spiked transfer devices in terms of worry/safety, ease/confidence and overall preference [13]. Likewise, BAXJECT (Baxter, Deerfield, IL, USA) was preferred to previous reconstitution methods and, for the first time, the device allowed young children to reconstitute factors [14].

Children's preference for transfer devices is an important factor in the treatment of their haemophilia. Severe haemophilia affects a significant number of children, most of whom can self-infuse by 8 years of age and are fully self-sufficient by the age of 14 years [4]. Children with chronic diseases, such as haemophilia, are experts in their own disease management [15]; their views about product enhancements or changes to treatment regimens are valuable as they allow healthcare workers and researchers to better understand their experiences [4].

The preference for one needleless transfer device, the ReFacto R2 (Wyeth, Madison, NJ, USA) has been assessed previously in 10 children who were able to self-infuse. In this study, nine of the 10 patients reported that the ReFacto R2 device was fairly, or very simple to use and that they were able to use the device within 5 min of its introduction. Overall, the device was found to be advantageous in terms of time and safety, although a small number of the patients had problems with leakage from the device [4].

Although preferences for many of the available transfer devices have been determined, to date, there has been no comparison of preference between devices. This study was designed to assess children's preferences and usability of four commercially available transfer devices, all of which were introduced following the introduction of the needlestick guidelines in 1998.

Methods

The study was submitted to the local research ethics committee for consideration and was subsequently declared to be non-interventional. Although ethical approval was not necessary, parental and participant consent/assent was sought from all participants before entering the study.

At the time of the study in August 2005, 150 children aged <18 years with severe haemophilia A or B, von Willebrands disease or mild/moderate haemophilia requiring factor concentrates were attending the haemophilia centre. Inclusion criteria for the study were that children had to have a bleeding disorder requiring home treatment; importantly, only children who were self-infusing (n = 51) were eligible for inclusion in the study. Children already using a needleless transfer device were excluded to reduce potential bias (n = 19). A further six children were excluded from the study due to developmental delay and inability to complete the study.

Participants tested the transfer capabilities of four commercially available needleless transfer devices, ReFacto® R2, BIOSET®, BAXJECT® and Mix2-Vial™ (CSL Behring, Marburg, Germany) in the clinical setting. Two of these devices, BIOSET and ReFacto R2, are pre-filled syringe devices. Dummy devices were used to mimic transfer of products; participants did not infuse after using the four devices. Initially, participants were asked to answer questions on their current treatment prior to the explanation of the devices including information on dosing, self-mixing and self-infusion of factors, as well as their satisfaction level with their current treatment. Participants were then shown the four devices and were asked which they would like to test first; this was performed to reduce the researcher bias. Having chosen the devices to test first, participants were given a brief explanation of how to use each device and were observed as they used the device; this was performed by the same person for all children and devices to avoid any trainer bias. Upon completion of the transfer, participants were asked

Table 1. Questions used to assess satisfaction with current treatment and ease of use of new transfer devices in children self-infusing treatment for bleeding disorders.

Question	Answer options
To be completed before training on devices	
Which factor do you use?	
What dose do you have?	
How often do you have this dose?	
Do you mix your factor yourself?	
Do you infuse your factor yourself?	
How happy are you at the moment with the way that you mix factor?	• Very happy • Fairly happy • Neither happy or unhappy • Fairly unhappy • Unhappy
To be completed after training on the use of each device (answered separately for each device)	
How easy was it to learn how to use the device?	• Very simple • Fairly simple • Neither • Fairly difficult • Very difficult
How long did it take you to learn how to use the device?	• 5 min or less • 10 min or less • 15 min or less • 20 min or less • More than 20 min
What do you like about the device?	
What do you not like about the device?	
To be completed after assessment of all devices	
Can you put the devices in order with 1 as the best, 2 nearly best, 3 nearly worst and 4 worst?	
Why is [device] the best?	
Why is [device] the worst?	

questions, agreed by all manufacturers before the study, about the device and their answers based on a five-point Likert scale (Table 1). This process was repeated for each device, with identical questions asked after each device had been used. Following use of all four devices, participants were asked to rank the devices in order of preference, providing reasons for this decision. Any further comments made by participants were also recorded. No statistical analysis was performed due to the low number of participants enrolled in the study.

Results

Fifteen children were recruited into the study, including 11 boys aged 7.5–17.0 years with haemophilia A (three with mild and eight with severe haemophilia, including one with current high titre inhibitor), three boys aged 13.0–16.5 years with severe haemophilia B and one girl aged 14 years with severe von Willebrand disease.

All participants completed the study. Based on the questionnaire, none of the participants had difficulty in learning to use any of the devices, with all able to use each device within 5 min. Overall, 78% of participants rated the devices as 'very simple' or 'fairly simple'. Analysing the data by device, the ReFacto R2 was rated as 'very simple' or 'fairly simple' by 86% (13/15) of participants, closely followed by BIOSET and Mix2Vial, which were rated similarly by 80% (12/15) of respondents. However, both the BIOSET and Mix2Vial had the highest number of participants reporting them to be 'very simple' to use (Fig. 1). BAXJECT had the lowest number of participants reporting it as 'very simple' or 'fairly simple' to use (68%; 10/15); furthermore, 27% (4/15) of participants rated this device as 'fairly difficult' to use compared with 20% (3/15) for BIOSET, 13% (2/15) for Mix2Vial and 7% (1/15) for ReFacto R2.

When the devices were ranked by preference, 26.7% of participants rated a pre-filled syringe device (one for ReFacto R2 device and three for BIOSET) as their preferred device (Table 2; Fig. 2); these devices were the second choice for 80% of participants. BIOSET was also the least preferred device overall, with six participants rating it as such. Four participants ranked the BAXJECT device as their device of preference, but nine children ranked it as their least, or least but one favourite. The Mix2Vial was the most popular device, with seven participants (47%) ranking it as their device of preference. In contrast, three children ranked it as their least favourite and four as their least but one favourite.

To assess the potential reasons for the preference, the choice of preferred device was stratified by

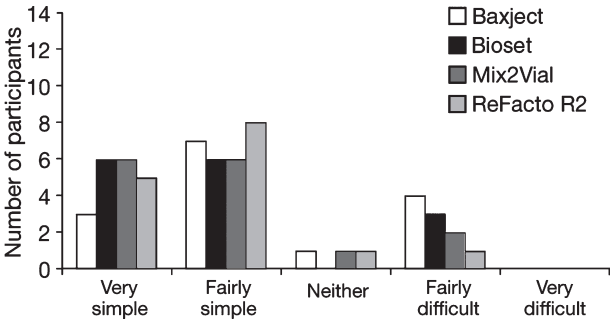

Fig. 1. Usability of transfer devices. Participants rated each of the four devices as 'very simple', 'fairly simple', 'neither', 'fairly difficult' or 'very difficult' to use.

Table 2. Children's preference ranking of new transfer devices used in the study.

	Device			
Rating	BIOSET* (number [%])	ReFacto R2* (number [%])	BAXJECT (number [%])	Mix2Vial (number [%])
1	1 [6.7]	3 [20.0]	4 [26.7]	7 [46.7]
2	5 [33.3]	7 [46.7]	2 [13.3]	1 [6.7]
3	3 [20.0]	4 [26.7]	4 [26.7]	4 [26.7]
4	6 [40.0]	1 [6.7]	5 [33.3]	3 [20.0]

*Pre-filled syringe devices.

disease and by age. When analysed by disease, there was no obvious trend in preference. However, when stratified by age, a number of trends were observed. In general, the ReFacto R2 was preferred by younger participants (three participants, age range 9–13 years) and the BAXJECT was preferred by older participants (four participants, age range 11–15 years). Of the seven participants whose preference was for the Mix2Vial, there was an even distribution of age from 8 to 17 years. Further to this observation, the data were stratified by current dose of treatment. The participants receiving the largest doses (due to either body weight in teenagers or, in one case, due to ongoing immune tolerance therapy) rated the pre-filled syringe devices lowest in terms of preference. This was due to their concerns over multiple vial/syringes and the need to 'syringe swap' whilst infusing, raising the concern that the butterfly could move leading to the infusion 'tissuing'. The two participants receiving the highest dose (4000 units) both preferred the Mix2Vial device. Those receiving lower doses (1000–2000 units) were less uniform in their preference, with ReFacto R2 receiving three nominations, and BIOSET, BAXJECT and Mix2Vial receiving one each.

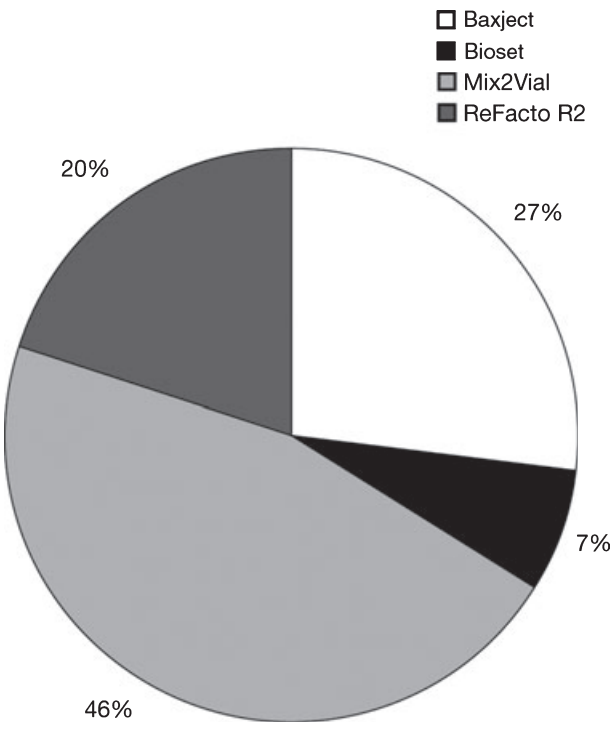

Fig. 2. Most preferred transfer device by participants. Participants ranked the four transfer devices in order of preference graded 1–4. The number of participants ranking each device as '1' is shown.

The comments that participants made during the study were collected and are shown in Table 3. Participants commented on their likes and dislikes about each device, and on their reasons for preference. Most participants commented made positive comments on the overall designs of the devices, the absence of needles and ease-of-use relative to their current system. Participants frequently commented on the volume of dose that could be reconstituted in each device. Whilst a number of participants commented that the large volume capacity of some devices was a particular like, participants requiring large doses stated a dislike for the low volume of the pre-filled syringe devices as this would require more than one syringe per infusion. The BAXJECT not only received positive comments on its ease of use, but also received a number of negative comments from participants regarding the three-way tap. Universally, this was the reason for its poor preference rating.

Discussion

Following the introduction of new health and safety guidelines in 1998, a number of new, advanced needleless reconstitution devices were introduced onto the UK market for use in the prophylactic treatment of haemophilia. Some of these devices (ReFacto R2, BAXJECT and BIOSET) have previously been reported to provide benefits over double-ended needle devices with regard to safety against needlestick injuries and ease of use [4,13,14]. However, this is the first published study to compare children's preferences of four commercially available reconstitution devices.

Although the four devices were all designed to avoid the use of double-ended needles, there are a number of practical and design differences between them, which are outlined in Table 4. The design of the devices, including labelling and instructions, is directly related to their ease of use, and therefore has a considerable impact on children's preferences. Furthermore, the volume that each device allows to

Table 3. Participants' comments about the transfer devices.

	ReFacto R2; pre-filled syringe device		BIOSET; pre-filled syringe device		BAXJECT; three-way tap device with separate syringe		Mix2Vial; filter device with separate syringe		Other comments
Participant	Likes	Dislikes	Likes	Dislikes	Likes	Dislikes	Likes	Dislikes	
1	2000 IU in one syringe	Not as basic as BIOSET, too much in it	Easier than current treatment, less ancillaries and waste	2000iu = 2 syringes	One device – is compact	Three-way tap – too hard to know which way round	Easy to use. No needles		
2	Easy to use	It leaks when it is not right in the middle	Pre-filled syringes				Easy to use and only one syringe		
3	R2 device itself – 'it's cool'			It's too long to hold properly when fully assembled The plunger is too delicate		Three-way tap		Too hard to use the two parts, hard to unscrew them	
4	Syringe and the way it fits to R2 device makes it easy to use		Easy to reconstitute, water goes in easily	Plunger is fiddly and easy to detach from rubber bung	The device makes it like science	Three-way tap	Mixes by itself and easy to disconnect the vials	Didn't mix as easily as the others	If I have a finger/hand bleed I'm not sure that the little syringes and the fiddling about will be so easy
5	The syringe is neat		It's fun, I like the syringe						
6	Easier, with less bits than I use currently. No sharp needles in it		Quite easy and not difficult		Simplistic to use	Too hard to suck the factor out	No sharp bits or needles	Separate syringes are difficult to use	
7	Simple to use	Having to make the syringe and spilling water when taking the cap off	Small volume	Takes a bit of getting use to: 'which part goes where?'	Mixes itself and draws back easily	Pressure before draw back and the Three-way tap	Simple, quick and easy		
8	Easy!			Leaks easily		Too hard to use the three-way tap	Easy to use more than one FVIII per dose	Hard to push the devices into the bottle	

Table 3. Continued

Participant	ReFacto R2; pre-filled syringe device		BIOSET; pre-filled syringe device		BAXJECT; three-way tap device with separate syringe		Mix2Vial; filter device with separate syringe		Other comments
	Likes	Dislikes	Likes	Dislikes	Likes	Dislikes	Likes	Dislikes	
9	Pre-filled syringe makes it quick to do	Screwing the syringe together	Pre-filled syringe	Packaging is too hard to get things out easily. Why not have one part syringe so I don't have to make that as well as my factor	Fairly simple to use	Three-way tap, Leur-lock syringes	Easy to mix and does it quickly	Takes longer than current method	
10	Simplicity itself	No markings on the syringe	Small amount of equipment	Lots of fiddling and screwing together, need to swap syringes three times for 300iu dose. No markings on the syringe	Less equipment and easy to use	Three-way tap	Easy to remember and to do	FVIII left in bottle which I could get out with a 'proper' filter needle	No need to change from double-ended needle; if I'm about to stick a needle in why should I worry about needles
11	Pre-filled syringe makes it easy			Activation step seems silly, too difficult to get the end off the syringe	Easy and fun			Hard to fit device onto the bottles	
12	Easy to do and learn	R2 device not very easy to fit into the factor bottle	Very simple and easy to use		Looks like a beer barrel	Takes too long and is complicated	Simple, quite like I use now		I prefer to use one big syringe than to use three of the small ones
13	Easy to join it all together, no danger of hurting yourself		Easy to get FVIII out	Too slow and is hard to use, need two syringes for 2000 IU		Complicated and hard to use	Simple and easy to use	Hard to screw syringe onto the device	
14	Not as much clutter as current method	Packaging not environmentally friendly, can't they change it? Need two syringes for 3000 IU and it is hard to swap syringes	Simplicity of it all	Need three bottles which means three syringes or one (different) big syringe which is wasteful	Easy, can just have one big syringe		Simple and easy to use. 3000 IU in one syringe		Doesn't really matter – the important thing is injecting it

Table 3. Continued

Participant	ReFacto R2; pre-filled syringe device		BIOSET; pre-filled syringe device		BAXJECT; three-way tap device with separate syringe		Mix2Vial; filter device with separate syringe		Other comments
	Likes	Dislikes	Likes	Dislikes	Likes	Dislikes	Likes	Dislikes	
15	Pre-filled syringe	Would need three syringes for my dose, or one big syringe	Pre-filled syringe, its small and neat	Would need three syringes for my dose, or one big syringe	Easy, no needles, two bottles, 1 syringe; the three-way tap is clever		Safer than the other three devices and would only need one syringe		Would be great if they made a 3000 IU vial so I still had to use only one syringe

Participants were asked for their specific likes/dislikes about the devices tested as well as general comments on their treatment.

be reconstituted has an impact on whether or not the device is considered easier than double-ended needle methods. Two of the devices assessed in the study, BIOSET and ReFacto R2 devices, are pre-filled syringe devices. Whilst these devices have advantages in terms of simplicity of use, the contained dose affected the preference expressed for them, with more than one syringe necessary for children requiring doses greater than 2000 IU. This was considered by participants to be a major disadvantage of these devices, however, as this study there has been a steady introduction of larger dose vials of recombinant factors VIII and IX making single vial dosing a reality for most children.

Mix2Vial was the preferred device for the majority of participants. This device has a large volume capacity, which was a major advantage for many of the participants who were using a dose of at least 3000 IU and were used to self-infusing with a large syringe, separate from the transfer device. As a result, this feature was specifically cited as a 'like' by a number of participants. This is further supported by the observation that the participants administering the highest doses preferred the Mix2Vial device. Overall, the pre-filled syringe devices received the lowest number of 'preferred choice' selections, and BIOSET was ranked lowest by the majority of participants. As previously discussed, the low rate of preference for pre-filled syringes probably reflects the relatively low doses contained within the syringes, necessitating syringe swap or the use of an additional larger syringe for children taking higher doses. The low acceptance of the BAXJECT device was universal, and was, almost unanimously, a result of its three-way tap. Since this study was undertaken, Baxter has launched a second-generation transfer device, the BAXJECT II. This improved device eliminates the three-way tap and is suggested to make mixing of haemophilia therapies easier and faster than its predecessor [16]. In a study by Greer *et al.*, this was found to be more preferable than BIOSET or ReFacto R2 devices [17]. An additional vial adapter (made by Medinet, Munster, Germany) has also been launched by Novo Nordisk. Bayer has also improved the ease of use of BIOSET with the introduction of a 2000-IU vial size in 2007. This larger dose reduces reconstitution time by eliminating the need to mix and pool multiple vials for patients requiring higher doses. The preference of these newer devices has yet to be reported, but they should be considered when deciding which coagulation product to use to treat children.

The results from this and previously published studies on preference of needleless transfer devices

Table 4. Comparison of the features of the four transfer devices used in the study.

Reconstituted dose (volume)	BIOSET 1000 IU (2.5 mL)	ReFacto R2 2000 IU (4 mL)	BAXJECT 1500 IU (5 mL)	Mix2Vial 1000 IU (2.5 mL)
Device components	Pre-filled syringe	Pre-filled syringe	Two bottles connected by transfer device with integral three-way tap; separate syringe	Water and concentrate adapters connected by an integrated filter; separate syringe
Packaging	Letter coded	Colour coded	Colour coded	Colour coded
Transfer	Activation step followed by injection of diluent	Injection of diluent	Automatic mixing	Automatic mixing
Instructions for use	Attach pre-filled syringe to vial; 'Activate' syringe by pushing down hard on 'wings'; Connect plunger to syringe and inject remainder of diluents into factor, mix product; Draw product back into syringe	Attach R2 syringe to product vial; Inject diluent into vial and mix product; Draw product back into syringe	Attach BAXJECT to diluent vial and product vials; Diluent automatically transfers; Transfer product via three-way tap to expel vacuum; Draw product into syringe	Fix adapters onto the product and diluent vials; Diluent automatically transfers; Disconnect device; Draw product into syringe

clearly demonstrate their advantages regarding safety and ease of use. Furthermore, their use is strongly advocated by patients, caregivers and healthcare professionals. Within the healthcare profession, a number of benefits of transfer devices for haemophilia have been reported. The most universally reported advantage relates to that of ease of use: newer devices may simplify transfer, which is especially important when performed by children. Secondly, the improved safety offered by needle-free devices reduces the risk of needlestick injury and is seen to promote the use of such devices by children who may be less able to self-infuse. Taken together, these benefits have the potential to lead to improved concordance [11], which is by far the largest reported barrier to uptake of prophylaxis in both the United Kingdom and United States [2]. When put into context of the high number of children currently self-infusing coagulation factors, these benefits provide support for the immediate adoption of needleless transfer devices in clinical practice.

The results from this study provide valuable data on children's views of, and ability to use, transfer devices. Without exception, children over 8 years of age were able to manage all the devices; most appreciated the need for changing reconstitution methods and were readily adaptable to this. For some children, in whom a daily dose comprises one vial, a pre-filled syringe is an easy option; however, children requiring higher doses found the transfer devices to be preferable to the pre-filled syringes. In line with this observation, the Mix2Vial device was the most preferred option overall. Newer, advanced transfer devices offer a more user-friendly and convenient and effective way to reconstitute factors in the treatment of haemophilia.

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Disclosures

The author stated that she had no interests which might be perceived as posing a conflict or bias.

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Curriculum Vitae

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Qualifications:	SRN	1982
	RSCN	1988
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English National Board Courses

237	Oncology nursing	1986
924	HIV and AIDS	1998
998	Teaching & assessing	1990
N21	Genetic counselling	1993
U01	Essentials of haemophilia	1999

RCN Institute BSc (hons) in child health nursing

CH304D	Leadership for child health nurses	2000
CH305D	The child health nurse as strategist	2002

UKCC Higher levels of practice pilot 2001

Non-medical prescribing 2005

Current Post:

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Previous Posts:

July 1990 – Sept 2003	Clinical Nurse Specialist - Haemophilia Great Ormond Street Hospital for Children NHS Trust. London. WC1N 3JH.
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March 1988 - Dec 1989	Senior Staff Nurse Haematology/Oncology Ward. The Hospitals For Sick Children Great Ormond Street. London
Jan. 1987 - March 1988	Post Registration Student Nurse. Registered Sick Children's Nursing Course. The Hospitals For Sick Children. London.
June - Dec. 1986	Staff Nurse. Intra-Venous Therapy Team. Queen Elizabeth Medical Centre, Birmingham.
Sept. 1985 - June 1986	Post Registered Nursing Student. ENB Oncological Nursing Course (435). The Royal Marsden Hospital. London.
Jan 1984 - Aug. 1985	Senior Staff Nurse. Male Adult Oncology. Queen Elizabeth Medical Centre. Birmingham.
Nov. 1982 - Jan. 1984	Staff Nurse. General/Vascular surgery. East Birmingham Hospital. Birmingham.
May 1979 - Nov. 1982	Student Nurse. (SRN Training) East Birmingham Hospital. Birmingham.

Research bodies & Committees:

June 2013 -	Editorial Board – Advances in Medicine
Feb 2013 -	Member GOS Clinical Research Adoptions Committee
Oct 2012 -	Clinical Advisory Committee UK Haemophilia Society
July 2012 -	Global Inhibitor Diagnosis and Management Group
Feb 2012 -	UK Haemophilia Nurses Association (chair)
May 2011 -	Trustee UK Haemophilia Society
April 2011 -	Editorial Board Case Histories in Haematology Journal
May 2010 -	Nurses Committee - World Federation of Haemophilia (Secretary July 2012 -)
Mar 2009 -	Haemophilia Society Inhibitor Support Group Medical Advisory Panel
Jan 2009 – March 2011	Healthcare for London Tertiary Paediatric Services Review Committee
2008- 2009	Baxter Research Nurses Committee
Nov 2005 – Aug 2007	Little Bruisers Parent mentor scheme
Nov 2005 - 2009	Clinovia national clinical governance advisory board
Jan 2005 -	Member of Pednet working party
Jan 2004 -	Baxter (UK) nurses advisory board (chair)
May 2003 – July 2005	Baxter haemophilia nurses website editorial board
Jan 2003 – July 2003	Pan Thames Haemophilia Consortium service review working party
May 2002 – May 2012	GOS (Bloomsbury) Research Ethics Committee
May 2002 – 2006	Haemophilia society research funding committee
April 2002 - 2009	British Society of Haematology nurses committee (chair)
Jul 2001 – May 2002	Haemophilia Nurses Association nursing competencies working party
Oct 2000 -Jul 2001	GOS Latex allergy working party

Sep 2000 – Jun 2003	London regional specialised commissioning group - defining specialised services (haemophilia) working group.
Aug 2000 -	Pan Thames Haemophilia Consortium expert advisory group
Feb 2000 - Sept 2000	GOS Rehabilitation review working party
September 1999 - 2006	UK representative on European quality of life in children with haemophilia working group (HaemoQoL)
Sep 1999 – Oct 2005	Haemophilia Society Medical Advisory Panel
Mar 1999 – Mar 2002	Royal College of Nursing - Haemophilia Nurses Association committee member
Feb 1999 – May 2001	CNS representative - GOS Vision for Nursing Working Party
Oct 1998 - Jan 2001	UK representative on Wyeth/Genetics Institute Global Haemophilia Nurses Advisory Board.
Jan 1998 – Mar 2004	Member of GOS Patient Information Management System clinical implementation group.
Sep 1997 – Jan 2001	Member of GOS nursing strategy committee.
Jul 1997 - Dec. 2000	Member of GOS health records committee.
Aug 1994 - Feb 2004	Member of GOS blood transfusion committee.

Research interests + projects:

- Developing Haemophilia Nursing (2012)
- Girls and Young women with inherited bleeding disorders (2011)
- Children and young people (with bleeding disorders) as expert patients (2011)
- Social networking and expert patients (2009)
- The use of NovoSeven in children with Glanzmanns Thrombasthenia (2009)
- The impact of sport on quality of life in children and adults with haemophilia (2008)
- The lived experience of children with haemophilia (2008)
- Primary investigator (UK) PAIR study (2007)
- Primary investigator Octim study (2005)
- Primary investigator Children's opinions of transfer devices (2005)
- Primary investigator 'MediMop' study (2004)
- Study co-ordinator for recombinant factor VIII and IX trials in previously untreated (PUP), minimally treated (MTP) or previously treated (PTP) children. (1994 onwards) (CANAL and RODIN study groups)
- Study co-ordinator for gene mutation analysis in Hermansky-Pudlack syndrome. (1999)
- Primary investigator for use of NovoSeven in children with platelet disorders, and 'off label' use in intractable bleeding. (2003) (Glanzmanns Thrombasthenia Registry)
- Study co-ordinator for PFA analysis in children undergoing investigation of bleeding disorders and/or non accidental injury. (2000)
- Primary investigator (UK) of European Quality of Life (in children with haemophilia) study.
- Haemophilia society research initiative committee member.

