

MAKING CARE AN INVESTMENT, NOT A COST

15 June 2021 HAAB Webinar: Discussion Report

During the 2021 Hemophilia Global Advocacy Leadership (GHAL) Summit, Sebastian Rohde presented on *The Future Advocacy Options for the Hemophilia Community*, provoking fruitful discussion on how to interact with policy makers and decision makers to help position investing in hemophilia care as mutually beneficial, for both patients and society. Based on the overwhelming feedback received, the Hemophilia Advocacy Advisory Board (HAAB) decided to explore this topic further throughout the 2021 HAAB Webinar Series – starting with the first webinar, ‘*Making Care an Investment, Not a Cost*’ on June 15th, 2021.

The webinar began with Mr. Rohde’s presentation on how to position hemophilia care as an investment, rather than a cost, (downloadable to view [here*](#)), followed by an audience Q&A. Below is a summary of the key points raised as part of the discussion.

In creating an incentivized policy, the first step is establishing the issue as a priority, but how do we as advocacy organizations move to elevate healthcare as a priority?

- The best way to show that incentivized policies work is to demonstrate to the policy community that they’ve done this in the past with great success.
- Over the past 25 years of hemophilia treatment and rare disease policy development, health ministries and policy teams have changed many times through elections and wider changes.
- This means current policy teams were often not in office when incentivized policy proved successful in the past, and so they must be reminded.
- In practical terms, advocates could provide case study examples of how incentivized policy in healthcare has been successful in the past. Through doing so, these can be brought forward, showing policy makers how they can be part of the successes now and in the future.

Civil society have unequal standing across different countries, how would you adapt this advice to countries outside the EU or North America?

- Europe and the United States both have highly developed civil societies, in the sense that patient communities are present and welcomed into policy conversations. It’s important to learn from these developed countries and what they did with patient advocacy groups to get to where they are now. It’s also important to take learnings from the policies that result from those advocacy activities, to serve as an end goal.
- Europe took longer to develop equivalent policies to the United States, as it took them longer to create the patient community engagements and to be welcomed into the conversation by policy makers. This demonstrates the value of transcending the ‘ingredients to success’ from more advanced markets to your own local activities.
- The hemophilia advocacy community is already globally connected, and so is able to transfer learnings between markets more easily.

The government or policy makers will change due to economic or political pressure. The hemophilia/ bleeding disorders community is small but can become the example of how it is possible to lead the way and achieve our own agenda at the same time.

- The hemophilia community is small, given the rarity of the disease, which can be an advantage as it’s easier to remain focused without other groups or agendas causing disruption.
- Climate change policy was discussed as an example of where small pieces of policy ladder up to bigger change. In particular, the electric car policy’s contribution to the wider climate change policy movement was discussed.
- The hemophilia community could be part of a great movement among all rare or genetic diseases through incentivizing legislation to boost investment into gene therapy or personalized medicine. Opportunity would be created not only for the hemophilia patient community, but for disease areas outside of hemophilia as well.
 - By being the first community to influence policy makers, the hemophilia community would be associated with innovative therapies, including gene therapy or personalized medicine, into the future.

What is your recommendation for advocacy leaders receiving demands from patients and their families to take to decision makers - knowing that realistically it is not possible for the State to comply?

- An important part of our role as patient organization leaders is setting realistic expectations with and for our members on what is practically achievable in a given time period, in addition to advocating for these objectives.
- The patient advocate must collect the demand from the patient/ family member and let them know what is realistically achievable. They must then translate this request into an understandable political language of the decision maker, as well as appeal to the decision maker's interests.
 - The main separation between the patient community and policy makers are the day-to-day dealings. Patients and families are faced with the real-life situation of the disease, whereas policy maker acts on a higher level of decision making. A good advocate must bridge this gap and translate both sides into a mutually agreeable request and language.
- It's also important to understand that partnership and group change is more effective than trying to make change alone – it's a lengthy process and it won't happen in a day.
- Effective preparation and objective setting are essential.
- Advocacy cannot just focus on the next best treatment option for the developed world but must also respect the needs of less developed economies.
- In practical terms, a global resource could be developed to demonstrate to policy makers from all markets that there is a correlation between good policy and good treatment. This resource could be localized to specific markets and could showcase past examples.
 - The hemophilia community has made great progress, but policy makers now need to take one step further – this resource could show them how.

