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ORIGINAL ARTICLE OPEN ACCESS

The Haemophilia Health Journey: Experiences and Unmet Care Needs of Caregivers and Their Young Children

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ABSTRACT

Introduction: Understanding patients' and caregivers' experiences, values and needs is essential to implement patient-centred care. However, specific insights into how young children with haemophilia and their caregivers experience the entire care trajectory, from diagnosis onwards, remain limited.

Aim: Gain an in-depth understanding of the experiences and needs of caregivers of young children (0–10 years) with haemophilia regarding haemophilia care and daily life, as well as children's own perspectives.

Methods: A qualitative interview study was conducted applying purposive sampling to select a varied sample regarding haemophilia type, disease severity, treatment modality and the child's age. Interviews were conducted until saturation was reached, followed by a comprehensive thematic content analysis including multiple phases of coding.

Results: In total, 27 interviews were conducted (38 caregivers, 15 children). Twenty-five subthemes emerged related to four care pathway phases, and daily life. Caregivers emphasized that haemophilia knowledge among healthcare providers outside treatment centres, information provision, access to peer support and remote healthcare can be improved. Haemophilia had psychological, social and occupational impacts on caregivers. Impact on daily life was variable and primarily influenced by bleeding phenotype, and family history. Lastly, caregivers highlighted the importance of child-centred care, maintaining haemophilia awareness and the benefits of prophylaxis and self-administration.

Conclusion: To further enhance patient-centred care, caregivers expressed the need for a tailored and centrally available information resource, expansion of remote healthcare services and increased haemophilia knowledge. Furthermore, healthcare providers should be more aware of the multifaceted impact of haemophilia on caregivers and the factors influencing haemophilia impact on daily life.

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1 | Introduction

In the Netherlands, care for persons with rare bleeding disorders is centralized and provided by six treatment centres [1]. This centralization not only improves patient outcomes, such as bleeding rates and joint health [2, 3], but also promotes patient-centred care [4]. Patient-centred care is defined as health care that is given within a partnership between healthcare professionals (HCPs), patients, and their families to ensure decisions align with patients' needs, values and preferences [5, 6]. Within this framework, patients should be supported to make informed decisions and actively participate in their own care. Previous research has shown that patient-centred care benefits quality of care, by improving self-management skills, treatment adherence and care satisfaction, and also health-related quality of life (HRQoL) [7, 8].

Advances in treatment, such as prophylaxis with factor concentrates and the arrival of subcutaneously administered bispecific antibodies and non-factor therapies, have substantially improved disease management and HRQoL [9, 10], particularly in haemophilia A. However, haemophilia still influences patients' daily life activities. Depending on disease severity and treatment intensity, children still experience limitations regarding participation in physical and social activities, recurrent bleeding, pain, and psychological stress [11–13]. Moreover, haemophilia and its management are known to have a psychosocial impact on caregivers and family life [12, 14, 15], as well as on reproductive decisions [16, 17].

To implement patient-centred care, it is essential that HCPs have a comprehensive understanding of patients' and caregivers' experiences, values and needs throughout the care trajectory. The influence of haemophilia on HRQoL and caregivers' burden has been well characterized [12, 14, 18, 19]. However, detailed knowledge on how caregivers of young children experience the broader haemophilia care trajectory, as well as how children themselves perceive their care and daily lives, remains limited. Previous studies have explored experiences with haemophilia care, but have often focused on specific aspects of care, such as treatment experiences, included predominantly adults with haemophilia and not caregivers, or examined healthcare delivery in specific settings, for example, care in non-metropolitan areas [20–23]. Studying experiences of caregivers and young children is particularly important given that haemophilia is typically diagnosed in the first years of life [24]. Building upon existing knowledge, this qualitative interview study aims to gain an in-depth understanding of the experiences and needs of caregivers of young children (0–10 years) with haemophilia regarding haemophilia care and daily life, as well as children's own perspectives, and identify areas to further improve haemophilia care.

2 | Materials and Methods

2.1 | Study Design

A qualitative interview study was performed, as this study design is particularly suitable to gain an in-depth understanding of patients' experiences and needs [25, 26]. The Medical Ethical Research Committee of the Erasmus MC, Rotterdam, the Netherlands (MEC-2022-0081) reviewed the study and determined that it was not subject to the Medical Research Involving Human Subjects Act (WMO). Informed consent was obtained for study participation and data use for research purposes. The reporting of this study followed the consolidated criteria for reporting qualitative research (COREQ) [27].

2.2 | Participant Selection and Recruitment

Young children with haemophilia (0–10 years) and their caregivers were eligible to participate. Interviews primarily focused on caregivers' experiences due to the age of the children. If considered old enough by caregivers, children were invited to participate during parts of the interview to express their own experiences and needs. In case both caregivers (and the child) were interested to participate, a joint interview was performed. All children were treated in the Erasmus MC Sophia Children's Hospital in Rotterdam or the Amsterdam UMC Emma Children's Hospital in Amsterdam, the Netherlands. Both are accredited treatment centres and recognized European centres of expertise for rare bleeding disorders.

Eligible study participants were approached for participation by their treating physician. Purposive sampling was used to select a varied sample regarding haemophilia type (A or B), disease severity (severe, moderate, mild), treatment modality (prophylaxis, on demand) and the child's age [28]. Interested study participants were then contacted by a researcher for additional study information. Initially, 22 interviews were conducted. After conducting five additional interviews, saturation was reached—indicating that no new insights emerged—and sampling of participants ended [29]. Participation was voluntary and no incentives were offered.