Awards:

Haemophilia Buddy award 2012
Awarded to outstanding haemophilia nurses

Lady Claude Hamilton research and presentation award for nurses and allied health professionals
Awarded for paper into leadership in paediatric nurses. 2003

The UKCC (NMC) higher levels of practice (pilot) 2001

The Betty Barchard Bursary
Awarded to undertake study of haemophilia care in Bangladesh. 1995

Research Grants:

2013
Evaluation of the GaitRITE in children with haemophilia aged seven years and under
£25.000 Pfizer UK

2011
An expert patient programme for girls and young women with inherited bleeding disorders
£20,000 Bayer Caregivers award 2011

2010
STELLAR programme
£7,500 Different Disciplines, Common Experience Programme (Roald Dahl's Marvellous Children's Charity and Burdett Trust for Nursing)

Social networking for boys with haemophilia (in conjunction with VivaSix.com)
£96,000 Pfizer Ltd

2008
An evaluation of the lived experience of children with haemophilia
£10,000 Bayer Caregivers award 2008

2005
Involving children/young people with genetic disease, in genetic research.
£2,000 Bayer Healthcare Ltd.

Children's views of devices used for reconstitution of factor VIII/IX
£12,500 Wyeth Biotechnology Ltd.

2004
Patient, parent and health care worker acceptability of the MediMop™ device
£10,000 Wyeth Biotechnology Ltd.

Publications:

Multi-methodology research with boys with severe haemophilia
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HAEMO-QOL

Questionnaire for Children and Adolescents | Parents' long Version | age: 4-7

Hello, dear parent!
We would like to know how your son has been feeling during the past 4 weeks, so we have worked out a few questions which we would like you to answer.
This questionnaire was made for parents of young children with haemophilia.

Date of completion: |_|_|_|_|_|_|
(day/month/year)

Country: |_|_| Centre: |_|_| ID No.: |_|_|_|_|

HAEMO-QOL

QUESTIONNAIRE FOR PARENTS

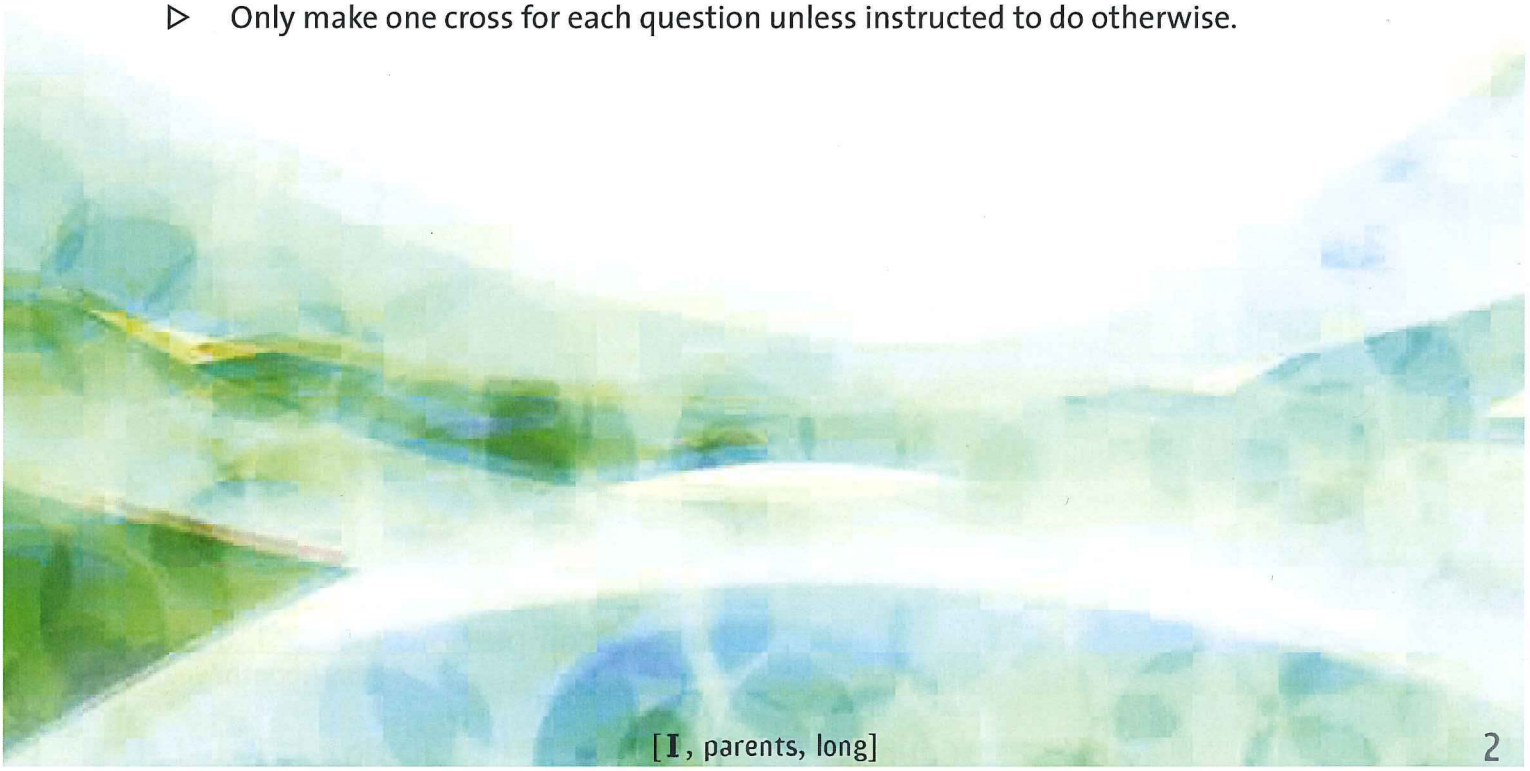
Dear Parent,

We really appreciate you are taking the time to complete this questionnaire about your son's well-being and health-related quality of life. This questionnaire is similar to the version for your children. We would like to know your assessment of your child's well-being. Please complete the questionnaire yourself according to the instructions, i.e. without asking your child.

All your answers will be treated with the strictest confidence!

For the following questions we would like to ask you to observe the instructions below:

- ▷ Only one parent, the person with whom the child relates most closely, should answer the questions and they should do so on their own.
- ▷ You are ☐ the mother? ☐ the father? ☐ Other:_____
- ▷ Read each question carefully.
- ▷ Think about how your child has been feeling during the past 4 weeks or what applies to your child.
- ▷ Please write the necessary answers on the lines provided or make a cross in the case of boxes.
- ▷ Put a cross in the box corresponding to the answer that fits your child best.
- ▷ Only make one cross for each question unless instructed to do otherwise.



Here we would like to know something about your child`s **BLEEDS** (JOINT BLEEDS).

1. How frequent were your child`s bleeds in the last 4 weeks?

☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many? ____

The following questions should only be answered if your child had bleeds.

2. How much was your child troubled by bleeds during the last 4 weeks?

☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit

3. How severe were your child`s bleeds during the last 4 weeks (if your child had several bleeds, please answer for the severest bleed)?

☐ slight ☐ moderate ☐ severe ☐ very severe

4. Did your child feel a strange sensation in his joints before he had a bleed?

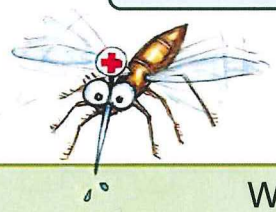
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. Did your child have to stay quiet (e.g. lie in bed) when he had bleeds?

☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

6. When your child had bleeds, did he inform you immediately?

☐ never ☐ seldom ☐ sometimes ☐ often ☐ always



We would like to know who gave your child **INJECTIONS**.

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child gave himself his injections	☐	☐	☐	☐	☐
2. ... I gave my child his injections	☐	☐	☐	☐	☐
3. ... my partner/spouse gave my child his injections	☐	☐	☐	☐	☐
4. ... a nurse gave my child his injections	☐	☐	☐	☐	☐
5. ... a doctor gave my child his injections	☐	☐	☐	☐	☐

Here we would like to know something about haemophilia and your child's **PHYSICAL HEALTH**.

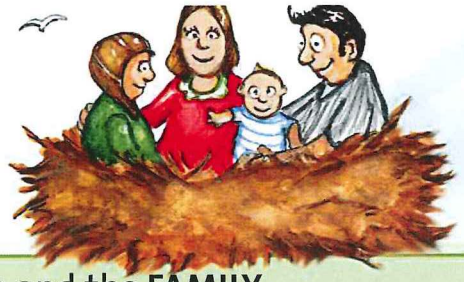
In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child's swellings hurt him	☐	☐	☐	☐	☐
2. ... it was difficult for my child to move his arms or legs	☐	☐	☐	☐	☐
3. ... my child was afraid of bleeds	☐	☐	☐	☐	☐
4. ... my child was afraid of hurting himself	☐	☐	☐	☐	☐

and now about how your child has been **FEELING** because of his haemophilia

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child was in a bad mood because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child was sad because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child's haemophilia made him angry	☐	☐	☐	☐	☐

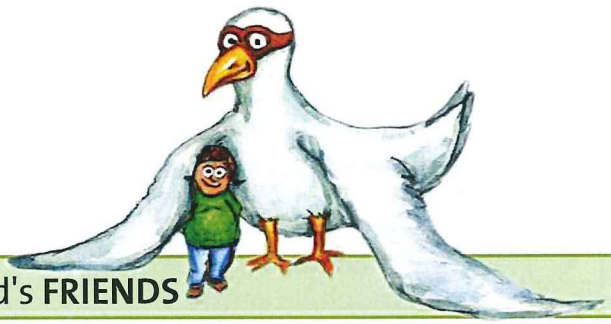
How does haemophilia affect your child's **VIEW OF HIMSELF**?

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child envied healthy boys his age	☐	☐	☐	☐	☐
2. ... my child felt embarrassed about his haemophilia	☐	☐	☐	☐	☐



The next questions are about haemophilia and the **FAMILY**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I protected my child too much	☐	☐	☐	☐	☐
2. ... my husband/wife protected our child too much	☐	☐	☐	☐	☐
3. ... I criticised my child when he hurt himself	☐	☐	☐	☐	☐
4. ... I forbade my child doing certain things because of his haemophilia	☐	☐	☐	☐	☐



and then about haemophilia and your child's **FRIENDS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child was unable to do as much with his friends because of his haemophilia	☐	☐	☐	☐	☐

These questions are about haemophilia and **OTHER PERSONS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child felt different from others because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child felt left out when others did things together	☐	☐	☐	☐	☐

[I, parents, long]

These questions are about **NURSERY SCHOOL/KINDERGARTEN**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child had to refrain from playing games that he enjoys because of haemophilia	☐	☐	☐	☐	☐
2. ... my child did just as much sports as any other kid	☐	☐	☐	☐	☐
3. ... my child was able to take part in games at nursery school/kinder-garten in spite of his haemophilia	☐	☐	☐	☐	☐

and the **TREATMENT**?

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
2. ... the injections annoyed my child	☐	☐	☐	☐	☐

your child' s **GLOBAL HEALTH**?

In general, ...	excellent	very good	good	fair	poor
1. ... would you say your child' s health is...	☐	☐	☐	☐	☐

1. How much is *your child* bothered by his haemophilia?
   ☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

2. How much are *you* bothered by his haemophilia?
   ☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

3. Which are the problems about having haemophilia and which are the problems about the treatment for haemophilia...

a) for your child

- _____
- _____
- _____
- _____

b) for yourself

- _____
- _____
- _____
- _____

Great job- congratulations!

THANK YOU FOR YOUR ASSISTANCE!

HAEMO-QOL

Questionnaire for Children and Adolescents | Kids' long Version | age: 8-12

Date of completion: |_|_|_|_|_|_|
(day/month/year)

Country: |_|_| Centre: |_|_| ID No.: |_|_|_|_|

Here we would like to know something about your **BLEEDS** (JOINT BLEEDS).

1. How frequent were your bleeds in the last 4 weeks?

☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many? ____

The following questions should only be answered if you had bleeds.

2. How much were you troubled by bleeds during the last 4 weeks?
☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit

3. How severe were your bleeds during the last 4 weeks
(if you had several bleeds, please answer for the severest bleed)?
☐ slight ☐ moderate ☐ severe ☐ very severe

4. Did you feel a strange sensation in your joints before you had a bleed?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. Did you have to stay quiet (e.g. lie in bed) when you had bleeds?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

6. When you had bleeds, did you inform your parents immediately?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

We would like to know who gave you **INJECTIONS** .

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I injected myself	☐	☐	☐	☐	☐
2. ... my mother injected me	☐	☐	☐	☐	☐
3. ... my father injected me	☐	☐	☐	☐	☐
4. ... a nurse injected me	☐	☐	☐	☐	☐
5. ... a doctor injected me	☐	☐	☐	☐	☐

Here we would like to know something about haemophilia and your **PHYSICAL HEALTH**.

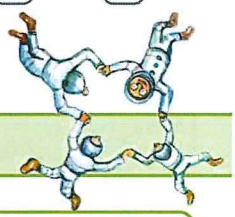
In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my swellings hurt	☐	☐	☐	☐	☐
2. ... I had pain in my joints	☐	☐	☐	☐	☐
3. ... it was painful for me to move	☐	☐	☐	☐	☐
4. ... my joints felt stiff	☐	☐	☐	☐	☐
5. ... it was difficult for me to move my arms or legs	☐	☐	☐	☐	☐
6. ... I had difficulty walking as far as I wanted to	☐	☐	☐	☐	☐
7. ... I was afraid of hurting myself	☐	☐	☐	☐	☐

and now about how you have been **FEELING** because of your haemophilia

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I was in a bad mood because of my haemophilia	☐	☐	☐	☐	☐
2. ... I was sad because of my haemophilia	☐	☐	☐	☐	☐
3. ... my haemophilia was a burden (real problem) for me	☐	☐	☐	☐	☐
4. ... my haemophilia made me angry	☐	☐	☐	☐	☐
5. ... I was worried because of my haemophilia	☐	☐	☐	☐	☐
6. ...I felt lonely because of my haemophilia	☐	☐	☐	☐	☐
7. ... I was afraid of bleeds	☐	☐	☐	☐	☐

How does haemophilia affect your **VIEW OF YOURSELF?**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I envied healthy boys my age	☐	☐	☐	☐	☐
2. ... I felt physically weaker than other boys	☐	☐	☐	☐	☐
3. ... I felt as well as other boys my age	☐	☐	☐	☐	☐
4. ... I felt contented about my body	☐	☐	☐	☐	☐
5. ... haemophilia made my life more difficult	☐	☐	☐	☐	☐
6. ... I felt embarrassed about my haemophilia	☐	☐	☐	☐	☐
7. ... I had difficulty doing things with other kids my age	☐	☐	☐	☐	☐
8. ... I was unable to do as much with my friends because of my haemophilia	☐	☐	☐	☐	☐
9. ... I felt healthy in spite of my haemophilia	☐	☐	☐	☐	☐



The next questions are about haemophilia and your **FAMILY**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my mother protected me too much	☐	☐	☐	☐	☐
2. ... my father protected me too much	☐	☐	☐	☐	☐
3. ... there were problems at home because of my haemophilia	☐	☐	☐	☐	☐
4. ... I felt I was causing my family trouble because of my haemophilia	☐	☐	☐	☐	☐
5. ... my parents limited their time at work or leisure because they had to look after me	☐	☐	☐	☐	☐

[Ⅱ, kids, long]

and then about haemophilia and your **FRIENDS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I was able to talk to my friends about my haemophilia	☐	☐	☐	☐	☐
2. ... my best friend cared about how I was feeling	☐	☐	☐	☐	☐
3. ... there was a best friend that I felt very close to	☐	☐	☐	☐	☐
4. ... my friends took care of me when I felt bad	☐	☐	☐	☐	☐

and then about haemophilia and your **PERCEIVED SUPPORT**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... others showed special consideration for me because of my haemophilia	☐	☐	☐	☐	☐
2. ... others showed understanding for my haemophilia	☐	☐	☐	☐	☐
3. ... I was able to talk to others about problems in connection with my haemophilia	☐	☐	☐	☐	☐
4. ... others stood by me	☐	☐	☐	☐	☐

These questions are about your haemophilia and **OTHER PERSONS**

In the past 4 weeks...

	never	seldom	some-times	often	all the time
1. ... I felt different from others because of my haemophilia	☐	☐	☐	☐	☐
2. ... I was bothered by others knowing about my haemophilia	☐	☐	☐	☐	☐
3. ... other kids teased me because of my haemophilia	☐	☐	☐	☐	☐
4. ... people behaved different towards me because of haemophilia	☐	☐	☐	☐	☐
5. ... I felt left out when others did things together	☐	☐	☐	☐	☐
6. ... others made dumb comments about my haemophilia	☐	☐	☐	☐	☐

These questions are about **SPORTS AND SCHOOL**

In the past 4 weeks...

	never	seldom	some-times	often	all the time
1. ... because of haemophilia I had to refrain from sports that I like	☐	☐	☐	☐	☐
2. ... I had to do indoor activities more than other kids because of my haemophilia	☐	☐	☐	☐	☐
3. ... I had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4. ... I did just as much sports as any other kid	☐	☐	☐	☐	☐
5. ... I participated in sports classes at school in spite of my haemophilia	☐	☐	☐	☐	☐
6. ... I was able to participate at school in spite of my haemophilia	☐	☐	☐	☐	☐
7. ... I had to refrain from special school events (e.g. outings) because of my haemophilia	☐	☐	☐	☐	☐
8. ... I found it difficult to concentrate at school because I was in pain	☐	☐	☐	☐	☐

[Ⅱ, kids, long]

The next questions are about **DEALING WITH HAEMOPHILIA**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2. ... I was careful with my body	☐	☐	☐	☐	☐
3. ... I was able to tell whether or not I was bleeding	☐	☐	☐	☐	☐
4. ... I was in control of my complaints due to haemophilia	☐	☐	☐	☐	☐
5. ... I felt well informed about haemophilia	☐	☐	☐	☐	☐
6. ... haemophilia was a normal part of my life	☐	☐	☐	☐	☐
7. ... I accepted having haemophilia	☐	☐	☐	☐	☐

and your **TREATMENT**?

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2. ... the treatment I got was okay	☐	☐	☐	☐	☐
3. ... I trusted my doctors and nurses	☐	☐	☐	☐	☐
4. ... I disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5. ... the injections annoyed me	☐	☐	☐	☐	☐
6. ... I was annoyed about the amount of time spent having injections	☐	☐	☐	☐	☐
7. ... I felt interrupted in my activities by the injections	☐	☐	☐	☐	☐

[Ⅱ, kids, long]

your **GLOBAL HEALTH?**

In general, ...	excellent	very good	good	fair	poor
1. ... would you say your health is...	☐	☐	☐	☐	☐

Open Questions

1. How much are you bothered by your haemophilia?
   ☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

2. What bothers you most about haemophilia? Please write down some things:
   - _____
   - _____
   - _____

3. How did you like the questionnaire?
   (Please put a mark on the line between "0" and "100", which most supplies you.)

   0 ——————————————————— 100
   very poor very good

4. Have we forgotten anything that is important to you?
   - _____
   - _____
   - _____

5. How long did it take to fill out the questionnaire? About ..... minutes

Great job- congratulations!

THANK YOU FOR YOUR ASSISTANCE!

HAEMO-QOL

Questionnaire for Children and Adolescents | Kids' long Version | age: 13-16

Date of completion: |_|_|_|_|_|_|
(day/month/year)

Country: |_|_| Centre: |_|_| ID No.: |_|_|_|_|

Here we would like to know something about your **BLEEDS** (JOINT BLEEDS).

1. How frequent were your bleeds in the last 4 weeks?

☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many? ____

The following questions should only be answered if you had bleeds.

2. How much were you troubled by bleeds during the last 4 weeks?
☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit

3. How severe were your bleeds during the last 4 weeks
(if you had several bleeds, please answer for the severest bleed)?
☐ slight ☐ moderate ☐ severe ☐ very severe

4. Did you feel a strange sensation in your joints before you had a bleed?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. Did you have to stay quiet (e.g. lie in bed) when you had bleeds?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

6. When you had bleeds, did you inform your parents immediately?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

We would like to know who gave you **INJECTIONS**.

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I injected myself	☐	☐	☐	☐	☐
2. ... my mother injected me	☐	☐	☐	☐	☐
3. ... my father injected me	☐	☐	☐	☐	☐
4. ... a nurse injected me	☐	☐	☐	☐	☐
5. ... a doctor injected me	☐	☐	☐	☐	☐

Here we would like to know something about haemophilia and your **PHYSICAL HEALTH**.

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my swellings hurt	☐	☐	☐	☐	☐
2. ... I had pain in my joints	☐	☐	☐	☐	☐
3. ... it was painful for me to move	☐	☐	☐	☐	☐
4. ... my joints felt stiff	☐	☐	☐	☐	☐
5. ... it was difficult for me to move my arms or legs	☐	☐	☐	☐	☐
6. ... I had difficulty walking as far as I wanted to	☐	☐	☐	☐	☐
7. ... I was afraid of hurting myself	☐	☐	☐	☐	☐

and now about how you have been **FEELING** because of your haemophilia

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I was in a bad mood because of my haemophilia	☐	☐	☐	☐	☐
2. ... I was sad because of my haemophilia	☐	☐	☐	☐	☐
3. ... my haemophilia was a burden (real problem) for me	☐	☐	☐	☐	☐
4. ... my haemophilia made me angry	☐	☐	☐	☐	☐
5. ... I was worried because of my haemophilia	☐	☐	☐	☐	☐
6. ... I felt lonely because of my haemophilia	☐	☐	☐	☐	☐
7. ... I was afraid of bleeds	☐	☐	☐	☐	☐
8. ... I felt excluded by my friends	☐	☐	☐	☐	☐

[III , kids, long]

How does haemophilia affect your **VIEW OF YOURSELF?**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I envied healthy boys my age	☐	☐	☐	☐	☐
2. ... I felt physically weaker than other boys	☐	☐	☐	☐	☐
3. ... I felt as well as other boys my age	☐	☐	☐	☐	☐
4. ... I felt contented about my body	☐	☐	☐	☐	☐
5. ... haemophilia made my life more difficult	☐	☐	☐	☐	☐
6. ... I was happy in spite of my haemophilia	☐	☐	☐	☐	☐
7. ... I felt embarrassed about my haemophilia	☐	☐	☐	☐	☐
8. ... I had difficulty doing things with other kids my age	☐	☐	☐	☐	☐
9. ... I was unable to do as much with my friends because of my haemophilia	☐	☐	☐	☐	☐
10. ... I felt healthy in spite of my haemophilia	☐	☐	☐	☐	☐

[Ⅲ, kids, long]

The next questions are about haemophilia and your **FAMILY**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I was treated differently by my family because of my haemophilia	☐	☐	☐	☐	☐
2. ... my mother protected me too much	☐	☐	☐	☐	☐
3. ... my father protected me too much	☐	☐	☐	☐	☐
4. ... my parents criticised me when I hurt myself	☐	☐	☐	☐	☐
5. ... my parents forbade me to do certain things because of my haemophilia	☐	☐	☐	☐	☐
6. ... there were problems at home because of my haemophilia	☐	☐	☐	☐	☐
7. ... I felt I was causing my family trouble because of my haemophilia	☐	☐	☐	☐	☐
8. ... my parents limited their time at work or leisure because they had to look after me	☐	☐	☐	☐	☐

and then about haemophilia and your **FRIENDS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I was able to talk to my friends about my haemophilia	☐	☐	☐	☐	☐
2. ... my best friend cared about how I was feeling	☐	☐	☐	☐	☐
3. ... there was a best friend that I felt very close to	☐	☐	☐	☐	☐
4. ... my friends took care of me when I felt bad	☐	☐	☐	☐	☐

These questions are about your haemophilia and **PERCEIVED SUPPORT**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... others showed special consideration for me because of my haemophilia	☐	☐	☐	☐	☐
2. ... others showed understanding for my haemophilia	☐	☐	☐	☐	☐
3. ... I was able to talk to others about problems in connection with my haemophilia	☐	☐	☐	☐	☐
4. ... others stood by me	☐	☐	☐	☐	☐

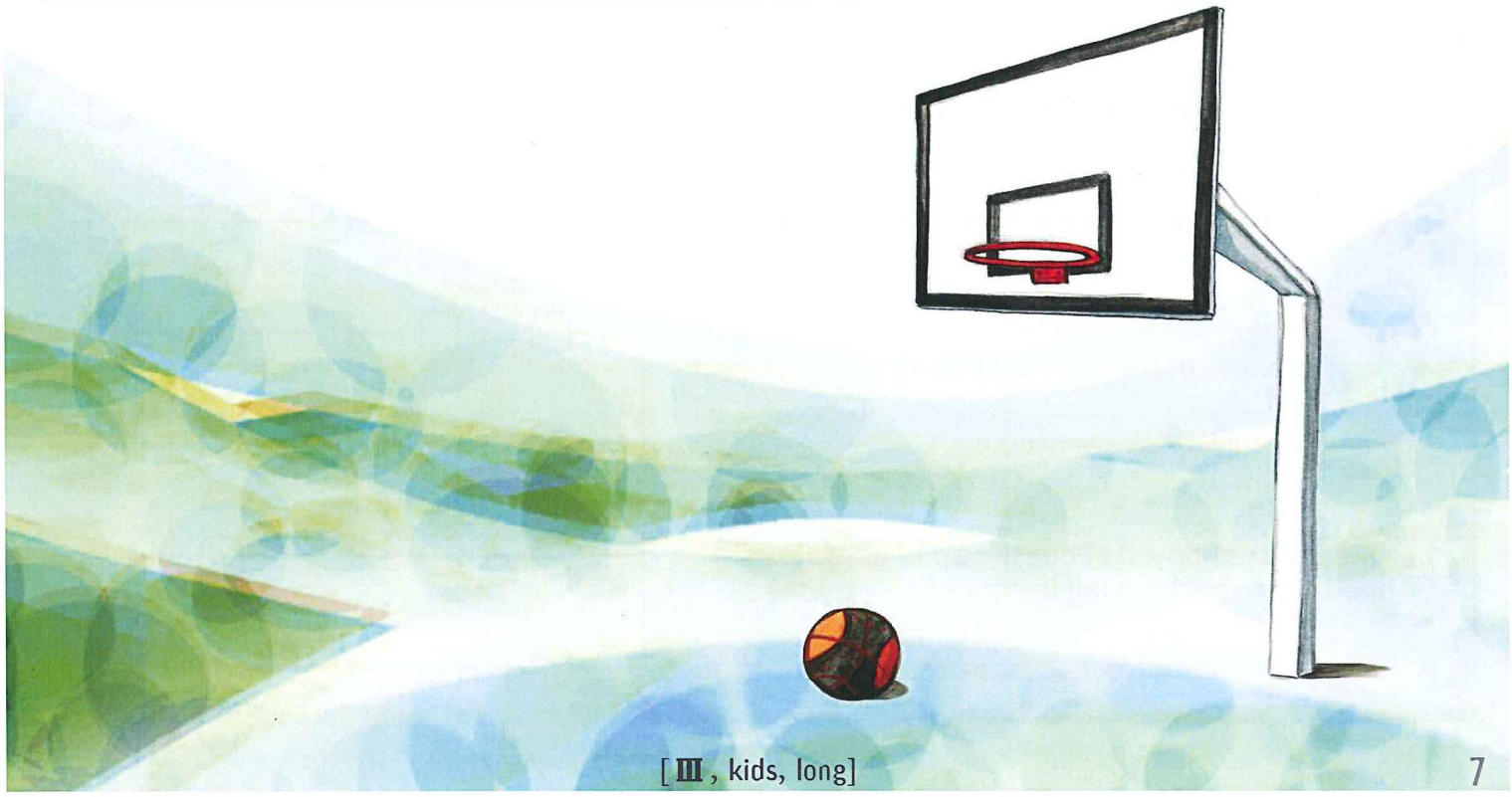
These questions are about your haemophilia and **OTHER PERSONS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I felt different from others because of my haemophilia	☐	☐	☐	☐	☐
2. ... I was bothered by others knowing about my haemophilia	☐	☐	☐	☐	☐
3. ... other kids teased me because of my haemophilia	☐	☐	☐	☐	☐
4. ... people behaved different towards me because of haemophilia	☐	☐	☐	☐	☐
5. ... I felt left out when others did things together	☐	☐	☐	☐	☐
6. ... others made dumb comments about my haemophilia	☐	☐	☐	☐	☐

These questions are about **SPORTS AND SCHOOL**

In the past 4 weeks...

	never	seldom	some-times	often	all the time
1. ... because of haemophilia I had to refrain from sports that I like	☐	☐	☐	☐	☐
2. ... I had to do indoor activities more than other kids because of my haemophilia	☐	☐	☐	☐	☐
3. ... I had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4. ... I did just as much sports as any other kid	☐	☐	☐	☐	☐
5. ... I was treated differently by teachers because of my haemophilia	☐	☐	☐	☐	☐
6. ... I participated in sports classes at school in spite of my haemophilia	☐	☐	☐	☐	☐
7. ... I was able to participate at school in spite of my haemophilia	☐	☐	☐	☐	☐
8. ... I had to refrain from special school events (e.g. outings) because of my haemophilia	☐	☐	☐	☐	☐
9. ... I found it difficult to pay attention at school because I was in pain	☐	☐	☐	☐	☐



The next questions are about **DEALING WITH HAEMOPHILIA**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2. ... I was careful with my body	☐	☐	☐	☐	☐
3. ... I was able to tell whether or not I was bleeding	☐	☐	☐	☐	☐
4. ... I was in control of my complaints due to haemophilia	☐	☐	☐	☐	☐
5. ... I felt well informed about haemophilia	☐	☐	☐	☐	☐
6. ... haemophilia was a normal part of my life	☐	☐	☐	☐	☐
7. ... I accepted having haemophilia	☐	☐	☐	☐	☐

and your **TREATMENT**?

