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CLINICAL PRACTICE

A multi-stakeholder collaborative approach to awareness, education and support needed in the haemophilia gene therapy patient journey – a report on insights from a European patient advisory board

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Gene therapy presents a potentially transformational approach to haemophilia management. The patient journey for gene therapy is unlike that for other haemophilia treatments, and its one-time, irreversible nature has led to broad agreement that informed shared decision-making is essential. Understanding the educational and support needs of people with haemophilia (PwH) who may consider or undergo gene therapy in the future is key to enabling this. A one-day patient advisory board was conducted with the aim of gaining insights on what education and support would enable PwH to have appropriate conversations with health care practitioners (HCPs) about gene therapy and participate in informed shared decision-making. The participants were 11 age-diverse men with severe haemophilia A or B, including six who had received gene therapy in phase 1-3 clinical trials. Two were members of a national patient organisation or a patient organisation leader. Participants agreed that informed shared

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decision-making was essential for gene therapy, but most felt the choice to have gene therapy would be limited by healthcare budgets and access arrangements. They identified key education needed by PwH to enable true informed decision-making, best delivered through a collaborative approach by patient organisations and HCPs. Eligibility criteria should be more widely shared within the community as part of general education around gene therapy. Gene therapy should be presented as one of a range of treatment options; understanding its risks, uncertainties and benefits is key to managing expectations and minimising treatment remorse. Awareness of how different factor levels impact bleeding tendency and frequency would be helpful, as success of gene therapy cannot be determined by factor activity alone. Other key educational needs included follow-up commitments, the role and potential side effects of corticosteroids or other immunosuppressants, impact on sense of identity, mental health, family planning, and managing bleeds after gene therapy. Teach-back could help ensure appropriate levels of understanding; psychological support could aid managing expectations. Peer-to-peer learning delivered across a variety of platforms, to share experiences of gene therapy and contextualise more 'formal' gene therapy education, was considered invaluable.

Keywords: *Haemophilia, Gene therapy, Patient education, Shared decision-making*

Haemophilia is an X-linked rare bleeding disorder characterised by reduced or absent clotting factor VIII (FVIII; haemophilia A) or factor IX (FIX; haemophilia B) [1], resulting in spontaneous joint and muscle bleeds and associated joint arthropathy, muscle contracture and disability [2]. For people with severe haemophilia (factor levels 1 IU/dL or less than 1%), standard of care involves prophylactic replacement of the missing factor to reduce spontaneous bleeding and consequent joint damage [1]. Factor replacement prophylaxis requires regular intravenous infusions, representing a significant treatment burden [3,4]. Recent advances in haemophilia treatment have included the development of longer-acting factors [5] and factor mimetics and rebalancing therapies that can be delivered subcutaneously [6,7], which have contributed to reducing treatment burden [8,9]. Gene therapy, which uses an adeno-associated virus to insert the relevant gene into hepatocytes to instigate production of FVIII or FIX, presents a potentially transformational approach to haemophilia management [10,11].

The patient journey for gene therapy is unlike that for other haemophilia treatments. It involves a long lead-up, once-only dosing and significant follow-up [12,13], alongside which it is irreversible. This has led to broad agreement that informed shared decision-making is essential for haemophilia gene therapy [4,14-19]. As haemophilia gene therapies have now received market approval in Europe, North America and Australia, there is a need to understand the educational and support needs of people with haemophilia (PwH) who may consider or undergo gene therapy in the future. Understanding the experiences of PwH who have had gene therapy, alongside the perspectives of PwH who are potentially eligible to have gene therapy, can help define this.

In May 2023, CSL Behring held a one-day patient advisory board meeting, involving men with severe haemophilia A or B who have had gene therapy or may be potentially eligible to have gene therapy, with the aim of gaining insights on what education and support would enable PwH to have appropriate conversations with health care practitioners (HCPs) about gene therapy and participate in informed shared decision-making on gene therapy. The meeting took the form of a guided discussion and group activities facilitated by researchers from Haemnet. Specific sections of the discussion focused on:

- Participants' perceptions and experience of shared decision-making in haemophilia treatment
- Educational needs around gene therapy
- Ensuring patient understanding of gene therapy, including the key stakeholders involved and the role of peer support.