2.3 | Data Collection

Prior to semi-structured interviews, caregivers and children received a sensitizing workbook consisting of several questions regarding their care trajectory and experiences with current haemophilia care. These questions were intended to support interview preparation, elicit preliminary reflections on experiences and needs, and support deeper engagement during the interview [30]. The workbooks were sent to caregivers' home addresses a few weeks before the scheduled interview, with a request to complete and return them before the interview. Completion was, however, not required, and children only completed the questions when considered old enough by caregivers.

Initial interviews were conducted between September first 2022 and November 30th 2022 either online using Microsoft Teams or in person at the participants home or hospital, depending on participant preference. Additional interviews were conducted between November first 2024 and December 31st 2024 to better reflect experiences and needs of children with severe haemophilia and their caregivers and to reach saturation. Interviews were conducted by one researcher (C.M.A.M., female medical doctor or E. Kerimoğlu, female master student in industrial design engineering) or two researchers from a group of eight male

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and female master students in industrial design engineering respectively, under supervision of C.M.A.M. or E.S.v.H. (female researcher). None of the researchers were directly involved in haemophilia care.

A topic guide, developed based on research team experiences and literature [20, 22, 23], was used to structure the interview, focusing on experiences with the provided care, diagnostic process, treatment, and influence of haemophilia on daily life (Supplement 1). Topic guides varied slightly between patient groups, as for example in severe haemophilia prophylaxis and treatment was discussed more extensively, whereas in mild haemophilia daily life without prophylaxis was more of a topic. Interviews were audio-recorded and lasted approximately 60 min.

2.4 | Data Analysis

Audio recordings and returned workbooks were transcribed verbatim and anonymized. A comprehensive thematic content analysis was performed using NVivo version R15 (QSR International) incorporating principles from the grounded theory [31–33], including multiple phases of coding (i.e. open, axial and selective), sampling until saturation, and constant comparison [26]. Transcripts were first reread for familiarization after which eight interviews were openly coded by one researcher (C.M.A.M.), and reviewed and supplemented by a second researcher (E.K. Kempers, female researcher), resulting in an unstructured list of open codes. In the axial coding phase, relationships between codes were identified and (sub)categories were created by one researcher (C.M.A.M.) to establish a structured list of codes, which was subsequently checked by and discussed with a second researcher (E.K. Kempers). Based on this hierarchical coding scheme, the remaining nineteen interviews were axially coded by one researcher (C.M.A.M.) and checked and complemented by another (E.K. Kempers), while constantly refining the coding scheme. New codes were added, and existing (sub)categories were refined, merged, or expanded. In the final phase of selective coding, main- and subthemes were identified and discussed within the research team (C.M.A.M., E.K. Kempers, M.H.C., female professor in paediatric hematology) until consensus was reached. Constant comparison was applied during the whole coding process by comparing each interpretation and finding with new data [34].

3 | Results

A total of 27 interviews were performed in which 38 caregivers (26 mothers and 12 fathers) and fifteen children participated. Characteristics of children with haemophilia and their caregivers are presented in Table 1.

Analysis resulted in 25 subthemes related to different phases within the haemophilia care pathway and daily life (Figure 1). Subthemes apply to both severe, moderate, and mild haemophilia unless stated otherwise.

3.1 | Referral and Diagnosis

Four subthemes were identified relating to the referral and diagnosis of haemophilia. Illustrative quotes are presented in Table 2.

3.1.1 | Family History Influences Perception of Haemophilia Diagnosis

Generally, diagnosis of haemophilia was experienced as less distressing in presence of a family history for the disease as caregivers were aware of a possible diagnosis and already familiar with the condition, making the diagnosis less unexpected and intense. Moreover, caregivers reported that experiences from family members reassured them haemophilia can be managed well in daily life, and emphasized that the familial presence facilitates early information exchange between partners, prenatal counselling and tailored perinatal care. Contrastingly, caregivers without prior cases in the family indicated to have been overwhelmed by the diagnosis, needing time to grieve and adjust.

3.1.2 | Limited Knowledge of Haemophilia Outside Treatment Centres Leads to Distress and Delay in Diagnosis

Caregivers experienced a lack of knowledge about haemophilia among HCPs working outside specialized treatment centres, for example, general practitioners, midwives, and HCPs in regional hospitals. Haemophilia was often not considered when atypical bleeding symptoms presented, delaying diagnosis and timely care. Some caregivers were suspected of child abuse, further contributing to caregiver distress and creating a barrier to seek care. Therefore, caregivers emphasized the need for increased knowledge and awareness.

3.1.3 | Peer Support Is Valued and Should be Offered Routinely at Diagnosis

Caregivers highlighted that peer support was most valuable shortly after diagnosis as it facilitated exchange of experiences, gave long-term perspectives, and provided opportunities to ask practical questions. Caregivers communicated that this need diminished over time as they gained more experience themselves. Although not all caregivers expressed the need for peer support, some reported to have missed it, underscoring the importance of offering it as part of routine care. While some caregivers favoured peer support through active peer matching or organized (information) sessions, others preferred more informal settings, such as through social media channels.

Caregivers additionally underlined social work as an important source of support, offering a trusted outlet for expressing concerns. Some caregivers highlighted the need for greater psychological support, particularly for coping with grief and emotional adjustment after diagnosis.

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TABLE 1 | Interview characteristics children with haemophilia and their caregivers.

