EAHAD 2026 Clinical and AI / ML Preview

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EAHAD 2026

DUBLIN, IRELAND
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EAHAD 2026 – General Overview

- **Focus on Innovation:** The EAHAD 2025 meeting will highlight cutting-edge treatments and gene therapies for hemophilia
- **Collaborative Research:** Leading global researchers will share insights on new findings in hemophilia and associated bleeding disorders
- **Clinical Trial Results:** Promising data from ongoing trials will be presented, particularly focusing on novel therapies
- **Patient-Centric Approaches:** Expect an emphasis on improving patient quality of life, focusing on bleeding control and disease management
- **Global Perspectives:** The meeting will feature research from diverse geographies, showcasing the global impact of hemophilia treatments
- **Multidisciplinary Dialogue:** Interactions between clinicians, researchers, and patients will be fostered to promote holistic care strategies

EAHAD 2026– Conference Themes

- **Gene Therapy Advancements:** Data on new gene therapies for hemophilia will take center stage, focusing on long-term effectiveness and safety
- **Non-Factor Therapies:** The application of antibody-based treatments for hemophilia will be explored, with focus on bleeding prevention and management
- **Prophylactic Regimens:** New insights into extended prophylactic treatment options and dosing schedules will be discussed
- **Real-World Outcomes:** Real-world evidence on the effectiveness of treatments like emicizumab will be showcased, with patient outcomes at the forefront
- **Joint Health and Bone Health:** Addressing joint damage prevention and treatment will be a major focus, with new insights on bone health in hemophilia patients
- **Psychosocial and Quality of Life:** Understanding the psychosocial impact of hemophilia treatment will be key, emphasizing patient well-being and long-term care strategies

Noteworthy Scientific presentations at EAHAD 2026

Key Topics From Notable Presentations (1/3)

- **Emicizumab and Real-World Prophylaxis Optimization:** The session will highlight Emicizumab's effectiveness in long-term bleeding control and joint health in pediatric hemophilia A patients
  - The Emicizumab Pediatric Cohort will demonstrate significant reductions in annual bleeding rates (ABR) and improvements in joint health, with MRI showing structural benefits
  - Presentations will focus on the real-world data regarding Emicizumab's cost-effective implementation in treating pediatric hemophilia A, with a focus on dosing reduction
- **Gene Therapy, RNA, and Novel Molecular Therapeutics:** Presentations will focus on the transformative potential of gene therapy for hemophilia A and B, with long-term data on Etranacogene Dezaparvovec and Valoctocogene Roxaparvovec
  - The HOPE-B trial will showcase the Etranacogene Dezaparvovec gene therapy's ability to significantly reduce bleeding in hemophilia B patients, with long-term efficacy data
  - GENEr8-1 study will provide data on Valoctocogene Roxaparvovec, demonstrating long-term liver safety and sustained factor activity, offering promising alternatives to factor-based treatments

Key Topics From Notable Presentations (2/3)

- **Factor Replacement Therapies, PK, and Long-Term Clinical Outcomes:** Session will underscore the durability and safety of Efanesoctocog Alfa and Damoctocog Alfa Pegol, highlighting their effectiveness for long-term prophylaxis and bleed prevention across age groups
  - Data from the XTEND-ed study will demonstrate Efanesoctocog Alfa's ability to provide durable bleed protection and effective treatment with a once-weekly regimen
  - The Alfa-PROTECT study will highlight Damoctocog Alfa Pegol's long-term safety and efficacy in pediatric patients, maintaining low bleeding rates and minimal adverse events
- **Non-Factor Therapies and Antibody-Based Approaches:** The focus will be on Marstacimab and Concizumab as effective non-factor prophylactic therapies, reducing both joint and non-joint bleeds in hemophilia patients with and without inhibitors
  - Marstacimab's ability to reduce joint bleeding by ~93% and target joint bleeding by ~88% will be emphasized in inhibitor patients
  - Concizumab will be presented as a highly effective once-daily option for reducing muscle bleeds and improving overall hemostatic control in hemophilia A and B

Key Topics From Notable Presentations (3/3)

- **Von Willebrand Disease and Rare Coagulation Disorders:** The session will explore new advancements in VWD and rare bleeding disorders, focusing on innovative prophylaxis strategies and personalized medicine approaches to optimize treatment outcomes
  - The WIL-33 study will highlight the safety and efficacy of pdVWF/FVIII prophylaxis in young children with severe VWD, demonstrating effective bleeding control with minimal side effects
  - PopPK-WILPROPHY will focus on personalized prophylaxis, aiming to reduce bleeding events while maintaining QOL and reducing treatment burden for VWD patients
- **Clinical Management, Adherence, QoL, Psychosocial, and Health Systems:** The session will focus on the anticipated impact of advanced tools for improving hemophilia management, enhancing adherence, and addressing psychosocial challenges faced by patients
  - Advanced systems will be explored for monitoring treatment adherence and better predicting patients' needs for individualized care
  - Real-world adherence data will highlight the significant impact of adherence to prophylaxis on functional outcomes and joint protection in hemophilia patients

Focus of Key Industry-Sponsored Sessions at EAHAD 2026 (1/3)

- **Takeda:**
  - Focus Areas: Prophylaxis in Hemophilia & von Willebrand Disease
  - The scientific debate on the use of prophylactic treatments in hemophilia and VWD will be explored, focusing on clinical trial results, patient outcomes, and future therapeutic directions
- **Pfizer:**
  - Focus Areas: Anti-TFPI Approaches in Hemophilia
  - Sessions will explore the latest advancements in anti-TFPI therapies, focusing on their role in advancing hemophilia care and improving treatment efficacy and safety for patients
- **Roche:**
  - Focus Areas: FVIII Mimetics in Hemophilia A Care
  - Discussions will cover the current and future horizons of FVIII mimetics in hemophilia A treatment, highlighting their potential benefits and challenges in real-world applications

Focus of Key Industry-Sponsored Sessions at EAHAD 2026 (2/3)

- **CSL:**
  - Focus Areas: Gene Therapy Durability in Hemophilia
  - The durability of gene therapy in hemophilia will be examined, bridging trial results with clinical practice to assess long-term benefits and challenges in patient care
- **Sobi:**
  - Focus Areas: Normalized Hemostasis in Hemophilia
  - Sessions will focus on moving toward normalized hemostasis in hemophilia treatment, emphasizing real-world data, patient outcomes, and advancements in therapeutic strategies
- **Novo Nordisk:**
  - Focus Areas: Hemophilia B and Anti-TFPI Approaches
  - Novo Nordisk will present patient-centric approaches for treating hemophilia B, highlighting the role of anti-TFPI therapies in improving clinical outcomes and patient satisfaction

Focus of Key Industry-Sponsored Sessions at EAHAD 2026 (3/3)

- **Sanofi:**
  - Focus Areas: Haemostatic Balance in Hemophilia Treatment
  - Sanofi will explore the evolving landscape of hemophilia treatment, focusing on finding a balance in hemostasis and integrating novel therapies into patient care
- **Octapharma:**
  - Focus Areas: Optimizing Prophylaxis in VWD
  - Sessions will address strategies for optimizing prophylaxis in von Willebrand disease, focusing on patient-specific treatment regimens and improving clinical outcomes with personalized approaches
- **Kedrion:**
  - Focus Areas: Hereditary Factor X Deficiency (HFCD)
  - Presentations will focus on personalized treatment strategies for HFCD, aiming to address patient-specific needs, optimize care, and improve clinical outcomes

Notable Presentations And Late-breaking Sessions At EAHAD 2026

Notable Presentations At EAHAD 2026
Non-Factor Therapies and Antibody-Based Approaches (1/3)

Date	Title	Author	Summary
06 Feb 2026	Joint Health in Participants With Hemophilia A or B With Inhibitors Treated With marstacimab in the Phase 3 BASIS Trial	J. Mahlangu	• **Introduction:** Patients with hemophilia A or B and inhibitors have limited prophylaxis options and remain vulnerable to recurrent joint bleeding and progressive arthropathy. Marstacimab offers factor-independent hemostatic rebalancing. • **Methodology:** In the phase 3 BASIS study, males ≥12 years with inhibitors completed a 6-month observational phase followed by 12 months of once-weekly subcutaneous marstacimab. Joint bleeds, target joints, and HJHS were assessed. • **Results:** Marstacimab reduced treated joint bleed ABR by ~93% and target joint bleed ABR by ~88%. Most target joints resolved. HJHS showed early numerical improvement. • **Conclusions:** Once-weekly marstacimab markedly improves joint bleeding control in inhibitor patients, supporting its role as a promising non-factor prophylactic strategy to prevent arthropathy.
06 Feb 2026	Subcutaneous Four-Week Dosing of the Novel Protein S Antibody VGA039 Demonstrates Safety and Clinically Meaningful Bleed Reduction in Patients With Von Willebrand Disease: Phase 1/2 Multi-Dose Study Results	G. Yamaguti-Hayakawa	• **Introduction:** VWD imposes recurrent bleeding and high infusion burden. VGA039, a non-factor IgG4 monoclonal antibody inhibiting Protein S, enhances thrombin generation to improve primary and secondary hemostasis. • **Methodology:** Open-label phase 1/2 multidose study in adolescents/adults with all VWD types received subcutaneous VGA039 every 4 weeks for 120 days, assessing safety, PK/PD, and bleeding outcomes. • **Results:** Sixteen patients showed favorable tolerability with no drug-related serious AEs, thrombosis, or D-dimer elevations. Patients with high baseline bleeding experienced 72–80% reductions in annualized bleeds, sustained into extension. • **Conclusions:** Monthly subcutaneous VGA039 demonstrates promising safety and efficacy across VWD subtypes, supporting non-factor prophylaxis and advancement to Phase 3 for reduced treatment burden.

