

2022 GLOBAL HEMOPHILIA ADVOCACY LEADERSHIP SUMMIT

Saturday 29 and Sunday 30 January 2022: Event discussion report

On Saturday 29 and Sunday 30 January, the 2022 Global Hemophilia Advocacy Leaderships (GHAL) Summit took place. The theme of this year's Summit was '*Advocating for Innovation: A Seat at the Table*', with particular focus on:

- ***Innovation, today and tomorrow:*** Advocating for innovative treatments, advancing the current standard of care in local settings.
- ***Securing your seat at the table:*** What is needed to become a partner in the policy makers' decision-making arena.

Full plenary session recordings from the 2022 GHAL Summit are available on the HAAB program microsite, at www.ghalsummit.com.

This report provides a summary and analysis of the key topics presented by the event speakers and discussed amongst event attendees, which were shared during sessions and parallel chat function entries.

Assessing the value of hemophilia treatment & innovation

- To assess the value of hemophilia treatment, both quality and length of life must be considered. Methodology must be flexible enough to work across both considerations.
- Value and cost must be defined separate from one another. Although a treatment may be costly, it may also improve a patient's quality of life considerably, making it also highly valuable.
- Evolving frameworks should allow for flexibility, for example in cost-effectiveness thresholds, particularly in a post-Covid environment. These global changes have made us more aware of global interaction in the healthcare space, and the value of having treatment solutions.
- The term "value of hope" is a great label, but a broad one. In the hemophilia community, what it really refers to is having the chance of a cure.
- The value framework is not intended for presentation to policy makers or other decision makers; instead, advocates should use the value framework to understand the relevant data and know the desired outcomes for patients.
- The value framework helps build a case by presenting data in an organized and coherent way, which will aid in policy maker understanding.
- The value of hemophilia treatment can no longer be defined by bleed rates alone – we need a framework to represent the value treatment brings to a patient's life.
- Evidence of patient-centered outcomes is limited, posing a challenge when taking clinical data into consideration for treatment decision-making.
- To prepare the world for gene therapy, there is a lot of work to do. There are major barriers such as a large upfront payment and how systems deal with this. The community is working toward a solution in which payers will help us address the issues across the breadth of different and emerging gene therapy treatments.
- We can look to existing analogs within the EU where a few cell and gene therapies have gained market authorization. In this situation, the life-long value vs. the one-off payment should be highlighted; hemophilia treatments are costly but the effects over a lifetime are significant. A large upfront payment could alleviate a long stream of costs over the patient's life.
- Most hemophilia patients have access to effective yet high-cost treatment that provides quantity and quality of life. This can make it difficult to convince payers of the value of a new innovative treatment.
 - To change the perception of payers and influence a payer's decision, data, metrics, and financial evidence must be utilized and presented – it must be made clear that value and cost are not the same.
 - Treatments are not unaffordable; to demonstrate this, advocates must explain what is important and meaningful to patients, have data to represent this and make clear that all elements must be reflected in the decision-making process.

Health technology assessment for innovation in hemophilia treatment

- When assessing new treatments or considering changes to disease management, mental health implications must be considered.

- If a patient suffers a loss of identity, they can often feel a separation from their patient community as they are no longer living with chronic illness – seen previously within the HIV/AIDS community.
- Increased levels of depression are evident among the hemophilia patient community, particularly among patients who use non-factor therapies.
- For gene therapy patients who are struggling with a loss of identity, mental health considerations must be accounted for in the ICER review process.
 - This is because if a patient suffers from depression, yet sees improvements in their physical outcomes, the net effect is that there is an impression of no incremental benefit to them having undergone treatment.
- It is beneficial to have a multidisciplinary team monitoring the clinical outcomes of these patients, ensuring accurate and complete representation of all treatment effects.
- With long-term chronic diseases, there are shortcomings, and the tools need to be pragmatic to articulate the instrumental value and benefit to the patient. Therefore, we must understand what drives meaningful innovation from the hemophilia community's perspective.
 - Data needs to be updated to reflect the progress made and patients' reality. Real world evidence must be used to show the long-term impact that a drug can have on an individual.
- HTA considerations must be led by what patients are experiencing in real-world terms. For example, the rise in depression in patients taking new treatments should be captured in the HTA assessment process.

Payment models for innovations in hemophilia treatment

- The emergence of gene therapy has prompted the need for alternative payment models: both outcome- and finance-based.
- To determine the best option for each country, the benefits and drawbacks of the treatment are assessed.
- When discussing payment models, there are several things we can bring to the table, particularly, insight regarding the tradeoffs presented by gene therapy.
- Payers and decision makers will be responsible for making innovative treatment accessible and they will require advice on how to do this.
- Like everyone else, senior stakeholders are trying to reduce uncertainty surrounding new treatments. As advocates, and as a community, we can support them by providing evidence to better understand the value of innovative therapies – both in terms of long-term data and evidence from clinical trials.
- Advocates should aim to explain all treatment options, including outcomes and potential risks associated with each as they relate to patient care. Advocates should highlight the current cost of treatments alongside the cost of the innovation in question, to reduce to economic negativity associated with gene therapy.
- It is an advocate's duty to be a partner to payers and support them in accessing the clinical data; innovation works by increasing certainty.