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2. ... the treatment I got was okay	☐	☐	☐	☐	☐
3. ... I trusted my doctors and nurses	☐	☐	☐	☐	☐
4. ... I disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5. ... I felt dependent on others because of my haemophilia	☐	☐	☐	☐	☐
6. ... the injections annoyed me	☐	☐	☐	☐	☐
7. ... I was annoyed about the amount of time spent having injections	☐	☐	☐	☐	☐
8. ... I felt interrupted in my activities by the injections	☐	☐	☐	☐	☐

What do you think about the **FUTURE**?

Recently...

	never	seldom	some-times	often	all the time
1. ... I have been thinking that it will be difficult for me to lead a normal life	☐	☐	☐	☐	☐
2. ... I have been expecting that things will get better as I grow older	☐	☐	☐	☐	☐
3. ... I have been worrying about my health	☐	☐	☐	☐	☐
4. ... I have been sure about having a family later on	☐	☐	☐	☐	☐

What about **RELATIONSHIPS**?

Recently...

	never	seldom	some-times	often	all the time
1. ... I have been finding it difficult to date because of my haemophilia	☐	☐	☐	☐	☐
2. ... I have been insecure in my relationship with girls because of my haemophilia	☐	☐	☐	☐	☐

your **GLOBAL HEALTH**?

In general, ...

	excellent	very good	good	fair	poor
1. ... would you say your health is...	☐	☐	☐	☐	☐

[Ⅲ, kids, long]

1. How much are you bothered by your haemophilia?
   ☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

2. What bothers you most about haemophilia? Please write down some things:

   • _____
   • _____
   • _____

3. How did you like the questionnaire?
   (Please put a mark on the line between "0" and "100", which most supplies you.)

   0 100
   very poor very good

4. Have we forgotten anything that is important to you?

   • _____
   • _____
   • _____

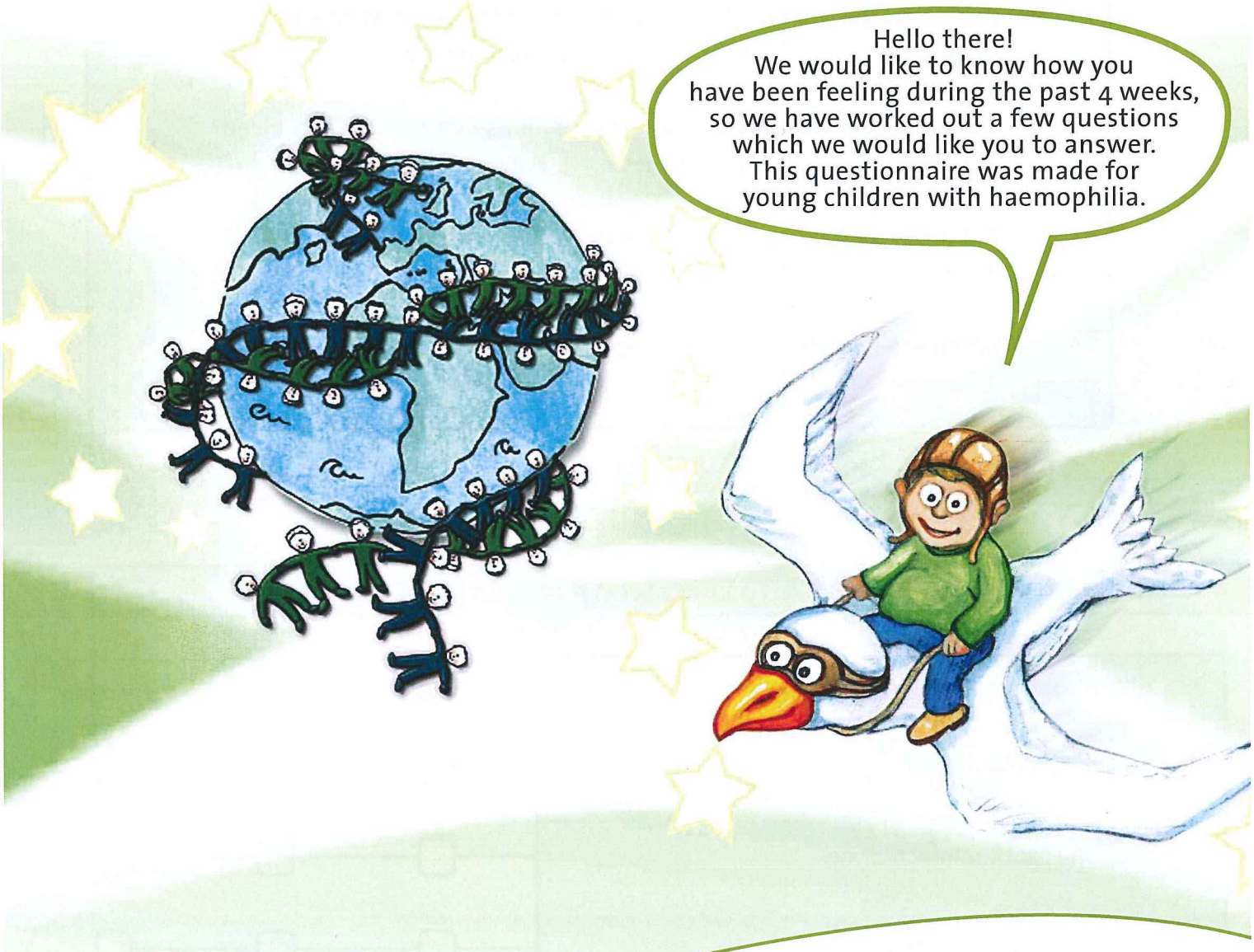
5. How long did it take to fill out the questionnaire? About ..... minutes

Great job- congratulations!

THANK YOU FOR YOUR ASSISTANCE!

HAEMO-QOL

Questionnaire for Children and Adolescents | Kids' long Version | age: 4-7



Date of completion: |_|_|_|_|_|_|
(day/month/year)

Country: |_|_| Centre: |_|_| ID No.: |_|_|_|_|

Here we would like to know something about your **BLEEDS** (JOINT BLEEDS).

1. How frequent were your bleeds in the last 4 weeks?

☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many? ____

The following questions should only be answered if the child had bleeds.

2. How much were you troubled by bleeds during the last 4 weeks?

☐ not at all ☐ somewhat ☐ very much

3. How severe were your bleeds during the last 4 weeks
(if you had several bleeds, please answer for the severest bleed)?

☐ slight ☐ moderate ☐ very severe

4. Did you feel a strange sensation in your joints before you had a bleed?

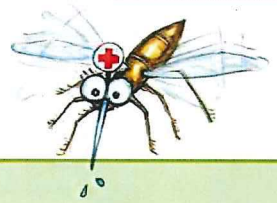
☐ never ☐ sometimes ☐ always

5. Did you have to stay quiet (e.g. lie in bed) when you had bleeds?

☐ never ☐ sometimes ☐ always

6. When you had bleeds, did you inform your parents immediately?

☐ never ☐ sometimes ☐ always



We would like to know who gave you **INJECTIONS** .

In the past 4 weeks...	never	sometimes	very often
1. ... I injected myself	☐	☐	☐
2. ... my mother injected me	☐	☐	☐
3. ... my father injected me	☐	☐	☐
4. ... a nurse injected me	☐	☐	☐
5. ... a doctor injected me	☐	☐	☐

Here we would like to know something about haemophilia and your **PHYSICAL HEALTH**.

In the past 4 weeks...	never	sometimes	very often
1. ... my swellings hurt	☐	☐	☐
2. ... it was difficult for me to move my arms or legs	☐	☐	☐
3. ... I was afraid of bleeds	☐	☐	☐
4. ... I was afraid of hurting myself	☐	☐	☐

How have you been **FEELING** because of your haemophilia?

In the past 4 weeks...	never	sometimes	very often
1. ... I was in a bad mood because of my haemophilia	☐	☐	☐
2. ... I was sad because of my haemophilia	☐	☐	☐
3. ... my haemophilia made me angry	☐	☐	☐

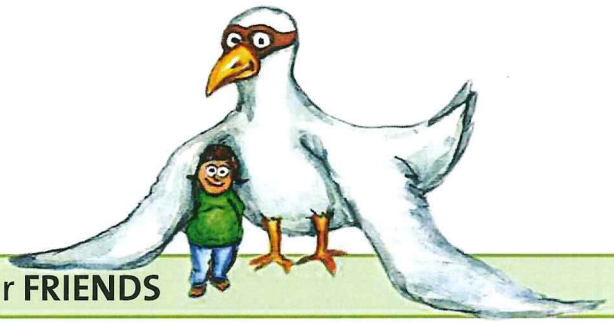
How does haemophilia affect your **VIEW OF YOURSELF**?

In the past 4 weeks...	never	sometimes	very often
1. ... I envied healthy boys my age	☐	☐	☐
2. ... I felt embarrassed about my haemophilia	☐	☐	☐

[I, kids, long]

The next questions are about haemophilia and your **FAMILY**

In the past 4 weeks...	never	sometimes	very often
1. ... my mother protected me too much	☐	☐	☐
2. ... my father protected me too much	☐	☐	☐
3. ... my parents told me off when I hurt myself	☐	☐	☐
4. ... my parents forbade me to do certain things because of my haemophilia	☐	☐	☐



and then about haemophilia and your **FRIENDS**

In the past 4 weeks...	never	sometimes	very often
1. ... I was unable to do as much with my friends because of my haemophilia	☐	☐	☐

These questions are about your haemophilia and **OTHER PEOPLE**

In the past 4 weeks...	never	sometimes	very often
1. ... I felt different from others because of my haemophilia	☐	☐	☐
2. ... I felt left out when others did things together	☐	☐	☐

These questions are about **NURSERY SCHOOL/KINDERGARTEN**

In the past 4 weeks...	never	sometimes	very often
1. ... because of haemophilia I had to refrain from playing games that I enjoy	☐	☐	☐
2. ... I did just as much sports as many other kids	☐	☐	☐
3. ... I was able to take part in games at nursery school/ kindergarten in spite of my haemophilia	☐	☐	☐

and your **TREATMENT**?

In the past 4 weeks...	never	sometimes	very often
1. ... I disliked visiting the haemophilia centre	☐	☐	☐
2. ... the injections annoyed me	☐	☐	☐

your **GLOBAL HEALTH**?

In general, ...	excellent	good	poor
1. ... would you say your health is...	☐	☐	☐

Open Questions

1. How much are you bothered by your haemophilia?
   ☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

2. What bothers you most about haemophilia? Please write down some things:

   • _____
   • _____
   • _____

3. How did you like the questionnaire?


don`t like it


ok


good

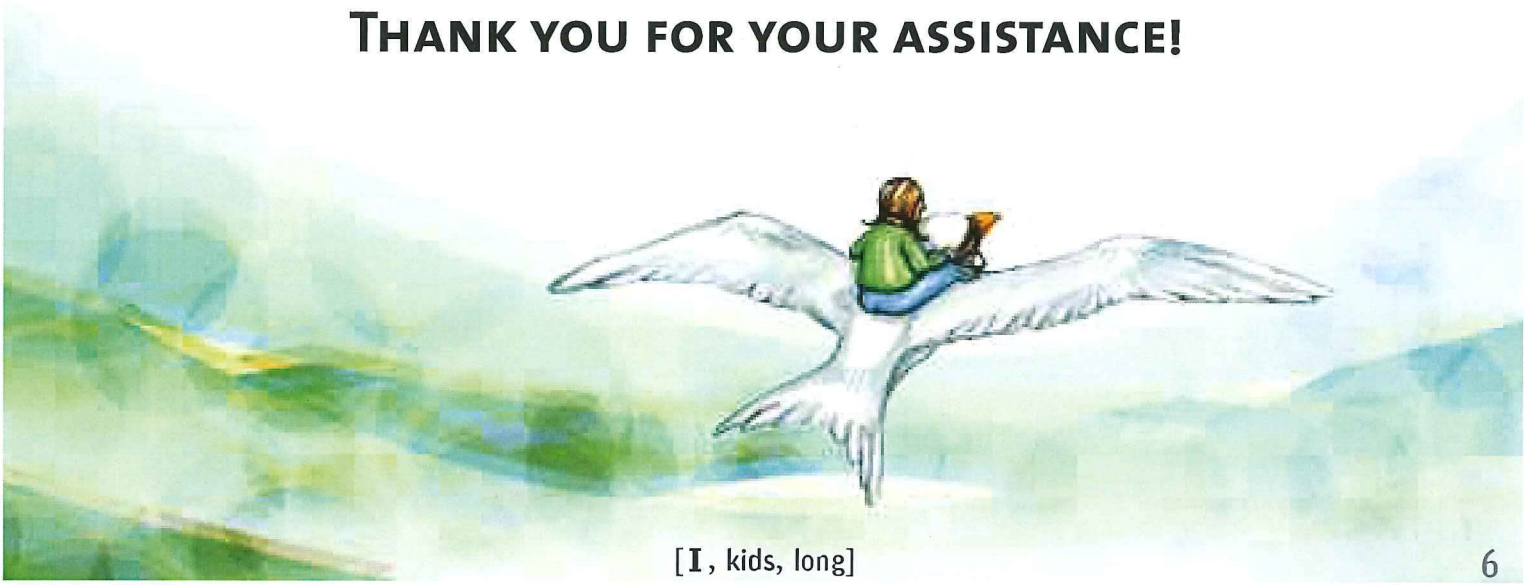
4. Have we forgotten anything that is important to you?

   • _____
   • _____
   • _____

5. How long did it take to fill out the questionnaire? About ..... minutes

Great job- congratulations!

THANK YOU FOR YOUR ASSISTANCE!



[I, kids, long]

HAEMO-QOL

Questionnaire for Children and Adolescents | Parents' long Version | age: 8-12

Date of completion: |_|_|_|_|_|_|
(day/month/year)

Country: |_|_| Centre: |_|_| ID No.: |_|_|_|_|

HAEMO-QOL

QUESTIONNAIRE FOR PARENTS

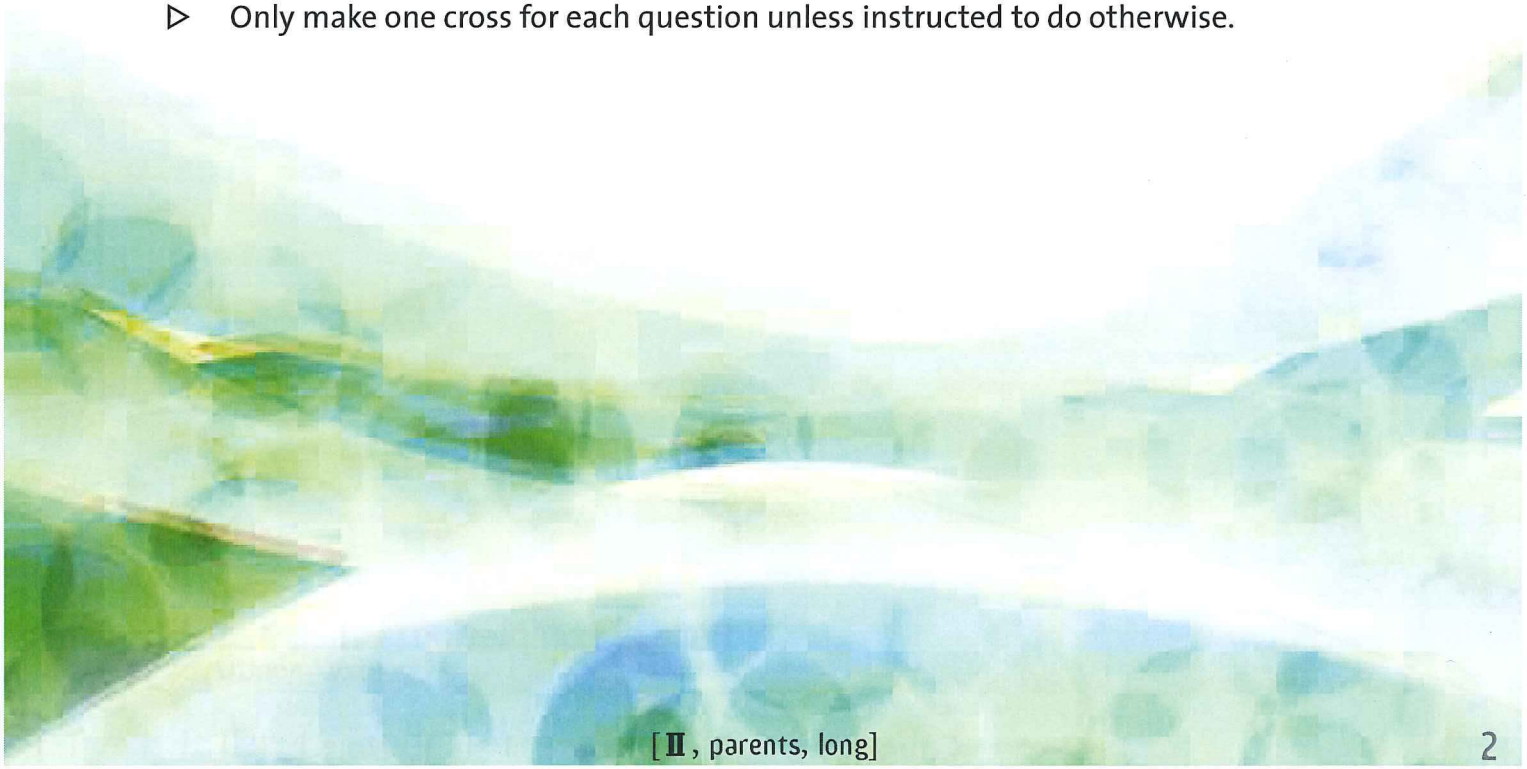
Dear Parent,

We really appreciate you are taking the time to complete this questionnaire about your son's well-being and health-related quality of life. This questionnaire is similar to the version for your children. We would like to know your assessment of your child's well-being. Please complete the questionnaire yourself according to the instructions, i.e. without asking your child.

All your answers will be treated with the strictest confidence!

For the following questions we would like to ask you to observe the instructions below:

- ▷ Only one parent, the person with whom the child relates most closely, should answer the questions and they should do so on their own.
- ▷ You are ☐ the mother? ☐ the father? ☐ Other:_____
- ▷ Read each question carefully.
- ▷ Think about how your child has been feeling during the past 4 weeks or what applies to your child.
- ▷ Please write the necessary answers on the lines provided or make a cross in the case of boxes.
- ▷ Put a cross in the box corresponding to the answer that fits your child best.
- ▷ Only make one cross for each question unless instructed to do otherwise.



Here we would like to know something about your child`s **BLEEDS** (JOINT BLEEDS).

1. How frequent were your child`s bleeds in the last 4 weeks?

☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many? ____

The following questions should only be answered if your child had bleeds.

2. How much was your child troubled by bleeds during the last 4 weeks?
☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit

3. How severe were your child`s bleeds during the last 4 weeks
(if your child had several bleeds, please answer for the severest bleed)?
☐ slight ☐ moderate ☐ severe ☐ very severe

4. Did your child feel a strange sensation in his joints before he had a bleed?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. Did your child have to stay quiet (e.g. lie in bed) when he had bleeds?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

6. When your child had bleeds, did he inform you immediately?
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

We would like to know who gave your child **INJECTIONS**.

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child gave himself his injections	☐	☐	☐	☐	☐
2. ... I gave my child his injections his injections	☐	☐	☐	☐	☐
3. ... my partner/spouse gave my child his injections	☐	☐	☐	☐	☐
4. ... a nurse gave my child his injections	☐	☐	☐	☐	☐
5. ... a doctor gave my child his injections	☐	☐	☐	☐	☐

Here we would like to know something about haemophilia and your child's **PHYSICAL HEALTH**.