Notable Presentations At EAHAD 2026
Non-Factor Therapies and Antibody-Based Approaches (2/3)

Date	Title	Author	Summary
All days	Non-Joint Bleeds in Patients With Haemophilia A or B Without Inhibitors: Results from the Concizumab Phase 3 explorer8 Study	V. Jiménez Yuste	• **Introduction:** Non-joint bleeds, particularly muscle bleeds, remain clinically relevant in hemophilia A and B without inhibitors. Concizumab, an anti-TFPI antibody, offers factor-independent prophylaxis. • **Methodology:** explorer8 was a phase 3, open-label study comparing once-daily subcutaneous concizumab with on-demand therapy, assessing non-joint bleeding outcomes up to 56 weeks. • **Results:** Concizumab significantly reduced muscle bleed ABRs versus on-demand therapy by 95% in HA and 85% in HB. Fewer patients experienced muscle bleeds, which were mostly mild/moderate and spontaneous, with durable control through 56 weeks. • **Conclusions:** Concizumab markedly reduces non-joint bleeding, supporting its efficacy as a daily non-factor prophylactic option in hemophilia without inhibitors.
All days	Concizumab Efficacy in Haemophilia A/B in an Intra-patient Comparison vs. Previous Prophylaxis (Phase 3 Explorer8 Study): Post-hoc Sensitivity Analysis	H. Eichler	• **Introduction:** Concizumab, a once-daily subcutaneous anti-TFPI antibody, is approved for prophylaxis in hemophilia A/B. Initial intra-patient comparisons versus prior prophylaxis showed variability driven by extreme bleeding rates. • **Methodology:** Post hoc sensitivity analyses from the phase 3 explorer8 study used negative binomial regression with imputation to assess the impact of outlier patients on annualized bleeding rate (ABR) comparisons. • **Results:** Post hoc sensitivity analyses from the phase 3 explorer8 study used negative binomial regression with imputation to assess the impact of outlier patients on annualized bleeding rate (ABR) comparisons. • **Conclusions:** Concizumab demonstrated non-inferior efficacy to prior prophylaxis for most patients, supporting its use as a reliable non-factor prophylactic option despite interindividual response variability.

Notable Presentations At EAHAD 2026
Non-Factor Therapies and Antibody-Based Approaches (3/3)

Date	Title	Author	Summary
All days	Dose Escalation of Marstacimab in Participants With Hemophilia A or B With Inhibitors in the BASIS Trial	A. Boban	• **Introduction:** Patients with hemophilia A or B and inhibitors remain at high bleeding risk despite non-factor prophylaxis; dose flexibility may be required to optimize bleed control with marstacimab. • **Methodology:** In the phase 3 BASIS trial, inhibitor patients on once-weekly marstacimab (150 mg) meeting prespecified criteria escalated to 300 mg. Treated ABR, compliance, PK/PD, and safety were descriptively assessed. • **Results:** Four inhibitor patients dose-escalated. Mean treated ABR decreased from 5.89 to 3.71 post-escalation with high compliance. No thrombotic events, serious AEs, or safety signals emerged. • **Conclusions:** Marstacimab dose escalation improved bleeding control without new safety concerns, supporting individualized non-factor prophylaxis in inhibitor patients needing enhanced efficacy.
All days	Immunogenicity of Marstacimab in Participants With Hemophilia A or Hemophilia B With Inhibitors: Results From the BASIS Trial	A. Coppola	• **Introduction:** Immunogenicity can affect efficacy and safety of non-factor therapies. This analysis evaluated anti-drug antibody (ADA) development to marstacimab in hemophilia A/B patients with inhibitors in BASIS. • **Methodology:** In the phase 3 BASIS study, inhibitor patients received weekly subcutaneous marstacimab for 12 months. ADAs and neutralizing antibodies (NAbs) were serially assessed alongside safety, PK/PD, and bleeding outcomes. • **Results:** ADAs developed in 19.6%, were low-titer and transient in most; NAbs occurred in 2 patients and resolved. Bleeding rates, safety, and PK/PD were comparable between ADA-positive and ADA-negative patients. • **Conclusions:** Marstacimab shows low, transient immunogenicity in inhibitor patients without clinically meaningful impact on efficacy or safety, supporting durable non-factor prophylaxis.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (1/9)

Date	Title	Author	Summary
04 Feb 2026	Clinical Characteristics of Glanzmann Thrombasthenia—First Results From the Glanzmann Thrombasthenia Natural History Study	R. Schutgens	• **Introduction:** Glanzmann Thrombasthenia (GT) is a rare, severe inherited platelet function disorder marked by lifelong mucocutaneous bleeding. Standardized bleeding assessment remains essential to characterize burden and guide management. • **Methodology:** An international multicenter observational cohort captured clinical characteristics and patient-reported bleeding using lifetime ISTH-BAT and monthly self-ITP-BAT, with longitudinal and correlational analyses. • **Results:** Among 22 patients (mean age 52), bleeding burden was high (mean ISTH-BAT 15.7). Cutaneous bleeding, epistaxis, and wound bleeding predominated. Self-ITP-BAT correlated with ISTH-BAT. Forty-one percent required recent medical intervention; 82% had lifetime platelet transfusions. • **Conclusions:** GT imposes substantial, persistent bleeding morbidity requiring frequent factor-independent supportive therapy. Correlated lifetime and recent bleeding scores support routine patient-reported tools to inform individualized treatment and real-world care strategies.
06 Feb 2026	Recombinant von Willebrand Factor Prophylaxis for Severe von Willebrand Disease: Final Results Focusing on Adults Receiving Once Weekly Prophylaxis in a Phase 3b Continuation Study	S. Susen	• **Introduction:** Prophylactic recombinant von Willebrand factor (rVWF) is standard for severe VWD, but reduced dosing frequency may lessen treatment burden while maintaining bleed control. • **Methodology:** Phase 3b open-label continuation study of adults with severe VWD receiving individualized rVWF prophylaxis; post hoc analysis focused on once-weekly dosing. Primary endpoint was treated spontaneous ABR over 12 months. • **Results:** Five once-weekly patients completed ~3 years' therapy with median sABR 1.0, comparable to overall prophylaxis. Non-rollover patients showed marked reductions versus prior on-demand therapy. Mean dose was ~64 IU/kg/week. Only two required escalation. No thromboembolic events, inhibitors, or rVWF-related adverse events occurred. • **Conclusions:** Once-weekly rVWF prophylaxis offers durable efficacy and excellent safety in selected adults with severe VWD, supporting individualized prophylaxis strategies to reduce infusion burden without compromising bleed protection.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (2/9)

Date	Title	Author	Summary
All days	Psychosocial Factors Worsened by Bleeding in VWD: The VWD360 Study	K. Khair	• **Introduction:** VWD is traditionally viewed as low-burden, yet patient-reported bleeding and its psychosocial impact remain underrecognized, potentially underestimating true disease severity • **Methodology:** VWD360 was a large mixed-methods natural history study using validated QoL, mental health, and self-efficacy instruments alongside modified ISTH self-BAT and weekly bleed reporting in adults. • **Results:** Among 611 adults, mean bleeding was 1.40 episodes/week across all VWD subtypes. Higher bleed frequency strongly correlated with worse QoL, increased anxiety and depression, reduced self-efficacy, and lower self-esteem (all $p<0.001$). • **Conclusions:** VWD carries a substantial, multidimensional burden independent of subtype. Findings support earlier diagnosis, improved clinician awareness, and broader access to effective prophylactic therapies to reduce bleeding and psychosocial harm.
All days	Clinical Outcomes of 1:1 pdVWF/FVIII Prophylaxis in Children Under 6 Years With Severe VWD: Results From the WIL-33 Study	A. Jain	• **Introduction:** Severe pediatric VWD causes early, frequent bleeding. Evidence for VWF prophylaxis in children <6 years is limited, creating uncertainty around efficacy and safety in this high-risk group. • **Methodology:** WIL-33 was a global phase 3, open-label study evaluating 1:1 pdVWF/FVIII prophylaxis (30–50 IU/kg, 2–3×/week) over 12 months. Primary endpoint was total annualized bleeding rate; PK and safety were assessed. • **Results:** Twelve children (median age 2 years) showed low TABR (median 3.0), predominantly minor bleeds, effectively treated with 1–2 infusions. PK was stable without accumulation; no thrombotic or treatment-related serious AEs occurred. • **Conclusions:** Early 1:1 pdVWF/FVIII prophylaxis is effective, safe, and clinically meaningful in young children with severe VWD, supporting early preventive treatment to minimize bleeding morbidity.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (3/9)