Leadership Development

- Being an effective advocate means becoming a positive resource with key decision makers as well as developing and maintaining constructive and engaged relationships based on good data and information.
- The next generation of leaders should be involved at all levels of the organization, including leadership and at the board level.
 - Board composition should ideally mirror the demographics of the patient organization itself.
- Until young people are entrusted with roles outside of the usual social media or technology-based positions, the gap between younger and more experience advocates in NMOs will not narrow. There are many ways to do this, including:
 - Normalize shadowing, to ensure younger talent understand and gain experience in the day-to-day operations of their organization.
 - Invite younger organization members to strategic planning meetings to present their ideas and better understand decision-making processes.
- To create a bright future and maintain progress, we must evolve and generate new and dynamic leaders for the future.
- There are several ways to attract younger advocates, and this varies from country to country.

- In some cases, younger advocates are energized by the opportunity to engage with elected officials and document their experience on social media. These platforms have been key to attaining new talent and elected officials thrive from social media engagement.
- In other countries, NMOs have an active youth group. Within these groups, it is important to have representatives of youth amongst the patient community.
- There is also the issue of time and dedication. It can be incredibly difficult for the youth to prioritize advocacy. The key is identifying individuals who are busy but are striving to build themselves up within this arena – particularly someone who is interested in engagement on a policy level.
- It is the current advocacy leaders' responsibility to provide information to young people to ensure that they are prepared for advocacy in the future. Ideally, there would be a natural succession and there would be clear people we will bring through the ranks, but this is a challenge for us as a community.
- The younger generations of patients have better access to treatment in many countries and can live their lives with their disease, meaning they may not readily come forward. We must proactively seek, engage, and persuade, younger people with bleeding disorders.
- Opportunities, mentorship and empowering future leaders is not limited by age; leadership is a skill we all develop over a course of our lifetime, and it is never too early or late to become a leader.
- When developing leaders, what is effective for one country may not work for another. We must take a broad view and acknowledge cultural considerations.
- The community should aim to continue to improve care for hemophilia patients – we must proactively seek talent with whom this goal is shared. Often, people do not come forward to join boards, they should be engaged with on an educational and personal level to bring them in directly to support the board, whether this be shadowing board members or through internships.
- The hope is that potential leaders will develop sufficient experience and expertise to take on leadership positions as current leaders retire.

Securing a seat at the table: Lessons from an advocate's perspective

- Speakers identified several means to secure a seat at the table, based on their experience:
 - Build a long-standing and productive community
 - Understand the decision-making structure of your country
 - Develop and actively seek out the right connections
 - Have all the information: do the research, collect the data, and seek patient experiences.
- In low-middle income countries, using data and patient experience is crucial. It is essential to use the evidence from developed countries as real-world examples of overcoming economic barriers.
- As an advocate, you must understand who the key decision makers are in your country. Once in the room, the focus should be on developing and maintaining constructive relationships, as well as providing good data and information. Advocates should be aware of which 'tables' are the most important to be at within local settings and aim to sit at as many of these as possible.
- An advocacy campaign can be one short item, which takes a limited period, or a medium to long term set of objectives.
 - These campaigns need to be strategically planned, particularly with longer and more complex cases.
 - The campaign must include clear objectives, identified resources, time application and a clear strategy for advocacy members, board members, the media, and political stakeholders.
- COVID-19 has forced the health system to speed up, digitally, for example with renewal of prescriptions online. As a community, we have reached the point now that these changes seem to be embedded rather than fleeting.
- Events such as 'Washington Day' can be complex and require significant logistical effort – so we must ensure that we have the right people to support these events.
 - These events are an experience, and it provides a platform for advocates to share their personal experiences of living with a bleeding disorder.
 - Most importantly, the opportunity provides access to relationships and key stakeholders which is the key to success.
- In large countries it is essential to identify the key advocacy requests of each region. It is important to collect data and use the experiences of others, both at home and abroad, to support advocacy campaigns.
- There is often no opportunity to challenge health authorities' processes, which becomes problematic when trying to advocate for improvements. This is partly caused by bureaucracy and the multiple steps needed for product approval.

- Decision makers often request evidence from large studies, as well as randomized and controlled trials, which puts rare disease populations at a significant disadvantage.
 - The best way to overcome this is by gathering the data, real-world evidence and being able to communicate that information effectively to decision makers.
- Where we don't have a seat at the table, advocates must focus on developing the evidence and make a convincing case for advancing standards of care where it is possible.
- Other advocate experience and local program successes are great sources of experience and inspiration for own activities. Local experiences can be documented, templated and shared with other advocates for implementation in their local countries.
 - One program which provides the opportunity for this is the Fellowship For Integrating Responsible Mentors (AFFIRM) program, sponsored by Bayer.
 - The program is an international advocacy and leadership development program, where younger advocates have access to mentors to help further develop and expand their leadership and advocacy skills. The program aims support advocates in bringing better care, programs and treatment to their respective communities.
 - Further information on the program is available [here](#). Applications are open to both male and female applicants, aged 26-38.