

Personalized Hemophilia Care: EHA 2026 Highlights Individualized Approaches

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The management of hemophilia has historically relied on standardized prophylactic regimens, aiming to prevent bleeding episodes and associated arthropathy. However, the inherent variability in patient response, bleeding phenotypes, and pharmacokinetic profiles necessitates a more refined approach. The EHA 2026 congress highlighted the emerging era of personalized medicine in hemophilia, emphasizing tailored treatment strategies to optimize outcomes for each individual.

KEY TAKEAWAYS

- ° The Pivot: Hemophilia care is shifting from uniform prophylaxis to individualized treatment plans, integrating patient-specific data.
- ° The Data: Pharmacokinetic-guided dosing and real-world data collection are central to optimizing factor replacement and non-factor therapies.
- ° The Action: Clinicians should consider incorporating patient-specific pharmacokinetic profiles and lifestyle factors into treatment decisions for hemophilia A and B.

Hemophilia, an X-linked bleeding disorder caused by a deficiency in clotting factor VIII (hemophilia A) or factor IX (hemophilia B), has seen significant advancements in treatment over recent decades. The introduction of recombinant factor concentrates and extended half-life (EHL) products has substantially improved the quality of life for many patients, reducing the frequency of bleeding episodes and preventing joint damage. Despite these advances, a 'one-size-fits-all' approach to prophylaxis often leads to suboptimal outcomes for some individuals, while others may receive more treatment than necessary. This variability underscores the clinical dilemma: how to best match the therapy to the patient's unique needs.¹

The concept of personalized medicine in hemophilia addresses this by advocating for treatment regimens that consider an individual's specific characteristics. These characteristics include their bleeding phenotype (e.g., spontaneous bleeds, trauma-induced bleeds), physical activity level, joint health status, and most critically, their pharmacokinetic (PK) profile. Traditional dosing often relies on population-averaged PK data, which may not accurately reflect an individual's factor clearance rate or half-life.²

Advancing Individualized Treatment Strategies

Discussions at EHA 2026 emphasized several key components of personalized hemophilia care. One central aspect is the increasing use of PK-guided dosing. This involves determining an individual patient's factor half-life and clearance rate through sparse sampling methods, often using software algorithms to estimate a full PK curve from a limited number

of blood samples. By understanding a patient's unique PK profile, clinicians can adjust the dose and frequency of factor concentrate infusions to maintain trough levels above a protective threshold, thereby minimizing bleeding risk while potentially reducing overall factor consumption. For instance, a patient with a faster factor clearance might require more frequent infusions or higher doses to achieve the same protective trough level as a patient with slower clearance.³

Beyond factor replacement, personalized medicine extends to the application of non-factor therapies. Efficizumab, a bispecific antibody mimicking factor VIII, has demonstrated efficacy in preventing bleeds in hemophilia A patients with and without inhibitors. Its fixed subcutaneous dosing schedule offers convenience, but even with this therapy, individual patient characteristics, such as baseline bleeding rates and activity levels, may influence the overall effectiveness and patient satisfaction. Gene therapy, an investigational treatment aiming for a sustained endogenous production of clotting factor, represents the ultimate form of personalized medicine, as its success hinges on individual patient eligibility, immune response, and long-term expression levels.⁴

The integration of real-world data and digital health tools is also a critical element. Patient-reported outcomes (PROs), electronic diaries for tracking bleeds and infusions, and wearable sensors to monitor physical activity can provide a comprehensive picture of a patient's disease burden and treatment effectiveness. This data can inform treatment adjustments in real-time, allowing for dynamic personalization of care plans. For example, an increase in reported bleeds despite adequate trough levels might prompt an investigation into adherence, activity levels, or the need for a higher protective threshold.⁵

Limitations to widespread implementation of personalized care include the logistical challenges of routine PK testing in all patients, particularly in resource-limited settings. The cost associated with frequent PK assessments and the need for specialized software and training for clinicians also present barriers. Furthermore, while PK-guided dosing aims to optimize factor levels, it does not fully account for other patient-specific factors that contribute to bleeding risk, such as joint status or the presence of comorbidities. Future research will need to refine predictive models that integrate a broader range of clinical and biological markers to achieve truly comprehensive personalized care. The development of more accessible and less invasive PK assessment methods, such as saliva-based testing or micro-sampling techniques, could help overcome some of these practical hurdles.⁶

CLINICAL IMPLICATIONS

The shift towards personalized medicine in hemophilia, as highlighted at EHA 2026, signals a necessary evolution in clinical practice. Relying solely on population-averaged dosing for factor replacement, even with extended half-life products, is increasingly insufficient given the available tools for individualization. Clinicians should actively consider incorporating pharmacokinetic-guided dosing into their management strategies for patients with hemophilia A and B, especially those experiencing breakthrough bleeds or those with highly active lifestyles. While the logistical burden of routine PK assessment is real, the long-term benefits of optimized bleeding prevention and potentially reduced factor consumption warrant the investment.

For the pharmaceutical industry, this emphasis on personalization means a continued focus on developing therapies that allow for flexible dosing and monitoring. The success of gene therapies, for instance, will depend not only on their efficacy but also on the ability to predict and manage individual patient responses, including immune reactions. Companies developing novel non-factor therapies should also consider how their products can be integrated into personalized regimens, rather than assuming a universal applicability. The market will

increasingly reward solutions that empower clinicians to tailor treatment, rather than simply offering another standardized option.

Patients stand to gain significantly from this individualized approach. Reduced bleeding episodes, improved joint health, and a better quality of life are direct consequences of treatments precisely matched to their needs. However, this also places a greater onus on patient engagement and education. Patients will need to understand the rationale behind their personalized regimen, adhere to monitoring protocols, and actively participate in reporting outcomes. The era of personalized medicine in hemophilia is not just about advanced therapeutics; it is about a more collaborative and data-driven partnership between patient and clinician.

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