Date	Title	Author	Summary
All days	Identifying the Challenges Posed by Living With VWD: The VWD360 Study	K. Khair	• **Introduction:** Although VWD is the most prevalent inherited bleeding disorder, its lived experience and real-world burden remain underexplored, contributing to underrecognition in clinical practice. • **Methodology:** A qualitative substudy of the VWD360 natural history program conducted in-depth online interviews with 30 adults across VWD subtypes, analyzed using a structured framework approach. • **Results:** Patients described persistent adaptation to unpredictable bleeding, treatment navigation challenges, self-education, gender-specific reproductive issues, and frequent need to educate healthcare professionals, reflecting substantial psychosocial and clinical burden. • **Conclusions:** VWD is not a low-burden condition. Delayed diagnosis, inconsistent care, and limited awareness necessitate earlier identification, standardized management, and improved access to effective therapies to reduce lifelong impact.
All days	Validation of a New Pharmacokinetic Population Model for Perioperative Dosing of a Highly Purified Plasma-derived von Willebrand Factor (pdVWF): The WIL-PKPOP-CHIR Study	M. Daniel	• **Introduction:** Marked interindividual PK variability of VWF/FVIII complicates perioperative management in VWD, risking under- or over-dosing during surgery. • **Methodology:** A prospective, multicentre real-world study will externally validate a population PK model for pdVWF using observed vs predicted VWF:Ag, VWF:Act, and FVIII:C levels, applying adaptive RMSE-based validation. • **Results:** ~50 surgical VWD patients will be enrolled. Model performance will be assessed sequentially, targeting RMSE ≤0.30 initially and <0.20 for final validation, with re-estimation if needed. • **Conclusions:** Validated PK-guided perioperative dosing could enable individualized therapy, reduce bleeding risk, avoid FVIII excess, and shorten hospitalization in VWD surgery care.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (4/9)

Date	Title	Author	Summary
All days	Disease Burden in Pediatric Patients With von Willebrand Disease (VWD): Post Hoc Analysis of a European Cross-Sectional Study	G. Castaman	• **Introduction:** Pediatric VWD is common, yet real-world data on disease burden and treatment effectiveness in children remain limited, particularly across prophylaxis strategies. • **Methodology:** Post hoc analysis of CVESS (Europe, 2018) evaluated 266 children (1–17 years) with congenital VWD, assessing bleeding outcomes, joint morbidity, pain, and treatment patterns over 12 months. • **Results:** Type 1 predominated (66%). Intermittent prophylaxis—mainly in Type 2—was associated with higher treated bleeds, more joints with limited ROM, target joints, and chronic damage versus continuous prophylaxis, despite lower baseline severity. • **Conclusions:** Intermittent prophylaxis in pediatric VWD is linked to poorer outcomes. Greater use of continuous prophylaxis, especially in Type 2 disease, may better prevent bleeding and joint morbidity.
All days	Management of the Preconception Phase, Pregnancy, and Childbirth in Women With Glanzmann Thrombasthenia: Findings From the European Association of Haemophila and Allied Disorders Survey	K. V. Galen	• **Introduction:** Glanzmann thrombasthenia (GT) confers high bleeding risk during pregnancy and childbirth, yet standardized guidance for reproductive management is lacking. • **Methodology:** An EAHAD-led international survey (30 items) captured current clinical practices for preconception, pregnancy, and delivery in women with GT across Europe. • **Results:** Thirty-seven clinicians from 18 countries responded. Most reported fertility challenges, endorsed multidisciplinary preconception counseling, and screened for platelet alloimmunisation, though timing varied. Delivery mode was largely obstetric-led. PPH prevention strategies were heterogeneous, commonly TXA with rFVIIa ± platelet transfusion. Views on thromboprophylaxis diverged. • **Conclusions:** Substantial practice variability highlights unmet need for consensus, evidence-based guidelines to optimize maternal–fetal outcomes and balance bleeding and thrombosis risks in GT.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (5/9)

Date	Title	Author	Summary
All days	Natural History of Anti-platelet Antibody Formation in Patients With Glanzmann Thrombasthenia: A French Multicenter Prospective Study	M. Fiore	• **Introduction:** GT patients frequently require platelet transfusions, predisposing to alloimmunization against αIIbβ3 and HLA, which can compromise hemostatic efficacy and complicate bleeding management. • **Methodology:** This multicenter prospective French cohort followed 55 GT patients (2018–2025). Anti-αIIbβ3 (MAIPA) and anti-HLA antibodies were longitudinally assessed, with functional assays evaluating fibrinogen binding, activation (CD62P), and complement deposition. • **Results:** Anti-αIIbβ3 antibodies occurred in 36%, predominantly in type I GT with null ITGA2B/ITGB3 variants. Antibodies emerged rapidly post-transfusion and were usually transient. Anti-HLA antibodies persisted longer. Anti-αIIbβ3 impaired fibrinogen binding without platelet activation or complement injury. • **Conclusions:** Immunization is frequent and genotype-driven in GT, supporting proactive antibody monitoring and judicious use of non-platelet therapies to preserve hemostatic options.
All days	Characterizing FXI Deficiency by Proteomic Profiling: Insights From the RBiN Study	B. Haisma	• **Introduction:** FXI deficiency shows marked genotype–phenotype discordance, limiting prediction of bleeding risk from FXI activity alone. Proteomic characterization may clarify functional consequences of F11 variants. • **Methodology:** Twenty-eight FXI-deficient patients and 20 controls underwent clinical phenotyping, F11 sequencing, and LC-MS/MS–based global, targeted, and immunocapture proteomic analyses to define FXI abundance and proteoforms. • **Results:**FXI levels correlated with activity (R=0.79), but some patients had normal protein with low activity, indicating qualitative defects. Proteoforms matched missense genotypes but did not correlate with bleeding severity. • **Conclusions:** Integrated proteomics reveals functional heterogeneity in FXI deficiency and aids variant interpretation, though bleeding risk remains multifactorial beyond FXI quantity or proteoform profile.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (6/9)

Date	Title	Author	Summary
All days	Living With Factor VII Deficiency: Data From the FVIID 360 Study	K. Khair	• **Introduction:** Factor VII deficiency (FVIID) causes unpredictable, potentially life-threatening bleeding with weak correlation to FVII levels, and current therapies often fail to adequately control disease burden. • **Methodology:** FVIID360 was a mixed-methods natural history study combining validated quantitative surveys (ISTH-BAT, PHQ-8, GAD-7) in 100 participants with qualitative interviews. • **Results:** Bleeding burden was high (mean 1.25 bleeds/week), especially in women, with frequent diagnostic delays and elevated bleeding scores. Despite prophylaxis, breakthrough bleeding persisted (~1.18/week), markedly impairing physical, social, and emotional quality of life. • **Conclusions:** FVIID imposes substantial unmet clinical and psychosocial burden, highlighting the need for more effective, durable prophylactic therapies and improved clinical recognition.
All days	Establishment of the First Long-term Registry and Cohort Study of Factor XIII Deficiency in Sistan and Baluchistan: A Global Hotspot for FXIII Mutations and Clinical Burden	M. Naderi	• **Introduction:** Factor XIII deficiency is ultra-rare globally but highly prevalent in southeastern Iran, with severe bleeding, obstetric complications, and mortality; longitudinal real-world data have been lacking. • **Methodology:** A prospective regional registry enrolled genetically or biochemically confirmed FXIII-deficient patients, capturing demographics, genotype, bleeding phenotype, prophylaxis, pregnancy outcomes, QoL, and long-term complications. • **Results:** Among 670 patients, consanguinity was high (92%). Early-life bleeding was common; ICH affected 34%, miscarriage 41%. Recurrent and novel F13A1 mutations were identified. Continuous prophylaxis reduced bleeding from 6.8 to 0.4 events/year. • **Conclusions:** This high-burden population demonstrates the life-saving impact of early diagnosis and sustained FXIII prophylaxis, supporting population screening and genotype-informed personalized care.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (7/9)