Is it better to not link hemophilia with other rare diseases, to avoid the pot becoming too large?

- Rare diseases are not so rare when you look at the numbers - out of the 550 million population in Europe, 30 million people have a rare disease, which is 6% of the total population.
- It's important to make hemophilia a pilot case, particularly when it comes to gene therapy. Developing and maintaining a good treatment pathway should be the characteristics of hemophilia community.
- The group also discussed that, particularly in the US, payers become more reluctant to spend on expensive therapies, such as gene therapy, for diseases like hemophilia that are treatable.
- One member of the audience shared that in bifurcated health systems, such as the US, in which patient care is often paid for by employers, in many cases expensive new therapies are invalid on health insurance for a few years post launch.
- The group discussed the advantage that hemophilia has above other rare disease areas, in that there is existing government spend in hemophilia. Therefore, advocates must demonstrate that investment in new therapies will result in a cost saving rather than a cost increase.

The World Health Organization (WHO) promotes multisectoral & intersectoral healthcare policies and programs. When countries follow such approaches, do you see risks of the hemophilia agenda being diluted? Or is being included in such multisectoral policies a more sustainable solution for better access to treatment and care?

- Public health policy is a multi-level political approach, with multi-national, national, regional and local approaches to be considered. It's important for advocacy organizations to develop an approach that targets all of these levels of policies in a consistent manner.
 - This can be difficult, particularly for organizations that lack global and local competence. However, given the global nature of the hemophilia community, an opportunity/ strength already exists.
- Multi-sectoral approaches aim to create positive health impacts through splintering health policy objectives into different policy sectors. It aims to seed consistent health policy through unrelated sectors, such as environmental or employment law.
- A similar multi-sectoral narrative could be adopted for the multi-sectoral benefit of investing in hemophilia care – both in terms of the lives of patients as well as the evolution from hemophiliac to a person with hemophilia contributing to society on a higher level.

- The hemophilia community can act as a first case example of how the multi-sectoral policy approach works in healthcare investment in practice.
- The current global and local activation within the hemophilia community places them in an optimal position for engaging with policy makers at all levels from local to global.

In the US, lower taxes due to increased unemployment and dealing with the repercussions of COVID could lead to more restrictive coverage policies in states. How can we make the case for investment in hemophilia care when policymakers have been so focused on mitigating the effects of COVID?

- Cost containment and reduction is an expected political response to a crisis, such as the COVID-19 pandemic. The major difference in this crisis is that it was caused by not investing in healthcare - we weren't ready for it.
- The hemophilia community must take this opportunity to demonstrate to decision makers that this crisis should result in increased investment in healthcare, because less investment will lead to an unhealthier society – we now have the proof.
- Quality of hemophilia care has been linked to innovation, e.g. biotech and gene therapy.
- Countries must now choose whether they will invest in technologies and remain in the driving seat of innovation or hand the lead over to countries willing to invest. We must convince decision makers that our markets must continue to drive innovation through investment.
- We can't allow policy makers to cost save to overcome the aftermath of the pandemic. It must act as the magnifying glass to prove that cost containment leads to crisis.

The patient population that would benefit most from the first generation of gene therapies cannot afford it - are there any innovative global access programs that could be considered?

- There has been an unorganized but effective approach to rare disease policy thus far, including frameworks and incentives, which has resulted in access to medicine for patients.
- However, for gene therapy and other innovative personalized medicines, a global approach is required to create a sustainable regulatory environment with incentives and access in all markets.
- It has been done in the past and can be done better - with an integrated approach. Rare disease approach was successful in the end but took many years to spread from the US to other markets in the world.
- A plan must be developed and taken to policy makers to make it as easy as possible for them to begin the development of such a global environment.
- The hemophilia community has everything it needs to do better this time, in terms of knowledge, experience, global and local activation.

**Note that the download link to Sebastian Rohde's presentation included in this document will expire on 4th August 2021. Please save the recording in your files for future reference.*