Interview number	Interview participants	Age[a]	Type of haemophilia[a]	Disease severity[a]	Treatment strategy[a]
1	Mother, father, child	8	Haemophilia A	Severe	Prophylaxis with subcutaneous bispecific antibody
2	Mother	1,5	Haemophilia B	Severe	Prophylaxis with factor concentrate
3	Mother, father, child	7	Haemophilia A	Severe	Prophylaxis with subcutaneous bispecific antibody
4	Mother	1,5	Haemophilia B	Severe	Prophylaxis with factor concentrate
5	Mother	4	Haemophilia A	Severe	Prophylaxis with subcutaneous bispecific antibody
6	Mother, child	11	Haemophilia B	Severe	Prophylaxis with factor concentrate
7	Mother, father, child	4	Haemophilia B	Severe	Prophylaxis with factor concentrate
8	Mother, father, child	9	Haemophilia B	Severe	Prophylaxis with factor concentrate
9	Mother, father	6 months	Haemophilia A	Severe	Prophylaxis with subcutaneous bispecific antibody
10	Mother, father, child	10	Haemophilia A	Severe	Prophylaxis with subcutaneous bispecific antibody
11	Mother, child	8	Haemophilia A	Severe	Prophylaxis with subcutaneous bispecific antibody
12	Mother, father	1,5	Haemophilia A	Severe	Prophylaxis with subcutaneous bispecific antibody
13	Mother, child	3	Haemophilia A	Mild	On-demand
14	Mother, child	7	Haemophilia A	Moderate	On-demand
15	Mother, child	3	Haemophilia A	Mild	On-demand
16	Mother, father	5	Haemophilia A	Mild	On-demand
17	Mother, father	1	Haemophilia A	Mild	On-demand
18	Mother, father	6	Haemophilia A	Mild	On-demand
19	Mother	5	Haemophilia A	Mild	On-demand
20	Mother, child	6	Haemophilia A	Moderate	On-demand
21	Mother, child	8	Haemophilia A	Mild	On-demand
22	Mother, child	8	Haemophilia A	Mild	On-demand
23	Mother, child	7	Haemophilia A	Mild	On-demand
24	Mother	1,5	Haemophilia A	Mild	On-demand
25	Father	5	Haemophilia A	Mild	On-demand
26	Mother	2	Haemophilia A	Mild	On-demand
27	Mother, father, child	3	Haemophilia A	Mild	On-demand

Note: Children with severe haemophilia received prophylaxis with intravenously administered factor concentrates or a subcutaneously administered bispecific antibody. Children with moderate and mild haemophilia received on-demand treatment with intravenously administered factor concentrates in case of bleeding or to prevent bleeding in case of head trauma or medical procedures. All children were boys with haemophilia.

[a]Age, type of haemophilia, disease severity and treatment strategy refers to the child.

3.1.4 | Information Provision Is Fragmented, Incomplete, and Insufficiently Adapted to Personal Needs

Caregivers emphasized that adequate disease knowledge supported effective haemophilia management, particularly in acute situations. They indicated that their HCPs were the most valuable information source, who incrementally provided clear, reliable, and up-to-date information. Yet, caregivers also reported to have experienced the first period after diagnosis as overwhelming, due to the amount of information and involved emotions, hampering recollection of oral information and necessitating written information which can be reread at home. In addition, caregivers underlined that information is currently difficult to find as it is fragmented across multiple offline and online resources, incomplete, often outdated and usually exclusively focused on severe haemophilia, making it less applicable to milder disease.

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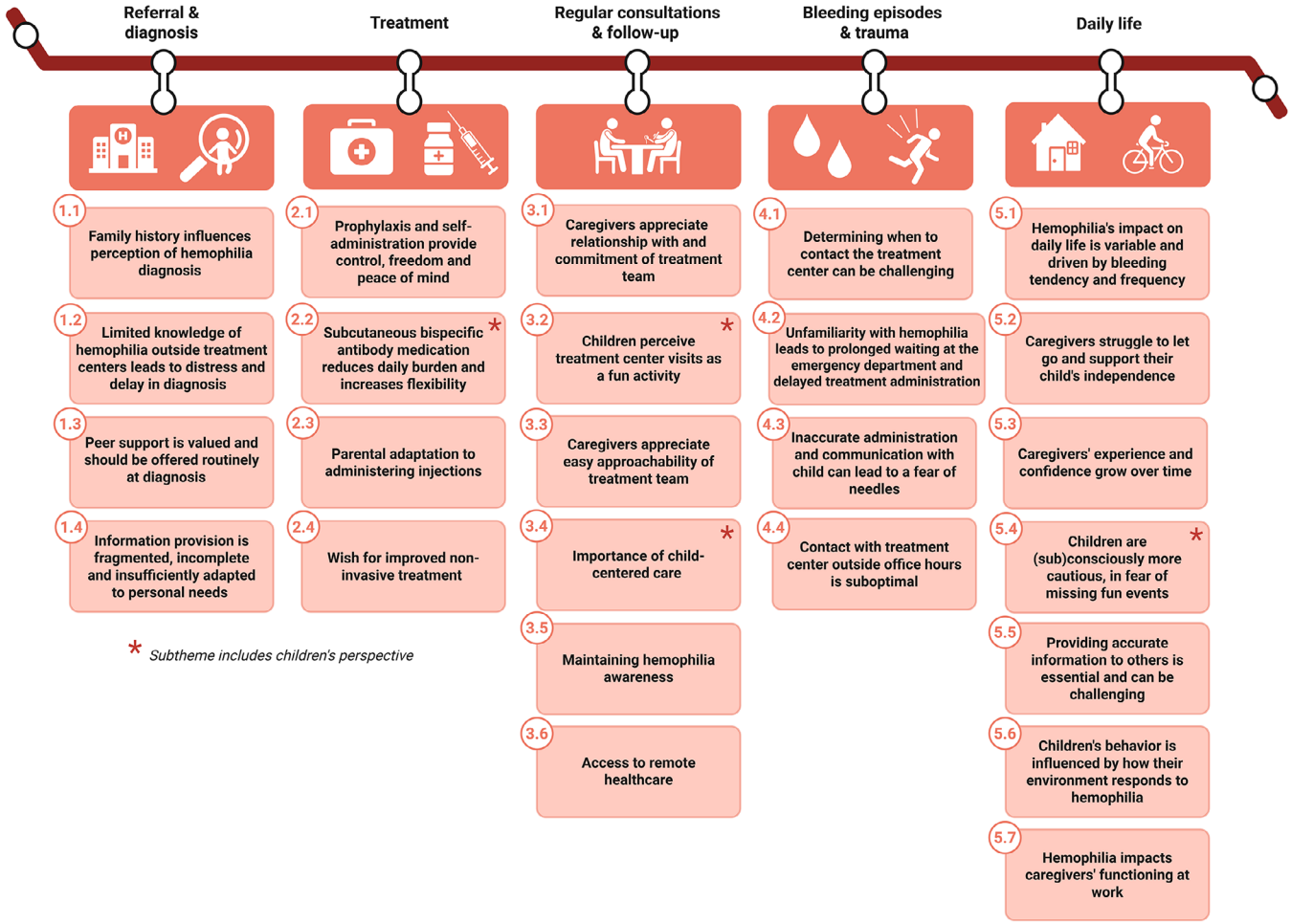