Date	Title	Author	Summary
All days	A Novel Deep Intronic Splicing Mutation in F5 Gene Resulting in a Mild Factor V Deficiency	O. Pshenichnikova	• **Introduction:** Congenital factor V deficiency is ultra-rare and genetically heterogeneous; deep intronic variants remain underrecognized causes of mild bleeding phenotypes. • **Methodology:** Comprehensive genomic work-up included Sanger sequencing, MLPA, whole-genome sequencing, and functional RNA/cDNA splicing analysis in a symptomatic adult patient. • **Results:** A novel heterozygous deep intronic F5 variant (c.1119-1103A>G) created an aberrant splice site, confirmed to cause exon 8 skipping and frameshift. FV activity remained mildly reduced (~44%), explaining lifelong mucocutaneous bleeding. • **Conclusions:** This study highlights deep intronic splicing defects as clinically relevant in FV deficiency and supports advanced genomic and RNA analyses to resolve unexplained mild bleeding disorders.
All days	Landscape of Inherited Platelet Disorders and Treatment Outcomes: A Single-Center Study	M. Usman	• **Introduction:** Inherited platelet disorders (IPDs) are rare but likely overrepresented in consanguineous populations, with significant bleeding morbidity and limited curative options beyond supportive care. • **Methodology:** A single-center cross-sectional analysis (2018–2024) evaluated 87 IPD patients in Pakistan, assessing clinical features, treatments, and outcomes, including fully HLA-matched allogeneic HSCT. • **Results:** Glanzmann thrombasthenia (44.8%) and Bernard–Soulier syndrome (41.4%) predominated, presenting early (median age 2 years) with bruising and epistaxis. Supportive therapies were widely used. Seventeen patients underwent HSCT, achieving 82.4% OS/DFS with no relapse. • **Conclusions:** IPDs impose substantial early-life bleeding burden. HSCT offers a viable curative option for severe disease in resource-limited, high-consanguinity settings when appropriately selected.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (8/9)

Date	Title	Author	Summary
All days	Rare Bleeding Disorders: A 10-Year Retrospective Study in Our Hospital	J. Cabral	• **Introduction:** Rare bleeding disorders (RBDs) are genetically heterogeneous coagulation factor deficiencies with highly variable bleeding phenotypes and poor correlation between factor levels and clinical severity, complicating diagnosis and management. • **Methodology:** A 10-year retrospective observational study assessed demographics, factor deficiencies, activity levels, diagnostic triggers, and clinic–laboratory correlations in patients followed at a single center. • **Results:**Eighteen patients were identified, most commonly with factor VII and XI deficiencies. Median diagnostic age was 28 years. Many had factor levels >30% and minimal bleeding; most diagnoses followed abnormal preoperative coagulation tests rather than symptoms. • **Conclusions:** RBDs are frequently underrecognized and clinically silent. Preoperative detection enables proactive hemostatic planning, underscoring the importance of vigilance despite weak factor–bleeding correlations.
All days	Caregiver Challenges in von Willebrand Disease: Insights From the PIVOT-vWD UK Study	S. Brighton	• **Introduction:** Caregivers of children with von Willebrand disease (VWD) shoulder substantial, underrecognized clinical and psychosocial burdens that may influence disease management and family well-being. • **Methodology:** Using PIVOT-vWD UK survey data, caregivers ranked caregiving challenges via a preference-based exercise, with attributes weighted by overall importance scores • **Results:** Among 20 caregivers (mostly mothers), the greatest challenges were balancing caregiving with other responsibilities (18%), managing bleeding episodes (17%), emotional/mental strain (16%), and administering treatments (16%), reflecting combined practical and psychological burden. • **Conclusions:** Caregiving in pediatric VWD imposes significant multidimensional strain. Integrating caregiver support into clinical care and treatment development is essential to optimize real-world outcomes.

Notable Presentations At EAHAD 2026
Von Willebrand Disease and Rare Coagulation Disorders (9/9)

Date	Title	Author	Summary
All days	Investigating the Effect of Population Pharmacokinetic-Guided pdVWF/FVIII (1:1) Prophylaxis for Severe VWD in a Real-World Setting: The PopPK-WILPROPHY Study	R. F. Sidonio, Jr	• **Introduction:** Severe VWD requires long-term VWF-containing prophylaxis, yet fixed dosing may inadequately address interindividual PK variability, risking bleeding or overtreatment. • **Methodology:** PopPK-WILPROPHY is a prospective, multicentre, single-arm study enrolling 70 severe VWD patients. After 6 months of standard pdVWF/FVIII 1:1 prophylaxis, PopPK-derived individualized dosing will guide a further 12 months. Primary endpoint is ABR comparison. • **Results:** Study initiation is planned for Q1 2026 across ~20 global centres, evaluating bleeding control, QoL, factor consumption, surgical prophylaxis efficacy, and safety. • **Conclusions:** PopPK-guided prophylaxis may optimize efficacy, reduce treatment burden, and advance personalized VWD management.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (1/10)

Date	Title	Author	Summary
03 Feb 2026	How to Improve Adherence to Visits, Protocols, and Procedures Required in Clinical Trials? Literature Review Applied to Haemophilia	S. García Barcenilla	• **Introduction:** Protocol non-compliance in haemophilia clinical trials undermines data quality and operational efficiency, yet evidence-based strategies to improve visit and procedure adherence are limited. • **Methodology:** A narrative literature review (last 5 years) evaluated adherence and retention strategies in clinical trials, categorizing factors into patient-, protocol-, and center-level domains and mapping them to stakeholder roles using the Vrijens adherence framework. • **Results:** Effective adherence is multi-component across initiation, implementation, and persistence phases. Key drivers included reduced patient burden, protocol flexibility, logistical support, digital tools, and coordinated multidisciplinary team involvement. • **Conclusions:** Trial adherence is system-driven rather than solely patient-dependent. Optimizing trial design, center practices, and digital support can meaningfully improve compliance and retention in haemophilia clinical trials.
All days	European Prospective Case–Control Study Evaluating the Prevalence of Bone Loss in Patients With Hemophilia: PHILEOS Study	B. Tardy	• **Introduction:** Hemophilia is associated with reduced bone mineral density (BMD), driven by bleeding severity, reduced mobility, and comorbidities. The protective impact of prophylaxis timing on bone health remains clinically relevant. • **Methodology:** A multicenter study (23 European centers) assessed BMD in 224 hemophilia A/B patients versus matched controls, stratified by severity and age at prophylaxis initiation, excluding HIV and inhibitors. • **Results:** Severe hemophilia without prophylaxis or with late initiation (>20 years) showed markedly higher osteoporosis risk, especially hemophilia A. Early prophylaxis (<20 years) normalized BMD. HCV infection further increased osteoporosis risk. • **Conclusions:** Early prophylaxis is bone-protective in hemophilia. Delayed or absent prophylaxis confers significant osteoporosis risk, supporting routine BMD monitoring and early preventive strategies.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (2/10)

Date	Title	Author	Summary
All days	Assessment of Joint Health and Patient-Physician Alignment on Joint Damage Reports in a Cohort of People With Haemophilia A: Real-World Insights From the CHESS III Study	N. Kragh	• **Introduction:** Joint deterioration remains a major long-term burden in hemophilia A, and misalignment between patient- and clinician-reported joint problems may hinder optimal management. • **Methodology:** Cross-sectional analysis of CHESS III data evaluated adult non-inhibitor PwHA with paired patient and HCP reports of problem joints (PJs), defined by chronic pain and/or limited motion. • **Results:** Among 133 PwHA (mean age 42), PwHA reported more PJs than HCPs (1.2 vs 0.9). Over 25%—notably severe HA—reported additional PJs. Chronic pain predominated and was underrecognized by HCPs. • **Conclusions:** Patient-reported outcomes reveal clinically relevant joint morbidity beyond clinician assessment. Integrating patient perspectives is essential to guide treatment and preserve joint function.
All days	Study of the Pediatric Hemophilia Activities List (PEDHAL) in Children With Hemophilia a in a Developing Country	M. Elshinawy	• **Introduction:** Functional impairment remains common in pediatric hemophilia A despite improved access to factor therapy, underscoring the need to identify determinants of daily activity limitations. • **Methodology:** A cross-sectional study (July 2024–June 2025) assessed children aged 4–18 years using the validated 22-item PedHAL short, with multivariable analysis of clinical and sociodemographic predictors. • **Results:** PedHAL scores were significantly reduced versus controls, particularly in sports and lower-limb domains. Adherence to prophylaxis was the strongest predictor of better function, alongside absence of inhibitors, no target joints, and higher socioeconomic status. • **Conclusions:** Optimal prophylaxis adherence is central to preserving function in pediatric hemophilia A, supporting strengthened care delivery, registry development, and equitable access strategies.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (3/10)

