Hematology research

Expertise to accelerate your inherited bleeding disorder clinical trials

Inherited bleeding disorders—such as hemophilia A (1 in 5,000 male births), hemophilia B (1 in 25,000–30,000), von Willebrand disease (0.6–1.3% prevalence), and hereditary hemorrhagic telangiectasia (1 in 5,000)—vary in frequency but significantly impact patients and families.

Treatment aims to manage bleeding risks and improve quality of life, with major advances including extended half-life therapies, gene and cell-based treatments, novel factor concentrates, and improved diagnostics.

Despite progress, challenges remain. Continued research is critical to develop more effective, accessible therapies. The PPD™ clinical research business of Thermo Fisher Scientific is your partner in accelerating development—combining global expertise, operational excellence, and a robust network of hematology sites to support recruitment, retention, and trial success.

Sources:

1. World Federation of Hemophilia Report on the Annual Global Survey 2023 WFH when Annual Global Survey- Access from Reports from October 2024 https://www1.wfh.org/publications/files/pdf-2525.pdf
2. https://doi.org/10.1016/j.rpth.2023.102264
3. https://doi.org/10.2147/JBM.S389241

Inherited bleeding disorders present several challenges across different domains, including medical, psychological, and societal aspects

Medical challenges

- **Frequent bleeding episodes:** Individuals with these disorders are prone to spontaneous and prolonged bleeding, which can be difficult to manage and may lead to severe complications. Severe forms of inherited bleeding disorders can develop life-threatening bleeding episodes.
- **Treatment side effects:** Long-term use of treatments like clotting factor concentrates can lead to the development of inhibitors (antibodies) that reduce treatment efficacy, contributes to joint disease and impact quality of life
- **Special considerations for pediatrics:** often due to genetic components – they frequently have early onset impacting quality of life in children and adolescents. Significant challenges occur during the transition, including switching healthcare systems and establishing independence to avoid a decline in quality of life. Awareness of the fundamental prerequisites for transition among patients, families, and healthcare professionals

Psychological and emotional challenges

- **Anxiety and stress:** The constant threat of bleeding and the need for ongoing management can cause significant anxiety and stress for both patients and their families.

Stigma

- **Stigma** associated with bleeding disorders in the general population. Need for better education of patients, family members, and non-specialist medical professionals about the disease

Socioeconomic challenges

- **Healthcare access:** Socioeconomic status can affect access to quality healthcare, leading to disparities in outcomes and ultimately contributing to.
- **Financial burden:** The cost of treatment can be a significant burden for many patients and their families.
- **Caregiving responsibilities:** Family members may need to provide significant support, which can be physically and emotionally taxing.

Genetic counseling and family planning

- Families may face decisions regarding genetic counseling and testing, especially if planning for future children.

Treatment adherence and management

- Development of extended half-life formulations to reduce dosing frequency. Development of chain of custody to support prophylaxis and care for acute treatment. Innovative therapies like emicizumab improve compliance with prophylaxis and provide alternatives for patients with inhibitors.
  - **Limited access to prophylaxis:** Approximately 75% of people with hemophilia worldwide are not regularly treated.
  - **Complex treatment regimens:** Managing the bleeding disorders often requires adherence to complex treatment regimens, which can be difficult to maintain consistently.
  - **Emergency preparedness:** Patients and families must be prepared to handle bleeding emergencies, which requires education, planning, and quick access to medical care.

Addressing these challenges requires a multifaceted approach involving advancements in research and treatment options, as well as improved access to care and support networks, which are essential to help individuals with inherited bleeding disorders lead healthier and more fulfilling lives.

Our strong global expertise in hematology diseases, pediatrics, and gene/cell therapy has collectively supported strategic offerings, solutions, and recent successes for rare hematology disease trials. We can tailor unique solutions to each protocol and proactively assess challenges that may arise.

In the last five years, we have conducted:

266
Hematology studies

607
Rare disease studies

174
Rare hematology studies

We have successfully delivered over 20 studies in bleeding disorders- including hemophilia A and B, hereditary hemorrhagic telangiectasia and von Willebrand disease, from early to late stage development.

Our experienced team can provide the following to your bleeding disorder trial:

- Accelerated site startup and increased enrollment using our selected sites
- Reduced global site activation timelines through the use of innovative site feasibility and activation strategies
- Earlier achievement of enrollment timelines by a unified team approach towards timely interventions to support high enrolling and non-enrolling sites
- Timely data analysis by close cross-departmental collaboration

End-to-end operational expertise for rare hematology conditions and inherited bleeding disorders

A **global network** of experienced hematology principal investigators

Characterization, endpoint determination, and patient recruitment expertise

Optimized, data-driven country and trial site selection

Site activation, enrollment and retention strategy development

Deep understanding of key protocol components impacting enrollment

Data management and hematology expertise in biostatistics, quality and regulatory expertise

Clinical and regulatory strategy consulting

Support services for optimizing access and minimizing patient burden

Cross-functional rare hematology research in our Center of Excellence

Our Rare Hematology Center of Excellence (COE) offers access to more than 20 rare disease experts, including former FDA officials, who provide strategic insights across all therapeutic areas to inform and optimize clinical and regulatory strategy development.

We recognize that there are special challenges with inherited bleeding disorders, so we provide patient and caregiver services informed by our long term partnerships with patient advocacy groups, as well as a compassionate, open discourse with individual participants and researchers.

Easing enrollment and increasing retention with patient-centric services

\We recognize how burdensome clinical trials in rare disease can be for patients and their caregivers. Our host of supportive concierge services reduces the burden for both sites and patients and **makes it easier for patients to participate in trials** by offering:

Telemedicine & Home Healthcare Services	**Digital & Decentralized Protocols**
Transportation Coordination & Verification	**eCOA/ePRO**
Flexible Reimbursement Options	**Wearable & Mobile Pagers**

The advantages of decentralizing clinical studies includes:

- Reduction in recruitment timelines
- Improved patient retention
- Patient readiness
- Improved patient diversity

Through strategic preferred partnerships and expanded vendor capabilities, we have implemented hematology-specific offerings such as:

- **Patient identification:** Identifying patients through registries and/or directly through the site's own EMR data, reducing site workload and improving the speed of enrollment
- **Hematology mobile sites:** Bringing the site to the patient by going to the patients' homes, setting up outside the site office, or alternate locations to assist with visit procedures, including exams and IP management

These services help **produce timely and high-quality data** for our clients while saving patients time and cost. Our patient-centric approach has led to over **90% patient retention** over five years for a recent long-term follow-up trial.

Unmatched inherited bleeding disorder knowledge and experience

We have significant expertise in early development studies with global reach to enable decreased timelines, improved success rates, global resource footprint with a niche CRO mindset. We offer tailored services to our partners, sites and patients, as well as leverage innovative new approaches to design more efficient studies.

We recognize that **Patient Advocacy Groups (PAGs)** bridge the gap between patients, caregivers, and the medical and pharmaceutical community. Involvement with PAGs to bring awareness to clinical trials is a win-win for the patients and our industry, as we gain firsthand knowledge of the burden of disease from patients and caregivers, and at the same time patients have an opportunity to participate in clinical trials for new treatment options. We offer a patient advocacy specialist role to leverage knowledge and experience with PAGs.

Our commitment to patients means that we will strategize to optimize patient and caregiver experience by offering decentralized solutions, home healthcare and patient concierge options as well as educational materials and experienced medical monitors that offer disease insights and engage patients.