Field notes were taken during the meeting, which was also audio-recorded and transcribed verbatim. This report summarises the views shared by the advisory board participants.

PARTICIPANT DEMOGRAPHICS

Participants included eleven age-diverse adult males (range 18-63 years) with severe haemophilia A or B recruited from Netherlands (n=4), the UK (n=4), Ireland (n=2) and Germany (n=1), some of whom had received gene therapy as part of a phase 1-3 clinical trial (n=6), and some of whom had not had gene therapy but were potentially eligible to do so (n=5). Two were members of a national patient organisation or a patient organisation leader.

Participants had differing experience in terms of their first awareness of gene therapy for haemophilia, with knowledge and information obtained from their own medical or biomedical educational/professional background, information presented during routine clinic appointments or larger group meetings arranged at their treatment centre, discussions with clinicians at youth camps, and general and/or anecdotal information about gene therapy in the public domain.

Six participants had received gene therapy for haemophilia A (n=1) or haemophilia B (n=5) as part of phase 1-3 clinical trials. All reported a positive impact on their lives, which they expressed in terms of not having to think or worry about their haemophilia, not having to have prophylactic treatment, and overall positive effects physically (e.g. accidental trauma no longer causing bleeding) and psychologically (e.g. a sense of freedom).

Five participants had not taken part in a gene therapy clinical trial but were potentially eligible to have gene therapy. They reported concerns about the time commitment involved in having gene therapy, wanting more information on gene therapy outcomes and current 'unknowns', and feeling that their current treatment was effective and not burdensome.

PERCEPTION AND EXPERIENCE OF SHARED DECISION-MAKING

Shared decision-making in haemophilia care involves a two-way flow of information between PwH and HCPs, whereby patient goals and values are balanced with consideration of risks, benefits and alternatives to reach a decision on treatment [14].

While participants felt that being informed should be key in any treatment choice, it was recognised that being informed does not always result in a fully shared decision. Participants identified that treatment choice, including the information presented to them and recommendations made by HCPs, was influenced by national tendering processes and the general availability/lack of availability of specific treatment products. In some cases, these influences effectively resulted in there being a lack of choice when considering treatment options. One participant noted:

> *"I've always been on the same product until it wasn't available anymore – and then my hospital switched me."*

Good shared decision-making with regard to factor-based and mimetic treatments was defined by participants as taking patient choice and lifestyle into consideration [14], including (but not limited to) therapeutic goals such as venous access, activity levels, and peaks and troughs.

Some participants felt that having a large range of treatment choices could make decision-making difficult. Some also reported a willingness to be guided by their clinician's recommendation based on trust:

> *"The doctors do push you in a way that they're thinking instead of giving you every option possible, but they normally give you the best option anyway."*

Although this may appear contradictory to shared decision-making, there was acknowledgement that, for some, shared decision-making could include the patient choosing to trust a clinician's recommendation. In all cases, however, participants felt that individualisation based on lifestyle and individual circumstances should be a key consideration in shared decisions about treatment, whether related to a specific product or a particular treatment paradigm.

Participants agreed that the one-time, irreversible nature of haemophilia gene therapy made informed shared decision-making particularly important, and it was suggested that shared decision-making had been far less a consideration in treatment choice before gene therapy. One participant stated:

> *"Shared decision-making is a very good thing to do for any haemophilia treatment but for gene therapy has to be mandatory."*

Differences in choice of treatment paradigm versus choice of specific treatment products were noted. One participant stated that decision-making in gene therapy could be seen as a binary choice in that, post-screening eligibility testing, it ultimately became a decision as to whether to have it or not. By contrast, shared decisions around switching to a different treatment product involved considerations around tailored dosage, frequency of administration and impact on lifestyle and activities, as well as including the option to switch back to a previous treatment.

