

ORIGINAL ARTICLE

Clinical haemophilia

From the voices of people with haemophilia A and their caregivers: Challenges with current treatment, their impact on quality of life and desired improvements in future therapies

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Abstract

Introduction: Haemophilia A is a chronic disease requiring frequent intravenous infusions of recombinant factor VIII. Previous studies have shown that challenges associated with current treatments may have significant impacts on quality of life (QoL) that are as important as the health outcomes conferred by the therapy. Emerging therapeutic innovations offer the potential to mitigate treatment-related challenges, and it is therefore important to develop a better understanding of patient and caregiver experiences with existing haemophilia A treatments in order to characterize the full value of new treatments.

Aim: To gather firsthand perspectives from people with haemophilia A (PWHA) and caregivers on the challenges with current treatment, their impact on QoL and desired improvements in future therapies.

Methods: Qualitative insights were gathered from 20 non-inhibitor PWHA or caregivers of PWHA across Canada through one-on-one interviews; insights were further explored through focus group sessions to uncover overarching themes and prioritize issues with current treatments.

Results: PWHA and caregivers identified several challenges, including administration of intravenous infusions, coordination of treatment schedules and ensuring adequate medication and supplies. Participants described how these challenges impact psychosocial well-being, physical health, personal/social life and work. Alternate modes of administration and longer-lasting treatment effects were identified as desired improvements over current treatments.

Conclusion: This study emphasizes the impact that existing haemophilia A treatments have on psychological well-being, employment opportunities and adherence to treatment regimens. These considerations may help to inform decision-making for policymakers and health systems around the true value of new therapies entering the haemophilia market.

Wiley, Khoury, Snihur and Williams contributed equally to the study.

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KEYWORDS

adherence, employment, haemophilia, psychological well-being, quality of life, treatment

1 | INTRODUCTION

With an influx of treatments recently approved or in late-stage clinical development, the haemophilia A landscape is evolving.¹ These therapies not only have the potential to improve health outcomes (eg reduced bleeds), but also offer improvements in mode of administration (eg subcutaneous injection, oral) and required frequency of administration (eg once a week or month). The emergence of therapies with modified value propositions might provide people with haemophilia A (PWHA) with additional treatment options that could influence how they experience the treatment itself.

Indeed, given that haemophilia A is a chronic disease that currently requires frequent intravenous infusions, experiences with current treatments may be as important as the health outcomes conferred by individual therapies. Studies have shown that PWHA and caregivers experience challenges with current treatments (eg financial, technical, educational)²⁻⁵ that can impact their quality of life (QoL; eg physical functioning, psychosocial health).⁶ In turn, these impacts may affect adherence to prescribed treatment, leaving PWHA at an increased risk of bleeding and joint damage.⁷ Given the broader innovations that new therapies are bringing to haemophilia treatment and their potential to address recognized challenges and impacts with current treatments, it will be important to build a deeper understanding of PWHA and caregiver experiences with existing options. This understanding will help characterize and prioritize the potential value of emerging therapies.

Previous studies exploring treatment-related challenges have primarily collected survey data^{6,8,9} or focused on sub-sets of the PWHA population.⁴ While these studies provide valuable information, survey-based approaches do not capture candid perspectives or allow for follow-up questions. By engaging in direct conversations with PWHA and caregivers, this study's objective was to complement existing literature with firsthand insights on experiences with current treatments and uncover associations between treatment-related challenges, impacts on QoL and desired improvements in future therapies.

2 | METHODS

2.1 | Approach

Our approach was modelled on a modified Delphi method to zero in on areas of consensus after successive rounds of participant engagement. The first stage involved one-on-one interviews using structured questionnaires to uncover challenges with current treatments, the impact of challenges on QoL and desired improvements in new therapies. The second stage re-engaged participants in focus

groups to present, validate and further characterize and prioritize findings from interviews. All participants were compensated for their time.

2.2 | Setting

Sixty-minute teleconference interviews were conducted by two researchers—one leading the interview and the other taking notes. Audio recordings were taken to revisit key points for further clarification; participant consent was received prior to initiating the interview. Two-hour teleconference focus groups with the initial cohort (divided into two groups to maintain a manageable number) were conducted after the completion of the one-on-one interviews. Two researchers were involved and audio recordings were taken (with consent).