Date	Title	Author	Summary
All days	Factor Levels and Carrier Status Distribution Among Female Relatives of People With Hemophilia: A Cross-Sectional Study From a Hemophilia Treatment Center in Brazil (HEMOES)	A. L. Prezotti	• **Introduction:** Hemophilia A/B carriers may exhibit reduced FVIII/FIX levels and clinically relevant bleeding, yet are frequently underdiagnosed and undertreated. • **Methodology:** A cross-sectional registry analysis at a Brazilian hemophilia center evaluated female relatives of HA/HB patients, classifying obligate vs potential carriers and measuring FVIII/FIX activity via one-stage assays. • **Results:** Among 415 carriers, median FVIII/FIX levels were often within normal ranges; however, substantial interindividual variability was observed, with some carriers showing reduced factor levels and bleeding symptoms irrespective of carrier category. • **Conclusions:** Bleeding risk in carriers cannot be excluded by normal factor levels alone. Systematic identification, regular monitoring, and integration of genetic testing are essential to optimize counseling and clinical management.
All days	Evaluation of the Effect of the ADAPTOADULT (ATA) Android/iOS Application on the Transition Process in Adolescents and Young Adults With Hemophilia: A Multicenter Randomized Controlled Trial	A. Unuvar	• **Introduction:** Transition to adult care is a high-risk period in hemophilia, with potential loss of adherence, knowledge, and self-management capacity. • **Methodology:** A randomized pilot study evaluated AdapToAdult, a hemophilia-specific mobile app, versus standard care over 3 months in inhibitor-negative adolescents and young adults, assessing knowledge, self-efficacy, and quality of life. • **Results:** App users showed greater improvements in disease knowledge and self-efficacy than controls. Knowledge correlated positively with self-efficacy and inversely with QoL scores. User satisfaction was high, particularly for educational content. • **Conclusions:** Mobile health tools can meaningfully support transition readiness by improving knowledge and self-efficacy, complementing structured hemophilia transition programs.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (4/10)

Date	Title	Author	Summary
All days	Early Indicators of Cardiovascular Risk in Paediatric Patients With Haemophilia: Results From 24-hour Blood Pressure Monitoring	N. Pasikowska	• **Introduction:** Hypertension is increasingly recognized in hemophilia, yet pediatric data are scarce. Early vascular or renal dysfunction may contribute to long-term cardiovascular risk. • **Methodology:** Seventeen boys with hemophilia A/B (ages 5–18) underwent 24-hour ambulatory blood pressure monitoring, biochemical assessment, and BMI evaluation to identify early hypertensive patterns and associated factors. • **Results:** Prehypertension was detected in 29%, predominantly in severe HA on prophylaxis. Non-dipping patterns, proteinuria, elevated uric acid, and overweight were common. One patient showed isolated systolic hypertension. • **Conclusions:** Subclinical blood pressure abnormalities are frequent in pediatric hemophilia, indicating early cardiovascular and renal risk. Routine BP monitoring and preventive strategies should be integrated into long-term care.
All days	Identifying Predictors of Disease Burden in Women With Bleeding Disorders: Analyses From the Cinderella Study	K. Khair	• **Introduction:** Women with inherited bleeding disorders and hemophilia carriers experience substantial but poorly characterized medical and psychosocial burden, often remaining underdiagnosed and underserved. • **Methodology:** The Cinderella mixed-methods study used an online survey with elastic net regression to identify independent predictors of disease burden, comparing diagnosed women versus hemophilia carriers. • **Results:** Missed work/school due to bleeding affected 56% of diagnosed women versus 27% of carriers. In diagnosed patients, burden correlated with markers of complex disease and treatment use; in carriers, it was driven by activity limitations and emotional distress. • **Conclusions:** Disease burden drivers differ between diagnosed women and carriers, underscoring the need for tailored clinical assessment, improved recognition, and implementation of EAHAD Principles of Care for women and girls.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (5/10)

Date	Title	Author	Summary
All days	Barriers and Challenges for Women With Bleeding Disorders: Preliminary Results of a Patient Survey Conducted by the European Haemophilia Consortium	E. Ferri Grazzi	• **Introduction:** Women with bleeding disorders (WBD) remain under-recognized, with delayed diagnosis and significant reproductive and quality-of-life impacts despite diagnostic advances. • **Methodology:** A 2025 EHC multinational survey (n=356; 28 countries) assessed symptoms, diagnosis timing, treatment access, and reproductive impact across WBD subtypes. • **Results:** HMB (69%) and anemia/fatigue (53%) were common, highest in platelet function disorders. Nearly half were diagnosed after age 18; family history did not ensure earlier diagnosis. HTC registration increased treatment likelihood 3.7-fold. Reproductive decisions were impacted in 69% (severe in 44%), especially PFDs. • **Conclusions:** Substantial unmet needs persist for WBD. Earlier recognition, HTC linkage, and targeted education are critical to reduce diagnostic delay and reproductive burden.
All days	Navigating Acquired Hemophilia A: A Six-Year Regional Study From Italy's Liguria	A. C. Molinari	• **Introduction:** Acquired hemophilia A (AHA) is rare, life-threatening, and frequently underdiagnosed. Regional data are critical to understand true incidence, optimize bleeding control, and standardize immunosuppression. • **Methodology:** Retrospective regional analysis (Liguria, 2018–2024) of AHA cases (FVIII <50%, inhibitor >0.6 BU), assessing epidemiology, diagnosis timing, hemostatic and immunosuppressive treatments, and outcomes. • **Results:** Twenty-two cases were identified (estimated incidence ≥3.6/million). Median diagnosis time was 4 days. All achieved remission; relapse occurred in 31%. Hemostasis required in 91%; rpFVIII (susoctocog alfa) was highly effective, often after BPA failure, with favorable safety. • **Conclusions:** AHA remains underrecognized with probable missed cases. Early diagnosis, standardized immunosuppression, and rpFVIII-based bleeding control are key to reducing mortality, particularly in elderly patients.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (6/10)

Date	Title	Author	Summary
All days	Empowering Nursing Leadership in Haemophilia Research: Consolidation of the Clinical Trial Nurse Role	M. Jover Gonzalez	• **Introduction:** Rapid innovation in haemophilia therapies increases trial complexity, creating a need for specialised clinical trial nurses (CTNs) to ensure quality, safety, and continuity of care. • **Methodology:** Vall d'Hebron University Hospital implemented a CTN role within its haemophilia unit. Role development was assessed through practice-based analysis, SWOT framework, and patient feedback using the validated SPFQ. • **Results:** Across 11 interventional and 11 observational studies, CTNs improved coordination, treatment administration, and follow-up. SWOT highlighted high expertise and research quality as strengths, with resource constraints and trial complexity as challenges. Patient satisfaction with trial information was high. • **Conclusions:** The CTN role strengthens haemophilia research delivery, patient experience, and multidisciplinary collaboration, supporting a scalable European model for advanced nursing leadership.
All days	Balance Improvement With the Use of Virtual Reality Training in Hydrotherapy:A Pilot Study	R. Tiktinsky	• **Introduction:** Older adults with hemophilia develop balance and gait impairments from chronic hemarthropathy, increasing fall risk. Virtual reality (VR)–assisted rehabilitation may address neuromuscular deficits. • **Methodology:** Two adults with hemophilia A (ages 50–54) completed 12 VR-based hydrotherapy sessions. Balance scales, muscle strength, range of motion, HJHS, and spatiotemporal gait parameters (Zebris treadmill) were assessed pre/post. • **Results:** Gait parameters improved by ~25%, including weight bearing and step metrics. Balance, strength, and range of motion showed non-significant improvements. • **Conclusions:** VR hydrotherapy is feasible and may meaningfully improve gait in older hemophilia patients, warranting larger studies to confirm functional benefits.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (7/10)

Date	Title	Author	Summary
All days	Barriers and Attitudes Toward Physical Activity and Healthy Lifestyle Among Adults With Haemophilia in Ireland: A Qualitative Study	M. Jacob	• **Introduction:** Adults with hemophilia face elevated cardiometabolic risk driven by low physical activity, obesity, pain, and arthropathy, yet determinants of lifestyle engagement remain poorly defined. • **Methodology:** A qualitative study used semi-structured interviews with adults with hemophilia A/B in Ireland, analyzed via reflexive thematic analysis to explore attitudes and barriers to physical activity and healthy lifestyle behaviors. • **Results:** Key emerging barriers included fear of injury, reduced motivation, chronic joint pain, arthropathy, bleed recovery, self-consciousness, and variable access to specialist advice. • **Conclusions:** Physical activity engagement in hemophilia is shaped by personal, physical, and social factors. Findings support co-designed, personalized interventions to mitigate cardiometabolic risk in ageing patients.
All days	Effectiveness of Post Physiotherapy Gym Protocol and Sports Activity to Enhancing Fitness, Psychosocial Well-being and Self Confidence in Young Adult Patients With Haemophilia: A Pilot Study	G. S. Sharma	• **Introduction:** Haemophilic arthropathy limits physical activity and psychosocial well-being despite modern prophylaxis. Evidence for structured exercise programs tailored to haemophilia remains limited. • **Methodology:** A 6-month feasibility study enrolled 10 inhibitor-negative adults with hemophilia A/B on EHL prophylaxis. A supervised gym and sports program was evaluated using validated physical activity, QoL, self-efficacy, self-esteem, and joint health tools. • **Results:** Participants showed marked improvements in self-efficacy, self-esteem, EQ-5D scores, perceived fitness, and confidence, with low bleed rates and preserved joint health (HJHS ≤2). • **Conclusions:** Supervised gym exercise with adequate prophylaxis is safe, feasible, and improves psychological well-being and activity levels in young adults with hemophilia.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (8/10)