While patient choice was seen as being very much part of whether or not to participate in a gene therapy clinical trial, there was general agreement that this would be limited when it came to licensed gene therapy products. Participants felt this would be influenced by healthcare budgets and funding arrangements, and that cost would ultimately dictate whether or not a

choice exists between prophylaxis with a longer acting treatment product, for example, and gene therapy.

Participants who had had gene therapy as part of a clinical trial described having taken months and, in some cases, years to make their decision. In the context of gene therapy being more widely available as a treatment option, participants believed that the process of decision-making could potentially take less time, but they recognised that the decision-making and consenting process would inevitably vary between individuals and should not be rushed [20,21].

EDUCATIONAL NEEDS AROUND GENE THERAPY

Participants discussed the key information that PwH should know ahead of making an informed decision about whether or not to have haemophilia gene therapy. Core topics identified included eligibility, expectation management, the potential need for adjunctive medication post-gene therapy (specifically, corticosteroids), follow-up commitments, sense of identity, family planning, managing bleeds after gene therapy, and the importance of access to up-to-date information.

Eligibility

Participants agreed that information about eligibility criteria for gene therapy should be available in the haemophilia community as part of general education, ideally through patient organisations and prior to any conversations about gene therapy with clinicians. There was uncertainty as to the general age at which education around gene therapy should start, with concern among some participants that children may be disappointed at not being eligible until they reach adulthood. Although those under 18 year of age are not eligible for gene therapy, it was suggested that gene therapy education for individuals should begin at around age 16. In the context of gene therapy as a treatment option, participants felt it was important that parents understood the need for children to be well treated and as healthy as possible. Prophylaxis and the availability of longer acting treatments means it is now possible for children with haemophilia to reach adulthood with minimal joint damage [1,22], and they can potentially approach future treatment decisions about gene therapy without mobility issues.

There was general agreement that establishing eligibility for gene therapy (i.e., AAV antibody testing, liver health screening) at an early stage should ensure PwH do not go too far along the gene therapy pathway before being told it is not possible for them [21].

> *"If you build up their hopes, you bring them all the way down the road, and then at the end they find out they're not eligible, I think that would be much more crushing."*

Participants also highlighted the importance of presenting gene therapy as one of a range of treatment options [23]. It was suggested that 'pre-selection' of gene therapy candidates by care teams (i.e., reviewing eligibility before raising the possibility of gene therapy) could be a positive act, and that clinicians should be aware of the inhibitor status and liver health of PwH under their care to help facilitate conversations about gene therapy eligibility.

Managing expectations

Participants agreed that it was essential for PwH to understand the pros/benefits and cons/drawbacks of gene therapy; this was described as key to informed shared decision-making. The potential tendency for individuals to focus only on the positive aspects of gene therapy was noted, with one participant who had received gene therapy admitting that his focus had been on the 'green lights' (pros) and not the 'red lights' (cons) when making his decision:

> *"It's like I said, you only see the green, you just, you don't think about the red."*

One participant who was potentially eligible to have gene therapy reported being unaware of some of the negative aspects associated with it (e.g., side effects of corticosteroids, if needed) prior to the advisory board meeting. Participants identified a need for education to ensure that PwH are aware of what is not yet known about haemophilia gene therapy and the possibility of there being other 'unknown unknowns' [15,24].

Understanding what different gene therapy outcomes would or could mean for the everyday lives of PwH was identified as important. Participants noted a tendency for gene therapy outcomes information to focus on numerical factor levels. If the numbers are not high this could be interpreted by PwH as gene therapy having 'failed', when in fact it may still have an impact on bleeding tendency and frequency [25]. As the success of gene therapy cannot be determined by factor activity alone, understanding what any change in factor production means for protection against bleeding, and the overall general impact this may have on the individual, was identified as being more helpful to PwH than numerical factor levels:

"It would be nice to see what that actually looks like, as opposed to it just being a number on a piece of paper, like how does that actually change your life."

Participants also highlighted the importance of ensuring that PwH understand that it is not possible to guarantee achievement of a particular factor level following gene therapy, and that some symptoms would not be alleviated by gene therapy.