FIGURE 1 | Overview of identified subthemes. Created in BioRender. Cnossen, M. (2026) https://BioRender.com/buzhqv0.

Therefore, they expressed the need for a centralized information resource, tailored to disease severity.

3.2 | Treatment

Illustrative quotes to the four subthemes regarding treatment are presented in Table 3.

3.2.1 | Prophylaxis and Self-Administration Provide Control, Freedom, and Peace of Mind

Caregivers of children with severe haemophilia emphasized that prophylaxis, in contrast to solely on-demand treatment, provided control and reduced uncertainty by preventing bleeds and lowering bleeding risk, making haemophilia more predictable. Moreover, self-administration of factor concentrates was described as offering both independence and freedom by not needing medical support after trauma. This was reported to reduce the overall impact of haemophilia on daily life.

Contrastingly, caregivers of children with mild haemophilia expressed a desire for prophylaxis or home treatment, as the dependence on treatment centre visits in case of trauma or bleeding was perceived as particularly burdensome. They noted that home treatment would provide independence, freedom and reassurance. Moreover, guaranteed availability of sufficient on-demand factor concentrates when traveling abroad would also reduce stress and limitations.

3.2.2 | Subcutaneous Bispecific Antibody Medication Reduces Daily Burden and Increases Flexibility

Caregivers of children with severe haemophilia A highlighted that subcutaneous bispecific antibody medication reduced the impact of haemophilia on daily life and increased treatment flexibility. Compared to intravenous factor concentrates, administration was easier, less frequent and more adaptable to daily routines. Some children described injections as more painful and intimidating at first, although they usually adapted over time.

3.2.3 | Parental Adaptation to Administering Injections

Initially, caregivers experienced administering injections as stressful and emotionally difficult, especially when their child was distressed, reinforcing parental feelings of causing pain.

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TABLE 2 | Illustrative quotes to referral and diagnosis.

Subthemes	Quotes
3.1.1 Family history influences perception of haemophilia diagnosis	*My father-in-law had hemophilia. So of course we already knew that when she became pregnant there was a chance the child could have hemophilia. […] I think that because of what we had seen with his grandfather, we were actually quite relaxed about the condition… We had seen for ourselves that you can live with it perfectly well. So in that respect I don't think we felt much stress about it.* — Hemophilia A, mild (CG18)
	When he was born we didn't know that our son had hemophilia. We only found out when he was seven months. That was really… besides the enormous grief we felt, I think we cried for a month. But after that hemophilia dominated our lives, in the sense that it was a big part of our daily schedule: the care, the fear, or not really afraid, but deeply worried and constantly thinking of it. — Hemophilia A, severe (CG5)
3.1.2 Limited knowledge of hemophilia outside treatment centers leads to distress and delay in diagnosis	*That doctor [medical specialist in regional hospital], who really had no knowledge of the disease, told us that our son had hemophilia A, in a very severe form, and that he would become disabled, would not live long, and would soon end up in a wheelchair.* — Hemophilia A, severe (CG3)
3.1.3 Peer support is valued and should be offered routinely at diagnosis	*I: You mentioned that you also attended an information session with other parents?* *CG: Yes, which we really appreciated. When he was just a week old, we also called a couple […] who have two sons with hemophilia […] We were able to talk with them briefly over Facetime, which helped to take away some of those initial worry, like, how do you manage this now, with two children.* — Haemophilia A, severe (CG9)
3.1.4 Information provision is fragmented, incomplete and insufficiently adapted to personal needs	*What you generally find [on the internet] is fairly outdated, information from the 1980s, 90s […] This information can be quite shocking. […] And there isn't a single [information] source that you can really use.* — Haemophilia A, severe (CG12)

TABLE 3 | Illustrative quotes to the treatment phase.