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child's swellings hurt	☐	☐	☐	☐	☐
2. ... my child had pain in his joints	☐	☐	☐	☐	☐
3. ... it was painful for my child to move	☐	☐	☐	☐	☐
4. ... my child's joints felt stiff	☐	☐	☐	☐	☐
5. ... it was difficult for my child to move his arms or legs	☐	☐	☐	☐	☐
6. ... my child had difficulty walking as far as he wanted to	☐	☐	☐	☐	☐
7. ... my child was afraid of hurting himself	☐	☐	☐	☐	☐

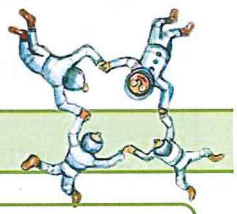
and now about how your child has been **FEELING** because of his haemophilia

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child was in a bad mood because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child was sad because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child's haemophilia was a burden to him	☐	☐	☐	☐	☐
4. ... my child was angry because of his haemophilia	☐	☐	☐	☐	☐
5. ... my child was worried about his haemophilia	☐	☐	☐	☐	☐
6. ... my child felt lonely because of his haemophilia	☐	☐	☐	☐	☐
7. ... my child was afraid of bleeds	☐	☐	☐	☐	☐

[II, parents, long]

How does haemophilia affect your child's **VIEW OF HIMSELF?**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child envied healthy boys his age	☐	☐	☐	☐	☐
2. ... my child felt physically weaker than other boys	☐	☐	☐	☐	☐
3. ... my child felt as well as other boys his age	☐	☐	☐	☐	☐
4. ... my child felt contented about his body	☐	☐	☐	☐	☐
5. ... haemophilia made my child's life more difficult	☐	☐	☐	☐	☐
6. ... my child felt embarrassed about his haemophilia	☐	☐	☐	☐	☐
7. ... my child had difficulty doing things with other kids his age	☐	☐	☐	☐	☐
8. ... my child was unable to do as much with his friends because of his haemophilia	☐	☐	☐	☐	☐
9. ... my child felt healthy in spite of his haemophilia	☐	☐	☐	☐	☐



The next questions are about haemophilia and the **FAMILY**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... I protected my child too much	☐	☐	☐	☐	☐
2. ... my husband/wife protected our child too much	☐	☐	☐	☐	☐
3. ... there were problems at home because of his haemophilia	☐	☐	☐	☐	☐
4. ... my child felt that he was causing his family trouble because of his haemophilia	☐	☐	☐	☐	☐
5. ... I limited my time at work or leisure because I had to look after my child	☐	☐	☐	☐	☐

[II, parents, long]

and then about haemophilia and your child's **FRIENDS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child was able to talk to his friends about his haemophilia	☐	☐	☐	☐	☐
2. ... my child's best friend cared about how my child was feeling	☐	☐	☐	☐	☐
3. ... there was a best friend that my child felt very close to	☐	☐	☐	☐	☐
4. ... my child's friends took care of him when he felt bad	☐	☐	☐	☐	☐

and then about haemophilia and **PERCEIVED SUPPORT**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... others showed special consideration for my child because of his haemophilia	☐	☐	☐	☐	☐
2. ... others showed understanding for my child's haemophilia	☐	☐	☐	☐	☐
3. ... my child was able to talk to others about problems connected with his haemophilia	☐	☐	☐	☐	☐
4. ... others stood by my child	☐	☐	☐	☐	☐

These questions are about your child's haemophilia and **OTHER PERSONS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child felt different from others because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child was bothered by others knowing about his haemophilia	☐	☐	☐	☐	☐
3. ... my child was teased by other kids because of his haemophilia	☐	☐	☐	☐	☐
4. ... people behaved different towards my child because of his haemophilia	☐	☐	☐	☐	☐
5. ... my child felt left out when others did things together	☐	☐	☐	☐	☐
6. ... other people made 'dumb' comments to my child about his haemophilia	☐	☐	☐	☐	☐

These questions are about **SPORTS AND SCHOOL**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child had to refrain from sports that he likes because of haemophilia	☐	☐	☐	☐	☐
2. ... my child had to do indoor activities more than other kids because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4. ... my child did just as much sports as any other kid	☐	☐	☐	☐	☐
5. ... my child participated in sports classes at school in spite of his haemophilia	☐	☐	☐	☐	☐
6. ... my child was able to participate at school in spite of his haemophilia	☐	☐	☐	☐	☐
7. ... my child had to refrain from special school events (e.g. outings) because of his haemophilia	☐	☐	☐	☐	☐
8. ... my child found it difficult to concentrate at school because he was in pain	☐	☐	☐	☐	☐

[II, parents, long]

The next questions are about **DEALING WITH HAEMOPHILIA**

In the past 4 weeks...

	never	seldom	some-times	often	all the time
1. ... my child tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2. ... my child was careful with his body	☐	☐	☐	☐	☐
3. ... my child was able to tell whether or not he was bleeding	☐	☐	☐	☐	☐
4. ... my child was in control of his complaints due to haemophilia	☐	☐	☐	☐	☐
5. ... my child felt well-informed about haemophilia	☐	☐	☐	☐	☐
6. ... haemophilia was a normal part of his life	☐	☐	☐	☐	☐
7. ... my child accepted having haemophilia	☐	☐	☐	☐	☐

and the **TREATMENT?**

In the past 4 weeks...

	never	seldom	some-times	often	all the time
1. ... my child was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2. ... the treatment my child got was okay	☐	☐	☐	☐	☐
3. ... my child trusted his doctors and nurses	☐	☐	☐	☐	☐
4. ... my child disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5. ... the injections annoyed my child	☐	☐	☐	☐	☐
6. ... my child was annoyed about the amount of time spent having the injections	☐	☐	☐	☐	☐
7. ... my child felt interrupted in his activities by the injections	☐	☐	☐	☐	☐

[Ⅱ, parents, long]

your child' s **GLOBAL HEALTH?**

In general, ...

1. ... would you say your child' s health is...

excellent | very good | good | fair | poor

Open Questions

1. How much is *your child* bothered by his haemophilia?
   ☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

2. How much are *you* bothered by his haemophilia?
   ☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

3. Which are the problems about having haemophilia and which are the problems about the treatment for haemophilia...

   a) for your child
   - _____
   - _____
   - _____
   - _____

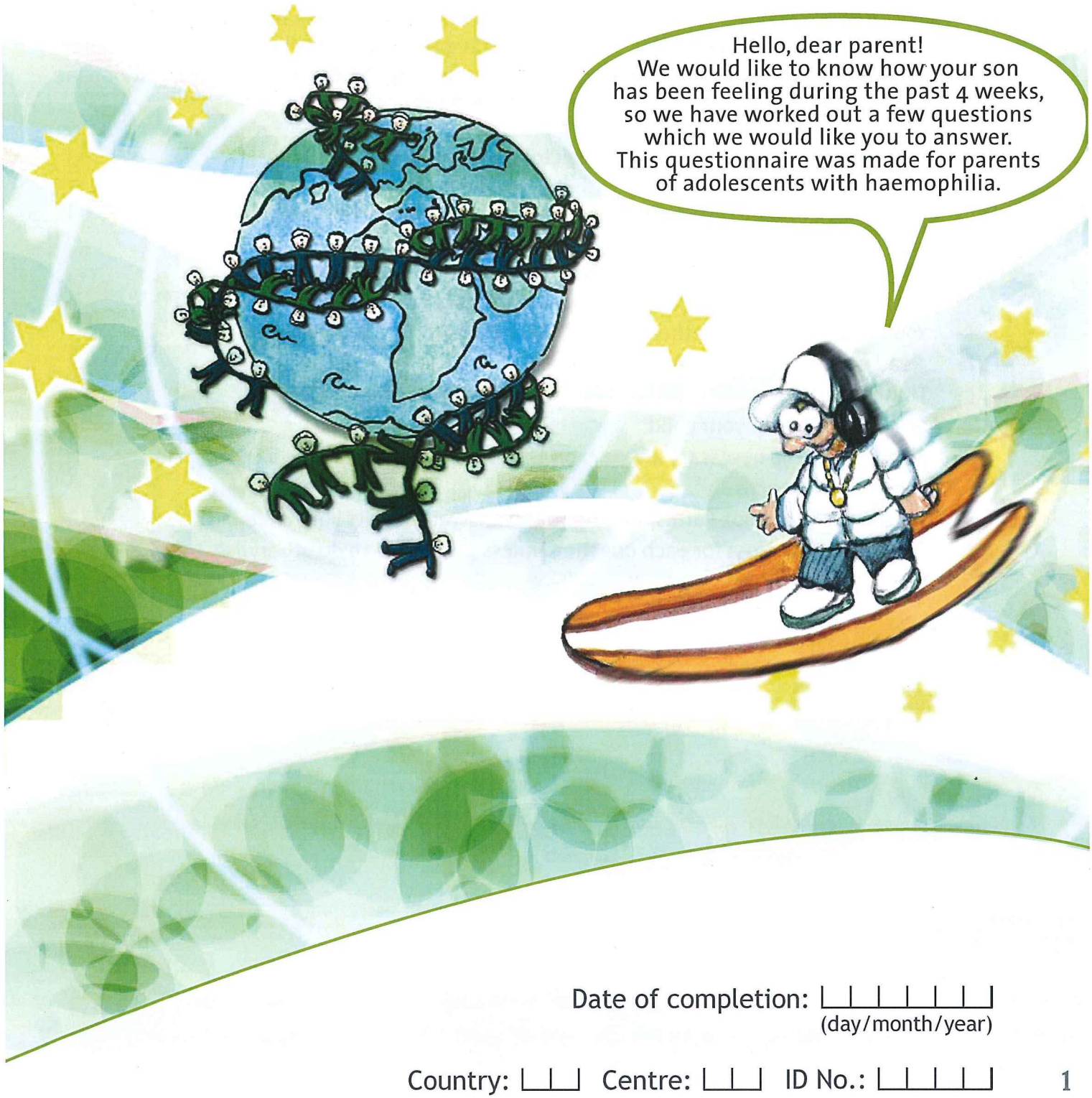
   b) for yourself
   - _____
   - _____
   - _____
   - _____

Great job- congratulations!

THANK YOU FOR YOUR ASSISTANCE!

HAEMO-QOL

Questionnaire for Children and Adolescents | Parents' long Version | age: 13-16



Date of completion: |_|_|_|_|_|_|
(day/month/year)

Country: |_|_| Centre: |_|_| ID No.: |_|_|_|_|

HAEMO-QOL

QUESTIONNAIRE FOR PARENTS

Dear Parent,

We really appreciate you are taking the time to complete this questionnaire about your son's well-being and health-related quality of life. This questionnaire is similar to the version for your children. We would like to know your assessment of your child's well-being. Please complete the questionnaire yourself according to the instructions, i.e. without asking your child.

All your answers will be treated with the strictest confidence!

For the following questions we would like to ask you to observe the instructions below:

- ▷ Only one parent, the person with whom the child relates most closely, should answer the questions and they should do so on their own.
- ▷ You are ☐ the mother? ☐ the father? ☐ Other:_____
- ▷ Read each question carefully.
- ▷ Think about how your child has been feeling during the past 4 weeks or what applies to your child.
- ▷ Please write the necessary answers on the lines provided or make a cross in the case of boxes.
- ▷ Put a cross in the box corresponding to the answer that fits your child best.
- ▷ Only make one cross for each question unless instructed to do otherwise.

Here we would like to know something about your child`s **BLEEDS** (JOINT BLEEDS).

1. How frequent were your child`s bleeds in the last 4 weeks?

☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many? ____

The following questions should only be answered if your child had bleeds.

2. How much was your child troubled by bleeds during the last 4 weeks?

☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit ☐ very much

3. How severe were your child`s bleeds during the last 4 weeks (if your child had several bleeds, please answer for the severest bleed)?

☐ slight ☐ moderate ☐ severe ☐ very severe

4. Did your child feel a strange sensation in his joints before he had a bleed?

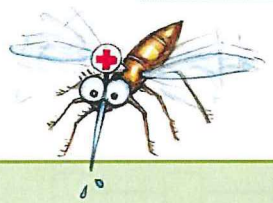
☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. Did your child have to stay quiet (e.g. lie in bed) when he had bleeds?

☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

6. When your child had bleeds, did he inform you immediately?

☐ never ☐ seldom ☐ sometimes ☐ often ☐ always



We would like to know who gave your child **INJECTIONS**.

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child gave himself his injections	☐	☐	☐	☐	☐
2. ... I gave my child his injections	☐	☐	☐	☐	☐
3. ... my partner/spouse gave my child his injections	☐	☐	☐	☐	☐
4. ... a nurse gave my child his injections	☐	☐	☐	☐	☐
5. ... a doctor gave my child his injections	☐	☐	☐	☐	☐

Here we would like to know something about haemophilia and your child's **PHYSICAL HEALTH**.

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child's swellings hurt	☐	☐	☐	☐	☐
2. ... my child had pain in his joints	☐	☐	☐	☐	☐
3. ... it was painful for my child to move	☐	☐	☐	☐	☐
4. ... my child's joints felt stiff	☐	☐	☐	☐	☐
5. ... it was difficult for my child to move his arms or legs	☐	☐	☐	☐	☐
6. ... my child had difficulty walking as far as he wanted to	☐	☐	☐	☐	☐
7. ... my child was afraid of hurting himself	☐	☐	☐	☐	☐

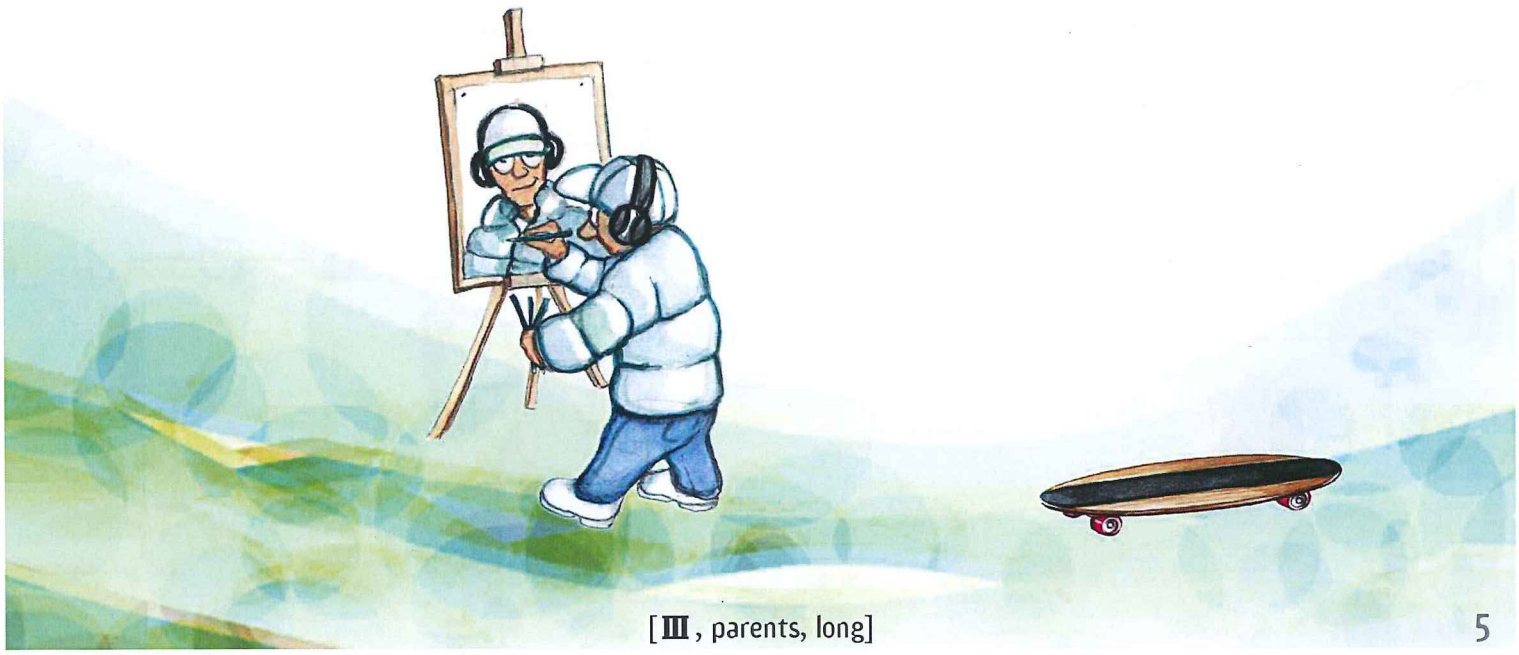
and now about how your child has been **FEELING** because of his haemophilia

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child was in a bad mood because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child was sad because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child's haemophilia was a burden to him	☐	☐	☐	☐	☐
4. ... my child was angry because of his haemophilia	☐	☐	☐	☐	☐
5. ... my child was worried about his haemophilia	☐	☐	☐	☐	☐
6. ... my child felt lonely because of his haemophilia	☐	☐	☐	☐	☐
7. ... my child was afraid of bleeds	☐	☐	☐	☐	☐
8. ... my child felt excluded by his friends	☐	☐	☐	☐	☐

[Ⅲ, parents, long]

How does haemophilia affect your child's **VIEW OF HIMSELF?**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child envied healthy boys his age	☐	☐	☐	☐	☐
2. ... my child felt physically weaker than other boys	☐	☐	☐	☐	☐
3. ... my child felt as well as other boys his age	☐	☐	☐	☐	☐
4. ... my child felt contented about his body	☐	☐	☐	☐	☐
5. ... haemophilia made my child's life more difficult	☐	☐	☐	☐	☐
6. ... my child was happy in spite of his haemophilia	☐	☐	☐	☐	☐
7. ... my child felt embarrassed about his haemophilia	☐	☐	☐	☐	☐
8. ... my child had difficulty doing things with other kids his age	☐	☐	☐	☐	☐
9. ... my child was unable to do as much with his friends because of his haemophilia	☐	☐	☐	☐	☐
10. ... my child felt healthy in spite of his haemophilia	☐	☐	☐	☐	☐



[III, parents, long]

The next questions are about haemophilia and the **FAMILY**

In the past 4 weeks...

	never	seldom	some-times	often	all the time
1. ... my child was treated differently by the family because of his haemophilia	☐	☐	☐	☐	☐
2. ... I protected my child too much	☐	☐	☐	☐	☐
3. ... my husband/wife protected our child too much	☐	☐	☐	☐	☐
4. ... I criticised my child when he hurt himself	☐	☐	☐	☐	☐
5. ... I forbade my child doing certain things because of his haemophilia	☐	☐	☐	☐	☐
6. ... there were problems at home because of his haemophilia	☐	☐	☐	☐	☐
7. ... my child felt that he was causing his family trouble because of his haemophilia	☐	☐	☐	☐	☐
8. ... I limited my time at work or leisure because I had to look after my child	☐	☐	☐	☐	☐

and then about haemophilia and your child's **FRIENDS**

In the past 4 weeks...

	never	seldom	some-times	often	all the time
1. ... my child was able to talk to his friends about his haemophilia	☐	☐	☐	☐	☐
2. ... my child's best friend cared about how my child was feeling	☐	☐	☐	☐	☐
3. ... there was a best friend that my child felt very close to	☐	☐	☐	☐	☐
4. ... my child's friends took care of him when he felt bad	☐	☐	☐	☐	☐

[III, parents, long]

and then about haemophilia and **PERCEIVED SUPPORT**

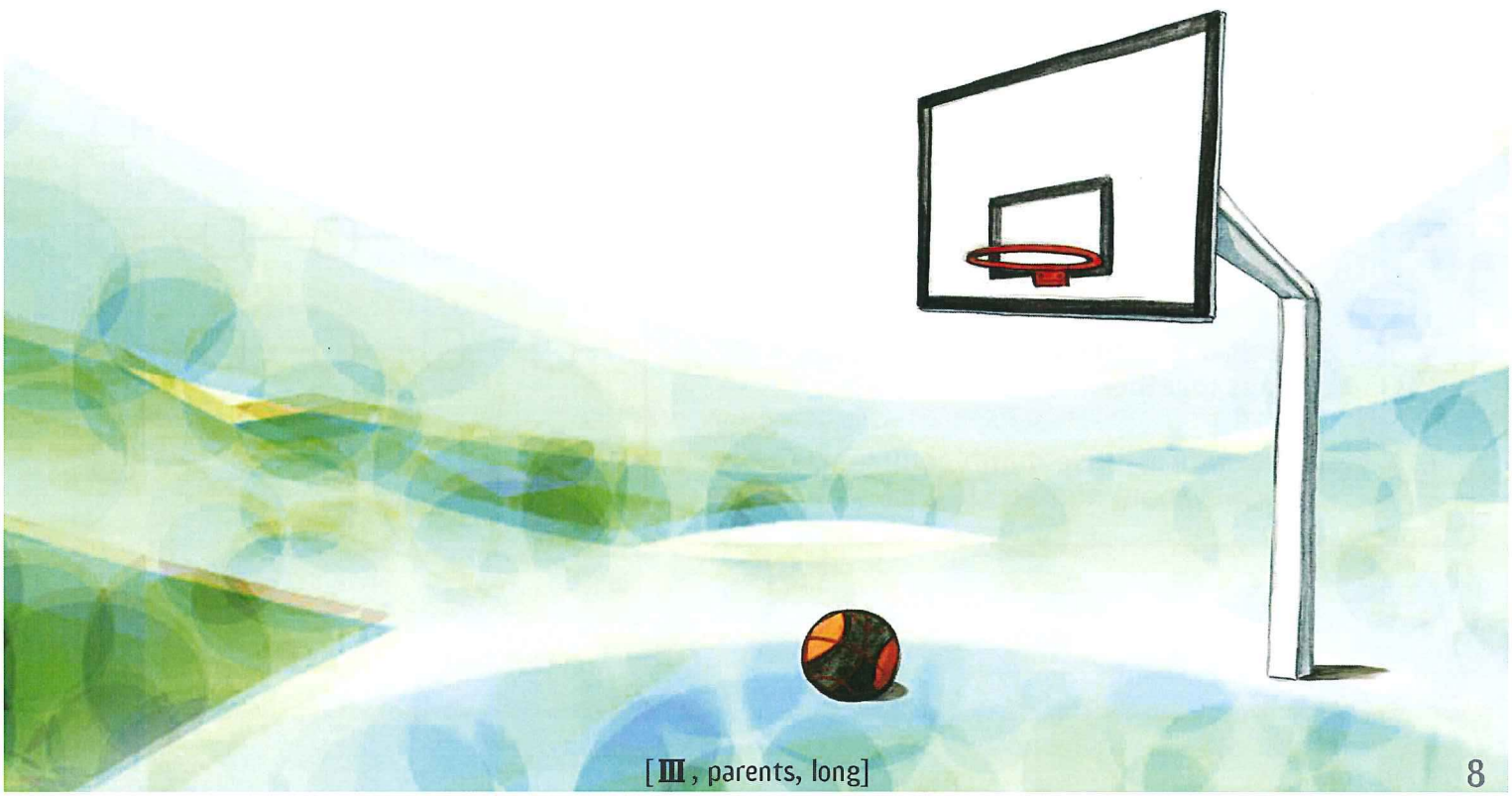
In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... others showed special consideration for my child because of his haemophilia	☐	☐	☐	☐	☐
2. ... others showed understanding for my child's haemophilia	☐	☐	☐	☐	☐
3. ... my child was able to talk to others about problems connected with his haemophilia	☐	☐	☐	☐	☐
4. ... others stood by my child	☐	☐	☐	☐	☐

These questions are about your child's haemophilia and **OTHER PERSONS**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child felt different from others because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child was bothered by others knowing about his haemophilia	☐	☐	☐	☐	☐
3. ... my child was teased by others because of his haemophilia	☐	☐	☐	☐	☐
4. ... people behaved different towards my child because of his haemophilia	☐	☐	☐	☐	☐
5. ... my child felt left out when others did things together	☐	☐	☐	☐	☐
6. ... other people made 'dumb' comments to my child about his haemophilia	☐	☐	☐	☐	☐

These questions are about **SPORTS AND SCHOOL**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child had to refrain from sports that he likes because of haemophilia	☐	☐	☐	☐	☐
2. ... my child had to do indoor activities more than other kids because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4. ... my child did just as much sports as any other kid	☐	☐	☐	☐	☐
5. ... my child was treated differently by teachers because of his haemophilia	☐	☐	☐	☐	☐
6. ... my child participated in sports classes at school in spite of his haemophilia	☐	☐	☐	☐	☐
7. ... my child was able to participate at school in spite of his haemophilia	☐	☐	☐	☐	☐
8. ... my child had to refrain from special school events (e.g. outings) because of his haemophilia	☐	☐	☐	☐	☐
9. ... my child found it difficult to concentrate at school because he was in pain	☐	☐	☐	☐	☐



[Ⅲ, parents, long]

The next questions are about **DEALING WITH HAEMOPHILIA**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2. ... my child was careful with his body	☐	☐	☐	☐	☐
3. ... my child was able to tell whether or not he was bleeding	☐	☐	☐	☐	☐
4. ... my child was in control of his complaints due to haemophilia	☐	☐	☐	☐	☐
5. ... my child felt well-informed about haemophilia	☐	☐	☐	☐	☐
6. ... haemophilia was a normal part of his life	☐	☐	☐	☐	☐
7. ... my child accepted having haemophilia	☐	☐	☐	☐	☐

and the **TREATMENT?**

In the past 4 weeks...	never	seldom	some-times	often	all the time
1. ... my child was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2. ... the treatment my child got was okay	☐	☐	☐	☐	☐
3. ... my child trusted his doctors and nurses	☐	☐	☐	☐	☐
4. ... my child disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5. ... my child felt dependent on others because of his haemophilia	☐	☐	☐	☐	☐
6. ... the injections annoyed my child	☐	☐	☐	☐	☐
7. ... my child was annoyed about the amount of time spent having the injections	☐	☐	☐	☐	☐
8. ... my child felt interrupted in his activities by the injections	☐	☐	☐	☐	☐

[III, parents, long]

What does your child think about the **FUTURE**?

Recently...

	never	seldom	some-times	often	all the time
1. ... my child has been thinking it will be difficult to lead a normal life	☐	☐	☐	☐	☐
2. ... my child has been expecting that things will get better as he grows older	☐	☐	☐	☐	☐
3. ... my child has been worried about his health	☐	☐	☐	☐	☐
4. ... my child has been sure about having a family of his own later on	☐	☐	☐	☐	☐

What about **RELATIONSHIPS**?

Recently...

	never	seldom	some-times	often	all the time
1. ... my son has found it difficult to date because of his haemophilia	☐	☐	☐	☐	☐
2. ... my son has been insecure in his relationships with girls becuase of his haemophilia	☐	☐	☐	☐	☐

your child‘ s **GLOBAL HEALTH**?

In general, ...

	excellent	very good	good	fair	poor
1. ... would you say your child‘ s health is...	☐	☐	☐	☐	☐

[Ⅲ, parents, long]

1. How much is *your child* bothered by his haemophilia?

☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

2. How much are *you* bothered by his haemophilia?

☐ not at all ☐ somewhat ☐ moderately ☐ considerably ☐ very much

3. Which are the problems about having haemophilia and which are the problems about the treatment for haemophilia...

a) for your child

- _____
- _____
- _____
- _____

b) for yourself

- _____
- _____
- _____
- _____

Great job- congratulations!

THANK YOU FOR YOUR ASSISTANCE!

Centre N°.: |__|__| ID N°.:|__|__|__| Initials: |__|__|

Questionnaire for children aged 6-7 years with haemophilia

Evaluation of the Impact of Sport Activities on Health-Related Qualify of Life of Haemophilia Patients

Hello there!

We have put together out a few questions which we would like you to answer. We would like to know how you have been feeling during the past weeks and something about your sporting activities.

We are happy that you and your mum or dad wants to participate in our project.

This questionnaire was made for children and adolescents with haemophilia.

- ⇨ I will now read each question to you.
- ⇨ Think about how things have been for you over the past weeks.
- ⇨ Please tell me, which answer fits you best.

There are no right or wrong answers. It's what you think that matters.

For example: 	**never**	**some-time**	**very often**
During the past week, I felt like eating ice-cream	☐	☐	☒
	☹	😐	☺

Date of completion: __ / __ / __ (day / month / year)

1. First of all, we would like to know about you...

1.	What is your date of birth?	☐☐.	☐☐.	☐☐☐☐
		Day	**Month**	**Year**
2.	Are you going to kindergarten or school?			
	☐ Kindergarten ☐ Primary			
3.	Do you have brothers or sisters?	☐ no ☐ yes How many? _____		

2. Questions about your Haemophilia

1. Are there things that you cannot do because of your health condition?

   ☐ No ☐ Yes If yes, which?