Participants felt that being able to hear and evaluate the experience of PwH who have had gene therapy should be facilitated as part of the educational process that supports informed shared decision-making. Peer-to-peer conversations of this kind were seen as valuable in ensuring that individuals considering gene therapy are prepared to accept the possibility of a range of different outcomes [21], and in understanding the decision-making process.

"You have to get to that point where they're willing to accept some uncertainty if they want to take gene therapy... this is all expectation management."

Adjunctive medication/corticosteroids

The potential need for adjunctive medication post-gene therapy – specifically, corticosteroids – was identified by participants as an important 'need to know', "even if it was just one chat before it all started off". Alongside understanding the potential need for corticosteroids and the rationale for this, participants considered it important that PwH understand they may experience side effects including mania and depression.

One participant who had required corticosteroids to address liver inflammation post-gene therapy identified a lack of support from clinicians involved in the clinical trial when he experienced side effects that had a negative impact on his mood and behaviour. It was also highlighted that previous experience of taking corticosteroids, particularly for different conditions, should not lead to an assumption that any side effects would be the same. In terms of the potential impact of corticosteroids on mood, it was suggested that having a 'baseline' knowledge of an individual's mental health status before having gene therapy would be helpful in terms of identifying changes/impacts that warrant supportive intervention.

"If they had a baseline, if you'd seen somebody, they could see the effect the steroids were having on you, they could actually maybe intervene a little bit."

This could also be useful in establishing whether there are longer-term improvements in mental health post-gene therapy.

Follow-up commitments

The time and commitment needed for follow-up was identified as an important point of education for PwH, particularly during the initial post-gene therapy period [15,21], including potential impacts on work. One participant who had received gene therapy reported not having been entirely prepared for the reality of an initially intense period of follow-up. Although the overall level of commitment needed for follow-up was perhaps greater than most post-gene therapy participants had anticipated, there was general agreement that it had been explained in advance but they had not been able to appreciate it fully.

"That amount of commitment is quite difficult to contemplate [...] I was definitely informed about it and told exactly what to expect, and I work for myself so I had complete freedom in that regard, but yeah, it is just, it's a lot."

Potential physical impacts related to tests during follow-up were also noted as something PwH should be made aware of. One participant reported feeling drained and developing anaemia as a result of weekly blood tests post-infusion. While acknowledging that the amount of blood taken may be less in the non-trial setting, and that his anaemia had been remedied with iron tablets, he said it would be helpful for PwH to know that this is a possibility.

Post-gene therapy participants agreed that, even with the knowledge they now had and their understanding of the inconvenience and time involved in follow-up testing and clinic appointments, they would still choose to have gene therapy.

"I had no idea how big an inconvenience it actually was, but in hindsight I would have done... I still would have done it and I would have done it even if it was, went on for longer."

Sense of identity

Participants raised the need for PwH to understand potential psychological impacts of having gene therapy in terms of their sense of identity.

"Certainly some people feel like haemophilia is part of who I am, it's part of my identity, I don't want to lose that."

Loss of identity as a person with haemophilia was likened to addiction by one participant, in the sense of "someone who's addicted to his disease". One participant also expressed surprise that PwH may miss experiencing bleeds: *"If you're no longer getting bleeds, how can you miss that?"*

Participants thought it important for PwH who have gene therapy to understand that haemophilia would still remain part of their identity, in that they would still carry the genetic mutation and this could still be passed on to future generations. They also pointed to the need for psychological support for PwH who have gene therapy in terms of preparing for transition to a 'haemophilia-free mind' [26], suggesting a focus on the positive impacts such as change in bleeding phenotype/potential normalisation of factor levels, rather than a change to individual 'status' within the haemophilia community. Encouraging people who have gene therapy to remain part of the haemophilia community was seen as valuable.

Family planning

Some participants raised questions around treatment timing in respect of family planning and sexual activity post-gene therapy, indicating a clear educational need. One participant asked whether any PwH who had undergone gene therapy had gone on to have children since.