Subthemes	Quotes
3.2.1 Prophylaxis and self-administration provide control, freedom and peace of mind	*From the moment we learned to give the injections ourselves, we experienced much more freedom. And the feeling that we were a lot less dependent on the hospital.* — Haemophilia A, severe (CG3)
	I think we are called by school about five times a week: he has a nosebleed again, he has fallen, can you come. If it is possible to see him separately for a moment, for example, at a party and give the factor concentrates ourselves, and then let him continue playing, that would feel so much more relaxed. — Haemophilia A, mild (CG16)
3.2.2 Subcutaneous bispecific antibody medication reduces daily burden and increases flexibility	*Because we were giving treatment through the port-a-cath three times a week, it really felt like, okay, we have to give the factor concentrate on this day and that day. Now it's like, oh I'll do it Wednesday, or Thursday… I just see when I'm free and then inject him. So yes, you have more freedom.* — Haemophilia A, severe (CG11)
3.2.3 Parental adaptation to administering injections	*I am always really nervous about the procedure. I always know which morning we have to give him the injection I have been dreading that day for days. It makes me really sad to have to hurt him. I find it awful to do. It breaks my heart to inject him while he is lying there so cheerfully.* —Haemophilia A, severe (CG9)
3.2.4 Wish for improved non-invasive treatment	*Well, that's an easy question [wishes regarding future care], because then I would say yes, a pill, no more injections, but just a pill to swallow with water or orange juice, and done. Yes, that would be the ideal situation.* — Haemophilia B, severe (CG8)

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TABLE 4 | Illustrative quotes to regular consultations and follow-up.

Subthemes	Quotes
3.3.1 Caregivers appreciate relationship with and commitment of treatment team	*You can feel that they are very involved at the [pediatric] hematology department, it really is a very nice group of doctors and nurses. You always feel, like, oh they know the child's name, and in that sense the contact is very personal and healthcare providers are approachable. […] I also always really appreciate it when they do a follow-up call [after a trauma].* — Haemophilia A, moderate (CG20)
3.3.2 Children perceive treatment centre visits as a fun activity	*He really loves it. Downstairs there is an aquarium with fish. […] Yes, going to the hospital is really an outing for him. […] They [healthcare providers] really make it fun, yes, I am full of praise for how they work.* — Haemophilia B, severe (CG2)
3.3.3 Caregivers appreciate easy approachability of treatment team	*The hemophilia team is always reachable, and you are always well supported. We even had an experience where a doctor called us while refueling the car, because she felt it was so important to contact us. So they are truly very accessible.* — Haemophilia A, severe (CG3)
3.3.4 Importance of child-centred care	*He [child] likes to be in control. […] For example, we agreed that he counts to three, and at three the needle goes in or comes out. That works really well. We also talk a lot about what is going to happen and why. During the very last step of the procedure, he can press the cap himself, which he enjoys. Being involved in the procedure also helps him feel more at ease.* — Haemophilia B, severe (CG8)
3.3.5 Keeping haemophilia top of mind	*Especially with a mild form, it [hemophilia] does fade into the background quite quickly. You're not always thinking of it. And when not much is occurring, you tend to downplay it a bit or think, oh, it's not so bad. But yes, of course, you still need to stay alert.* — Haemophilia A, mild (CG18)
3.3.6 Access to remote healthcare	*What I would desire, some doctors already do this here, […] is that, for example, you could video call if something happens […] like, 'Hey, take a look, do we need to come to the treatment center or not?' […] Maybe who knows, it might be resolved in that way, and that would save us an hour of travel time.* — Haemophilia A, mild (CG19)

These feelings gradually diminished as prophylaxis became routine.

3.2.4 | Wish for Improved Non-Invasive Treatment

When asked about desired healthcare improvements in an ideal world, caregivers emphasized their wish for even better therapies, ideally culminating in non-invasive treatment modalities not consisting of intravenous and subcutaneous injections.

3.3 | Regular Consultations and Follow-Up

Six subthemes focused on regular consultations and follow-up, of which illustrative quotes are presented in Table 4.

3.3.1 | Caregivers Appreciate the Relationship With and Commitment of Treatment Team

Caregivers appreciated the commitment of their warmhearted and supportive HCPs, emphasizing the importance of a familiar and consistent point of contact. HCPs were described as engaged, accessible and attentive, which promoted sharing of grief, worries and doubts.

3.3.2 | Children Perceive Treatment Centre Visits as a Fun Activity

Children often experienced treatment centre visits as an enjoyable outing. They appreciated the child-friendly environment of the waiting area and enjoyed interacting with their HCPs, who are interested in both haemophilia and their daily life activities.

3.3.3 | Caregivers Appreciate Easy Approachability of Treatment Team

Caregivers valued the treatment centre's easy approachability, noting easy contact by phone and through a digital platform for non-urgent questions, with the ability to attach photos, and prompt responses.

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3.3.4 | Importance of Child-Centred Care

Caregivers stressed the importance of involving children in treatment administration and decision-making, even at a young age. Preserving sense of control, particularly during venipunctures and prophylaxis, was reported to reduce anxiety and frustration. They also emphasized the value of honest communication and active engagement in discussions, rather than talking about the child in their presence. Involvement should be individualized, as some children preferred full participation, while others benefited from limited information or distraction. Caregivers highlighted the importance of preparing children for upcoming treatment centre visits and expressed the need for more insight into procedures during a visit, such as venipunctures. Children acknowledged this fact as important.

3.3.5 | Maintaining Haemophilia Awareness

Caregivers, especially of children with mild haemophilia, noted that regular consultations and information sessions helped to maintain awareness, as haemophilia plays a minor role in daily life when bleeding episodes are less common.

3.3.6 | Access to Remote Healthcare

Because of traveling distances, caregivers expressed their desire for more remote healthcare options, including digital consultations for routine follow-up in the absence of care demand, home delivery of medication, and the possibility to administer on demand treatment at regional hospitals closer to home.

3.4 | Bleeding Episodes and Trauma

Four subthemes were identified focusing on bleeding episodes and trauma. Illustrative quotes are presented in Table 5.

