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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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