Bleeds after gene therapy

Participants discussed the need for PwH to have access to information and reassurance on what to do in the event of a suspected bleed post-gene therapy. One participant shared his belief that he had experienced a joint bleed after having gene therapy but that there was uncertainty around this:

> *"I feel like I had a bleed and the doctors were all sort of saying 'no, you can't have one' because my levels are too high."*

In addition to his care team's belief that his raised factor levels made a bleed unlikely, he explained that no longer having recourse to the pain>infuse>resolution process of his previous factor product meant he also could not be sure as it was a different experience. He described feeling that he would only be certain that he had had a bleed if he infused a factor product.

> *"I'm not convinced. See if I was on prophylaxis, if I had factor for three days that would have sorted that out."*

Access to updated information

Participants agreed on the importance of access to reliable updated information about all aspects of haemophilia gene therapy as knowledge increases.

> *"You want the information to be reliable, and you want it to be pointed out that there has been a change in what you knew."*

PATIENT UNDERSTANDING OF GENE THERAPY

Alongside broad information, the need for an individualised approach to gene therapy education is recognised, focused on the particular needs of each person [12,14-16,18]. Participants discussed the stakeholders who should be involved in education around gene therapy and ensuring individuals considering gene therapy understand enough about it to make an informed shared decision.

Stakeholder collaboration

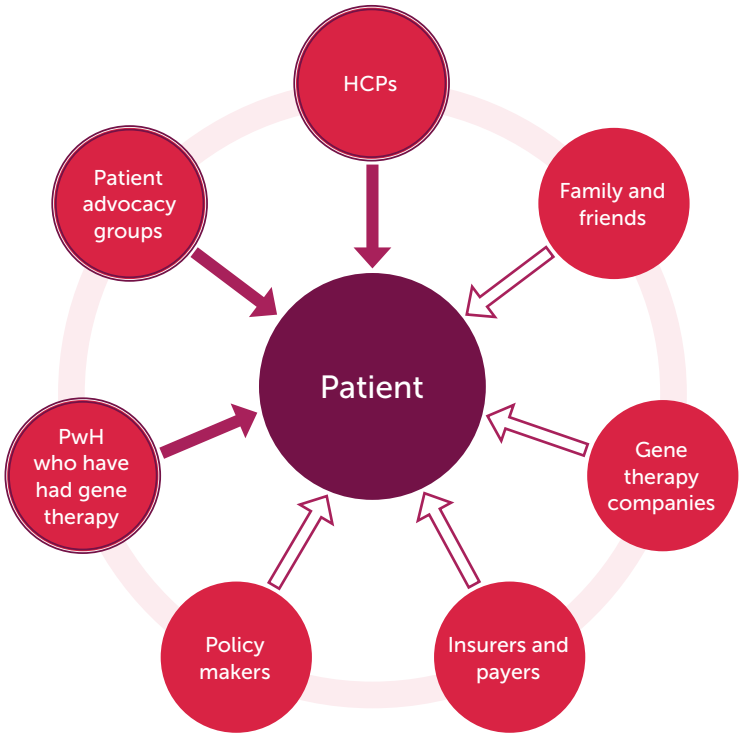
The advisory board participants identified multiple stakeholders in the bleeding disorders community who may play a role in awareness, education and support around in haemophilia gene therapy (Figure 1), among whom HCPs, patient advocacy groups and PwH who have had gene therapy being seen as key.

Collaboration between different stakeholders was seen by participants as important for ensuring that PwH have access to up-to-date information in an understandable format. It was suggested that HCPs and patient advocacy groups should work together to enable a coordinated approach to patient education, making sure to strike a balance between providing enough information and 'information overload'. Participants felt that reliable information on trial outcomes and ongoing treatment should be provided by HCPs, with input from gene therapy manufacturers. Providing psychological support to PwH considering or undergoing gene therapy was also identified as an HCP role, as was checking individual understanding of gene therapy and other haemophilia treatments. Patient organisations and advocacy groups could act as facilitators of peer-to-peer support, enabling PwH who have already had gene therapy to contextualise information provided by both HCPs and patient groups through sharing personal experiences.

Ensuring understanding

As on overall approach to gene therapy education, it was suggested that basic information should be provided in the first instance, and gradually built up as interest in pursuing gene therapy as a treatment option increases.

