

Executive Summary

EHC Round Table on “Working Together on Rare Diseases – Shared Decision-Making for Advanced Technologies”

On December 10, 2024, the European Haemophilia Consortium (EHC) hosted its second post-pandemic Round Table on “Working Together on Rare Diseases – Shared Decision-Making for Advanced Technologies” in Brussels, Belgium. The event took place at the Warwick Hotel from 10:00 to 13:30, and was chaired by Dr Maria Elisa Mancuso, Chair of the EHC Medical Advisory Group. The Round Table was attended by a total of 17 participants, representing a diverse group of stakeholders. This included EHC staff and Steering Committee members and National Member Organisation (NMO) representatives. In addition, third-party organisations, clinicians, and industry representatives were present. The variety of attendees fostered rich discussions and allowed for a broad exchange of perspectives on the key challenges and opportunities in shared decision-making for advanced therapies.

Session Highlights

The event commenced with a warm welcome and introduction from the chair, setting the stage for an informative and interactive session.

1. Towards a more patient-centered EU policy on rare diseases:

MEP Andriukaitis (S&D, Lithuania) and MEP Sokol (EPP, Croatia) provided an overview of EU policies on rare diseases. They highlighted the critical impact of the lack of diagnosis, access to care and prevailing health inequalities and the role of EU Pharmaceutical Legislation in addressing these challenges. Both MEPs emphasised the importance of overcoming these challenges through better education for the general public, patients, and healthcare professionals.

Key points included the need for a comprehensive strategy on rare diseases that would include data collection, AI, & education of healthcare professionals. The need for a Pan-european level of cooperation to leave no patient behind was also emphasised by MEP Andriukaitis. MEP Sokol stressed the need to bridge the gap between EU members with ambitious funding. He also noted that health equity requires the EU Pharmaceutical Legislation that balances innovation for rare diseases with guaranteed access and strengthened ERNs. Additionally, it was highlighted that improved cross-border access pathways with the necessary infrastructure can mitigate inequalities, tightening the gap



across Member States, and ensuring all EU patients can access life-saving therapies, regardless of their place of residence.

2. Innovative therapies for rare bleeding disorders, new challenges on sharing and deciding:

Dr Quentin Van Thillo presented an overview of the innovative therapies for rare bleeding disorders, focusing on new challenges on sharing and deciding. He discussed the different treatment options available for bleeding disorders, and stressed the need for more innovation is needed to overcome health inequity in rare bleeding disorders. Dr Quentin Van Thillo stressed that the access to treatment is not distributed equally and that future treatments should be tailored to and discussed with the patient.

3. Working together on rare diseases: a common design between patients and clinicians:

Dominik Cepic shared a personal account of his experiences with haemophilia, covering the broad spectrum of bleeding symptoms he has experienced, his journey to diagnosis and getting the right treatment. His powerful story illustrated the ongoing challenges of accessing and the need for appropriate and tailored treatment, as he illustrated by comparing his treatment dosage to a standard patient. Dominik emphasised the critical importance of patient education and self-advocacy. His story highlighted the need for healthcare professionals to listen closely to patient experiences and ensure timely, appropriate interventions to manage the various symptoms of bleeding disorders.

4. Q&A Session:

The event concluded with a Q&A session, where attendees raised important questions and shared insights on the challenges surrounding shared-decision making. A key discussion point was the limited access to tailored treatment, with attendees exploring the main obstacles to wider access. It was noted that collaboration between physicians and patients play a significant role to improve patient outcomes. The challenge of educating and empowering patients was also highlighted. Learning from haemophilia treatment models could provide valuable insights, but awareness among clinicians remains insufficient, necessitating further education and awareness-raising efforts.

Increased collaboration between clinicians and patients was discussed as a potential option, but missing data from patients not reporting all bleeding episodes and data-sharing challenges between hospitals were flagged as issues. Additionally, 83% of patients desire more involvement in their treatment choices. This underlines the importance of improving shared decision-making processes between patients and clinicians to enhance the quality of life for those with hemophilia and other bleeding disorders

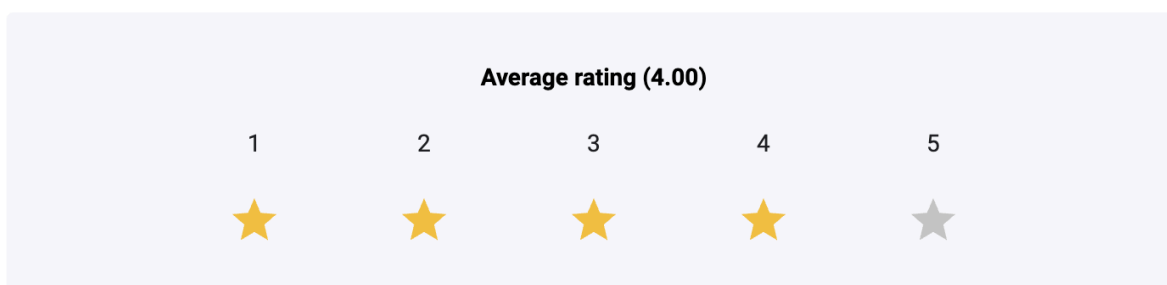


Attendees also discussed the need for clinicians to listen to the patients. It was highlighted that patients bring unique insights from lived experiences. These discussions underscored the ongoing need for more tailored and accessible care for patients with bleeding disorders across Europe.

The Round Table concluded with a synthesis of the discussions by Dr Mancuso, reaffirming the EHC’s commitment to addressing ongoing issues in care of bleeding disorders and encouraging collaborative efforts to improve access to treatment and patient support across Europe.

Post-Event Survey

Attendees were invited to complete a post-event survey to evaluate the effectiveness of the sessions, the relevance of the topics covered, and the overall experience of participants. Following the event, attendees responded to the post-event survey, with an overall rating of 4.0. All three individual sessions were rated as “excellent” and “very good”, reflecting the high quality of the presentations. Attendees particularly appreciated hearing the patient perspective, which added valuable real-world insight to the discussions. The positive feedback will help guide future EHC Round Tables, ensuring they continue to meet the needs of the bleeding disorder community.



Conclusion and Acknowledgements

The EHC Round Table on “Working Together on Rare Diseases – Shared Decision-Making for Advanced Technologies” successfully fostered meaningful dialogue among healthcare professionals, patients and patient advocates, third-party organisations, and industry representatives, addressing key challenges in diagnosis, treatment, and patient care. The event emphasised the importance of education, awareness, and collaboration to improve outcomes for bleeding disorder patients across Europe. The EHC extends its heartfelt thanks to Sobi, Regeneron and Biomarin for their generous sponsorship, which made this important gathering possible and contributed to the advancement of discussions on improving care for people living with bleeding disorders.

