2022 Global Hemophilia Advocacy Leadership (GHAL) Summit

Advocating for Innovation: A Seat at the Table

Event Information & Pre-read

Event information

The 2022 GHAL Summit will take place on January 29-30, 2022 at 9:00 – 13:00 EDT / 14:00 – 18:00 BST / 15:00 – 19:00 CET / 22:00 – 02:00 JST.

The theme of this year's event is ***Advocating for Innovation: A Seat at the Table,*** during which we will 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

Agenda details: Saturday, January 29

Day 1 of this year's Summit will see the following speakers join us, expanding on the topic they presented at the final webinar of the 2021 HAAB webinar series.

- **Dr. Louis Garrison**, *Professor in the Pharmaceutical Outcomes Research and Policy Program in the School of Pharmacy, and Adjunct Professor in the Departments of Global Health and Health Services at the University of Washington*
- **Mark Skinner**, *Institute for Policy Advancements, Ltd.*
- **Jamie O'Hara**, *CEO at HCD Economics*
- **Cliff Goodman**, *Senior Vice President and Director, Center for Comparative Effectiveness Research, The Lewin Group*

Following the plenary presentations, audience members will be distributed among breakout groups to discuss each speakers' presentation topic in turn.

Agenda details: Sunday, January 30

Day 2 will see the following leading advocates from all over the world join us to share their own advocacy experiences. They will focus on how they were able to secure a seat at the table and become partners to decision makers in their local markets.

- **Nathan Schaefer**, *Vice President of Public Policy, National Hemophilia Foundation*
- **Dejan Petrovic**, *President of the Serbian Hemophilia Society*
- **Ekawat Suwantaroj,** *National Hemophilia Foundation of Thailand*
- **Tania Pietrobelli**, *President of Federação Brasileira de Hemofilia*
- **Boyan Pirnat**, *Hemophilia Ontario*

Speakers will focus on barriers they faced, the actions they took to overcome these barriers, as well as share their key advice for anyone in a similar position to them.

The event will end with a group workshop activity, during which the event speakers and HAAB members will be available to share thoughts and guidance. Audience members are encouraged to submit topics of discussion for this, including personal advocacy objectives or barriers being faced in your local market. HAAB members could provide guidance on how to achieve your objective or address a barrier you are facing.

If you wish to submit a topic for discussion, please share this with the board for consideration at info@ghalsummit.org.

Pre-read materials

Day 1 of this year's GHAL Summit will focus on the topics that presented during the final webinar of the 2021 HAAB webinar series. To recap, the webinar's speakers and their respective presentation topic can be found below.

- **Dr Louis Garrison** – How ready is the world for gene therapy?
- **Mark Skinner** – Assessing the value of hemophilia treatment
- **Jamie O'Hara** – Health Technology Assessment for Gene Therapy in Hemophilia
- **Cliff Goodman** – Designing alternative payment models for durable therapies: The case of gene therapy for hemophilia A

To read the webinar discussions, a full report can be found here, or on the HAAB program microsite within the on-demand materials here.

During day 1 of this year's Summit, we will be joined by these speakers who will delve into deeper detail on their webinar 3 topics. Workshop groups will also be held in relation to these topics.

To prepare for this, a summary of each of the speakers' presentation topics and research papers can be found below, along with a recording of each speakers' webinar presentation.

Dr. Louis Garrison: How ready is the world for gene therapy?

Focus: Perspectives and insights on the introduction of gene therapy in hemophilia

Purpose: To prepare stakeholders for the introduction of gene therapy into the market along with important context to the three key publications in the supplement that comprise a hemophilia "toolkit".

Key findings: Discussion on the importance of innovative discoveries that have culminated in gene therapy, realistic expectations for gene therapy specific to persons with hemophilia, and the importance of enabling equitable global access to this promising new therapy. Furthermore, they address current and anticipated challenges, while encouraging collaboration between key stakeholders as a necessary mechanism to prepare for entry of this new technology.

Dr. Garrison's webinar 3 presentation recording is available here.

Mark Skinner: Assessing the value of hemophilia treatment

Focus: An in-depth literature review and analysis conducted across emerging therapies for hemophilia, including extended half-life [EHL] replacement factor, non-replacement factor therapies, and gene therapies.

Purpose: To differentiate important patient-centered outcomes between different classes of therapies.

Key findings: Revealed important observations about patient outcomes not limited to bleeding and joint complications, but also encompassing those that affect patients' lifestyles and activities. These observations serve as important considerations for decision-making by patients, clinicians, and within health technology assessment agencies.

Mark Skinner's webinar 3 presentation recording is available here.

Jamie O'Hara: Health Technology Assessment for Gene Therapy in Hemophilia[i]

Focus: Describes the important role of health technology assessment (HTA) in decision-making for new therapies and highlights the need for continued evolution of processes to accommodate the complexities with evidence collection for rare diseases such as hemophilia.

Purpose: Until recently, the hemophilia treatment landscape has been dominated by many variations of replacement factor products that generally share similar features and yield similar outcomes. As gene therapies for hemophilia are anticipated to enter the hemophilia market in the foreseeable future, it is important that HTA bodies are prepared to provide the best possible assessment of value, allowing flexibility in processes used for other, more common afflictions.

Key findings: HTA should take into consideration the rarity and complexity of the disorder, as well as lifelong impacts beyond traditionally monitored clinical outcomes of bleeding or joint scores. HTA assessments that have been used for hemophilia therapies including gene therapy are described, and important factors, such as long-term extrapolation of clinical data and inclusion of a larger set of endpoints, are discussed.

Jamie O'Hara's webinar 3 presentation recording is available here.

Cliff Goodman: Designing alternative payment models for durable therapies: The case of gene therapy for hemophilia A[ii]

Focus: Describes new alternative models (APMs), outcomes-based models (value or performance-based models) and finance-based models, that the authors believe will help to mitigate or manage financial risks, to allow patients to have access to gene therapy in hemophilia A.

Purpose: With the recent emergence of potentially curative cell and gene therapies for persons with hemophilia, there is the potential for improvements in treatment outcomes and in quality of life with a single gene therapy injection (albeit with a high upfront price tag). This brings both economic and clinical uncertainties to the forefront for physicians, patients, payers, providers, and manufacturers alike.

Key findings: APMs offer a different approach to the traditional fee-for-service models. As relatively few gene therapies have been approved in the US or EU, and none, to date, in hemophilia A, it is important to initiate discussions now on reimbursement and payment approaches in readiness for their

approval and introduction to our treatment protocols. The ultimate purpose for this paper is to present the case for APMs for gene therapy for hemophilia A, mindful that they need to be appropriate for a particular health care system and include multi-stakeholder collaboration.

Cliff Goodman's webinar 3 presentation recording is available here.

[i] Health technology assessment for gene therapy in hemophilia. O'Hara J, Neymann P, Jonsson B, *Haemophilia 2021;* supp in press.
24 (6), 873-879.
[ii] Alternative payment models for durable and potentially curative therapies: the case for gene therapy for haemophilia A. Goodman C, Berntorp E, Wong O. *Haemophilia* 2021; supp in press.