Date	Title	Author	Summary
All days	Clinical, Psychosocial, Quality of Life Outcomes and Patient-Physician Discrepancies in Joint Damage Reporting in a Cohort of People With Haemophilia A: Real-World Insights From the CHESS III Study	B. O'Mahony	• **Introduction:** Joint damage in hemophilia A may be under-recognized when patient-reported symptoms diverge from clinician assessments, potentially worsening outcomes and overall disease burden. • **Methodology:** CHESS III cross-sectional data were analyzed in adult non-inhibitor PwHA, comparing those with concordant vs discordant patient–HCP reports of problem joints, alongside bleeding, HRQoL, and psychosocial metrics. • **Results:** PwHA reporting more problem joints than HCPs had significantly higher ABR, worse EQ-5D-5L scores, and increased anxiety and depression. Discordance was more frequent in severe HA. • **Conclusions:** Under-detection of joint morbidity is associated with higher clinical and psychosocial burden. Integrating systematic patient-reported joint assessments may improve hemophilia outcomes.
All days	Emotional Distress and Coping Patterns in Adolescents With Haemophilia: A Preliminary Study	G. Goula	• **Introduction:** Adolescents with haemophilia face physical, emotional, and social challenges; adaptive coping is crucial for psychological well-being during autonomy development. • **Methodology:** A cross-sectional study of 14 Greek adolescents (12–17 years; HA/HB, all severities) assessed distress (DASS-21) and coping (SAKA), with reliability testing and Pearson correlations. • **Results:** Emotional distress was low overall. Adaptive, problem-focused coping predominated (family support, problem solving, cognitive reappraisal). No significant correlations emerged between distress and coping, while adaptive strategies were strongly interrelated. • **Conclusions:** Comprehensive care and prophylaxis are associated with low distress and effective coping in adolescents with haemophilia. Strengthening family and cognitive coping may further support resilience and transition to adulthood.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (9/10)

Date	Title	Author	Summary
All days	Comparative Evaluation of Psychosocial and Functional Outcomes in Young Individuals With Hemophilia Treated With on-Demand Factor VIII Therapy or Emicizumab Prophylaxis: A One-Year Cohort Study	A. Thomas	• **Introduction:** Hemophilia significantly impairs physical function and psychosocial well-being in children and young adults. Non-factor prophylaxis may offer broader benefits than on-demand factor therapy. • **Methodology:** A 12-month observational cohort compared emicizumab prophylaxis versus on-demand FVIII in 20 patients aged 6–25 years, assessing EQ-5D-5L domains, EQ-VAS, ABR, and qualitative psychosocial outcomes. • **Results:** Emicizumab prophylaxis led to near-normal EQ-5D-5L scores, minimal bleeding, higher EQ-VAS, and improved independence and social participation. On-demand therapy showed persistent bleeding, anxiety, and functional limitations. • **Conclusions:** Emicizumab provides superior functional and psychosocial outcomes, supporting routine prophylaxis with integrated psychosocial care in young hemophilia patients.
All days	Adherence to Prophylactic Treatment With Coagulation Factor Concentrates in People With Hemophilia: A Cross-Sectional Study in a Reference Center in Brazil	D. M. Do Carmo Rocha	• **Introduction:** Adherence to factor prophylaxis is critical for long-term joint protection in hemophilia, yet real-world adherence data from Brazil remain limited. • **Methodology:** A cross-sectional analysis at a Brazilian hemophilia center evaluated adherence by comparing prescribed versus dispensed FVIII/FIX units in 150 PwHA/B on prophylaxis, alongside clinical outcomes. • **Results:** Despite low reported joint bleeding and arthropathy, adherence was poor: 57% non-adherent, 11% suboptimal. A substantial gap existed between prescribed and dispensed factor, indicating underuse. Adherence did not correlate with short-term bleeding. • **Conclusions:** Suboptimal adherence is prevalent and may drive silent joint damage despite apparent bleed control. Strengthened monitoring, patient engagement, and multidisciplinary support are essential to realize prophylaxis benefits.

Notable Presentations At EAHAD 2026
Clinical Management, Adherence, QoL, Psychosocial, and Health Systems (10/10)

Date	Title	Author	Summary
All days	Cerebral Hemorrhage in Patients With Hemophilia: Results of a 10-Year Prospective Study on Clinical Features and Risk Factors Prospective From EMO.REC Registry	E. Zanon	• **Introduction:** Intracranial hemorrhage (ICH) is a life-threatening complication in hemophilia with limited prospective data on long-term outcomes and risk modifiers. • **Methodology:** A 10-year multicenter Italian cohort (2012–2022) analyzed hemophilia A/B patients with ICH, capturing severity, prophylaxis status, comorbidities, imaging features, treatments, and neurological outcomes. • **Results:** Thirty-two patients were included (81% HA; 66% severe). Prophylaxis significantly reduced ICH incidence. In adults, hypertension—especially in mild hemophilia—was a major risk factor. Trauma was more common in severe disease. Overall survival exceeded 75% at 5 years. • **Conclusions:** Early and sustained prophylaxis and aggressive cardiovascular risk management, particularly hypertension control in adults, are critical to reducing ICH risk and improving long-term outcomes in hemophilia.

Notable Presentations At EAHAD 2026
Emicizumab and Real-World Prophylaxis Optimization (1/5)

Date	Title	Author	Summary
04 Feb 2026	MRI-Based Assessment of Joint Outcomes in Children With Severe Hemophilia A on Emicizumab Prophylaxis: A Prospective Observational Study	L. M. Sherief	• **Introduction:** Children with severe hemophilia A develop recurrent hemarthroses leading to progressive arthropathy. While emicizumab's bleeding control is established, its real-world impact on joint structure remains limited. • **Methodology:** Prospective, multicenter observational study of 39 boys (<18 years) on emicizumab prophylaxis. Clinical (ABR, HJHS) and MRI (IPSG v1.0) assessments of 54 index joints were performed at baseline and 12 months. • **Results:** Mean ABR fell from 50 to 0.46 and HJHS improved from 13 to 8 (both p<0.001). MRI showed marked reductions in effusions, synovial hypertrophy, and haemosiderin, with 88.9% of joints improving and minimal progression. • **Conclusions:** Emicizumab delivered profound bleeding suppression and meaningful structural joint improvement, supporting early non-factor prophylaxis to prevent arthropathy and optimize long-term musculoskeletal outcomes in pediatric hemophilia A.
04 Feb 2026	Long-term Results of Patients With Acquired Haemophilia A in the Era of Emicizumab	M. Erard	• **Introduction:** Acquired hemophilia A (AHA) is driven by FVIII inhibitors, with high bleeding and treatment-related morbidity. Emicizumab enables effective bleed prevention and may allow delayed immunosuppression. • **Methodology:** Retrospective multicenter Belgian analysis of 33 emicizumab-treated AHA patients (2020–2025), assessing bleeding, IST use, inhibitor eradication, thrombosis, and survival • **Results:** Major bleeding occurred in 47.1%, mostly early; no major bleeds followed emicizumab initiation. Recombinant FVIIa was required in 26.5%. Inhibitor eradication succeeded in 76.7%, allowing emicizumab discontinuation after median 151 days. One thrombotic event occurred. No bleed-related deaths were observed. • **Conclusions:** Emicizumab provided strong, safe hemostatic control in AHA, enabling delayed IST, low thrombotic risk, and shorter treatment courses, supporting its role as frontline non-factor therapy.

