Executive Summary

EHC Round Table "No Patient Left Behind: Unmet Needs in Extremely Rare Bleeding Disorders"

On March 17, 2026, the European Haemophilia Consortium (EHC) hosted the Round Table entitled "*No Patient Left Behind: Unmet Needs in Extremely Rare Bleeding Disorders*" at Thon EU Hotel in Brussels, Belgium. The event took place from 14:00 to 16:00, and was chaired by Tatjana Marković, Vice-President of the European Haemophilia Consortium (EHC). The Round Table was attended by a total of 23 participants, representing a diverse group of stakeholders. This included EHC staff, Steering Committee members, European Rare and Inhibitor Network (ERIN) Committee members and National Member Organisation (NMO) representatives. Additionally, representatives from third-party organisations, clinicians, and the industry were present. The variety of attendees fostered rich discussions and allowed for a broad exchange of perspectives on how to address gaps in diagnosis, treatment, and access to care for rare bleeding disorders.

Session Highlights

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

1. **Rare Diseases in EU policy:Extremely Rare Bleeding Disorders: A Joint Look at Unmet Needs:**

Dr Maria Elisa Mancuso, Chair of EHC Medical Advisory Group, presented an overview of rare bleeding disorders in Europe. She emphasised that accurate diagnosis relies on careful clinical evaluation, specialised laboratory testing, and multidisciplinary expertise. She highlighted the wide variability in bleeding symptoms and underscored the importance of comprehensive treatment centres, as well as the need for continuous medical education to support timely and effective care.

Dr Katharina Holstein, Vice Chair of EAHAD Very Rare Coagulation Factor Deficiencies Working Group, presented the major challenges in this field, including highly variable bleeding phenotypes, limited clinical evidence, a lack of guidelines, and insufficient research. She stressed that even patients with mild deficiencies may experience significant bleeding if not appropriately treated. She also outlined key research gaps and highlighted emerging therapeutic approaches, such as rebalancing therapies and gene therapy, alongside collaborative European efforts to improve data collection, education, and patient care.

2. Living with an Extremely Rare Bleeding Disorder:

Nathan Roubaty shared a personal account of his experiences with afibrinogenemia, detailing early severe complications, the transition to self-managed prophylactic treatment, and the challenges of treatment logistics, while emphasising that strong support systems and modern therapies had enabled him to live a relatively normal life.

Inge Pieters presented a caregiver's perspective that stood in stark contrast. Her story illustrated the unpredictability of bleeding, repeated emergencies, and a lack of medical awareness. She also highlighted a critical gap: unlike Nathan, her daughter does not benefit from a reliable treatment capable of effectively protecting her from bleeding risks. She further described the heavy emotional, social, and psychological burden on both the child and the family, including limitations in daily activities and constant anxiety about the future. Her and Nathan Roubaty's stories underscored the need for better knowledge, faster diagnosis, improved care pathways, and greater support for patients and families.

3. The European Rare Inhibitor Network: Building Knowledge, Pathways and Hope:

Maja Johanne Søndergaard Knudsen, member of the EHC ERIN Committee, provided an overview of the activities of the EHC European Rare and Inhibitor Network (ERIN) Committee. She explained that the European Rare & Inhibitor Network, established in 2022 from the EHC Inhibitor Working Group, aims to expand support for people with extremely rare bleeding disorders by building on earlier advocacy efforts. She outlined how the network has identified key unmet needs—including limited data, underdiagnosis, insufficient education, and social isolation—and has responded by setting objectives focused on patient empowerment, advancing research, strengthening advocacy, and enhancing engagement with policymakers.

She also highlighted ERIN's key activities, such as webinars, awareness campaigns, and annual summits. In closing, she presented a long-term vision centred on improved diagnosis, better access to treatment, and enhanced quality of life, supported by stronger collaboration, increased investment in research, and more inclusive European rare disease policies.

4. Panel discussion:

During the Q&A session, previous speakers were joined by Cédric Hermans, Head of Division of Haematology at the Saint-Luc University Hospital, and Marina Pimentel, member of the EHC ERIN Committee. Together with participants, they engaged in a lively discussion on how to address gaps in diagnosis, treatment, and access to care for rare bleeding disorders. The conversation emphasised the importance of placing lived experiences at the centre of hospital practices to better inform research priorities, healthcare policies, and treatment pathways. The round table also reinforced that addressing gaps in care requires comprehensive and systematic data collection and comprehensive registries. As the discussions concluded, participants underscored the need to ensure that people with rare bleeding disorders receive appropriate care to manage their symptoms while awaiting effective treatments for their conditions.

The Round Table concluded with a synthesis of the discussions by Tatjana Marković, reaffirming the EHC's commitment to addressing ongoing issues in the diagnosis and care for people with rare bleeding disorders and encouraging collaborative efforts to improve diagnosis and access to care across Europe.

Conclusion and Acknowledgements

The EHC Round Table on *"No Patient Left Behind: Unmet Needs in Extremely Rare Bleeding Disorders"* successfully fostered meaningful dialogue among healthcare professionals, patients, patient advocates, third-party organisations, and industry representatives, addressing key challenges faced by people living with rare bleeding disorders. The event highlighted the critical importance of education, awareness, and support in improving care for individuals with rare bleeding disorders across Europe.