Figure 1. Stakeholders in the bleeding disorders community who may play a role in gene therapy awareness, education and support



HCPs: Health care practitioners
PwH: People with haemophilia

The importance of striking a balance between avoiding information overload while providing enough information to enable PwH to be fully involved in making an informed shared decision about gene therapy was noted, as was the need to ensure that information about gene therapy is accessible to PwH of all backgrounds and levels of education. Teach-back to check understanding was identified as important in confirming that PwH understand the information they are presented with [12,16,27,28].

> *"They need to know what they're getting into and I think you use the teach-back method to check that and they have to understand, for example, the monitoring and follow up requirements."*

> *"You have to check the accuracy of the information in someone's head after you've given it. So you give information and then you check 'Okay, what does it mean to you? What do you now see as the biggest pro? What do you see as the biggest con?'"*

Patient organisations were seen as playing a key role in the general education that would enable PwH to have informed conversations with HCPs about gene therapy as a treatment option [29]. However, participants felt that providing more detailed information and checking understanding of gene therapy and any other treatment option being considered was a role for clinicians [15]. They discussed the issue of the trust PwH may place in clinicians to direct them to the most appropriate treatment:

> *"A doctor wouldn't give you anything that's going to harm you, would they?"*

In the case of gene therapy, participants generally felt that its 'one-time-only' nature and lifelong impact made patient understanding particularly important, including understanding of the fact that outcomes may be unpredictable. Managing expectations and addressing outcome uncertainty were seen as highly important [15,16,19,26,28]. In presenting information on outcome uncertainty, it was again suggested that focusing on the impact of different outcomes on number of bleeds rather than factor percentages would support better patient understanding. Some participants suggested that there should be a degree of psychological intervention before making a decision on whether to go ahead with gene therapy. Ascertaining how individual PwH have previously managed other 'unknowns' in their lives could provide HCPs with foresight around potential support needs.

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"You can't talk about what you don't know but you do want to know how someone has been dealing with unknowns in his life [...] how do we think he will deal with this and can we foresee some problems there?"

Although haemophilia gene therapy is only available as a treatment option to adults, i.e. those over the age of 18, some participants questioned the capability of an 18-year-old to make an informed decision based on a clear understanding of treatment. While acknowledging that the late teenage years and early twenties may be a good time to have gene therapy – *"just because this is your most active time in life"* – there was some doubt as to whether individuals in this age group would have a sufficiently *"mature understanding"* of gene therapy or the *"possible repercussions"*. Parental influence was also raised as a potential impact on decision-making for PwH in this age group.

Overall, participants agreed that PwH who decide to have gene therapy should have at least a minimum level of understanding about what it involves, including the importance of follow-up, and focused on benefits, risks and managing expectations [15,16,18,19,28]. Ensuring this should help to avoid 'treatment remorse', as well as meaning that PwH are confident that their decision is well thought through and, if they go ahead with gene therapy, that they are able to accept their individual outcome.

"Whatever decision you've taken, whatever outcome you get you should be confident that you've thought all that through beforehand."

Peer support

Participants saw great value in enabling PwH to speak with people who have had haemophilia gene therapy as part of the education process, illustrating clinical facts with experience, both positive and negative (Table 1).

"To see what it means, you have to hear from a person who has experienced it."

Within the group, the participants who had undergone gene therapy reflected the known variation in individual experiences and outcomes.

"If there was somebody out there with haemophilia who's thinking about gene therapy, they'd get much more benefit from coming into this room and talking to each of you individually and then collectively than he would from any one of us, because it's different experiences. We all went into this with different expectations, different life experiences, different experiences with our haemophilia, different concerns."

It was seen as important for PwH to speak with or hear from multiple people who have had gene therapy – basing decisions on the experience of a single person was described as 'dangerous' as it would not be representative of this range.