   _____

2. Did you suffer from chronic pain in the past 6 months?

   (Definition: pain present without an apparent bleed, recurrent at least twice a week and lasting >3 hours without treatment)

   ❑ No ❑ Yes If YES, please specify the average intensity of pain on this visual scale *(put an X on the line)*

   0 10

   |_____|

   (no pain) (max. pain)

HERE YOU WILL FIND SOME QUESTIONS ABOUT YOUR HEALTH-RELATED QUALITY OF LIFE

3. We would like to know about your physical health...

	During the past week...	never	sometimes	very often
1.	... I felt ill	☐	☐	☐
2.	... I had a headache or tummy-ache	☐	☐	☐
3.	... I was afraid that my illness might get worse	☐	☐	☐

4. ... then about how you've been feeling in general...

	During the past week...	never	sometimes	very often
1.	... I had fun and laughed a lot	☐	☐	☐
2.	... I was bored	☐	☐	☐
3.	... I was sad because of my illness	☐	☐	☐

5. ... and how you have been feeling about yourself.

	During the past week...	never	sometimes	very often
1.	... I was proud of myself	☐	☐	☐
2.	... I felt pleased with myself	☐	☐	☐
3.	... I was able to cope well with my illness	☐	☐	☐

6. The next questions are about your family ...

	During the past week...	never	sometimes	very often
1.	... I got on well with my parents	☐	☐	☐
2.	... I felt fine at home	☐	☐	☐
3.	... my parents treated me like a baby because of my illness	☐	☐	☐

7. ... and then about your friends.

	During the past week...	never	sometimes	very often
1.	... I played with friends	☐	☐	☐
2.	... I got along well with my friends	☐	☐	☐
3.	... I avoided others to notice my illness	☐	☐	☐

8. Now, we would like to know about nursery school/kindergarten.

	During the past week...	never	sometimes	very often
1.	... I coped well with the assignments set in nursery school/kindergarten	☐	☐	☐
2.	... I enjoyed nursery school/ kindergarten	☐	☐	☐
3.	...I missed something at nursery school/kindergarten because of my illness	☐	☐	☐

NOW WE WOULD LIKE TO ASK YOU SEVERAL QUESTIONS ABOUT YOUR HAEMOPHILIA!

9. Here we would like to know about your BLEEDS (JOINT BLEEDS).

1. How frequent were your bleeds in the last 4 weeks?
   ☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many?___

The following questions should only be answered if the child had bleeds.

2. How much were you troubled by bleeds during the last 4 weeks?
   ☐ not at all ☐ somewhat ☐ very much

3. How severe were your bleeds during the last 4 weeks *(if you had several bleeds, please answer for the most severe bleed)*?
   ☐ slight ☐ moderate ☐ very severe

4. Did you feel a strange sensation in your joints before you had a bleed?
   ☐ never ☐ sometimes ☐ always

5. Did you have to stay quiet (e.g. lie in bed) when you had bleeds?
   ☐ never ☐ sometimes ☐ always

6. When you had bleeds, did you inform your parents immediately?
   ☐ never ☐ sometimes ☐ always

10. Here we would like to know about haemophilia and your HEALTH.

In the past 4 weeks ...	never	sometimes	very often
1. ... my swellings hurt	☐	☐	☐
2. ... it was difficult for me to move my arms or legs	☐	☐	☐
3. ... I was afraid of bleeds	☐	☐	☐
4. ... I was afraid of hurting myself	☐	☐	☐

11. How have you been FEELING because of your haemophilia?

In the past 4 weeks ...	never	sometimes	very often
1. ... I was in a bad mood because of my haemophilia	☐	☐	☐
2. ... I was sad because of my haemophilia	☐	☐	☐
3. ... my haemophilia made me angry	☐	☐	☐

12. How does haemophilia affect your VIEW OF YOURSELF?

In the past 4 weeks ...	never	sometimes	very often
1. ... I envied healthy boys my age	☐	☐	☐
2. ... I felt embarrassed about my haemophilia	☐	☐	☐

13. The next questions are about haemophilia and your FAMILY

In the past 4 weeks ...	never	sometimes	very often
1. ... my mother protected me too much	☐	☐	☐
2. ... my father protected me too much	☐	☐	☐
3. ... my parents told me off when I hurt myself	☐	☐	☐
4. ... my parents forbade me to do certain things because of my haemophilia	☐	☐	☐

14. and then about haemophilia and your FRIENDS

In the past 4 weeks ...	never	sometimes	very often
1. ... I was unable to do as much with my friends because of my haemophilia	☐	☐	☐

15. These questions are about your haemophilia and OTHER PEOPLE

In the past 4 weeks ...	never	sometimes	very often
1. ... I felt different from others because of my haemophilia	☐	☐	☐
2. ... I felt left out when others did things together	☐	☐	☐

16. These questions are about nursery school/kindergarten

In the past 4 weeks ...	never	sometimes	very often
1. ... because of haemophilia I had to refrain from playing games that I enjoy	☐	☐	☐
2. ... I did just as much sport as many other kids	☐	☐	☐
3. ... I was able to take part in games at nursery school/kindergarten in spite of my haemophilia	☐	☐	☐

17. and your Treatment?

In the past 4 weeks ...	never	sometimes	very often
1. ... I disliked visiting the haemophilia centre	☐	☐	☐
2. ... the injections annoyed me	☐	☐	☐

IN THE NEXT SECTION WE WILL ASK YOU SOME QUESTIONS ABOUT YOUR PHYSICAL ACTIVITIES

18. TV/Video Viewing or Video/Computer Games

Average of the last 12 months ...	never	< 1 hour a day	1-2 hours a day	3-4 hours a day	> 4 hours a day
1. On a weekday	☐	☐	☐	☐	☐
2. On a weekend	☐	☐	☐	☐	☐

19. Attitudes towards sport

1. Do you think doing sport is good?

   ☐ no ☐ yes

1a. If yes, what do you think is good?

2. Do you think that sport is bad or dangerous?

   ☐ no ☐ yes

2a. If yes, what do you think is bad or dangerous?

20. We would like to know about your sports activities

1. Do you do sport?

   ☐ no ☐ yes

If you do NOT do sport, continue with QUESTION 27

2. Where do you do sport?

   ☐ in school ☐ in the public recreation ground ☐ in a team/sports club ☐ somewhere else (specify?) ……

3. With whom do you do sports?

   ☐ alone ☐ with friends ☐ with my team ☐ with others (specify?) ……

4. Have you ever injured yourself doing sports?

   ☐ never ☐ sometimes ☐ often ☐ always

5.	Are there sports which you like, but you have been told not do because of your haemophilia? ☐ no ☐ yes If yes, which?_____
5a.	Have you ever tried one of these sports? ☐ no ☐ yes If yes, which?_____
5b.	What happened? _____

21. How often do you do the following sports?

Per week…	never	once	twice	three times	more than three times
1. Football	☐	☐	☐	☐	☐
2. Swimming	☐	☐	☐	☐	☐
3. Gymnastics	☐	☐	☐	☐	☐
4. Jogging	☐	☐	☐	☐	☐
5. Others (specify?) ……	☐	☐	☐	☐	☐
6. Others (specify?) ……	☐	☐	☐	☐	☐

22. How long do you do the following sports for?

Every time…	1 hour	2 hours	more than 2 hours
1. Football	☐	☐	☐
2. Swimming	☐	☐	☐
3. Gymnastics	☐	☐	☐
4. Jogging	☐	☐	☐
5. Others (specify?) ……	☐	☐	☐

6.	Others (specify?) ……	☐	☐	☐

23. …. and your friends?

1.	Do your friends do sport? ☐ no ☐ yes
2.	Which sports do your friends do? ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) ……
3.	Do your friends do any sport which you would like to do? ☐ no ☐ yes If yes, which? _____

24. …. and your parents?

1.	Do your parents do sport? ☐ no ☐ yes
1a.	Which sports do your parents do? ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) ……
2.	Are your parents interested in sport? ☐ no ☐ yes

25. Here we would like to know about competitive sports

n(playing in competition with regular training)

1.	Are you a member of a sports team? ☐ no ☐ yes
2.	Do you participate in competitive sports? ☐ no ☐ yes
3.	Have you done competitive sports in the past? ☐ no ☐ yes
3a.	Have you had to stop doing competitive sports? ☐ no ☐ yes If yes, which? _____

3b. If yes, why did you have to stop it? *(you can tick more than one box)*

☐ because of bleeding complications

☐ because of my decision

☐ because of the decision of my parents

☐ because of the decision of my doctor

☐ other reasons (specify?)

_____

4. Would you like to do competitive sports?

☐ no ☐ yes

26. We have some questions about the future

1. Would you like to continue sports when you are older?

☐ no ☐ yes

2. Would you like to try another sport?

☐ no ☐ yes

3. If yes, which?

☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?)

…….

27. Here are questions only for kids who are NOT doing sports

1. Even though you are not doing sports would you like to do sports?

☐ no ☐ yes

2. If yes, which?

☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?)

…….

3. Why do you not do sport?

☐ because I do not like it (I am not interested in)

☐ because I am afraid to hurt myself

☐ because my mother does not want me to do it

☐ because my father does not want me to do it

☐ because my doctor does not allow it

☐ other reasons, which?

_____

3a. If you do not do sports because of your parents, do you know why your parents do not want you to do sports?

☐ no ☐ yes

3b. Why your parents do not want you to do sports?

☐ because sport is not important to them

☐ because they are afraid that I will hurt myself

☐ because my doctor does not allow it

☐ other reasons, which?

_____

28. Would you like to tell us about your sporting life?

_____

_____

_____

29. What are your main interests?

_____

_____

_____

30. If you could express a wish, what would it be?

_____

_____

_____

THANKS FOR YOUR HELP!

Centre N°.: |__|__| ID N°.:|__|__|__| Initials: |__|__|

Questionnaire for children aged 8-12 years with haemophilia

Evaluation of the Impact of Sport Activities on Health-Related Qualify of Life of Haemophilia Patients

Hello there!

We have put together out a few questions which we would like you to answer. We would like to know how you have been feeling during the past weeks and something about your sporting activities.

We are happy that you and your mum or dad wants to participate in our project.

This questionnaire was made for children and adolescents with haemophilia.

- ⇨ Please read each question carefully.
- ⇨ Think about how things have been for you over the past weeks.
- ⇨ Choose the answer that fits you best and cross the appropriate box.

There are no right or wrong answers. It's what you think that matters.

For example: ✎	**never**	**seldom**	**some-times**	**often**	**all the time**
During the past week, I felt like eating ice-cream	☐	☐	☐	☒	☐

Date of completion: __ / __ / __ (day / month / year)

1. First of all, we would like to know about you...

1.	What is your date of birth?	☐☐.	☐☐.	☐☐☐☐
		Day	Month	Year
2.	What kind of school do you attend?			
	☐ Primary ☐ Secondary ☐ 6th Form College ☐ Special School	☐ I no longer go to school ☐ Other _____		
3.	What grade are you in?	☐☐ grade		
4.	Do you have siblings?	☐ no ☐ yes How many? _____		

2. Questions about your Haemophilia

1. Are there things that you cannot do because of your health condition?

☐ No ☐ Yes If yes, which?

_____

2. Did you suffer from chronic pain in the past 6 months?

(Definition: pain present without an apparent bleed, recurrent at least twice a week and lasting >3 hours without treatment)

❑ No ❑ Yes If YES, please specify the average intensity of pain on this visual scale

(put an X on the line)

0 10

|_____|

(no pain) (max. pain)

HERE YOU WILL FIND SOME QUESTIONS ABOUT YOUR HEALTH-RELATED QUALITY OF LIFE

3. We would like to know about your physical health...

	During the past week...	never	seldom	some-times	often	all the time
1.	... I felt ill	☐	☐	☐	☐	☐
2.	... I had a headache or tummy-ache	☐	☐	☐	☐	☐
3.	... I was tired and worn-out	☐	☐	☐	☐	☐
4.	... I felt strong and full of energy	☐	☐	☐	☐	☐
5.	... I was afraid that my illness might get worse	☐	☐	☐	☐	☐

4. ... then about how you've been feeling in general...

	During the past week...	never	seldom	some-times	often	all the time
1.	... I had fun and laughed a lot	☐	☐	☐	☐	☐
2.	... I was bored	☐	☐	☐	☐	☐
3.	... I felt alone	☐	☐	☐	☐	☐
4.	... I was scared	☐	☐	☐	☐	☐
5.	... I was sad because of my illness	☐	☐	☐	☐	☐

5. ... and how you have been feeling about yourself.

	During the past week...	never	seldom	some-times	often	all the time
1.	... I was proud of myself	☐	☐	☐	☐	☐
2.	... I felt on top of the world	☐	☐	☐	☐	☐
3.	... I felt pleased with myself	☐	☐	☐	☐	☐
4.	... I had lots of good ideas	☐	☐	☐	☐	☐
5.	... I was able to cope well with my illness	☐	☐	☐	☐	☐

6. The next questions are about your family ...

During the past week...	never	seldom	some-times	often	all the time
1. ... I got on well with my parents	☐	☐	☐	☐	☐
2. ... I felt fine at home	☐	☐	☐	☐	☐
3. ... We quarrelled at home	☐	☐	☐	☐	☐
4. ... My parents stopped me from doing certain things	☐	☐	☐	☐	☐
5. ... My parents treated me like a baby because of my illness	☐	☐	☐	☐	☐

7. ... and then about your friends.

During the past week...	never	seldom	some-times	often	all the time
1. ... I played with friends	☐	☐	☐	☐	☐
2. ... Other kids liked me	☐	☐	☐	☐	☐
3. ... I got along well with my friends	☐	☐	☐	☐	☐
4. ... I felt different from other children	☐	☐	☐	☐	☐
5. ... I avoided others to notice my illness	☐	☐	☐	☐	☐

8. Now, we would like to know about school.

During the past week...	never	seldom	some-times	often	all the time
1. ... doing my schoolwork was easy	☐	☐	☐	☐	☐
2. ... I enjoyed my lessons	☐	☐	☐	☐	☐
3. ... I looked forward to the weeks ahead	☐	☐	☐	☐	☐
4. ... I was afraid of bad marks or grades	☐	☐	☐	☐	☐
5. ... I missed something at school because of my illness	☐	☐	☐	☐	☐

NOW WE WOULD LIKE TO ASK YOU SEVERAL QUESTIONS ABOUT YOUR HAEMOPHILIA!

9. Here we would like to know about your BLEEDS (JOINT BLEEDS).

1. How frequent were your bleeds in the last 4 weeks?

☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many?___

The following questions should only be answered if you had bleeds.

2.	How much were you troubled by bleeds during the last 4 weeks?				
	☐ not at all	☐ somewhat	☐ moderately	☐ quite a bit	
3.	How severe were your bleeds during the last 4 weeks *(if you had several bleeds, please answer for the most severe bleed)*?				
	☐ slight	☐ moderate	☐ severe	☐ very severe	
4.	Did you feel a strange sensation in your joints before you had a bleed?				
	☐ never	☐ seldom	☐ sometimes	☐ often	☐ always
5.	Did you have to stay quiet (e.g. lie in bed) when you had bleeds?				
	☐ never	☐ seldom	☐ sometimes	☐ often	☐ always
6.	When you had bleeds, did you inform your parents immediately?				
	☐ never	☐ seldom	☐ sometimes	☐ often	☐ always

10. Here we would like to know about haemophilia and your PHYSICAL HEALTH.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my swellings hurt	☐	☐	☐	☐	☐
2.	... I had pain in my joints	☐	☐	☐	☐	☐
3.	... it was painful for me to move	☐	☐	☐	☐	☐
4.	... my joints felt stiff	☐	☐	☐	☐	☐
5.	... it was difficult for me to move my arms or legs	☐	☐	☐	☐	☐
6.	... I had difficulty walking as far as I wanted to	☐	☐	☐	☐	☐
7.	... I was afraid of hurting myself	☐	☐	☐	☐	☐

11. and now about how you have been FEELING because of your haemophilia

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... I was in a bad mood because of my haemophilia	☐	☐	☐	☐	☐
2.	... I was sad because of my haemophilia	☐	☐	☐	☐	☐
3.	... my haemophilia was a burden (real problem) for me	☐	☐	☐	☐	☐
4.	... my haemophilia made me angry	☐	☐	☐	☐	☐
5.	... I was worried because of my haemophilia	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
6.	...I felt lonely because of my haemophilia	☐	☐	☐	☐	☐
7.	... I was afraid of bleeds	☐	☐	☐	☐	☐

12. How does haemophilia affect your VIEW OF YOURSELF?

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... I envied healthy boys my age	☐	☐	☐	☐	☐
2.	... I felt physically weaker than other boys	☐	☐	☐	☐	☐
3.	... I felt as well as other boys my age	☐	☐	☐	☐	☐
4.	... I felt contented about my body	☐	☐	☐	☐	☐
5.	... haemophilia made my life more difficult	☐	☐	☐	☐	☐
6.	... I felt embarrassed about my haemophilia	☐	☐	☐	☐	☐
7.	... I had difficulty doing things with other kids my age	☐	☐	☐	☐	☐
8.	... I was unable to do as much with my friends because of my haemophilia	☐	☐	☐	☐	☐
9.	... I felt healthy in spite of my haemophilia	☐	☐	☐	☐	☐

13. The next questions are about haemophilia and your FAMILY

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my mother protected me too much	☐	☐	☐	☐	☐
2.	... my father protected me too much	☐	☐	☐	☐	☐
3.	... there were problems at home because of my haemophilia	☐	☐	☐	☐	☐
4.	... I felt I was causing my family trouble because of my haemophilia	☐	☐	☐	☐	☐
5.	... my parents limited their time at work or leisure because they had to look after me	☐	☐	☐	☐	☐

14. and then about haemophilia and your FRIENDS

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... I was able to talk to my friends about my haemophilia	☐	☐	☐	☐	☐
2. ... my best friend cared about how I was feeling	☐	☐	☐	☐	☐
3. ... there was a best friend that I felt very close to	☐	☐	☐	☐	☐
4. ... my friends took care of me when I felt bad	☐	☐	☐	☐	☐

15. and then about haemophilia and your PERCEIVED SUPPORT

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... others showed special consideration for me because of my haemophilia	☐	☐	☐	☐	☐
2. ... others showed understanding for my haemophilia	☐	☐	☐	☐	☐
3. ... I was able to talk to others about problems in connection with my haemophilia	☐	☐	☐	☐	☐
4. ... others stood by me	☐	☐	☐	☐	☐

16. These questions are about your haemophilia and OTHER PERSONS

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... I felt different from others because of my haemophilia	☐	☐	☐	☐	☐
2. ... I was bothered by others knowing about my haemophilia	☐	☐	☐	☐	☐
3. ... other kids teased me because of my haemophilia	☐	☐	☐	☐	☐
4. ... people behaved differently towards me because of haemophilia	☐	☐	☐	☐	☐
5. ... I felt left out when others did things together	☐	☐	☐	☐	☐
6. ... others made dumb comments about my haemophilia	☐	☐	☐	☐	☐

17. These questions are about SPORTS AND SCHOOL

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... because of haemophilia I had to refrain from sports that I like	☐	☐	☐	☐	☐
2. ... I had to do indoor activities more than other kids because of my haemophilia	☐	☐	☐	☐	☐
3. ... I had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4. ... I did just as much sport as any other kid	☐	☐	☐	☐	☐
5. ... I participated in sports classes at school in spite of my haemophilia	☐	☐	☐	☐	☐
6. ... I was able to participate at school in spite of my haemophilia	☐	☐	☐	☐	☐
7. ... I had to refrain from special school events (e.g. outings) because of my haemophilia	☐	☐	☐	☐	☐
8. ... I found it difficult to concentrate at school because I was in pain	☐	☐	☐	☐	☐

18. The next questions are about DEALING WITH HAEMOPHILIA

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... I tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2. ... I was careful with my body	☐	☐	☐	☐	☐
3. ... I was able to tell whether or not I was bleeding	☐	☐	☐	☐	☐
4. ... I was in control of my complaints due to haemophilia	☐	☐	☐	☐	☐
5. ... I felt well informed about haemophilia	☐	☐	☐	☐	☐
6. ... haemophilia was a normal part of my life	☐	☐	☐	☐	☐
7. ... I accepted having haemophilia	☐	☐	☐	☐	☐

19. and your TREATMENT?

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... I was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2. ... the treatment I got was okay	☐	☐	☐	☐	☐
3. ... I trusted my doctors and nurses	☐	☐	☐	☐	☐
4. ... I disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5. ... the injections annoyed me	☐	☐	☐	☐	☐
6. ... I was annoyed about the amount of time spent having injections	☐	☐	☐	☐	☐
7. ... I felt interrupted in my activities by the injections	☐	☐	☐	☐	☐

HERE WE WOULD LIKE TO KNOW ABOUT YOUR PHYSICAL STATUS UND YOUR PHYSICAL ACTIVITIES

20. First we would like to know about your PHYSICAL STATUS…

	excellent	very good	good	fair	poor
1. How do you evaluate your actual physical activity?	☐	☐	☐	☐	☐

	much better	somewhat better	about the same as 1 year ago	somewhat worse	much worse
2. How do you evaluate your physical activity compared to last year?	☐	☐	☐	☐	☐

21. Now we would like to know about your MOBILITY

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. … I had stiff joints	☐	☐	☐	☐	☐
2. ... my mobility was affected	☐	☐	☐	☐	☐
3. … my physical activity was affected because of pain	☐	☐	☐	☐	☐
4. … my physical activity was affected because of chronic pain (e.g. aches)	☐	☐	☐	☐	☐

22. We would like to know what you think about your STRENGTH & COORDINATION

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. ... I had a lot of energy	☐	☐	☐	☐	☐
2. … I felt secure walking	☐	☐	☐	☐	☐
3. … I had problems climbing stairs	☐	☐	☐	☐	☐
4. … it was difficult for me to climb stairs without holding onto something	☐	☐	☐	☐	☐
5. … I had problems walking downstairs	☐	☐	☐	☐	☐
6. ... I had difficulty lifting or carrying heavy things	☐	☐	☐	☐	☐
7. … I had problems with keeping my balance on rough ground	☐	☐	☐	☐	☐

23. what about your ENDURANCE?

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. ... I could cover far distances (about 30 minutes or 2 km)	☐	☐	☐	☐	☐
2. ... I was exhausted after physical activities of medium difficulty (e.g. climbing stairs and carrying shopping)	☐	☐	☐	☐	☐
3. ... I was easily out of breath	☐	☐	☐	☐	☐
4. ... I could do physical activities longer than usual	☐	☐	☐	☐	☐
5. …I was easily tired	☐	☐	☐	☐	☐
6. … I could carry out exhausting activities (e.g. walking fast, cycling)	☐	☐	☐	☐	☐
7. … I did a lot with others	☐	☐	☐	☐	☐
8. … I was physically more active than usual	☐	☐	☐	☐	☐

24. these questions are concerning your BODY PERCEPTION

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. … I felt steady on my feet	☐	☐	☐	☐	☐
2. … I felt fine in my body	☐	☐	☐	☐	☐
3. … I felt confident in my body	☐	☐	☐	☐	☐
4. … I felt my body was strong	☐	☐	☐	☐	☐
5. … I was self-confident	☐	☐	☐	☐	☐

IN THE NEXT SECTION WE WILL ASK YOU SOME QUESTIONS ABOUT YOUR PHYSICAL ACTIVITIES

25. TV/Video Viewing or Video/Computer Games

Average of the last 12 months ...	never	< 1 hour a day	1-2 hours a day	3-4 hours a day	> 4 hours a day
1. On a weekday	☐	☐	☐	☐	☐
2. On a weekend	☐	☐	☐	☐	☐

26. Attitudes towards sport

1.	Do you think doing sport is good? ☐ no ☐ yes
1a.	If yes, what do you think is good?
2.	Do you think that sport is bad or dangerous? ☐ no ☐ yes
2a.	If yes, what do you think is bad or dangerous?

27. We would like to know about your sports activities

1.	Do you do sport? ☐ no ☐ yes

If you do <u>NOT</u> do sport, continue with QUESTION 34

2.	Where do you do sport? ☐ in school ☐ in the public recreation ground ☐ in a team/sports club ☐ somewhere else (specify?) …….
3.	With whom do you do sports? ☐ alone ☐ with friends ☐ with my team ☐ with others (specify?) …….
4.	Have you ever injured yourself doing sports? ☐ never ☐ sometimes ☐ often ☐ always
5.	Are there sports which you like, but you have been told not do because of your haemophilia? ☐ no ☐ yes If yes, which?_____
5a.	Have you ever tried one of these sports? ☐ no ☐ yes If yes, which?_____
5b.	What happened? _____

28. How often do you do the following sports?

Per week…	never	once	twice	three times	more than three times
1. Football	☐	☐	☐	☐	☐
2. Swimming	☐	☐	☐	☐	☐
3. Gymnastics	☐	☐	☐	☐	☐
4. Jogging	☐	☐	☐	☐	☐
5. Others (specify?) …….	☐	☐	☐	☐	☐
6. Others (specify?) …….	☐	☐	☐	☐	☐

29. How long do you do the following sports for?

Every time…	1 hour	2 hours	more than 2 hours
1. Football	☐	☐	☐
2. Swimming	☐	☐	☐
3. Gymnastics	☐	☐	☐
4. Jogging	☐	☐	☐
5. Others (specify?) ……	☐	☐	☐
6. Others (specify?) ……	☐	☐	☐

30. …. and your friends?

1. Do your friends do sport?
   ☐ no ☐ yes
2. Which sports do your friends do?
   ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) ……
3. Do your friends do any sport which you would like to do?
   ☐ no ☐ yes If yes, which? _____

31. …. and your parents?