2.3 | Sampling method

We worked closely with national and regional representatives from the Canadian Hemophilia Society (CHS) and healthcare professionals from institutions providing haemophilia care to gather input on ideal characteristics for our cohort of participants (Table 1). Specifically, we sought to speak with both PWHA directly (>18 years of age) as well as caregivers of PWHA (to gather insights from the caregiver perspective and on behalf of PWHA <18 years of age). The target population was non-inhibitor PWHA, specifically individuals with more severe clinical manifestations of the disease and, subsequently, a greater need for treatment. Other characteristics were identified to reflect the diversity of the Canadian PWHA population, including the following: age, provincial distribution and urban/rural setting (based on the distance to the nearest haemophilia treatment centre [HTC]). Based on these ideal characteristics, regional CHS representatives engaged with prospective participants and received consent from 28 individuals to be included in the selection process for the study. A final cohort of 20 PWHA and caregivers of PWHA was selected to align with our ideal breakdown (Table 1); these individuals were engaged to re-confirm participation in the study and initiate interviews.

2.4 | Data collection and analysis

Interview guides were developed for both PWHA and caregivers. Questions focused on confirming demographic information (age, factor VIII levels, disease severity, joint damage, distance to HTC), understanding challenges and impacts related to current treatment and identifying future desired treatment improvements. Consensus findings were presented during focus groups in a semi-structured, facilitated manner; researchers asked open-ended questions to

TABLE 1 Ideal and actual cohort characteristics

Characteristics	Ideal cohort (Prestudy targets)	Actual cohort
Disease type	100% non-inhibitor PWHA	100% non-inhibitor PWHA
Ratio	Approximately even distribution of PWHA and caregivers	60% PWHA 40% Caregivers ^a
Disease severity ^b	75% Severe 25% Mild/moderate	75% Severe 10% Moderate 15% Mild
Age	Broad distribution—children, pre-teens/teens (through caregivers), young adults, adults and seniors	20% Children (0-9 y) 30% Pre-teens/teens (10-19 y) 15% Young adults (20-24 y) 20% Adults (25-59 y) 15% Seniors (60+ y)
Sex	Primarily males (given rarity in females) A caregiver could be male or female	100% Males 75% of Caregivers were female
Provincial distribution	Representation aligned with distribution of PWHA across Canada—40% Ontario 20% Québec 10% British Columbia 10% Alberta 10% Manitoba/Saskatchewan 10% Atlantic Canada	45% Ontario 15% Québec 10% British Columbia 10% Alberta 20% Manitoba/Saskatchewan 0% Atlantic Canada
Rural/Urban Setting	Majority urban with some rural representation (>100 km from the nearest haemophilia treatment centre)	85% Urban 15% Rural

^aCaregivers were interviewed to gather their own firsthand perspectives and reflect insights on behalf of their children (ie <18 y of age).

^bDisease Severity: Mild (5%-40% factor VIII activity); Moderate (1%-5% factor VIII activity); Severe (<1% factor VIII activity).

gauge initial reactions to findings, dive deeper into responses, clarify perspectives and further characterize insights. Notes and audio recordings were reviewed to identify overarching themes and elucidate both consensus and key issues. This process involved both independent analysis by the researchers and team discussions.

3 | RESULTS

3.1 | Participants

Of the 20 participants selected for this study, 12 were PWHA and 8 were caregivers (either the mother or father of children or pre-teens/teens who are <18 years of age). Initial one-on-one consultations with PWHA or caregivers confirmed that the individuals with haemophilia who were receiving treatment did not have inhibitors at the time of the study. The majority of PWHA (16 in total; including all severe and one moderate PWHA) were receiving

prophylactic treatment; the remainder were receiving on-demand treatment. Our cohort ranged in age from 2.5 to 84 years, and the distribution across disease severity and province was representative of the distribution of PWHA across Canada (see Table 1 for a comparison of ideal and actual cohort characteristics). All 20 participants (PWHA and caregivers) were involved in the one-on-one interviews. Sixteen individuals participated in focus groups, while four were unable to attend due to scheduling conflicts. The demographic characteristics for each participant (ie the PWHA—whether engaged directly or indirectly through caregiver discussions) can be found in Table 2.