3.4.1 | Determining When to Contact the Treatment Center Can be Challenging

Caregivers reported insecurity in deciding when to contact the treatment centre and assessing the severity of trauma or bleeding. These difficulties were most pronounced shortly after diagnosis and lessened with growing haemophilia experience. Some caregivers living at greater distances from the treatment centre noted that travel constraints sometimes influenced their decision-making about contacting HCPs.

3.4.2 | Unfamiliarity With Haemophilia Leads to Prolonged Waiting at the Emergency Department and Delayed Treatment Administration

Caregivers underlined that limited haemophilia knowledge among HCPs working at the emergency department (ED) or regional hospitals sometimes resulted in prolonged waiting times and delayed treatment administration. While caregivers were understanding, they indicated that assertiveness towards HCPs was often necessary to ensure timely care, especially administration of factor concentrates. They noted that their assertiveness increased as haemophilia experience grew. Greater attention for the child's needs during treatment at the ED, including anxiety and pain management, was also requested.

3.4.3 | Inaccurate Administration and Communication With Child Can Lead to a Fear of Needles

Caregivers noted that negative experiences at the ED, such as long waits, inadequate communication, and difficult venipunctures, can cause a fear of needles in their child. Such experiences subsequently complicated the administration of prophylactic treatment at home.

3.4.4 | Contact With Treatment Center outside Office Hours Is Suboptimal

Caregivers shared that contact with the treatment centre during office hours was highly satisfactory, with direct access to their own treatment team and prompt responses. However, contact outside office hours was experienced as suboptimal due to unfamiliarity of the first contact person with haemophilia and failures in referral or call-back procedures.

3.5 | Daily Life

Seven subthemes comprised daily life with haemophilia. Illustrative quotes are presented in Table 6.

3.5.1 | Haemophilia's Impact on Daily Life Is Variable and Driven by Bleeding Tendency and Frequency

Caregivers communicated that haemophilia had minimal impact during infancy, increased as children began walking due to frequent trauma and associated treatment centre visits, and decreased as motor skills improved. The extent of this impact was described as primarily driven by bleeding tendency and the frequency of trauma and bleeding episodes, rather than disease severity. Caregivers of children with severe haemophilia receiving prophylaxis generally noted that haemophilia only minimally influenced daily life activities, apart from prophylaxis administration moments and occasional bleeds.

3.5.2 | Caregivers Struggle to Let Go and Support Their Child's Independence

Although caregivers emphasized the importance of not restricting their child and allowing them to explore, some reported having difficulty relinquishing control. Granting more independence was described as stressful and required gradual adaptation.

3.5.3 | Caregivers' Experience and Confidence Grow Over Time

Caregivers indicated that their knowledge and confidence in managing haemophilia increased over time. They highlighted that responding to acute situations, recognizing a bleed and

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TABLE 5 | Illustrative quotes to bleeding episodes and trauma.

Subthemes	Quotes
3.4.1 Determining when to contact the treatment centre can be challenging	*Especially when it comes to what to do if he falls really hard? I still find that difficult. Especially with head trauma, when do we need to call [the treatment center]?* — Haemophilia B, severe (CG4)
3.4.2 Unfamiliarity with haemophilia leads to prolonged waiting at the emergency department and delayed treatment administration	*When we arrive at the emergency department and instead of being registered under [pediatric] hematology, he is registered under neurology, because, he has fallen on his head. This leads to delay in consultation with the [pediatric] hematologist, and no timely infusion of his factor concentrates. […] Sometimes we are waiting for it [factor concentrates] for hours. […] Even when we say, 'hey, this is not the usual procedure', they really do not listen. They say 'yes ma'am, but you're at the ED'. I think, yes, I know […]. Afterwards [factor infusion] we can wait an hour, that's no problem, but he just urgently needs the factor concentrates. […] He gets the label of a child with head trauma, instead of the label of a hemophilia patient who needs medication quickly.* — Haemophilia A, mild (CG16)
3.4.3 Inaccurate administration and communication with child can lead to a fear of needles	*Something that was quite bothersome was when he fell outside office hours. At the ED it took a long time to be helped. There were also a few failed venipunctures. Because of this, he became afraid to go to the hospital for a while.* — Haemophilia B, severe (CG2)
3.4.4 Contact with treatment centre outside office hours is suboptimal	*During office hours we call the hemophilia nurse directly […] Outside office hours you call the ED. The ED calls the pediatric hematologist, who then calls back to the ED, and the ED calls us back […] This very often goes wrong. There are so many lines of communication, and mainly because your first point of contact is the pediatric nurse at the ED, who of course is not specialized in hemophilia.* — Haemophilia A, mild (CG16)

assessing bleeding risk became easier and less stressful with more experience, and as a result they became more relaxed.

3.5.4 | Children Are (Sub)Consciously More Cautious, in Fear of Missing Fun Events

Children shared that missing fun moments was the most burdensome aspect of haemophilia. Some caregivers noticed that their child played more cautiously, either consciously or subconsciously, or avoided certain activities due to prior bleeds and the associated consequences (e.g. hospital visit and administration of factor concentrates).

3.5.5 | Providing Accurate Information to Others Is Essential and Can be Challenging

Caregivers emphasized the importance of accurately informing their social environment, for example, family, friends, daycare and school, both to safeguard the child and alleviate anxiety among others. However, information provision was sometimes experienced challenging, as it relied predominantly on oral communication and the formulation influenced how risks were perceived and managed. Misunderstandings often resulted in either excessive concern and unnecessary restrictions, or underestimation of haemophilia, leading to inadequate responses in acute situations. To support sharing of information, caregivers expressed their need for accessible and comprehensible information materials tailored to their social environment.