1. Do your parents do sport?
   ☐ no ☐ yes

1a. Which sports do your parents do?
   ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) ……

2. Are your parents interested in sport?
   ☐ no ☐ yes

32. Here we would like to know about competitive sports

(playing in competition with regular training)

1.	Are you a member of a sports team? ☐ no ☐ yes
2.	Do you participate in competitive sports? ☐ no ☐ yes
3.	Have you done competitive sports in the past? ☐ no ☐ yes
3a.	Have you had to stop doing competitive sports? ☐ no ☐ yes If yes, which? _____
3b.	If yes, why did you have to stop it? *(you can tick more than one box)* ☐ because of bleeding complications ☐ because of my decision ☐ because of the decision of my parents ☐ because of the decision of my doctor ☐ other reasons (specify?) _____
4.	Would you like to do competitive sports? ☐ no ☐ yes

33. We have some questions about the future

1.	Would you like to continue sports when you are older? ☐ no ☐ yes
2.	Would you like to try another sport? ☐ no ☐ yes
3.	If yes, which? ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) …….

34. Here are questions only for kids who are NOT doing sports

1. Even though you are not doing sports would you like to do sports?

   ☐ no ☐ yes

2. If yes, which?

   ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?)

   …….

3. Why do you not do sport?

   ☐ because I do not like it (I am not interested in)

   ☐ because I am afraid to hurt myself

   ☐ because my mother does not want me to do it

   ☐ because my father does not want me to do it

   ☐ because my doctor does not allow it

   ☐ other reasons, which?

   _____

3a. If you do not do sports because of your parents, do you know why your parents do not want you to do sports?

   ☐ no ☐ yes

3b. Why your parents do not want you to do sports?

   ☐ because sport is not important to them

   ☐ because they are afraid that I will hurt myself

   ☐ because my doctor does not allow it

   ☐ other reasons, which?

   _____

35. Would you like to tell us about your sporting life?

_____

_____

_____

36. What are your main interests?

_____

_____

_____

37. If you could express a wish, what would it be?

_____

_____

_____

THANKS FOR YOUR HELP!

Centre N°.: |__|__| ID N°.:|__|__|__| Initials: |__|__|

Questionnaire for adolescents aged 13-17 years with haemophilia

Evaluation of the Impact of Sport Activities on Health-Related Qualify of Life of Haemophilia Patients

Hello there!

We have put together out a few questions which we would like you to answer. We would like to know how you have been feeling during the past weeks and something about your sporting activities.

We are happy that you and your mum or dad wants to participate in our project.

This questionnaire was made for children and adolescents with haemophilia.

- ⇨ Please read each question carefully.
- ⇨ Think about how things have been for you over the past weeks.
- ⇨ Choose the answer that fits you best and cross the appropriate box.

There are no right or wrong answers. It's what you think that matters.

For example:	**never**	**seldom**	**some-times**	**often**	**all the time**
During the past week, I felt like eating ice-cream	☐	☐	☐	☒	☐

Date of completion: __ / __ / __ (day / month / year)

1. First of all, we would like to know about you...

1.	What is your date of birth?	☐☐.	☐☐.	☐☐☐☐
		Day	Month	Year
2.	What kind of school do you attend?			
	☐ Primary ☐ Secondary ☐ 6th Form College ☐ Special School	☐ I no longer go to school ☐ Other _____		
3.	What year are you in?	☐☐		
4.	Do you have brothers or sisters?	☐ no ☐ yes How many? _____		

2. Questions about your Haemophilia

1. Are there things that you cannot do because of your health condition?

☐ No ☐ Yes If yes, which?

_____

2. Did you suffer from chronic pain in the past 6 months?

(Definition: pain present without an apparent bleed, recurrent at least twice a week and lasting >3 hours without treatment)

❑ No ❑ Yes If YES, please specify the average intensity of pain on this visual scale

(put an X on the line)

0 10

|_____|

(no pain) (max. pain)

HERE YOU WILL FIND SOME QUESTIONS ABOUT YOUR HEALTH-RELATED QUALITY OF LIFE

3. We would like to know about your physical health...

	During the past week...	never	seldom	some-times	often	all the time
1.	... I felt ill	☐	☐	☐	☐	☐
2.	... I was in pain	☐	☐	☐	☐	☐
3.	... I was tired and worn-out	☐	☐	☐	☐	☐
4.	... I felt strong and full of energy	☐	☐	☐	☐	☐
5.	... I was afraid that my illness might get worse	☐	☐	☐	☐	☐

4. ... then about how you've been feeling in general...

	During the past week...	never	seldom	some-times	often	all the time
1.	... I had fun and laughed a lot	☐	☐	☐	☐	☐
2.	... I was bored	☐	☐	☐	☐	☐
3.	... I felt alone	☐	☐	☐	☐	☐
4.	... I felt scared or unsure of myself	☐	☐	☐	☐	☐
5.	... I was sad because of my illness	☐	☐	☐	☐	☐

5. ... and how you have been feeling about yourself.

	During the past week...	never	seldom	some-times	often	all the time
1.	... I was proud of myself	☐	☐	☐	☐	☐
2.	... I felt on top of the world	☐	☐	☐	☐	☐
3.	... I felt pleased with myself	☐	☐	☐	☐	☐
4.	... I had lots of good ideas	☐	☐	☐	☐	☐
5.	... I was able to cope well with my illness	☐	☐	☐	☐	☐

6. The next questions are about your family ...

During the past week...	never	seldom	some-times	often	all the time
1. ... I got on well with my parents	☐	☐	☐	☐	☐
2. ... I felt fine at home	☐	☐	☐	☐	☐
3. ... We quarrelled at home	☐	☐	☐	☐	☐
4. ... I felt restricted by my parents	☐	☐	☐	☐	☐
5. ... my parents treated me like a baby because of my illness	☐	☐	☐	☐	☐

7. ... and then about your friends.

During the past week...	never	seldom	some-times	often	all the time
1. ... I did things together with my friends	☐	☐	☐	☐	☐
2. ... I was a "success" with my friends	☐	☐	☐	☐	☐
3. ... I got along well with my friends	☐	☐	☐	☐	☐
4. ... I felt different from other people	☐	☐	☐	☐	☐
5. ... I avoided others to notice my illness	☐	☐	☐	☐	☐

8. Now, we would like to know about school.

During the past week...	never	seldom	some-times	often	all the time
1. ... doing my schoolwork was easy	☐	☐	☐	☐	☐
2. ... I found school interesting	☐	☐	☐	☐	☐
3. ... I worried about my future	☐	☐	☐	☐	☐
4. ... I worried about getting bad marks or grades	☐	☐	☐	☐	☐
5. ...I missed something at school because of my illness	☐	☐	☐	☐	☐

NOW WE WOULD LIKE TO ASK YOU SEVERAL QUESTIONS ABOUT YOUR HAEMOPHILIA!

9. Here we would like to know about your BLEEDS (JOINT BLEEDS).

1.	How frequent were your bleeds in the last 4 weeks? ☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many?___

The following questions should only be answered if you had bleeds.

2.	How much were you troubled by bleeds during the last 4 weeks? ☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit
3.	How severe were your bleeds during the last 4 weeks *(if you had several bleeds, please answer for the most severe bleed)*? ☐ slight ☐ moderate ☐ severe ☐ very severe
4.	Did you feel a strange sensation in your joints before you had a bleed? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
5.	Did you have to stay quiet (e.g. lie in bed) when you had bleeds? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
6.	When you had bleeds, did you inform your parents immediately? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

10. Here we would like to know about haemophilia and your PHYSICAL HEALTH.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my swellings hurt	☐	☐	☐	☐	☐
2.	... I had pain in my joints	☐	☐	☐	☐	☐
3.	... it was painful for me to move	☐	☐	☐	☐	☐
4.	... my joints felt stiff	☐	☐	☐	☐	☐
5.	... it was difficult for me to move my arms or legs	☐	☐	☐	☐	☐
6.	... I had difficulty walking as far as I wanted to	☐	☐	☐	☐	☐
7.	... I was afraid of hurting myself	☐	☐	☐	☐	☐

11. and now about how you have been FEELING because of your haemophilia

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... I was in a bad mood because of my haemophilia	☐	☐	☐	☐	☐
2.	... I was sad because of my haemophilia	☐	☐	☐	☐	☐
3.	... my haemophilia was a burden (real problem) for me	☐	☐	☐	☐	☐
4.	... my haemophilia made me angry	☐	☐	☐	☐	☐
5.	... I was worried because of my haemophilia	☐	☐	☐	☐	☐
6.	... I felt lonely because of my haemophilia	☐	☐	☐	☐	☐
	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
7.	... I was afraid of bleeds	☐	☐	☐	☐	☐
8.	... I felt excluded by my friends	☐	☐	☐	☐	☐

12. How does haemophilia affect your VIEW OF YOURSELF?

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... I envied healthy boys my age	☐	☐	☐	☐	☐
2.	... I felt physically weaker than other boys	☐	☐	☐	☐	☐
3.	... I felt as well as other boys my age	☐	☐	☐	☐	☐
4.	... I felt contented about my body	☐	☐	☐	☐	☐
5.	... haemophilia made my life more difficult	☐	☐	☐	☐	☐
6.	... I was happy in spite of my haemophilia	☐	☐	☐	☐	☐
7.	... I felt embarrassed about my haemophilia	☐	☐	☐	☐	☐
8.	... I had difficulty doing things with other kids my age	☐	☐	☐	☐	☐
9.	... I was unable to do as much with my friends because of my haemophilia	☐	☐	☐	☐	☐
10.	... I felt healthy in spite of my haemophilia	☐	☐	☐	☐	☐

13. The next questions are about haemophilia and your FAMILY

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... I was treated differently by my family because of my haemophilia	☐	☐	☐	☐	☐
2. ... my mother protected me too much	☐	☐	☐	☐	☐
3. ... my father protected me too much	☐	☐	☐	☐	☐
4. ... my parents criticised me when I hurt myself	☐	☐	☐	☐	☐
5. ... my parents forbade me to do certain things because of my haemophilia	☐	☐	☐	☐	☐
6. ... there were problems at home because of my haemophilia	☐	☐	☐	☐	☐
In the past 4 weeks ...	never	seldom	some-times	often	all of the time
7. ... I felt I was causing my family trouble because of my haemophilia	☐	☐	☐	☐	☐
8. ... my parents limited their time at work or leisure because they had to look after me	☐	☐	☐	☐	☐

14. and then about haemophilia and your FRIENDS

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... I was able to talk to my friends about my haemophilia	☐	☐	☐	☐	☐
2. ... my best friend cared about how I was feeling	☐	☐	☐	☐	☐
3. ... there was a best friend that I felt very close to	☐	☐	☐	☐	☐
4. ... my friends took care of me when I felt bad	☐	☐	☐	☐	☐

15. These questions are about your haemophilia and PERCEIVED SUPPORT

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... others showed special consideration for me because of my haemophilia	☐	☐	☐	☐	☐
2. ... others showed understanding for my haemophilia	☐	☐	☐	☐	☐

3.	... I was able to talk to others about problems in connection with my haemophilia	☐	☐	☐	☐	☐
4.	... others stood by me	☐	☐	☐	☐	☐

16. These questions are about your haemophilia and OTHER PERSONS

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... I felt different from others because of my haemophilia	☐	☐	☐	☐	☐
2.	... I was bothered by others knowing about my haemophilia	☐	☐	☐	☐	☐
3.	... others kids teased me because of my haemophilia	☐	☐	☐	☐	☐
	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
4.	... people behaved differently towards me because of haemophilia	☐	☐	☐	☐	☐
5.	... I felt left out when others did things together	☐	☐	☐	☐	☐
6.	... others made dumb comments about my haemophilia	☐	☐	☐	☐	☐

17. These questions are about SPORTS AND SCHOOL

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... because of haemophilia I had to refrain from sports that I like	☐	☐	☐	☐	☐
2.	... I had to do indoor activities more than other kids because of my haemophilia	☐	☐	☐	☐	☐
3.	... I had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4.	... I did just as much sport as any other kid	☐	☐	☐	☐	☐
5.	... I was treated differently by teachers because of my haemophilia	☐	☐	☐	☐	☐
6.	... I participated in sports classes at school in spite of my haemophilia	☐	☐	☐	☐	☐
7.	... I was able to participate at school in spite of my haemophilia	☐	☐	☐	☐	☐

8.	... I had to refrain from special school events (e.g. outings) because of my haemophilia	☐	☐	☐	☐	☐
9.	... I found it difficult to pay attention at school because I was in pain	☐	☐	☐	☐	☐

18. The next questions are about DEALING WITH HAEMOPHILIA

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	… I tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2.	... I was careful with my body	☐	☐	☐	☐	☐
3.	... I was able to tell whether or not I was bleeding	☐	☐	☐	☐	☐
	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
4.	... I was in control of my complaints due to haemophilia	☐	☐	☐	☐	☐
5.	... I felt well informed about haemophilia	☐	☐	☐	☐	☐
6.	... haemophilia was a normal part of my life	☐	☐	☐	☐	☐
7.	... I accepted having haemophilia	☐	☐	☐	☐	☐

19. and your TREATMENT?

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... I was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2.	... the treatment I got was okay	☐	☐	☐	☐	☐
3.	... I trusted my doctors and nurses	☐	☐	☐	☐	☐
4.	... I disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5.	... I felt dependent on others because of my haemophilia	☐	☐	☐	☐	☐
6.	... the injections annoyed me	☐	☐	☐	☐	☐
7.	... I was annoyed about the amount of time spent having injections	☐	☐	☐	☐	☐
8.	... I felt interrupted in my activities by the injections	☐	☐	☐	☐	☐

20. What do you think about the FUTURE?

Recently ...	never	seldom	some-times	often	all of the time
1. ... I have been thinking that it will be difficult for me to lead a normal life	☐	☐	☐	☐	☐
2. ... I have been expecting that things will get better as I grow older	☐	☐	☐	☐	☐
3. ... I have been worrying about my health	☐	☐	☐	☐	☐
4. ... I have been sure about having a family later on	☐	☐	☐	☐	☐

21. What about RELATIONSHIPS?

Recently ...	never	seldom	some-times	often	all of the time
1. ... I have been finding it difficult to date because of my haemophilia	☐	☐	☐	☐	☐
2. ... I have been insecure in my relation-ships with girls because of my haemophilia	☐	☐	☐	☐	☐

HERE WE WOULD LIKE TO KNOW ABOUT YOUR PHYSICAL STATUS UND YOUR PHYSICAL ACTIVITIES

22. First we would like to know about your PHYSICAL STATUS…

	excellent	very good	good	fair	poor
1. How do you evaluate your actual physical activity?	☐	☐	☐	☐	☐

	much better	somewhat better	about the same as 1 year ago	somewhat worse	much worse
2. How do you evaluate your physical activity compared to last year?	☐	☐	☐	☐	☐

23. Now we would like to know about your MOBILITY

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. … I had stiff joints	☐	☐	☐	☐	☐
2. ... my mobility was affected	☐	☐	☐	☐	☐

3. … my physical activity was affected because of pain	☐	☐	☐	☐	☐
4. … my physical activity was affected because of chronic pain (e.g. aches)	☐	☐	☐	☐	☐

24. We would like to know what you think about your STRENGTH & COORDINATION

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. ... I had a lot of energy	☐	☐	☐	☐	☐
2. … I felt secure walking	☐	☐	☐	☐	☐
3. … I had problems climbing stairs	☐	☐	☐	☐	☐
4. … it was difficult for me to climb stairs without holding onto something	☐	☐	☐	☐	☐
5. … I had problems walking downstairs	☐	☐	☐	☐	☐
6. ... I had difficulty lifting or carrying heavy things	☐	☐	☐	☐	☐
7. … I had problems with keeping my balance on rough ground	☐	☐	☐	☐	☐

25. what about your ENDURANCE?

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. ... I could cover far distances (about 30 minutes or 2 km)	☐	☐	☐	☐	☐
2. ... I was exhausted after physical activities of medium difficulty (e.g. climbing stairs and carrying shopping)	☐	☐	☐	☐	☐
3. ... I was easily out of breath	☐	☐	☐	☐	☐
4. ... I could do physical activities longer than usual	☐	☐	☐	☐	☐
5. …I was easily tired	☐	☐	☐	☐	☐
6. … I could carry out exhausting activities (e.g. walking fast, cycling)	☐	☐	☐	☐	☐
7. … I did a lot with others	☐	☐	☐	☐	☐
8. … I was physically more active than usual	☐	☐	☐	☐	☐

26. these questions are concerning your BODY PERCEPTION

In the past 4 weeks ...	never	seldom	sometimes	often	always
1. … I felt steady on my feet	☐	☐	☐	☐	☐
2. … I felt fine in my body	☐	☐	☐	☐	☐
3. … I felt confident in my body	☐	☐	☐	☐	☐
4. … I felt my body was strong	☐	☐	☐	☐	☐
5. … I was self-confident	☐	☐	☐	☐	☐

IN THE NEXT SECTION WE WILL ASK YOU SOME QUESTIONS ABOUT YOUR PHYSICAL ACTIVITIES

27. TV/Video Viewing or Video/Computer Games

Average of the last 12 months ...	never	< 1 hour a day	1-2 hours a day	3-4 hours a day	> 4 hours a day
1. On a weekday	☐	☐	☐	☐	☐
2. On a weekend	☐	☐	☐	☐	☐

28. Attitudes towards sport

1.	Do you think doing sport is good? ☐ no ☐ yes
1a.	If yes, what do you think is good?
2.	Do you think that sport is bad or dangerous? ☐ no ☐ yes
2a.	If yes, what do you think is bad or dangerous?

29. We would like to know about your sports activities

1.	Do you do sport? ☐ no ☐ yes

If you do <u>NOT</u> do sport, continue with QUESTION 36

2.	Where do you do sport? ☐ in school ☐ in the public recreation ground ☐ in a team/sports club ☐ somewhere else (specify?) …….
3.	With whom do you do sports? ☐ alone ☐ with friends ☐ with my team ☐ with others (specify?) …….
4.	Have you ever injured yourself doing sports? ☐ never ☐ sometimes ☐ often ☐ always
5.	Are there sports which you like, but you have been told not do because of your haemophilia? ☐ no ☐ yes If yes, which?_____
5a.	Have you ever tried one of these sports? ☐ no ☐ yes If yes, which?_____
5b.	What happened? _____

30. How often do you do the following sports?

Per week…	never	once	twice	three times	more than three times
1. Football	☐	☐	☐	☐	☐
2. Swimming	☐	☐	☐	☐	☐
3. Gymnastics	☐	☐	☐	☐	☐
4. Jogging	☐	☐	☐	☐	☐
5. Others (specify?) …….	☐	☐	☐	☐	☐
6. Others (specify?) …….	☐	☐	☐	☐	☐

31. How long do you do the following sports for?

Every time…	1 hour	2 hours	more than 2 hours
1. Football	☐	☐	☐
2. Swimming	☐	☐	☐
3. Gymnastics	☐	☐	☐
4. Jogging	☐	☐	☐
5. Others (specify?) ……	☐	☐	☐
6. Others (specify?) ……	☐	☐	☐

32. …. and your friends?

1.	Do your friends do sport? ☐ no ☐ yes
2.	Which sports do your friends do? ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) ……
3.	Do your friends do any sport which you would like to do? ☐ no ☐ yes If yes, which? _____

33. …. and your parents?

1.	Do your parents do sport? ☐ no ☐ yes
1a.	Which sports do your parents do? ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) ……
2.	Are your parents interested in sport? ☐ no ☐ yes

34. Here we would like to know about competitive sports

(playing in competition with regular training)

1.	Are you a member of a sports team? ☐ no ☐ yes
2.	Do you participate in competitive sports? ☐ no ☐ yes
3.	Have you done competitive sports in the past? ☐ no ☐ yes
3a.	Have you had to stop doing competitive sports? ☐ no ☐ yes If yes, which? _____
3b.	If yes, why did you have to stop it? *(you can tick more than one box)* ☐ because of bleeding complications ☐ because of my decision ☐ because of the decision of my parents ☐ because of the decision of my doctor ☐ other reasons (specify?) _____
4.	Would you like to do competitive sports? ☐ no ☐ yes

35. We have some questions about the future

1.	Would you like to continue sports when you are older? ☐ no ☐ yes
2.	Would you like to try another sport? ☐ no ☐ yes
3.	If yes, which? ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?) …….

36. Here are questions only for kids who are NOT doing sports

1. Even though you are not doing sports would you like to do sports?

   ☐ no ☐ yes

2. If yes, which?

   ☐ football ☐ swimming ☐ gymnastics ☐ jogging ☐ others (specify?)

   ……..

3. Why do you not do sport?

   ☐ because I do not like it (I am not interested in)

   ☐ because I am afraid to hurt myself

   ☐ because my mother does not want me to do it

   ☐ because my father does not want me to do it

   ☐ because my doctor does not allow it

   ☐ other reasons, which?

   _____

3a. If you do not do sports because of your parents, do you know why your parents do not want you to do sports?

   ☐ no ☐ yes

3b. Why your parents do not want you to do sports?

   ☐ because sport is not important to them

   ☐ because they are afraid that I will hurt myself

   ☐ because my doctor does not allow it

   ☐ other reasons, which?

   _____

37. Would you like to tell us about your sporting life?

_____

_____

_____

38. What are your main interests?

_____

_____

_____

39. If you could express a wish, what would it be?

_____

_____

_____

THANKS FOR YOUR HELP!

Centre N°.: |__|__| ID N°.:|__|__|__| Initials: |__|__|

Questionnaire for parents of children aged 6-7 with haemophilia

Evaluation of the Impact of Sport Activities on Health-Related Qualify of Life of Haemophilia Patients

Dear _____

we have put together a few questions, which we would like you to answer. The questionnaire was made for patients with haemophilia.

We really appreciate you taking the time to complete this questionnaire about your health-related quality of life and your sporting activities.

We would like to understand more about the life of patients with haemophilia and how to improve the quality of haemophilia care.

For the following questions please observe the instructions below:

- Read each question carefully.
- Put a cross in the box corresponding to the answer that fits you best.
- Please write the answers on the lines provided
- Only make one cross for each question unless instructed to do otherwise
- Date of completing questionnaire:

  ___ / ___ / ___ (day / month / year)

All your answers will be treated with the strictest confidence!

1. First at all, we would like to know about you…

1.	Are you?	☐ Mother ☐ Father ☐ Others Who? _____
2.	How old are you?	☐☐ years
3.	What is your marital status?	☐ Single ☐ Married ☐ Widowed ☐ Divorced ☐ Separated
4.	Do you live together with a partner?	☐ Yes ☐ No
5.	How many children do you have?	☐☐
5a	How many have haemophilia?	☐☐
6.	With what kind of educational qualification did you finish school? (Please tick the box describing your highest educational qualification)	☐ no qualifications ☐ GCSE/O-Level ☐ NVQ ☐ A-Level ☐ university degree ☐ higher university degree
7.	Are you working?	☐ working full-time ☐ working part-time ☐ studying ☐ looking after the home full-time ☐ not employed, looking for work
8.	Where does your child live?	☐ a big city ☐ the suburbs or outskirts of a big city ☐ a town or a small city ☐ a country village ☐ a farm or home in the country

2. Some general questions

1. Did you know about the possibility of having a child with haemophilia?

   ☐ no ☐ yes

2. Do you think that your child's haemophilia has affected your life in some way (family, work, other…)?

   ☐ no ☐ yes, in what way?.....
   .....

3. Questions about your Child's Haemophilia

1. Have any other members of your family got/had haemophilia?

   ❑ No ❑ Yes If yes, please give detailed information about relationship, e.g. uncle, etc. _____

2. Is any member of your family physically disabled because of their haemophilia?

   ❑ No ❑ Yes If yes,
   who?_____

3. Are there things that your child cannot do because of his condition?

   ☐ No ☐ Yes If yes, which?

   _____

4. Did your child suffer from chronic pain in the past 6 months?

   (Definition: pain present without an apparent bleed, recurrent at least twice a week and lasting >3 hours without treatment)

   ❑ No ❑ Yes If YES, please specify the average intensity of pain on this visual scale
   (put an X on the line)

   0 10
   |_____|

   (no pain) (max. pain)

5. In the past 6 months how many days of school did your child lose because of his haemophilia?

   ____ days

6. In the past 6 months how many days of work did you lose because of your child's haemophilia?

   ____ days

NOW WE WOULD LIKE TO ASK YOU SEVERAL QUESTIONS ABOUT YOUR CHILD'S HAEMOPHILIA!

4. Here we would like to know about your child's BLEEDS (JOINT BLEEDS).

1.	How frequent were your child's bleeds in the last 4 weeks? ☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many?___

The following questions should only be answered if your child's had bleeds.

2.	How much was your child troubled by bleeds during the last 4 weeks? ☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit
3.	How severe were your child's bleeds during the last 4 weeks *(if your child had several bleeds, please answer for the most severe bleed)*? ☐ slight ☐ moderate ☐ severe ☐ very severe
4.	Did your child feel a strange sensation in his joints before he had a bleed? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
5.	Did your child have to stay quiet (e.g. lie in bed) when he had bleeds? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
6.	When your child had bleeds, did he inform you immediately? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. We would like to know who gave your child INJECTIONS.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my child gave himself his injections	☐	☐	☐	☐	☐
2.	... I gave my child his injections	☐	☐	☐	☐	☐
3.	... my partner/spouse gave my child his injections	☐	☐	☐	☐	☐
4.	... a nurse gave my child his injections	☐	☐	☐	☐	☐
5.	... a doctor gave my child his injections	☐	☐	☐	☐	☐

6. Here we would like to know about haemophilia and your child's PHYSICAL HEALTH.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my child's swellings hurt	☐	☐	☐	☐	☐
2.	... it was difficult for my child to move his arms or legs	☐	☐	☐	☐	☐
3.	... my child was afraid of bleeds	☐	☐	☐	☐	☐
4.	... my child was afraid of hurting himself	☐	☐	☐	☐	☐

7. ...and now about how your child has been FEELING because of his haemophilia

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... my child was in a bad mood because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child was sad because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child was angry because of his haemophilia	☐	☐	☐	☐	☐

8. How does haemophilia affect your child's VIEW OF HIMSELF?

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... my child envied healthy boys his age	☐	☐	☐	☐	☐
2. ... my child felt embarrassed about his haemophilia	☐	☐	☐	☐	☐

9. The next questions are about haemophilia and the FAMILY

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... I protected my child too much	☐	☐	☐	☐	☐
2. ... my husband/wife protected our child too much	☐	☐	☐	☐	☐
3. ... I criticised my child when he hurt himself	☐	☐	☐	☐	☐
4. ... I forbade my child doing certain things because of his haemophilia	☐	☐	☐	☐	☐

10. and then about haemophilia and your child's FRIENDS

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... my child was unable to do as much with his friends because of his haemophilia	☐	☐	☐	☐	☐

11. These questions are about your child's haemophilia and OTHER PERSONS

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child felt different from others because of his haemophilia	☐	☐	☐	☐	☐
2.	... my child felt left out when others did things together	☐	☐	☐	☐	☐

12. NURSERY SCHOOL/KINDERGARTEN

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child had to refrain from playing games that he enjoys because of haemophilia	☐	☐	☐	☐	☐
2.	... my child did just as much sport as any other kid	☐	☐	☐	☐	☐
3.	... my child was able to take part in games at nursery school/kinder-garten in spite of his haemophilia	☐	☐	☐	☐	☐

13. and the TREATMENT?