Notable Presentations At EAHAD 2026
Emicizumab and Real-World Prophylaxis Optimization (2/5)

Date	Title	Author	Summary
All days	Real-World Outcomes of PK/PD-Guided Individualized Emicizumab Dose Reduction in Chinese Pediatric Patients With Hemophilia A: A Prospective Study	Q. Mao	• **Introduction:** Emicizumab provides effective bleed prevention in hemophilia A, but high costs limit access. PK/PD-guided dose reduction may preserve hemostasis while improving affordability, though real-world pediatric data are scarce. • **Methodology:** Prospective single-center study in Chinese children with severe hemophilia A (± inhibitors). After ≥12 months of standard prophylaxis, eligible patients underwent PK/PD-guided tapering targeting FVIII-equivalent 10–15 IU/dL and trough >30 µg/mL, with ≥6 months follow-up. • **Results:** Fifteen patients reduced median monthly dose from 5.4 to 3.2 mg/kg. FVIII-equivalent activity remained therapeutic, with no bleeds, thrombosis, adverse events, or joint deterioration. • **Conclusions:** PK/PD-guided emicizumab dose reduction was safe, effective, and cost-efficient, supporting personalized non-factor prophylaxis, particularly in resource-limited settings.
All days	Evolution of Joint Health and Physical Activity in People With Haemophilia A Without Factor VIII Inhibitors Switching to Emicizumab Prophylaxis: 12-Month Interim Analysis of the BEYOND ABR Study	M. N. Dario Di Minno	• **Introduction:** With reduced bleeding in hemophilia A (HA), treatment goals now emphasize joint health, function, and physical activity beyond ABR. BEYOND ABR evaluates these outcomes after switching from FVIII to emicizumab. • **Methodology:** Phase IV, multicenter, open-label study in 136 adolescents/adults with moderate/severe HA without inhibitors. Joint health (HJHS 2.1), problem joints, physical activity (IPAQ), bleeding, and treatment preference were assessed over 12 months. • **Results:** HJHS improved numerically at 6 and 12 months; ~26% achieved clinically meaningful improvement. Problem joints decreased in ~30%. Zero treated bleeds occurred in ~80%. Physical activity shifted toward higher levels. Most patients preferred emicizumab. • **Conclusions:** Switching to emicizumab maintained excellent bleed control with favorable joint and functional outcomes, supporting holistic benefits of non-factor prophylaxis in routine practice.

Notable Presentations At EAHAD 2026
Emicizumab and Real-World Prophylaxis Optimization (3/5)

Date	Title	Author	Summary
All days	Enhancing Physical Activity in Patients Treated With Emicizumab in Hemophilia A: Results From the HEMO-ACT Cross-Sectional Study	M. Carrasco Expósito	• **Introduction:** Hemophilia A limits physical activity and quality of life due to bleeding risk. Emicizumab offers stable non-factor prophylaxis that may enable safer engagement in daily and recreational activities. • **Methodology:** Cross-sectional, multicenter Spanish study of 56 adolescents/adults with moderate–severe hemophilia A (± inhibitors) on emicizumab ≥6 months, using CATCH recreational activity scores, activity surveys, ABR, target joints, and satisfaction measures. • **Results:** Patients reported moderate baseline activity restriction but low pain. Post-emicizumab, 66% increased activity, 79% felt safer, ABR declined (2.03→0.91), and target joints decreased. Pediatric patients showed higher activity and lower perceived risk. • **Conclusions:** Emicizumab improved perceived safety, activity participation, and joint outcomes, supporting meaningful lifestyle and QoL benefits beyond bleeding control.
All days	Long-term Outcomes of Emicizumab Prophylaxis: Results From a Single-center Cohort Study	A. Colombo	• **Introduction:** Emicizumab enables effective subcutaneous prophylaxis in hemophilia A (HA), though real-world long-term bleeding control and correlation with laboratory activity remain uncertain beyond clinical trials. • **Methodology:** Retrospective single-center cohort of 40 PwHA (± inhibitors) on emicizumab, median follow-up 5.7 years. Clinical bleeding outcomes were correlated with emicizumab plasma levels and FVIII-like activity via chromogenic assays. • **Results:** Thirty percent remained bleed-free; 109 bleeds occurred in others. Median emicizumab level was 49 µg/mL with FVIII-like activity ~20 IU/dL. Bleeding phenotype did not correlate with laboratory measures. Four patients discontinued due to recurrent bleeds. • **Conclusions:** Long-term emicizumab provided sustained but heterogeneous bleeding control, highlighting limitations of laboratory surrogates and the need for individualized clinical monitoring in routine practice.

Notable Presentations At EAHAD 2026
Emicizumab and Real-World Prophylaxis Optimization (4/5)

Date	Title	Author	Summary
All days	Real-world Experience With emicizumab in Acquired Hemophilia A: A Multicenter Study in Southern Spain	F. López Jaime	• **Introduction:** Acquired hemophilia A causes severe bleeding with high treatment-related morbidity. Emicizumab is increasingly used off-label to improve hemostatic control and reduce complications. • **Methodology:** Retrospective multicenter analysis of six AHA patients treated with emicizumab, assessing bleeding outcomes, immunosuppression, transfusions, hospital stay, safety, and costs. • **Results:** No major bleeds occurred after emicizumab initiation. Transfusion needs and hospital stay decreased, all patients achieved remission, and no thrombotic events were observed. Estimated costs fell by ~80% • **Conclusions:** Emicizumab enabled rapid, safe, and cost-efficient hemostatic control in AHA, supporting its role in real-world management.
All days	Impact of Emicizumab Treatment on Patients With Severe Hemophilia A: A Retrospective Descriptive Study in Chile (REDEEM)	A. Ibarra	• **Introduction:** Real-world evidence on emicizumab use in hemophilia A (HA) remains limited in Chile, particularly in patients with inhibitors and severe disease. • **Methodology:** Retrospective descriptive review of five severe HA patients treated with emicizumab at a single Chilean center, assessing bleeding, ABR, joint health (HJHS, HEAD-US), and healthcare utilization pre/post treatment. • **Results:** Total bleeds dropped from 164 to 5, with ABR reductions in all patients and complete bleed elimination in two. Joint health improved across cases. • **Conclusions:** Emicizumab demonstrated strong real-world efficacy in severe HA, including inhibitor patients, with meaningful bleeding and joint outcome improvements.

Notable Presentations At EAHAD 2026
Emicizumab and Real-World Prophylaxis Optimization (5/5)

Date	Title	Author	Summary
All days	Use of the Multidimensional Haemophilia Pain Questionnaire (MHPQ) in Patients With Severe Haemophilia A Treated With Emicizumab: A Pilot Cross-Sectional, Single-Centre Study	G. D'errico	• **Introduction:** Despite excellent bleed prevention with emicizumab, pain remains a significant unmet need in adults with severe hemophilia A (sHA). Disease-specific tools may better capture its burden. • **Methodology:** Single-center, cross-sectional pilot study of 27 adults with sHA on emicizumab ≥6 months, using the Multidimensional Hemophilia Pain Questionnaire. Associations with inhibitors and arthropathy were explored. • **Results:** Lifetime pain was reported by 85% and recent pain by 78%, mainly affecting ankles, knees, and elbows, with major impact on activity and mobility. Arthropathy correlated with higher pain frequency. • **Conclusions:** Pain persists despite emicizumab, highlighting the need for integrated, patient-centered pain management alongside effective prophylaxis.

Notable Presentations At EAHAD 2026
Factor Replacement Therapies, PK, and Long-Term Clinical Outcomes (1/6)

Date	Title	Author	Summary
06 Feb 2026	Efanesoctocog Alfa Prophylaxis for People with Severe Haemophilia A: Third Interim Results from the XTEND-ed Long-Term Extension Study	Pratima Chowdary	• **Introduction:** Efanesoctocog alfa is a first-in-class ultra–long half-life FVIII designed to enable effective once-weekly prophylaxis in severe hemophilia A across age groups. • **Methodology:** European subgroup analysis from the XTEND-ed extension, including 109 rollover patients from XTEND-1 and XTEND-Kids, evaluating inhibitors, ABR, bleed treatment efficacy, and safety. • **Results:** Over long-term follow-up, mean ABRs remained low (~0.6). Most bleeds (92.8%) resolved with a single injection. No inhibitors developed. Safety was favorable, with no treatment-related serious events. • **Conclusions:** Once-weekly efanesoctocog alfa provided durable bleed protection, excellent treatment efficacy, and sustained safety in real-world European practice.
All days	Preliminary Results of the Evaluation of Pharmacokinetic Profiles in Haemophilia A Patients Receiving Efanesoctocog Alfa	M. Pikta	• **Introduction:** Efanesoctocog alfa (Altuvoct) is a next-generation extended half-life FVIII enabling once-weekly prophylaxis. Real-world FVIII exposure data are needed to contextualize its effectiveness versus earlier EHL products • **Methodology:** Single-center Estonian study of 19 hemophilia A patients (15 adults, 4 children) assessing FVIII levels at baseline, 4 h, 72 h, and 7–8 days post-infusion using one-stage assays. • **Results:** Median FVIII was 21% at day 7 (up to 54%); no inhibitors occurred. FVIII levels at 72 h exceeded those seen with Adynovi/Elocta. • **Conclusions:** Efanesoctocog alfa achieved sustained FVIII coverage with fewer injections, supporting effective once-weekly prophylaxis.