3.2 | Challenges with current treatment

Key findings across challenges, impacts on QoL and desired improvements for future therapies are summarized in Table 3. The majority of PWHA and caregivers acknowledged that challenges associated

TABLE 2 Demographic characteristics for each of the 20 study participants

Participant type	Disease severity ^a	Age category ^b (Actual age)	Province	Treatment regimen	Setting ^c
Person with haemophilia A	Mild	Pre-teen/teen (19)	Québec	On-demand	Urban
	Mild	Senior (64)	Ontario	On-demand	Urban
	Mild	Senior (84)	British Columbia	On-demand	Urban
	Moderate	Young adult (24)	Manitoba	On-demand	Urban
	Severe	Pre-teen/teen (19)	Québec	Prophylactic (3/wk)	Urban
	Severe	Young adult (20)	Ontario	Prophylactic (4/wk)	Rural
	Severe	Young adult (21)	Québec	Prophylactic (7/wk)	Urban
	Severe	Adult (26)	Alberta	Prophylactic (4/wk)	Urban
	Severe	Adult (33)	Ontario	Prophylactic (3/wk)	Urban
	Severe	Adult (29)	Manitoba	Prophylactic (2/wk)	Urban
	Severe	Adult (38)	Manitoba	On-demand	Urban
	Severe	Senior (60)	Ontario	Prophylactic (4/wk)	Urban
Caregiver	Moderate	Pre-teen/teen (11)	Manitoba	Prophylactic (2/wk)	Urban
	Severe	Child (2.5)	Ontario	Prophylactic (3/wk)	Urban
	Severe	Child (5)	Ontario	Prophylactic (2/wk)	Rural
	Severe	Child (6)	Ontario	Prophylactic (2/wk)	Urban
	Severe	Child (8)	British Columbia	Prophylactic (4/wk)	Urban
	Severe	Teen (10)	Alberta	Prophylactic (3/wk)	Rural
	Severe	Teen (11)	Ontario	Prophylactic (2/wk)	Urban
	Severe	Teen (14)	Ontario	Prophylactic (2/wk)	Urban

^aDisease Severity: Mild (5%-40% factor VIII activity), Moderate (1%-5% factor VIII activity), Severe (<1% factor VIII activity).

^bAge Category: Child (0-9 y), Pre-teen/teen (10-19 y), Young adult (20-24 y), Adult (25-59 y), Senior (60+ y).

^cRural: >100 km from the nearest haemophilia treatment centre; Urban: <100 km from the nearest haemophilia treatment centre.

with treatment administration and coordinating schedules were most problematic.

3.2.1 | Administering an intravenous infusion

Caregivers identified the time commitment required for treatment as the most significant issue with current treatments. Complications associated with intravenous infusions (eg inability to find a vein or self-administer due to injury) can cause additional inconveniences by extending the administration protocol or necessitating visits to an HTC. For individuals with moderate/severe haemophilia who require frequent infusions, it can be challenging to find a viable vein due to the formation of scar tissue. Interstitial IV is another major challenge inherent to infusions. Further, most PWHA and caregivers noted the steep learning curve associated with infusions, requiring considerable time investment and multiple visits to HTCs to acquire the necessary skills.

Many caregivers mentioned that infusing a child presents unique challenges, including helping the child overcome a fear of needles, alleviating the perception that the caregiver (often a parent) is

hurting the child, and psychologically and physically preparing the child for their regular infusions. Most caregivers find it challenging to infuse children through a port, given the precision required and potential blockages. Health concerns were also raised by most caregivers around the use of a port, including an increased risk of infection, the need for adjunctive treatment and follow-on surgeries. Most caregivers also noted that the transition from injections using a port to peripheral infusions offers additional challenges such as becoming familiar with a new administration protocol.

Some PWHA and caregivers mentioned that intravenous infusions can be painful, especially when multiple attempts are required. One individual with severe haemophilia noted that he intentionally foregoes treatment if he is at a lower risk of injury (eg when staying at home) because of the pain the infusion causes.