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
2.	... the injections annoyed my child	☐	☐	☐	☐	☐

14. Here we would like to know about your CHILD'S SCHOOL

1. Are there any sports that school forbids your child to do?

   ☐ no ☐ yes If yes, specify _____

2. Are you satisfied with the teachers understanding about haemophilia and their cooperation?

   ☐ no ☐ yes

3. Are you confident that teachers know what to do if your child has a bleed?

   ☐ no ☐ yes

4. Are you confident that teachers know what to do if your child has an injury?

   ☐ no ☐ yes

IN THE NEXT SECTION WE WILL ASK YOU SOME QUESTIONS ABOUT YOUR CHILD's PHYSICAL ACTIVITIES

15. TV/Video Viewing or Video/Computer Games

Average of the last 12 months ...	never	< 1 hour a day	1-2 hours a day	3-4 hours a day	> 4 hours a day
1. On a weekday	☐	☐	☐	☐	☐
2. On a weekend	☐	☐	☐	☐	☐

16. Attitudes towards Sport

1.	Do you think sport is beneficial? ☐ no ☐ yes
1a.	If yes, what in your opinion are the main benefits?
2.	Do you think there are any disadvantages due to sport? ☐ no ☐ yes
2a.	If yes, what in your opinion are the main disadvantages?

17. We would like to know about the sports activities of your child

1.	Does your child do sports? ☐ no ☐ yes ☐ yes, which?..... .....
2.	Are there other sports your child could do? ☐ no ☐ yes, which?..... .....
3.	How many sports can your child safely do? ☐ none ☐ some ☐ several ☐ all
3a	In you opinion how many sports can be safely done with prophylaxis? ☐ none ☐ some ☐ several ☐ all

If your child does NOT do sport, go the QUESTION 20

4.	Has your child ever injured himself doing sports? ☐ never ☐ sometimes ☐ often ☐ always
5.	Are there sports which he likes, but he can not do because of his haemophilia? ☐ no ☐ yes If yes, which?_____

18. How often does your child do the following sports?

Per week…	never	once	twice	three times	more than three times
1. Football	☐	☐	☐	☐	☐
2. Swimming	☐	☐	☐	☐	☐
3. Gymnastics	☐	☐	☐	☐	☐
4. Jogging	☐	☐	☐	☐	☐
5. Others (specifiy?) ……	☐	☐	☐	☐	☐
6. Others (specifiy?) ……	☐	☐	☐	☐	☐

19. How long does he do the following sports for?

Every time…	1 hour	2 hours	more than 2 hours
1. Football	☐	☐	☐
2. Swimming	☐	☐	☐
3. Gymnastics	☐	☐	☐
4. Jogging	☐	☐	☐
5. Others (specifiy?) ……	☐	☐	☐
6. Others (specifiy?) ……	☐	☐	☐

20. If your child does NOT do sports…

1.	Why does he not do sports? ☐ because he does not like it ☐ because he is afraid of hurting himself ☐ because I do not want him to do any sports

☐ because my partner does not want him to do any sports

☐ if you do not want him to do sport, please specify

which sport _____

and why_____

☐ because our doctor does not allow it

☐ other reasons, which?_____

21. What do you think about competitive sports for children?

(playing in competition with regular training)

1. Sports competition …

- ☐ are only for healthy children
- ☐ are based on the type of sport
- ☐ are dangerous for children with haemophilia
- ☐ depends on the child's ability in the particular sport
- ☐ depends on a doctor's advice
- ☐ should be possible for children, who have the desire to participate in competitive sports

WE WOULD NOW LIKE TO ASK YOU ABOUT YOUR AND YOUR PARTNER'S SPORTING ACTIVITIES?

22. Sporting Activities of the Mother

22.1. Present sporting activities

1. Do you/Does she do sports?

☐ no ☐ yes

1a. If yes, which sports do you/does she do?

☐ Aerobics ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?)

……

1b. How many hours do you/does she do per week?

☐ 1 hour ☐ 2 hours ☐ more than 2 hours

1c. If you do not/she does not make sports, why don't you?

- ☐ no interest
- ☐ I have/she has no time
- ☐ I do not/she does not have the opportunity
- ☐ because of my/her health condition
- ☐ other reasons (specify)……..

22.2. Past sporting activities

2.	Did you/she do sports in the past? ☐ no ☐ yes
2a.	If yes, which sports did you/she do? ☐ Aerobics ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
2b.	How many days did you/she do per week? ☐ 1 day ☐ 2 days ☐ 3 days ☐ more than 3 days
2c.	How many hours did you/she do per week? ☐ 1 hour ☐ 2 hours ☐ 3 hours ☐ 4 hours ☐ more than 4 hours:____

23. Sporting Activities of the Father

23.1. Present sporting activities

1.	Do you/Does he do sports? ☐ no ☐ yes
1a.	If yes, which sports do you/does he do? ☐ Football ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
1b.	How many hours do you/does he do per week? ☐ 1 hour ☐ 2 hours ☐ more than 2 hours
1c.	If you do not/he does not make sports, why don't you? ☐ no interest ☐ I have/he has no time ☐ I do not/he does not have the opportunity ☐ because of my/his health condition ☐ other reasons (specify)……

23.2. Past sporting activities

2.	Did you/he do sports in the past? ☐ no ☐ yes
2a.	If yes, which sports did you/he do? ☐ Football ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
2b.	How many days did you/he do per week? ☐ 1 day ☐ 2 days ☐ 3 days ☐ more than 3 days

2c. How many hours did you/he do per week?

☐ 1 hour ☐ 2 hours ☐ 3 hours ☐ 4 hours ☐ more than 4 hours:____

FINALLY YOU HAVE THE POSSIBILITY TO EXPRESS SOME PERSONAL THOUGHTS

24. Please would you give us your opinion on the implications of having haemophilia for your child and your family?

_____

_____

_____

25. How do you find your haemophilia centre in supporting you in making decisions about sport?

_____

_____

_____

26. The next questions ask for your VIEWS ABOUT YOUR OWN HEALTH.

This information will help keep track of how you feel and how well you are able to do your usual activities.

1. In general, would you say your health is: (circle one)

Excellent 1

Very good 2

Good 3

Fair 4

Poor 5

2. Compared to one year ago, how would you rate your health in general now? (circle one)

Much better now than one year ago 1

Somewhat better now than one year ago 2

About the same as one year ago 3

Somewhat worse now than one year ago 4

Much worse now than one year ago 5

3. The following questions are about activities you might do during a typical day. Does your health now limit you in these activities? If so, how much?

ACTIVITIES	**Yes, Limited A Lot**	**Yes, Limited A Little**	**No, Not Limited At All**
a. **Vigorous activities**, such as running, lifting heavy objects, participating in strenuous sports	1	2	3
b. **Moderate activities**, such as moving a table, pushing a vacuum cleaner, bowling, or playing golf	1	2	3
c. Lifting or carrying groceries	1	2	3
d. Climbing **several** flights of stairs	1	2	3
e. Climbing **one** flight of stairs	1	2	3

ACTIVITIES	**Yes, Limited A Lot**	**Yes, Limited A Little**	**No, Not Limited At All**
f. Bending, kneeling, or stooping	1	2	3
g. Walking **more than a mile**	1	2	3
h. Walking **half a mile**	1	2	3
i. Walking **one hundred yards**	1	2	3
j. Bathing or dressing yourself	1	2	3

4. During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of your physical health?

(circle one number on each line)

	YES	**NO**
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Were limited in the **kind** of work or other activities	1	2
d. Had **difficulty** performing the work or other activities (for example, it took extra effort)	1	2

5. During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of any emotional problems (such as feeling depressed or anxious)?

(circle one number on each line)

	YES	**NO**
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Didn't do work or other activities as **carefully** as usual	1	2

6. During the past 4 weeks, to what extent has your physical health or emotional problems interfered with your normal social activities with family, friends, neighbours, or groups?
(circle one)

Not at all 1

Slightly 2

Moderately 3

Quite a bit 4

Extremely 5

7. How much bodily pain have you had during the past 4 weeks?
(circle one)

None 1

Very mild 2

Mild 3

Moderate 4

Severe 5

Very severe 6

8. During the past 4 weeks, how much did pain interfere with your normal work (including both work outside the home and housework)?
(circle one)

Not at all 1

A little bit 2

Moderately 3

Quite a bit 4

Extremely 5

9. These questions are about how you feel and how things have been with you during the past 4 weeks. For each question, please give the one answer that comes closest to the way you have been feeling. How much of the time during the past 4 weeks -

(circle one number on each line)

	All of the Time	Most of the Time	A Good Bit of the Time	Some of the Time	A Little of the Time	None of the Time
a. Did you feel full of life?	1	2	3	4	5	6
b. Have you been a very nervous person?	1	2	3	4	5	6
c. Have you felt so down in the dumps that nothing could cheer you up?	1	2	3	4	5	6
d. Have you felt calm and peaceful?	1	2	3	4	5	6
e. Did you have a lot of energy?	1	2	3	4	5	6
f. Have you felt downhearted and low?	1	2	3	4	5	6
g. Did you feel worn out?	1	2	3	4	5	6
h. Have you been a happy person?	1	2	3	4	5	6
i. Did you feel tired?	1	2	3	4	5	6

10. During the past 4 weeks, how much of the time has your physical health or emotional problems interfered with your social activities (like visiting with friends, relatives, etc.)?

(circle one)

All of the time.....1

Most of the time.....2

Some of the time.....3

A little of the time.....4

None of the time.....5

11. How TRUE or FALSE is each of the following statements for you?

(circle one number on each line)

	Definitely True	**Mostly True**	**Don't Know**	**Mostly False**	**Definitely False**
a. I seem to get ill more easily than other people	1	2	3	4	5
b. I am as healthy as anybody I know	1	2	3	4	5
c. I expect my health to get worse	1	2	3	4	5
d. My health is excellent	1	2	3	4	5

THANKS FOR YOUR HELP!

Centre N°.: |__|__| ID N°.:|__|__|__| Initials: |__|__|

Questionnaire for parents of children aged 8-12 with haemophilia

Evaluation of the Impact of Sport Activities on Health-Related Qualify of Life of Haemophilia Patients

Dear _____

we have put together a few questions, which we would like you to answer. The questionnaire was made for patients with haemophilia.

We really appreciate you taking the time to complete this questionnaire about your health-related quality of life and your sporting activities.

We would like to understand more about the life of patients with haemophilia and how to improve the quality of haemophilia care.

For the following questions please observe the instructions below:

- Read each question carefully.
- Put a cross in the box corresponding to the answer that fits you best.
- Please write the answers on the lines provided
- Only make one cross for each question unless instructed to do otherwise
- Date of completing questionnaire:

  ___ / ___ / ___ (day / month / year)

All your answers will be treated with the strictest confidence!

1. First at all, we would like to know about you…

1.	Are you?	☐ Mother ☐ Father ☐ Others Who? _____
2.	How old are you?	☐☐ years
3.	What is your marital status?	☐ Single ☐ Married ☐ Widowed ☐ Divorced ☐ Separated
4.	Do you live together with a partner?	☐ Yes ☐ No
5.	How many children do you have?	☐☐
5a	How many have haemophilia?	☐☐
6.	With what kind of educational qualification did you finish school? (Please tick the box describing your highest educational qualification)	☐ no qualifications ☐ GCSE/O-Level ☐ NVQ ☐ A-Level ☐ university degree ☐ higher university degree
7.	Are you working?	☐ working full-time ☐ working part-time ☐ studying ☐ looking after the home full-time ☐ not employed, looking for work
8.	Where does your child live?	☐ a big city ☐ the suburbs or outskirts of a big city ☐ a town or a small city ☐ a country village ☐ a farm or home in the country

2. Some general questions

1. Did you know about the possibility of having a child with haemophilia?

   ☐ no ☐ yes

2. Do you think that your child's haemophilia has affected your life in some way (family, work, other…)?

   ☐ no ☐ yes, in what way?.....
   .....

3. Questions about your Child's Haemophilia

1. Have any other members of your family got/had haemophilia?

   ❑ No ❑ Yes If yes, please give detailed information about relationship, e.g. uncle, etc. _____

2. Is any member of your family physically disabled because of their haemophilia?

   ❑ No ❑ Yes If yes,
   who?_____

3. Are there things that your child cannot do because of his condition?

   ☐ No ☐ Yes If yes, which?

   _____

4. Did your child suffer from chronic pain in the past 6 months?

   (Definition: pain present without an apparent bleed, recurrent at least twice a week and lasting >3 hours without treatment)

   ❑ No ❑ Yes If YES, please specify the average intensity of pain on this visual scale
   (put an X on the line)

   0 10
   |_____|

   (no pain) (max. pain)

5. In the past 6 months how many days of school did your child lose because of his haemophilia?

   ____ days

6. In the past 6 months how many days of work did you lose because of your child's haemophilia?

   ____ days

NOW WE WOULD LIKE TO ASK YOU SEVERAL QUESTIONS ABOUT YOUR CHILD'S HAEMOPHILIA!

4. Here we would like to know about your child's BLEEDS (JOINT BLEEDS).

1.	How frequent were your child's bleeds in the last 4 weeks? ☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many?___

The following questions should only be answered if your child's had bleeds.

2.	How much was your child troubled by bleeds during the last 4 weeks? ☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit
3.	How severe were your child's bleeds during the last 4 weeks *(if your child had several bleeds, please answer for the most severe bleed)*? ☐ slight ☐ moderate ☐ severe ☐ very severe
4.	Did your child feel a strange sensation in his joints before he had a bleed? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
5.	Did your child have to stay quiet (e.g. lie in bed) when he had bleeds? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
6.	When your child had bleeds, did he inform you immediately? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. We would like to know who gave your child INJECTIONS.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my child gave himself his injections	☐	☐	☐	☐	☐
2.	... I gave my child his injections	☐	☐	☐	☐	☐
3.	... my partner/spouse gave my child his injections	☐	☐	☐	☐	☐
4.	... a nurse gave my child his injections	☐	☐	☐	☐	☐
5.	... a doctor gave my child his injections	☐	☐	☐	☐	☐

6. Here we would like to know about haemophilia and your child's PHYSICAL HEALTH.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my child's swellings hurt	☐	☐	☐	☐	☐
2.	... my child had pain in his joints	☐	☐	☐	☐	☐
3.	... it was painful for my child to move	☐	☐	☐	☐	☐
4.	... my child's joints felt stiff	☐	☐	☐	☐	☐

In the past 4 weeks ...	never	seldom	some-times	often	always
5. ... it was difficult for my child to move his arms or legs	☐	☐	☐	☐	☐
6. ... my child had difficulty walking as far as he wanted to	☐	☐	☐	☐	☐
7. ... my child was afraid of hurting himself	☐	☐	☐	☐	☐

7. ...and now about how your child has been FEELING because of his haemophilia

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... my child was in a bad mood because of his haemophilia	☐	☐	☐	☐	☐
2. ... my child was sad because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child's haemophilia was a burden to him	☐	☐	☐	☐	☐
4. ... my child was angry because of his haemophilia	☐	☐	☐	☐	☐
5. ... my child was worried about his haemophilia	☐	☐	☐	☐	☐
6. ... my child felt lonely because of his haemophilia	☐	☐	☐	☐	☐
7. ... my child was afraid of bleeds	☐	☐	☐	☐	☐

8. How does haemophilia affect your child's VIEW OF HIMSELF?

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... my child envied healthy boys his age	☐	☐	☐	☐	☐
2. ... my child felt physically weaker than other boys	☐	☐	☐	☐	☐
3. ... my child felt as well as other boys his age	☐	☐	☐	☐	☐
4. ... my child felt contented about his body	☐	☐	☐	☐	☐
5. ... haemophilia made my child's life more difficult	☐	☐	☐	☐	☐
6. ... my child felt embarrassed about his haemophilia	☐	☐	☐	☐	☐
7. ... my child had difficulty doing things with other kids his age	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
8.	... my child was unable to do as much with his friends because of his haemophilia	☐	☐	☐	☐	☐
9.	... my child felt healthy in spite of his haemophilia	☐	☐	☐	☐	☐

9. The next questions are about haemophilia and the FAMILY

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... I protected my child too much	☐	☐	☐	☐	☐
2.	... my husband/wife protected our child too much	☐	☐	☐	☐	☐
3.	... there were problems at home because of his haemophilia	☐	☐	☐	☐	☐
4.	... my child felt that he was causing his family trouble because of his haemophilia	☐	☐	☐	☐	☐
5.	... I limited my time at work or leisure because I had to look after my child	☐	☐	☐	☐	☐

10. and then about haemophilia and your child's FRIENDS

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child was able to talk to his friends about his haemophilia	☐	☐	☐	☐	☐
2.	... my child's best friend cared about how my child was feeling	☐	☐	☐	☐	☐
3.	... there was a best friend that my child felt very close to	☐	☐	☐	☐	☐
4.	... my child's friends took care of him when he felt bad	☐	☐	☐	☐	☐

11. These questions are about your child's haemophilia and PERCEIVED SUPPORT

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... others showed special consideration for my child because of his haemophilia	☐	☐	☐	☐	☐
2.	... others showed understanding for my child's haemophilia	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
3.	... my child was able to talk to others about problems connected with his haemophilia	☐	☐	☐	☐	☐
4.	... others stood by my child	☐	☐	☐	☐	☐

12. These questions are about your child's haemophilia and OTHER PERSONS

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child felt different from others because of his haemophilia	☐	☐	☐	☐	☐
2.	... my child was bothered by others knowing about his haemophilia	☐	☐	☐	☐	☐
3.	... my child was teased by others because of his haemophilia	☐	☐	☐	☐	☐
4.	... people behaved differently towards my child because of his haemophilia	☐	☐	☐	☐	☐
5.	... my child felt left out when others did things together	☐	☐	☐	☐	☐
6.	... other people made 'dumb' comments to my child about his haemophilia	☐	☐	☐	☐	☐

13. These questions are about SPORTS AND SCHOOL

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child had to refrain from sports that he likes because of haemophilia	☐	☐	☐	☐	☐
2.	... my child had to do indoor activities more than other kids because of his haemophilia	☐	☐	☐	☐	☐
3.	... my child had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4.	... my child did just as much sport as any other kid	☐	☐	☐	☐	☐
5.	... my child participated in sports classes at school in spite of his haemophilia	☐	☐	☐	☐	☐
6.	... my child was able to participate at school in spite of his haemophilia	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
7.	... my child had to refrain from special school events (e.g. outings) because of his haemophilia	☐	☐	☐	☐	☐
8.	... my child found it difficult to concentrate at school because he was in pain	☐	☐	☐	☐	☐

14. The next questions are about DEALING WITH HAEMOPHILIA

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2.	... my child was careful with his body	☐	☐	☐	☐	☐
3.	... my child was able to tell whether or not he was bleeding	☐	☐	☐	☐	☐
4.	... my child was in control of his complaints due to haemophilia	☐	☐	☐	☐	☐
5.	... my child felt well-informed about haemophilia	☐	☐	☐	☐	☐
6.	... haemophilia was a normal part of his life	☐	☐	☐	☐	☐
7.	... my child accepted having haemophilia	☐	☐	☐	☐	☐

15. and the Treatment?

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2.	... the treatment my child got was okay	☐	☐	☐	☐	☐
3.	... my child trusted his doctors and nurses	☐	☐	☐	☐	☐
4.	... my child disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5.	... the injections annoyed my child	☐	☐	☐	☐	☐
6.	... my child was annoyed about the amount of time spent having the injections	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
7.	... my child felt interrupted in his activities by the injections	☐	☐	☐	☐	☐

16. Here we would like to know about your CHILD'S SCHOOL

1.	Are there any sports that school forbids your child to do? ☐ no ☐ yes If yes, specify _____
2.	Are you satisfied with the teachers understanding about haemophilia and their cooperation? ☐ no ☐ yes
3.	Are you confident that teachers know what to do if your child has a bleed? ☐ no ☐ yes
4.	Are you confident that teachers know what to do if your child has an injury? ☐ no ☐ yes

IN THE NEXT SECTION WE WILL ASK YOU SOME QUESTIONS ABOUT YOUR CHILD's PHYSICAL ACTIVITIES

17. TV/Video Viewing or Video/Computer Games

Average of the last 12 months ...	never	< 1 hour a day	1-2 hours a day	3-4 hours a day	> 4 hours a day
1. On a weekday	☐	☐	☐	☐	☐
2. On a weekend	☐	☐	☐	☐	☐

18. Attitudes towards Sport

1.	Do you think sport is beneficial? ☐ no ☐ yes
1a.	If yes, what in your opinion are the main benefits?
2.	Do you think there are any disadvantages due to sport? ☐ no ☐ yes
2a.	If yes, what in your opinion are the main disadvantages?

19. We would like to know about the sports activities of your child

1.	Does your child do sports? ☐ no ☐ yes ☐ yes, which?..... .....
2.	Are there other sports your child could do? ☐ no ☐ yes, which?..... .....
3.	How many sports can your child safely do? ☐ none ☐ some ☐ several ☐ all
3a	In you opinion how many sports can be safely done with prophylaxis? ☐ none ☐ some ☐ several ☐ all

If your child does NOT do sport, go the QUESTION 22

4.	Has your child ever injured himself doing sports? ☐ never ☐ sometimes ☐ often ☐ always
5.	Are there sports which he likes, but he can not do because of his haemophilia? ☐ no ☐ yes If yes, which?_____

20. How often does your child do the following sports?

Per week…	never	once	twice	three times	more than three times
1. Football	☐	☐	☐	☐	☐
2. Swimming	☐	☐	☐	☐	☐
3. Gymnastics	☐	☐	☐	☐	☐
4. Jogging	☐	☐	☐	☐	☐
5. Others (specifiy?) ……	☐	☐	☐	☐	☐
6. Others (specifiy?) ……	☐	☐	☐	☐	☐

21. How long does he do the following sports for?

Every time…	1 hour	2 hours	more than 2 hours
1. Football	☐	☐	☐
2. Swimming	☐	☐	☐
3. Gymnastics	☐	☐	☐
4. Jogging	☐	☐	☐
5. Others (specifiy?) ……	☐	☐	☐
6. Others (specifiy?) ……	☐	☐	☐

22. If your child does NOT do sports…

1. Why does he not do sports?

☐ because he does not like it

☐ because he is afraid of hurting himself

☐ because I do not want him to do any sports

☐ because my partner does not want him to do any sports

☐ if you do not want him to do sport, please specify

which sport _____

and why_____

☐ because our doctor does not allow it

☐ other reasons, which?_____

23. What do you think about competitive sports for children?

(playing in competition with regular training)

1. Sports competition …

☐ are only for healthy children

☐ are based on the type of sport

☐ are dangerous for children with haemophilia

☐ depends on the child's ability in the particular sport

☐ depends on a doctor's advice

☐ should be possible for children, who have the desire to participate in competitive sports

WE WOULD NOW LIKE TO ASK YOU ABOUT YOUR AND YOUR PARTNER'S SPORTING ACTIVITIES?

24. Sporting Activities of the Mother

24.1. Present sporting activities

1.	Do you/Does she do sports? ☐ no ☐ yes
1a.	If yes, which sports do you/does she do? ☐ Aerobics ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
1b.	How many hours do you/does she do per week? ☐ 1 hour ☐ 2 hours ☐ more than 2 hours

1c.	If you do not/she does not make sports, why don't you? ☐ no interest ☐ I have/she has no time ☐ I do not/she does not have the opportunity ☐ because of my/her health condition ☐ other reasons (specify)……

24.2. Past sporting activities

2.	Did you/she do sports in the past? ☐ no ☐ yes
2a.	If yes, which sports did you/she do? ☐ Aerobics ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
2b.	How many days did you/she do per week? ☐ 1 day ☐ 2 days ☐ 3 days ☐ more than 3 days
2c.	How many hours did you/she do per week? ☐ 1 hour ☐ 2 hours ☐ 3 hours ☐ 4 hours ☐ more than 4 hours:_____

25. Sporting Activities of the Father

25.1. Present sporting activities

1.	Do you/Does he do sports? ☐ no ☐ yes
1a.	If yes, which sports do you/does he do? ☐ Football ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
1b.	How many hours do you/does he do per week? ☐ 1 hour ☐ 2 hours ☐ more than 2 hours
1c.	If you do not/he does not make sports, why don't you? ☐ no interest ☐ I have/he has no time ☐ I do not/he does not have the opportunity ☐ because of my/his health condition ☐ other reasons (specify)……

25.2. Past sporting activities

2.	Did you/he do sports in the past? ☐ no ☐ yes
2a.	If yes, which sports did you/he do? ☐ Football ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
2b.	How many days did you/he do per week? ☐ 1 day ☐ 2 days ☐ 3 days ☐ more than 3 days
2c.	How many hours did you/he do per week? ☐ 1 hour ☐ 2 hours ☐ 3 hours ☐ 4 hours ☐ more than 4 hours:____

FINALLY YOU HAVE THE POSSIBILITY TO EXPRESS SOME PERSONAL THOUGHTS

26. Please would you give us your opinion on the implications of having haemophilia for your child and your family?

_____

_____

_____

27. How do you find your haemophilia centre in supporting you in making decisions about sport?

_____

_____

_____

28. The next questions ask for your VIEWS ABOUT YOUR OWN HEALTH.

This information will help keep track of how you feel and how well you are able to do your usual activities.