3.5.6 | Children's Behaviour Is Influenced by How Their Environment Responds to Haemophilia

Caregivers stressed that the attitude and behaviour of their social environment towards haemophilia can influence the child's behaviour. Children were described as sensitive to stress and concern expressed by caregivers or others, whereas a calm and relaxed attitude promoted confidence.

3.5.7 | Haemophilia Impacts Caregivers' Functioning at Work

Caregivers indicated that haemophilia affected their performance at work, with some having to frequently leave or even stop work due to frequent hospital visits following trauma. Some described they constantly felt the need to be available for potential accidents necessitating treatment centre visits, and emphasized the benefit of having a flexible job to accommodate this.

4 | Discussion

This qualitative study focused on gaining an in-depth understanding of the experiences and needs of young children with

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TABLE 6 | Illustrative quotes regarding daily life.

Subthemes	Quotes
3.5.1 Haemophilia's impact on daily life is dynamic and driven by bleeding tendency and frequency	*In the beginning we actually did not have much to do with it. He was a baby, well, they just lie, sleep and poop. […] When he started standing, crawling and climbing, there was a period when we had to go to the hospital very often, and we thought, wow, what is this going to be? […] In time, this became better, because of course he developed motorically, and he was able to support himself better and fell less often.* — Haemophilia A, mild (CG18)
3.5.2 Caregivers struggle to let go and support their child's independence	*You have to let him do as much as possible and not put him in a golden cage. That's something I'm very aware of, because I find many things stressful… This week we had a birthday, and they asked if he could join the group to visit the playground. I said yes, he can and I didn't join them during the activity. […] Of course I would have preferred to join, but I also know he won't appreciate having his mom tagging along everywhere for the rest of his life.* — Haemophilia A, mild (CG13)
3.5.3 Caregivers' experience and confidence grows over time	*We have gained so much more experience in assessing situations when something happens. […] We are able to decide much more easily whether we should go to the hospital or not. Of course in the beginning that was very difficult, because you actually had no idea.*—Haemophilia A, mild (CG18)
3.5.4 Children are (sub)consciously more cautious, in fear of missing fun events	*What's really annoying is, for example, if you have a bleed just right when you are about to go on a school trip or something. When you are going to do something fun with the class, or you have a party or something, that's really… I think that's the biggest drawback.* — Haemophilia B, severe (child CG6)
3.5.5 Providing accurate information to others is essential and can be challenging	*At daycare I really noticed a tendency to sort of put him in a safe corner. Not too much activity… because they find it [the risk of bleeding] very stressful. The responsibility, of course, is so big. — Haemophilia A, mild (CG24)*
3.5.6 Children's behaviour is influenced by how their environment responds to haemophilia	*Then we heard from them that during the first two days of his stay they felt he really should have had a flashing light on top of his head. They really wanted to keep a close eye on him. […] We also heard that a lot from others. […] You notice now that [name child] is more afraid to try something new or to jump off a jungle gym or do something, compared with his sister. And that is mainly due to the environment.* — Haemophilia A, severe (CG3)
3.5.7 Haemophilia impacts caregivers' functioning at work	*I stopped working. […] I have only been back at work for the past six months. I really felt like doing something for myself again […] and not just sitting around waiting for him to fall again. Some weeks were really terrible, because we had to go to the treatment center so often.* — Haemophilia A, moderate (CG14)

haemophilia and their caregivers regarding haemophilia care and daily life. Although both caregivers and children are generally satisfied with the provided care, they still encounter different challenges throughout their care trajectory.

Caregivers highlighted that confidence in managing haemophilia grows with time and experience, particularly by the handling of acute situations and bleeding episodes. However, new treatments are significantly reducing bleeding [35, 36], which may hamper caregivers to gain these experiences. Additionally, minimal bleeding also diminishes haemophilia's prominence in daily life [14, 37], potentially lowering caregivers' awareness of disease risks. This underscores the importance of maintaining haemophilia awareness, for example, through regular consultations and organized information sessions.

Moreover, haemophilia not only affects children, but also has considerable psychological, social, and occupational impacts on caregivers. This is not surprising, as haemophilia is typically diagnosed prenatally or in early childhood, with care burden resting primarily on caregivers [24]. Caregivers experienced administering injections as stressful and reinforcing feelings of causing pain, as also has been reported by Berrueco et al. [38]. Also, haemophilia affected their ability to and performance at work, which aligns with previous research [19, 23, 39, 40], as well as a need for employment and financial support [40]. This

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underscores the necessity for HCPs to systematically address caregivers' needs by offering peer support and involve social work as part of standard care [40]. Such support is particularly important for caregivers without a prior family history of haemophilia, for whom the diagnosis may be more unexpected and emotionally disruptive [21, 41]. In contrast, those with affected relatives may benefit from pre-existing knowledge and practical guidance from their family [21, 41, 42], reducing this need for peer support.

The impact of haemophilia on daily life appeared to depend primarily on bleeding tendency and the frequency of trauma and bleeding episodes, rather than the traditional classification of disease severity. This traditional classification has longer been debated, as it does not always accurately correlate with bleeding phenotype [43–45]. Classification based on clinical phenotype has been suggested [43]. Accordingly, severe bleeding phenotype and on-demand treatment have been associated with higher disease burden and lower HRQoL compared to milder phenotypes and prophylaxis [46]. This highlights that mild haemophilia does not necessarily translate into a lower impact on daily life.