3.2.2 | Coordinating treatment schedules

All participants noted that it is challenging early on to establish and maintain a consistent treatment schedule, leading to

TABLE 3 Summary of key findings

Key topic	Overarching themes	Specific issues	Example quote
Challenges with current treatment	Administering an intravenous infusion	<ul style="list-style-type: none"> • Considerable time commitment • Challenges with injecting a child • Complex administration process • Challenges with using a port • Painful process 	“Every time he sees me prepare [the medication] he tries to hide and run off. He is scared of it. I remember once he said mommy you are hurting me.”—Caregiver of a 6-y-old boy with severe haemophilia A from Ontario
	Coordinating treatment schedules	<ul style="list-style-type: none"> • Establishing/maintaining a routine • Scheduling treatments for children 	“Coordinating treatment schedules and remembering to organize [is a challenge]. We both work full-time, if [our child] has an in-service day or is on a field trip, we need to reconfigure treatments. Sometimes we get busy and are scrambling at the last minute.”—Caregiver of an 11-y-old boy with moderate haemophilia A from Manitoba
	Ensuring an adequate supply of medication and supplies	<ul style="list-style-type: none"> • Frequent need to replenish medication and supplies • Picking up medication 	“We are also rural, 1 hour and 20-minute drive. I usually sit and wait for an hour. Now we pick up 36 boxes, since we are using double the dose, so I have to sit there for longer because there is more stuff to put through the system.”—Caregiver of a 10-y-old boy with severe haemophilia A from Alberta
Impact of current treatment on QoL	Psychological well-being	<ul style="list-style-type: none"> • Anxiety/stress • Negative associations 	“He literally goes into mild panic attacks when I infuse, he knows there is an increased chance I will miss versus mom who can do it with her eyes closed.”—Caregiver of an 11-y-old boy with severe haemophilia A from Ontario
	Physical impact	<ul style="list-style-type: none"> • Trauma • Exhaustion 	“You can see it everywhere on my skin [where I] infuse, as a kid it was on my hand, those veins are now between blue and purple.”—21-y-old man with severe haemophilia A from Québec
	Impact on personal/ social life	<ul style="list-style-type: none"> • Personal relationships • Social stigma 	“At my age, having a girlfriend or whatever and having to explain the disease and the treatment can be difficult, that this is what I live with ... can be hard sometimes.”—20-y-old man with severe haemophilia A from Ontario
	Work-related issues	<ul style="list-style-type: none"> • Coordinating work schedules • Finding a job 	“Up until 2 years ago we were going to the clinic 3 days a week [for treatments], so finding a job was not on the cards because there was no time to work.”—Caregiver of an 8-y-old boy with severe haemophilia A from British Columbia
Desired improvements in future therapies	<ul style="list-style-type: none"> • Disease-modifying therapies • Treatment with an alternate mode of delivery • Longer-lasting treatment effect 	“Administration of the medication without going intravenously would be a huge leap forward ... it would be a huge improvement on our quality of life.”—Caregiver of a 2.5-y-old boy with severe haemophilia A from Ontario	

potential adherence issues. This is complicated for caregivers providing treatment to young children (who are more temperamental), for teens and young adults with demanding schedules (eg work, school, extracurricular) and when disruptions to regular

schedules occur (eg summer holidays). Many caregivers noted that two people must be present to provide support for a child's infusion, which becomes challenging given conflicting schedules, work responsibilities or other competing/conflicting priorities.

3.2.3 | Ensuring an adequate supply of medication and supplies

Most PWHA and caregivers mentioned the time investment and, in some cases, logistical challenges that come with medication/supply management, including anticipating the need for emergency supplies and gathering ancillary supplies for port infusions. Many PWHA and caregivers also highlighted challenges with picking up medications, such as limited periods of time and places for pick-up, transportation challenges and long distances to HTC's (especially for PWHA in rural settings).

3.3 | Impact of current treatment on QoL

While impacts across multiple dimensions of QoL were identified, the majority of PWHA and caregivers stated that treatment-related challenges have the greatest impact on their psychosocial well-being.