1. In general, would you say your health is: (circle one)

Excellent.....1

Very good.....2

Good.....3

Fair.....4

Poor.....5

2. Compared to one year ago, how would you rate your health in general now? (circle one)

Much better now than one year ago.....1

Somewhat better now than one year ago.....2

About the same as one year ago.....3

Somewhat worse now than one year ago 4

Much worse now than one year ago.....5

3. The following questions are about activities you might do during a typical day. Does your health now limit you in these activities? If so, how much?

ACTIVITIES	**Yes, Limited A Lot**	**Yes, Limited A Little**	**No, Not Limited At All**
a. **Vigorous activities**, such as running, lifting heavy objects, participating in strenuous sports	1	2	3
b. **Moderate activities**, such as moving a table, pushing a vacuum cleaner, bowling, or playing golf	1	2	3
c. Lifting or carrying groceries	1	2	3
d. Climbing **several** flights of stairs	1	2	3
e. Climbing **one** flight of stairs	1	2	3
ACTIVITIES	**Yes, Limited A Lot**	**Yes, Limited A Little**	**No, Not Limited At All**
f. Bending, kneeling, or stooping	1	2	3
g. Walking **more than a mile**	1	2	3
h. Walking **half a mile**	1	2	3
i. Walking **one hundred yards**	1	2	3
j. Bathing or dressing yourself	1	2	3

4. During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of your physical health?

(circle one number on each line)

	YES	**NO**
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Were limited in the **kind** of work or other activities	1	2
d. Had **difficulty** performing the work or other activities (for example, it took extra effort)	1	2

5. During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of any emotional problems (such as feeling depressed or anxious)?

(circle one number on each line)

	YES	NO
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Didn't do work or other activities as **carefully** as usual	1	2

6. During the past 4 weeks, to what extent has your physical health or emotional problems interfered with your normal social activities with family, friends, neighbours, or groups?

(circle one)

Not at all 1

Slightly 2

Moderately 3

Quite a bit 4

Extremely 5

7. How much bodily pain have you had during the past 4 weeks?

(circle one)

None 1

Very mild 2

Mild 3

Moderate 4

Severe 5

Very severe 6

8. During the past 4 weeks, how much did pain interfere with your normal work (including both work outside the home and housework)?

(circle one)

Not at all 1

A little bit 2

Moderately.....3

Quite a bit.....4

Extremely.....5

9. These questions are about how you feel and how things have been with you during the past 4 weeks. For each question, please give the one answer that comes closest to the way you have been feeling. How much of the time during the past 4 weeks -

(circle one number on each line)

	All of the Time	Most of the Time	A Good Bit of the Time	Some of the Time	A Little of the Time	None of the Time
a. Did you feel full of life?	1	2	3	4	5	6
b. Have you been a very nervous person?	1	2	3	4	5	6
c. Have you felt so down in the dumps that nothing could cheer you up?	1	2	3	4	5	6
d. Have you felt calm and peaceful?	1	2	3	4	5	6
e. Did you have a lot of energy?	1	2	3	4	5	6
f. Have you felt downhearted and low?	1	2	3	4	5	6
g. Did you feel worn out?	1	2	3	4	5	6
h. Have you been a happy person?	1	2	3	4	5	6
i. Did you feel tired?	1	2	3	4	5	6

10. During the past 4 weeks, how much of the time has your physical health or emotional problems interfered with your social activities (like visiting with friends, relatives, etc.)?

(circle one)

All of the time.....1

Most of the time.....2

Some of the time.....3

A little of the time.....4

None of the time.....5

11. How TRUE or FALSE is each of the following statements for you?

(circle one number on each line)

	Definitely True	**Mostly True**	**Don't Know**	**Mostly False**	**Definitely False**
a. I seem to get ill more easily than other people	1	2	3	4	5
b. I am as healthy as anybody I know	1	2	3	4	5
c. I expect my health to get worse	1	2	3	4	5
d. My health is excellent	1	2	3	4	5

THANKS FOR YOUR HELP!

Centre N°.: |__|__| ID N°.:|__|__|__| Initials: |__|__|

Questionnaire for parents of children aged 13-17 with haemophilia

Evaluation of the Impact of Sport Activities on Health-Related Qualify of Life of Haemophilia Patients

Dear _____

we have put together a few questions, which we would like you to answer. The questionnaire was made for patients with haemophilia.

We really appreciate you taking the time to complete this questionnaire about your health-related quality of life and your sporting activities.

We would like to understand more about the life of patients with haemophilia and how to improve the quality of haemophilia care.

For the following questions please observe the instructions below:

- Read each question carefully.
- Put a cross in the box corresponding to the answer that fits you best.
- Please write the answers on the lines provided
- Only make one cross for each question unless instructed to do otherwise
- Date of completing questionnaire:

  ___ / ___ / ___ (day / month / year)

All your answers will be treated with the strictest confidence!

1. First at all, we would like to know about you…

1.	Are you?	☐ Mother ☐ Father ☐ Others Who? _____
2.	How old are you?	☐☐ years
3.	What is your marital status?	☐ Single ☐ Married ☐ Widowed ☐ Divorced ☐ Separated
4.	Do you live together with a partner?	☐ Yes ☐ No
5.	How many children do you have?	☐☐
5a	How many have haemophilia?	☐☐
6.	With what kind of educational qualification did you finish school? (Please tick the box describing your highest educational qualification)	☐ no qualifications ☐ GCSE/O-Level ☐ NVQ ☐ A-Level ☐ university degree ☐ higher university degree
7.	Are you working?	☐ working full-time ☐ working part-time ☐ studying ☐ looking after the home full-time ☐ not employed, looking for work
8.	Where does your child live?	☐ a big city ☐ the suburbs or outskirts of a big city ☐ a town or a small city ☐ a country village ☐ a farm or home in the country

2. Some general questions

1. Did you know about the possibility of having a child with haemophilia?

   ☐ no ☐ yes

2. Do you think that your child's haemophilia has affected your life in some way (family, work, other…)?

   ☐ no ☐ yes, in what way?.....
   .....

3. Questions about your Child's Haemophilia

1. Have any other members of your family got/had haemophilia?

   ❑ No ❑ Yes If yes, please give detailed information about relationship, e.g. uncle, etc. _____

2. Is any member of your family physically disabled because of their haemophilia?

   ❑ No ❑ Yes If yes,
   who?_____

3. Are there things that your child cannot do because of his condition?

   ☐ No ☐ Yes If yes, which?

   _____

4. Did your child suffer from chronic pain in the past 6 months?

   (Definition: pain present without an apparent bleed, recurrent at least twice a week and lasting >3 hours without treatment)

   ❑ No ❑ Yes If YES, please specify the average intensity of pain on this visual scale
   (put an X on the line)

   0 10
   |_____|

   (no pain) (max. pain)

5. In the past 6 months how many days of school did your child lose because of his haemophilia?

   ____ days

6. In the past 6 months how many days of work did you lose because of your child's haemophilia?

   ____ days

NOW WE WOULD LIKE TO ASK YOU SEVERAL QUESTIONS ABOUT YOUR CHILD'S HAEMOPHILIA!

4. Here we would like to know about your child's BLEEDS (JOINT BLEEDS).

1.	How frequent were your child's bleeds in the last 4 weeks? ☐ no bleeds ☐ 1 ☐ 2 ☐ more than 2 How many?___

The following questions should only be answered if your child's had bleeds.

2.	How much was your child troubled by bleeds during the last 4 weeks? ☐ not at all ☐ somewhat ☐ moderately ☐ quite a bit
3.	How severe were your child's bleeds during the last 4 weeks *(if your child had several bleeds, please answer for the most severe bleed)*? ☐ slight ☐ moderate ☐ severe ☐ very severe
4.	Did your child feel a strange sensation in his joints before he had a bleed? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
5.	Did your child have to stay quiet (e.g. lie in bed) when he had bleeds? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always
6.	When your child had bleeds, did he inform you immediately? ☐ never ☐ seldom ☐ sometimes ☐ often ☐ always

5. We would like to know who gave your child INJECTIONS.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my child gave himself his injections	☐	☐	☐	☐	☐
2.	... I gave my child his injections	☐	☐	☐	☐	☐
3.	... my partner/spouse gave my child his injections	☐	☐	☐	☐	☐
4.	... a nurse gave my child his injections	☐	☐	☐	☐	☐
5.	... a doctor gave my child his injections	☐	☐	☐	☐	☐

6. Here we would like to know about haemophilia and your child's PHYSICAL HEALTH.

	In the past 4 weeks ...	never	seldom	some-times	often	always
1.	... my child's swellings hurt	☐	☐	☐	☐	☐
2.	... my child had pain in his joints	☐	☐	☐	☐	☐
3.	... it was painful for my child to move	☐	☐	☐	☐	☐
4.	... my child's joints felt stiff	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	always
5.	... it was difficult for my child to move his arms or legs	☐	☐	☐	☐	☐
6.	... my child had difficulty walking as far as he wanted to	☐	☐	☐	☐	☐
7.	... my child was afraid of hurting himself	☐	☐	☐	☐	☐

7. ...and now about how your child has been FEELING because of his haemophilia

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child was in a bad mood because of his haemophilia	☐	☐	☐	☐	☐
2.	... my child was sad because of his haemophilia	☐	☐	☐	☐	☐
3.	... my child's haemophilia was a burden to him	☐	☐	☐	☐	☐
4.	... my child was angry because of his haemophilia	☐	☐	☐	☐	☐
5.	... my child was worried about his haemophilia	☐	☐	☐	☐	☐
6.	... my child felt lonely because of his haemophilia	☐	☐	☐	☐	☐
7.	... my child was afraid of bleeds	☐	☐	☐	☐	☐
8.	... my child felt excluded by his friends	☐	☐	☐	☐	☐

8. How does haemophilia affect your child's VIEW OF HIMSELF?

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child envied healthy boys his age	☐	☐	☐	☐	☐
2.	... my child felt physically weaker than other boys	☐	☐	☐	☐	☐
3.	... my child felt as well as other boys his age	☐	☐	☐	☐	☐
4.	... my child felt contented about his body	☐	☐	☐	☐	☐
5.	... haemophilia made my child's life more difficult	☐	☐	☐	☐	☐
6.	... my child was happy in spite of his haemophilia	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
7.	... my child felt embarrassed about his haemophilia	☐	☐	☐	☐	☐
8.	... my child had difficulty doing things with other kids his age	☐	☐	☐	☐	☐
9.	... my child was unable to do as much with his friends because of his haemophilia	☐	☐	☐	☐	☐
10.	... my child felt healthy in spite of his haemophilia	☐	☐	☐	☐	☐

9. The next questions are about haemophilia and the FAMILY

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child was treated differently by the family because of his haemophilia	☐	☐	☐	☐	☐
2.	... I protected my child too much	☐	☐	☐	☐	☐
3.	... my husband/wife protected our child too much	☐	☐	☐	☐	☐
4.	... I criticised my child when he hurt himself	☐	☐	☐	☐	☐
5.	... I forbade my child doing certain things because of his haemophilia	☐	☐	☐	☐	☐
6.	... there were problems at home because of his haemophilia	☐	☐	☐	☐	☐
7.	... my child felt that he was causing his family trouble because of his haemophilia	☐	☐	☐	☐	☐
8.	... I limited my time at work or leisure because I had to look after my child	☐	☐	☐	☐	☐

10. and then about haemophilia and your child's FRIENDS

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child was able to talk to his friends about his haemophilia	☐	☐	☐	☐	☐
2.	... my child's best friend cared about how my child was feeling	☐	☐	☐	☐	☐
3.	... there was a best friend that my child felt very close to	☐	☐	☐	☐	☐

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
4.	... my child's friends took care of him when he felt bad	☐	☐	☐	☐	☐

11. These questions are about your child's haemophilia and PERCEIVED SUPPORT

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... others showed special consideration for my child because of his haemophilia	☐	☐	☐	☐	☐
2.	... others showed understanding for my child's haemophilia	☐	☐	☐	☐	☐
3.	... my child was able to talk to others about problems connected with his haemophilia	☐	☐	☐	☐	☐
4.	... others stood by my child	☐	☐	☐	☐	☐

12. These questions are about your child's haemophilia and OTHER PERSONS

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child felt different from others because of his haemophilia	☐	☐	☐	☐	☐
2.	... my child was bothered by others knowing about his haemophilia	☐	☐	☐	☐	☐
3.	... my child was teased by others because of his haemophilia	☐	☐	☐	☐	☐
4.	... people behaved differently towards my child because of his haemophilia	☐	☐	☐	☐	☐
5.	... my child felt left out when others did things together	☐	☐	☐	☐	☐
6.	... other people made ‘dumb’ comments to my child about his haemophilia	☐	☐	☐	☐	☐

13. These questions are about SPORTS AND SCHOOL

	In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1.	... my child had to refrain from sports that he likes because of haemophilia	☐	☐	☐	☐	☐

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
2. ... my child had to do indoor activities more than other kids because of his haemophilia	☐	☐	☐	☐	☐
3. ... my child had to refrain from sports like rollerblading or soccer	☐	☐	☐	☐	☐
4. ... my child did just as much sport as any other kid	☐	☐	☐	☐	☐
5. ... my child was treated differently by teachers because of his haemophilia	☐	☐	☐	☐	☐
6. ... my child participated in sports classes at school in spite of his haemophilia	☐	☐	☐	☐	☐
7. ... my child was able to participate at school in spite of his haemophilia	☐	☐	☐	☐	☐
8. ... my child had to refrain from special school events (e.g. outings) because of his haemophilia	☐	☐	☐	☐	☐
9. ... my child found it difficult to concentrate at school because he was in pain	☐	☐	☐	☐	☐

14. The next questions are about DEALING WITH HAEMOPHILIA

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... my child tried to recognise early on when a bleed developed	☐	☐	☐	☐	☐
2. ... my child was careful with his body	☐	☐	☐	☐	☐
3. ... my child was able to tell whether or not he was bleeding	☐	☐	☐	☐	☐
4. ... my child was in control of his complaints due to haemophilia	☐	☐	☐	☐	☐
5. ... my child felt well-informed about haemophilia	☐	☐	☐	☐	☐
6. ... haemophilia was a normal part of his life	☐	☐	☐	☐	☐
7. ... my child accepted having haemophilia	☐	☐	☐	☐	☐

15. and the Treatment?

In the past 4 weeks ...	never	seldom	some-times	often	all of the time
1. ... my child was satisfied with the haemophilia centre	☐	☐	☐	☐	☐
2. ... the treatment my child got was okay	☐	☐	☐	☐	☐
3. ... my child trusted his doctors and nurses	☐	☐	☐	☐	☐
4. ... my child disliked visiting the haemophilia centre	☐	☐	☐	☐	☐
5. ... my child felt dependent on others because of his haemophilia	☐	☐	☐	☐	☐
6. ... the injections annoyed my child	☐	☐	☐	☐	☐
7. ... my child was annoyed about the amount of time spent having the injections	☐	☐	☐	☐	☐
8. ... my child felt interrupted in his activities by the injections	☐	☐	☐	☐	☐

16. What does your child think about the FUTURE?

Recently ...	never	seldom	some-times	often	all of the time
1. ... my child has been thinking it will be difficult to lead a normal life	☐	☐	☐	☐	☐
2. ... my child has been thinking that things will get better as he grows older	☐	☐	☐	☐	☐
3. ... my child has been worried about his health	☐	☐	☐	☐	☐
4. ... my child has been sure about having a family of his own later on	☐	☐	☐	☐	☐

17. What about RELATIONSHIPS?

Recently ...	never	seldom	some-times	often	all of the time
1. ... my son has found it difficult to date because of his haemophilia	☐	☐	☐	☐	☐
2. ... my son has been insecure in his relationships with girls because of his haemophilia	☐	☐	☐	☐	☐

18. Here we would like to know about your CHILD'S SCHOOL

1.	Are there any sports that school forbids your child to do? ☐ no ☐ yes If yes, specify _____
2.	Are you satisfied with the teachers understanding about haemophilia and their cooperation? ☐ no ☐ yes
3.	Are you confident that teachers know what to do if your child has a bleed? ☐ no ☐ yes
4.	Are you confident that teachers know what to do if your child has an injury? ☐ no ☐ yes

IN THE NEXT SECTION WE WILL ASK YOU SOME QUESTIONS ABOUT YOUR CHILD's PHYSICAL ACTIVITIES

19. TV/Video Viewing or Video/Computer Games

Average of the last 12 months ...	never	< 1 hour a day	1-2 hours a day	3-4 hours a day	> 4 hours a day
1. On a weekday	☐	☐	☐	☐	☐
2. On a weekend	☐	☐	☐	☐	☐

20. Attitudes towards Sport

1.	Do you think sport is beneficial? ☐ no ☐ yes
1a.	If yes, what in your opinion are the main benefits?
2.	Do you think there are any disadvantages due to sport? ☐ no ☐ yes
2a.	If yes, what in your opinion are the main disadvantages?

21. We would like to know about the sports activities of your child

1.	Does your child do sports? ☐ no ☐ yes ☐ yes, which?..... .....
2.	Are there other sports your child could do? ☐ no ☐ yes, which?..... .....
3.	How many sports can your child safely do? ☐ none ☐ some ☐ several ☐ all
3a	In you opinion how many sports can be safely done with prophylaxis? ☐ none ☐ some ☐ several ☐ all

If your child does NOT do sport, go the QUESTION 24

4.	Has your child ever injured himself doing sports? ☐ never ☐ sometimes ☐ often ☐ always
5.	Are there sports which he likes, but he can not do because of his haemophilia? ☐ no ☐ yes If yes, which?_____

22. How often does your child do the following sports?

Per week…	never	once	twice	three times	more than three times
1. Football	☐	☐	☐	☐	☐
2. Swimming	☐	☐	☐	☐	☐
3. Gymnastics	☐	☐	☐	☐	☐
4. Jogging	☐	☐	☐	☐	☐
5. Others (specifiy?) ……	☐	☐	☐	☐	☐
6. Others (specifiy?) ……	☐	☐	☐	☐	☐

23. How long does he do the following sports for?

Every time…	1 hour	2 hours	more than 2 hours
1. Football	☐	☐	☐
2. Swimming	☐	☐	☐
3. Gymnastics	☐	☐	☐
4. Jogging	☐	☐	☐
5. Others (specifiy?) ……	☐	☐	☐
6. Others (specifiy?) ……	☐	☐	☐

24. If your child does NOT do sports…

1. Why does he not do sports?
   - ☐ because he does not like it
   - ☐ because he is afraid of hurting himself
   - ☐ because I do not want him to do any sports
   - ☐ because my partner does not want him to do any sports
   - ☐ if you do not want him to do sport, please specify
     which sport _____
     and why_____
   - ☐ because our doctor does not allow it
   - ☐ other reasons, which?_____

25. What do you think about competitive sports for children?

(playing in competition with regular training)

1. Sports competition …
   - ☐ are only for healthy children
   - ☐ are based on the type of sport
   - ☐ are dangerous for children with haemophilia
   - ☐ depends on the child's ability in the particular sport
   - ☐ depends on a doctor's advice
   - ☐ should be possible for children, who have the desire to participate in competitive sports

WE WOULD NOW LIKE TO ASK YOU ABOUT YOUR AND YOUR PARTNER'S SPORTING ACTIVITIES?

26. Sporting Activities of the Mother

26.1. Present sporting activities

1.	Do you/Does she do sports? ☐ no ☐ yes
1a.	If yes, which sports do you/does she do? ☐ Aerobics ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
1b.	How many hours do you/does she do per week? ☐ 1 hour ☐ 2 hours ☐ more than 2 hours
1c.	If you do not/she does not make sports, why don't you? ☐ no interest ☐ I have/she has no time ☐ I do not/she does not have the opportunity ☐ because of my/her health condition ☐ other reasons (specify)……..

26.2. Past sporting activities

2.	Did you/she do sports in the past? ☐ no ☐ yes
2a.	If yes, which sports did you/she do? ☐ Aerobics ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
2b.	How many days did you/she do per week? ☐ 1 day ☐ 2 days ☐ 3 days ☐ more than 3 days
2c.	How many hours did you/she do per week? ☐ 1 hour ☐ 2 hours ☐ 3 hours ☐ 4 hours ☐ more than 4 hours:____

27. Sporting Activities of the Father

27.1. Present sporting activities

1.	Do you/Does he do sports? ☐ no ☐ yes
1a.	If yes, which sports do you/does he do? ☐ Football ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
1b.	How many hours do you/does he do per week? ☐ 1 hour ☐ 2 hours ☐ more than 2 hours
1c.	If you do not/he does not make sports, why don't you? ☐ no interest ☐ I have/he has no time ☐ I do not/he does not have the opportunity ☐ because of my/his health condition ☐ other reasons (specify)……..

26.2. Past sporting activities

2.	Did you/he do sports in the past? ☐ no ☐ yes
2a.	If yes, which sports did you/he do? ☐ Football ☐ Swimming ☐ Gymnastics ☐ Jogging ☐ other (specify?) ……
2b.	How many days did you/he do per week? ☐ 1 day ☐ 2 days ☐ 3 days ☐ more than 3 days
2c.	How many hours did you/he do per week? ☐ 1 hour ☐ 2 hours ☐ 3 hours ☐ 4 hours ☐ more than 4 hours:____

FINALLY YOU HAVE THE POSSIBILITY TO EXPRESS SOME PERSONAL THOUGHTS

28. Please would you give us your opinion on the implications of having haemophilia for your child and your family?

_____

_____

_____

29. How do you find your haemophilia centre in supporting you in making decisions about sport?

_____

_____

_____

30. The next questions ask for your VIEWS ABOUT YOUR OWN HEALTH.

This information will help keep track of how you feel and how well you are able to do your usual activities.

1. In general, would you say your health is: (circle one)

Excellent.....1

Very good.....2

Good.....3

Fair.....4

Poor.....5

2. Compared to one year ago, how would you rate your health in general now? (circle one)

Much better now than one year ago.....1

Somewhat better now than one year ago.....2

About the same as one year ago.....3

Somewhat worse now than one year ago 4

Much worse now than one year ago.....5

3. The following questions are about activities you might do during a typical day. Does your health now limit you in these activities? If so, how much?

ACTIVITIES	**Yes, Limited A Lot**	**Yes, Limited A Little**	**No, Not Limited At All**
a. **Vigorous activities**, such as running, lifting heavy objects, participating in strenuous sports	1	2	3
b. **Moderate activities**, such as moving a table, pushing a vacuum cleaner, bowling, or playing golf	1	2	3
c. Lifting or carrying groceries	1	2	3
d. Climbing **several** flights of stairs	1	2	3
e. Climbing **one** flight of stairs	1	2	3
ACTIVITIES	**Yes, Limited A Lot**	**Yes, Limited A Little**	**No, Not Limited At All**
f. Bending, kneeling, or stooping	1	2	3
g. Walking **more than a mile**	1	2	3
h. Walking **half a mile**	1	2	3
i. Walking **one hundred yards**	1	2	3
j. Bathing or dressing yourself	1	2	3

4. During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of your physical health?

(circle one number on each line)

	YES	**NO**
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Were limited in the **kind** of work or other activities	1	2
d. Had **difficulty** performing the work or other activities (for example, it took extra effort)	1	2

5. During the past 4 weeks, have you had any of the following problems with your work or other regular daily activities as a result of any emotional problems (such as feeling depressed or anxious)?

(circle one number on each line)

	YES	NO
a. Cut down on the **amount of time** you spent on work or other activities	1	2
b. **Accomplished less** than you would like	1	2
c. Didn't do work or other activities as **carefully** as usual	1	2

6. During the past 4 weeks, to what extent has your physical health or emotional problems interfered with your normal social activities with family, friends, neighbours, or groups?

(circle one)

Not at all 1

Slightly 2

Moderately 3

Quite a bit 4

Extremely 5

7. How much bodily pain have you had during the past 4 weeks?

(circle one)

None 1

Very mild 2

Mild 3

Moderate 4

Severe 5

Very severe 6

8. During the past 4 weeks, how much did pain interfere with your normal work (including both work outside the home and housework)?

(circle one)

Not at all 1

A little bit 2

Moderately.....3

Quite a bit.....4

Extremely.....5

9. These questions are about how you feel and how things have been with you during the past 4 weeks. For each question, please give the one answer that comes closest to the way you have been feeling. How much of the time during the past 4 weeks -

(circle one number on each line)

	All of the Time	Most of the Time	A Good Bit of the Time	Some of the Time	A Little of the Time	None of the Time
a. Did you feel full of life?	1	2	3	4	5	6
b. Have you been a very nervous person?	1	2	3	4	5	6
c. Have you felt so down in the dumps that nothing could cheer you up?	1	2	3	4	5	6
d. Have you felt calm and peaceful?	1	2	3	4	5	6
e. Did you have a lot of energy?	1	2	3	4	5	6
f. Have you felt downhearted and low?	1	2	3	4	5	6
g. Did you feel worn out?	1	2	3	4	5	6
h. Have you been a happy person?	1	2	3	4	5	6
i. Did you feel tired?	1	2	3	4	5	6

10. During the past 4 weeks, how much of the time has your physical health or emotional problems interfered with your social activities (like visiting with friends, relatives, etc.)?

(circle one)

All of the time.....1

Most of the time.....2

Some of the time.....3

A little of the time.....4

None of the time.....5

11. How TRUE or FALSE is <u>each</u> of the following statements for you?

(circle one number on each line)

	Definitely True	**Mostly True**	**Don't Know**	**Mostly False**	**Definitely False**
a. I seem to get ill more easily than other people	1	2	3	4	5
b. I am as healthy as anybody I know	1	2	3	4	5
c. I expect my health to get worse	1	2	3	4	5
d. My health is excellent	1	2	3	4	5

THANKS FOR YOUR HELP!