Caregivers of children with mild haemophilia emphasized their wish to administer treatment more independently, for example, access to prophylaxis or medication at home, as dependence on treatment centre visits in acute situations was particularly perceived as burdensome, and travelling distances influenced decisions to contact HCPs. This aligns with previous research showing that prophylaxis, especially when administered at home, is less disruptive, offers freedom and control, and improves caregivers' HRQoL [22, 39, 47–49]. Specifically, Berrueco et al. similarly described a feeling of better control and security in managing haemophilia after initiation of home treatment [38]. However, prophylaxis is generally restricted to people with severe haemophilia or people with moderate haemophilia and a severe bleeding phenotype due to costs, treatment burden, and consensus of greatest benefits in those with very low factor levels [50, 51]. Moreover, in the Netherlands reimbursement of prophylaxis with bispecific antibodies is restricted to these groups, complicating the realization of this wish within current clinical practice [52]. Facilitating more flexible access to home medication or availability of factor concentrates at regional shared care hospitals, may reduce caregiver burden and better tailor care, particularly in children with frequent trauma or bleeding.

Several study limitations need to be considered. First, additional interviews were performed two years after the initial interviews due to staffing constraints. As no structural changes in care delivery occurred, this delay is however unlikely to have affected our findings. Second, several researchers performed the interviews, which may have introduced variation, though a standardized topic guide was used and data saturation was reached. Therefore, we do not expect interviewer-related variation to have influenced the results. We only included boys with haemophilia, therefore, our findings may not be directly generalizable to haemophilia carriers or girls with haemophilia, as we did not examine girl-specific experiences and needs. Nevertheless, differences are not most prominent before the age of 10 years due to absence of girl-specific bleeding.

This study also has notable strengths. We included a varied sample of caregivers to obtain maximum variation in experiences, with a relatively high number of fathers, who are often underrepresented in paediatric research [28, 53, 54]. Most interviews were performed at participants' homes, ensuring a confidential and comfortable interview setting [26]. Our thorough qualitative methodology, with multiple phases of coding and achieved saturation, strengthens the robustness and validity of our results [26, 29, 31–33]. Lastly, sampling was performed in two large specialized treatment centres. Given that haemophilia care in the Netherlands is organized similarly in all treatment centres according to national guidelines [55], we believe our findings are representative for other Dutch centres. Furthermore, in light of the similarities observed with the experiences reported in a Spanish qualitative study, our findings may also be generalizable to healthcare settings abroad with similar centralization of haemophilia care, geographical distribution of treatment centres and availability of (subcutaneous) prophylactic treatment.

5 | Conclusion

In conclusion, our qualitative interview study provided an in-depth understanding of the experiences and needs of young children with haemophilia and their caregivers, and identified several areas for improvement to further enhance patient-centred care. Increasing knowledge of haemophilia among HCPs working outside specialized treatment centres, for example, through medical education for general practitioners, paediatricians and nurses, could reduce healthcare delays, particularly in emergency settings. A tailored and centrally available information resource could support caregivers and their social environment. Furthermore, caregivers emphasized the importance of child-centred care to preserve a sense of control and alleviate anxiety. Haemophilia care can be further improved by expanding remote healthcare services with care delivery closer to patients' homes, facilitating more flexible access to medication at shared care hospitals or at home for milder haemophilia, and routinely offering peer support and social work. Concomitantly, HCPs should be aware of the multifaceted impact of haemophilia on caregivers themselves as well as the influence of a prior family history, developmental phase of the child and frequency of trauma and bleeding episodes on the impact of haemophilia on daily life. These insights are valuable to incorporate in follow-up schedules, currently often mainly based on classification of haemophilia severity according to residual factor values.

Author Contributions

Caroline M.A. Mussert: data collection, data analysis, data interpretation, original draft, review process, writing. **Eva K. Kempers**: data analysis, data interpretation, original draft, review process, writing. **Evelien S. van Hoorn**: study design, data collection, review and editing. **Ekin Kerimoğlu**: data collection, review and editing. **Marijke Melles**: review and editing. **Mathieu A. Gielen**: study design, review and editing. **Armağan Albayrak**: study design, review and editing. **Kees T.B. Ahaus**: review and editing. **Simone H. Reitsma**: review and editing. **Samantha C. Gouw**: review and editing. **Marjon H. Cnossen**: study design, data interpretation, review and editing. This manuscript was approved for submission by all authors.

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Ethics Statement

This study was conducted in accordance with the ethical principles outlined in the Declaration of Helsinki and national guidelines. This study was reviewed and determined not to fall under the Medical Research Involving Human Subjects Act (WMO) by the Medical Ethical Research Committee of the Erasmus Medical Centre, Rotterdam, the Netherlands (MEC-2022-0081). All participants signed informed consent to participate in the study and to use their data for research purposes.

Conflicts of Interest

Samantha C. Gouw received unrestricted research grant from Sobi. Marjon H. Cnossen has received grants from governmental and societal research institutes for example, NWO-ZonMW, NWO-NWA, Innovation fund, and unrestricted investigator initiated research grants from private funds, as well as institutional grants, trial funding/educational and travel funding from the following companies over the years: Pfizer, Baxter/Baxalta/Shire Takeda, Bayer Schering Pharma, CSL Behring, Sobi Biogen, Novo Nordisk, Novartis, Nordic Pharma, Roche and het Sikkelcelfonds, and has served as a member on steering boards of Roche and Bayer. All grants, awards and fees go to the Erasmus MC as an institution. All other authors have no competing interest relevant to the contents of this manuscript.

Data Availability Statement

The data that support the study findings is available on request from the corresponding author. These data are not publicly available due to privacy and ethical restrictions.

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Supporting Information

Additional supporting information can be found online in the Supporting Information section.

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