3.3.1 | Psychological well-being

Both PWHA and caregivers expressed a worry about whether their current treatment will result in the development of inhibitors. Additionally, most caregivers noted the anguish they feel in seeing their child experience stress (eg from a fear of needles) and pain during the infusion. Most caregivers indicated that the relationship with their child is temporarily affected during the infusion process due to the pain the child experiences, while a few caregivers highlighted negative associations their child has with hospitals and/or health-care professionals given the intravenous infusions.

3.3.2 | Physical impact

Individuals with severe haemophilia noted substantial vein damage due to frequent infusions, including permanent scar tissue/bruises. Most caregivers stated that they are often physically exhausted by attempting to infuse a child that is not sitting still or fighting back.

3.3.3 | Impact on personal/social life

A few caregivers noted that it can be difficult to find time to spend with significant others due to treatment (and disease-related) challenges or finding a partner who understands the disease and is willing to assist with treatment. Some young adults with severe haemophilia mentioned their general reluctance to infuse in public due to infusion (and disease-related) stigma. A similar social stigma has been observed by caregivers when their child is taken aside to receive an infusion (eg at school).

3.3.4 | Work-related issues

All caregivers and some PWHA mentioned the need to take time off work for treatment, and the impact on productivity and co-worker perceptions. A few caregivers noted that the need to manage their

child's treatment hinders their employment options, as they need sufficient flexibility (and understanding) to accommodate the treatment schedule. In certain cases, caregivers had to switch to a job that could be performed from home to better manage their child's treatment schedules.

3.4 | Desired improvements for future therapies

Most PWHA and caregivers recognized the impact that a disease-modifying treatment or, better yet, a cure (eg gene therapy) would have on their lives. Most PWHA and caregivers indicated that an alternate mode of delivery (eg oral, subcutaneous) would represent a significant improvement and compel them to switch therapies, provided comparable efficacy/safety. This would reduce the time for administration, minimize recognized inconveniences and mitigate physical and psychosocial impacts. A more efficacious, longer-acting effect of factor VIII (eg longer than 12-18 hours) was also cited as a desirable innovation by many. This would alleviate the mental strain of calculating factor levels and anticipating treatment needs and reduce the frequency of infusions.

4 | DISCUSSION AND CONCLUSION

This study offers firsthand perspectives from PWHA and caregivers across Canada on treatment outcomes and impacts that matter most to them, supporting findings from previous studies^{3,4,6} and offering new insights on the association across treatment challenges, impacts and desired improvements. PWHA and caregivers experience many treatment-related challenges associated with administration, coordination and supply, leading to impacts on psychosocial well-being, physical health, social well-being/relationships and employment. Indeed, PWHA and caregivers highlighted desired innovations in future therapies that would help to circumvent current challenges—including alternate modes of administration and longer-lasting treatment effects. Taken together, the study contributes to the body of scientific literature about the experiences of PWHA and caregivers, and can potentially inform the scientific community, health systems and policymakers in thinking about the broader definition of value for existing and future treatments.

While the burden of treatment-related challenges was clearly acknowledged across multiple dimensions, our findings indicate that the impact on the psychosocial well-being of individuals, their employment opportunities and the ability to adhere to prescribed treatment regimens are especially notable, and may have social, economic and health implications.

4.1 | Psychosocial well-being

The impact of haemophilia on psychosocial well-being is well-known. Studies have shown that one-third of PWHA have depression symptoms,^{10,11} while other psychosocial outcomes (eg self-esteem/

self-autonomy) are also negatively impacted.¹² Our study shows that the nature of haemophilia treatment can compound the psychosocial impact of the disease. Many individuals worry about the development of inhibitors with current treatments—which occurs in 33% of individuals with severe haemophilia A and requires a shift in prescribed medication.¹³ Other treatment impacts—from constant infusions to physical scars to personal/professional relationships—were also recognized as negatively affecting psychosocial well-being. Poorer mental/social well-being can pose economic challenges through reduced production and consumption opportunities, as well as increased health and social care expenditures.¹⁴ Recognizing the broad impact of treatment-related challenges on psychosocial well-being and implications on health systems and economic prosperity, the study reinforces the importance of psychosocial support to help mitigate these issues.