Participants agreed on the importance of peer experience sharing being conducted or presented in a way that does not influence the decision-making process of the person considering gene therapy. Providing basic training to PwH who have had gene therapy and who are willing to share their stories with peers would help to ensure objectivity and contextualisation of personal experience. One participant suggested that peer conversations should focus on the decision-making process, i.e. why they decided to have gene therapy/what considerations led to this decision, rather than outcomes. This would help to highlight the

Table 1. Gene therapy information sharing in 'formal' and 'informal' settings
'Formal' gene therapy education is provided by health care practitioners (HCPs) and patient organisations, with peer-to-peer sharing of experiences providing context.

	FORMAL: HCPS, PATIENT ORGANISATIONS	INFORMAL: PEER-TO-PEER
INFORMATION / DISCUSSION	• Ensuring patient understanding of gene therapy for haemophilia • Pros, cons and potential risks • Motivations for having gene therapy (patient preferences and goals) • Specifics of particular gene therapy products • Follow-up requirements and rationale	• Adding context/detail to pros, cons and risks explained by the clinical team • Best and worst parts of individual gene therapy experience • Experience of follow-up requirements • Impact(s) on family (e.g. corticosteroid side effects, family planning) • Alcohol avoidance

importance of individuals being happy with their decision and being reconciled to whatever outcome they obtain.

In terms of the form that peer support should take, participants saw face-to-face conversations as being ideal but acknowledged the difficulties inherent in enabling peer conversations given the rarity of haemophilia and the small number of PwH who have undergone gene therapy to date. It was suggested that patient organisations could play a role in facilitating peer conversations in a group setting, and that this could involve partners, carers and family members as well as PwH considering gene therapy. Being able to speak with or hear from PwH from the same country about their experience of gene therapy was not a high priority; participants felt it was more important to hear about different individual experiences, regardless of where the individual was treated.

Participants suggested that online technology could be a useful facilitator in sharing peer experiences of gene therapy. Video 'talking heads', based on individuals answering a shared set of questions, and videoed group conversations about gene therapy were also proposed as a way of sharing experience without face-to-face contact. These could include the sharing of stories from PwH who do not want to have gene therapy or who would like to have gene therapy but are unable to. A perceived benefit of access to video of this kind was that it could be accessed repeatedly. Being able to read about peer experiences was also seen as valuable.

CONCLUSION

Shared decision-making is already established as a best practice approach in bleeding disorders care [14], and participants in the patient advisory board agreed on its importance in the context of gene therapy for haemophilia. This belief is based on a need, recognised by all participants, to ensure that PwH considering gene therapy as a potential treatment option understand what it entails and that their expectations are managed appropriately.

A question remains as to how 'informed' shared decision-making is assessed as different individuals will consider different levels of information as constituting them being 'informed'. These differences should be accommodated within the overall individualisation of the shared decision-making process.

PwH participating in the advisory board identified clear areas of educational need pre-, peri- and post-haemophilia gene therapy, including initial eligibility, comparison with other therapeutic options, expectation management, gene therapy administration, and short- and long-term follow-up. To address these needs, PwH require access to consistent, accessible information, starting with the basics and building up the level of detail as their interest in gene therapy as a treatment option progresses. HCPs and patient organisations should be the key deliverers of gene therapy education, ideally working in collaboration. PwH must have a certain level of understanding of gene therapy before making a decision about having it, and a teach-back approach should be employed to ensure this. Gene therapy should be presented as one of a range of treatment options. Patient understanding of its risks, uncertainties and benefits is key to managing expectations and minimising the risk of treatment remorse.

Peer-to-peer learning has a valuable role to play in gene therapy education. Learning about the unique experiences of other PwH who have had gene therapy would help to contextualise the information provided through 'formal' gene therapy education, bringing a sense of reality to the range of possible outcomes and adding colour to potential positives and negatives. Peer learning will require facilitation, with patient organisations well placed to support this. Consideration must be given to different methods of enabling peer learning to ensure that it is accessible to all PwH interested in gene therapy.

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Disclosures

Brian O'Mahony participated in the advisory board and has acted as a paid consultant to CSL Behring, Biomarin, Freeline, Pfizer and Roche; he is Chief Executive of the Irish Haemophilia Society.

Manuel Baarslag and Daan Breederveld participated in the advisory board and have acted as paid consultants to CSL Behring.

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