4.2 | Employment

The study offers support for an impact of treatment schedules on the ability to attain desired employment. Individuals often feel limited in their job choice and ability to attain professional goals due to treatment-related challenges, noting that treatment protocols impact productivity and influence co-worker perceptions. In certain cases, caregivers were compelled to switch to more flexible jobs to better manage their child's treatment. These qualitative insights—particularly for caregivers—complement quantitative findings from the HERO initiative, which reported that 29% of caregivers selected their job and 17% voluntarily left their job to care for their child.¹⁵ Job-related challenges also have substantial economic implications; indirect costs (eg lost wages, part-time work) to manage prophylactic treatment in adults/children with severe haemophilia in the United States amount to \$8867 annually per individual.² In addition to reducing household income, this impact on employment also affects the ability of PWHA and caregivers to contribute to the economy.

4.3 | Adherence to prescribed treatment regimens

Our study affirms that the complexity of current treatment protocols compels individuals to make tough decisions around treatment adherence. PWHA and caregivers acknowledge many challenges that lead to intermittent delays in administration or missed infusions, including difficulty finding a vein, physical impediments and unexpected changes in schedules. It is well documented that adherence to prophylaxis is essential for maintaining circulating factor levels above established targets and critical to better health outcomes (eg preventing bleeds and arthropathy).¹⁶⁻¹⁸ A longitudinal study examining adherence to early prophylactic therapy in 49 PWHA reported that 69% of patients interrupted prophylactic treatment one or more times during the study and had significantly more arthropathy.¹⁷ Other studies have shown that increasing the time between treatments is associated with a greater number of total bleeds and hemarthroses, and lowered adherence is the most important

determinant of increased bleeding.¹⁹ There is also a substantial cost to the health system associated with bleeding episodes—ranging from €6650 to €14 138 per bleed (data from Germany, Sweden, the United Kingdom and the Netherlands).²⁰ While not examined in this study, it is reasonable to conclude that lower adherence in this study's cohort could result in an increased risk of bleeds and have a negative impact on PWHA and caregiver QoL.

Several limitations must be considered in interpreting the results of this study. This study focused on non-inhibitor PWHA only and cannot be generalized to haemophilia B or inhibitor populations. Further, the study focused on gathering perspectives from PWHA and caregivers across Canada; it is possible perspectives from individuals in other countries would yield different outcomes. The cohort of PWHA in our study also included a higher proportion of individuals with severe haemophilia A (75%) compared to the Canadian average, with less representation from individuals with mild (15%) and moderate (10%) haemophilia A. Future studies may wish to more closely examine these sub-populations to contextualize the outcomes from this study.

This study offers perspectives from PWHA and caregivers on their experiences with current haemophilia treatments, impacts on QoL and desired improvements in future therapies. Current treatments were shown to negatively impact psychosocial well-being and employment, which can hinder adherence to prescribed treatment regimens. By offering improved modes of administration and reduced treatment frequency, future treatments have the potential to minimize the burden of these challenges and improve adherence. This may lead to an enhanced ability to manage bleeds, reduce joint damage and mitigate other impacts on QoL. Indeed, PWHA and caregivers expressed their hope for innovations in mode of delivery and the ability to maintain elevated factor levels and reduce administration frequency.

The outcomes of this study could serve as an important, complementary input for policymakers and health systems to better understand the true value of new therapies entering the haemophilia market, the relevance of these innovations to PWHA and caregivers, and their importance to improving clinical value. These qualitative insights could be combined with quantitative information appraised using traditional health technology assessment frameworks—including safety, efficacy and cost-effectiveness—to present a more complete picture of the potential value and impact of new haemophilia therapies on the health, social and economic well-being of both PWHA and caregivers. For example, this complete picture could be particularly valuable in the context of treatment procurement strategies.²¹ By broadening the definition of value for innovative haemophilia treatments, PWHA and caregivers could experience decreased disease burden and live fuller, more productive lives.

DISCLOSURES

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AUTHOR CONTRIBUTIONS

REW, CPK, AWKS and MW designed the study and performed the research. DP, NG, LL, CMW and JMS contributed to identifying ideal cohort characteristics and prospective participants. All authors contributed to the analysis and interpretation of findings, and writing of this manuscript.

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