Notable Presentations At EAHAD 2026
Factor Replacement Therapies, PK, and Long-Term Clinical Outcomes (2/6)

Date	Title	Author	Summary
All days	Physical Activity and Efficacy in Patients With Severe Haemophilia A Treated With Efanesoctocog Alfa: 12-Month Interim Results From FREEDOM	J. Astermark	• **Introduction:** As bleeding control improves in severe hemophilia A, attention has shifted to functional outcomes such as physical activity. FREEDOM evaluates activity and joint health during efanesoctocog alfa prophylaxis. • **Methodology:** Phase 3b study of 93 patients ≥12 years switching to weekly efanesoctocog alfa, assessing bleeds, IPAQ-reported activity, wearable tracker data, and safety over 12 months. • **Results:** Physical activity levels were high at baseline and remained stable at 12 months. Eighty-four percent had zero treated bleeds; mean ABR was 0.3. No new safety signals emerged. • **Conclusions:** Efanesoctocog alfa sustained excellent bleed protection despite high physical activity, supporting effective once-weekly FVIII prophylaxis.
All days	Damoctocog Alfa Pegol in Children With Severe Haemophilia A Aged 7 to < 12 Years of Age: Final Analysis of the Alfa-PROTECT Extension Study	M. C. Ozelo	• **Introduction:** Extended half-life FVIII aims to sustain bleed prevention with reduced infusion burden in pediatric hemophilia A. Alfa-PROTECT evaluated long-term outcomes of damoctocog alfa pegol in children. • **Methodology:** Phase 3, open-label extension of Alfa-PROTECT in 32 previously treated children (7–<12 years), receiving twice-weekly or every-5-day prophylaxis. Safety, ABR, and FVIII use were assessed over 18 months. • **Results:** Mean ABR was 0.72. Zero treated bleeds occurred in ~72% and zero joint bleeds in ~79% during the last 12 months. No inhibitors, anti-drug antibodies, or safety signals emerged. • **Conclusions:** Damoctocog alfa pegol showed sustained efficacy and favorable long-term safety, supporting durable prophylaxis in pediatric severe hemophilia A.

Notable Presentations At EAHAD 2026
Factor Replacement Therapies, PK, and Long-Term Clinical Outcomes (3/6)

Date	Title	Author	Summary
All days	Real-World Effectiveness and Usage of rFIXFc in Haemophilia B: Final Paediatric Data From the B-MORE Study	A. M. Taylor	• **Introduction:** Real-world pediatric data are essential to optimize long-term prophylaxis in hemophilia B. rFIXFc (eftrenonacog alfa) offers extended half-life replacement with reduced treatment burden. • **Methodology:** B-MORE was a 24-month prospective, non-interventional study evaluating pediatric PwHB (<18 years) on rFIXFc prophylaxis, assessing ABR, AJBR, injection frequency, factor consumption, safety, and satisfaction. • **Results:** Across age groups, ABR and AJBR were consistently low, with median weekly injection frequency of ~1 and moderate factor consumption. No inhibitors or treatment-related serious adverse events occurred. Satisfaction was high. • **Conclusions:** rFIXFc provided durable, safe, and effective bleed prevention with low burden in pediatric hemophilia B real-world practice.
All days	Real-World Safety of Damoctocog Alfa Pegol in Severe and Nonsevere Hemophilia A: Results From the Sixth Interim Analysis of the HEM-POWR Study	M. T. A. Román	• **Introduction:** Real-world safety data are essential to confirm long-term tolerability of extended half-life FVIII products. HEM-POWR evaluates damoctocog alfa pegol in routine clinical practice. • **Methodology:** Phase 4, prospective real-world study of 370 previously treated patients ≥12 years with hemophilia A, assessing treatment-emergent adverse events via diaries and physician records. • **Results:** Over ~2.5 years' median follow-up, 41.1% experienced TEAEs and 14.1% serious TEAEs. Adverse events of special interest were rare (0.8%), including one transient inhibitor. Discontinuations were uncommon. • **Conclusions:** Damoctocog alfa pegol showed an acceptable long-term safety profile in real-world hemophilia A management.

Notable Presentations At EAHAD 2026
Factor Replacement Therapies, PK, and Long-Term Clinical Outcomes (4/6)

Date	Title	Author	Summary
All days	Treatment of Bleeding Episodes With Efanesoctocog Alfa in Adults, Adolescents, and Children With Severe Haemophilia A: Third Interim Analysis of the XTEND-ed Long-Term Extension Study	J. Oldenburg	• **Introduction:** Efanesoctocog alfa is an ultra–long half-life FVIII enabling once-weekly prophylaxis and effective bleed treatment in severe hemophilia A. Long-term real-world bleed treatment data remain important. • **Methodology:** XTEND-ed extension followed 217 previously treated patients continuing weekly 50 IU/kg prophylaxis. Bleeds were treated primarily with single-dose efanesoctocog alfa; ABRs and treatment response were analyzed over ~3 years. • **Results:** Mean ABR remained low (~0.6). Of 341 treated bleeds, 93% resolved with one injection, median total dose ~51 IU/kg. Most responses were rated good/excellent. • **Conclusions:** Long-term efanesoctocog alfa provided durable bleed prevention and highly effective single-dose bleed treatment across bleed types and ages.
All days	Real-World Effectiveness of Damoctocog Alfa Pegol in Severe and Nonsevere Hemophilia A: Results From the HEM-POWR Study Sixth Interim Analysis	M. T. A. Román	• **Introduction:** Real-world effectiveness data are critical to confirm the clinical benefit of extended half-life FVIII products beyond trials. HEM-POWR evaluates damoctocog alfa pegol in routine hemophilia A care. • **Methodology:** Prospective Phase 4 study in previously treated patients ≥12 years with severe or nonsevere hemophilia A. Effectiveness was assessed via ABR using patient diaries and physician records. • **Results:** In 279 evaluable patients, 61.9% reported zero bleeds over 6 months. Mean ABR decreased from 2.7 on prior FVIII to 2.0 on damoctocog alfa pegol, with consistent reductions in spontaneous and joint bleeds. • **Conclusions:** Damoctocog alfa pegol showed sustained real-world effectiveness with reduced bleeding across patient subgroups.

Notable Presentations At EAHAD 2026
Factor Replacement Therapies, PK, and Long-Term Clinical Outcomes (5/6)

Date	Title	Author	Summary
All days	Long-Term Damoctocog Alfa Pegol Prophylaxis and Joint Health in Adults With Haemophilia A: The Observational JOIHA Study	M. N. D. Di Minno	• **Introduction:** Progressive joint damage from recurrent hemarthroses remains a key morbidity in hemophilia A. Ultrasound allows sensitive detection of early arthropathy and monitoring of prophylactic benefit. • **Methodology:** JOIHA is an open-label, multicenter Italian observational study of previously treated adults with FVIII ≤2% on damoctocog alfa pegol, assessing joint outcomes via HEAD-US over 12 months. • **Results:** Among evaluable patients, mean HEAD-US scores improved modestly (−0.4). At 12 months, 82% showed stable or improved joint scores, with reductions in synovitis signals. • **Conclusions:** Interim real-world data suggest damoctocog alfa pegol may preserve or improve joint health, supporting its role in long-term prophylaxis.
All days	Real-world Insights Into rVIII-SingleChain for Haemophilia A: A European Multicentre Study	M. Olivieri	• **Introduction:** Real-world evidence complements trial data for rVIII-SingleChain (AFSTYLA) by characterizing bleeding control, treatment response, and tolerability across ages in routine hemophilia A care. • **Methodology:** Multicenter European non-interventional study of HA patients receiving ≥12 weeks of prophylaxis or on-demand rVIII-SingleChain, assessing ABR, AsBR, bleed treatment efficacy, safety, and QoL. • **Results:** Median ABR was low on prophylaxis (0.57). Most bleeds were mild and rated excellent/good in 85%. About half resolved with one infusion. No inhibitors or treatment-related AEs occurred. • **Conclusions:** rVIII-SingleChain showed effective, safe bleed control in real-world hemophilia A practice.

Notable Presentations At EAHAD 2026
Factor Replacement Therapies, PK, and Long-Term Clinical Outcomes (6/6)

Date	Title	Author	Summary
All days	Real-World Use of Simoctocog Alfa in Severe Haemophilia A: Preliminary Results From the Hungarian Cohort of the Long-Term Non-Interventional Investigation (Nuwiq NIS)	C. Kiss	• **Introduction:** Achieving near-zero bleeding remains the goal in severe hemophilia A. Simoctocog alfa, a human cell line–derived rFVIII, has shown strong efficacy in trials; real-world confirmation is needed. • **Methodology:** Prospective Phase 4 Hungarian multicenter NIS of 42 previously treated males ≥2 years with severe hemophilia A on physician-tailored simoctocog alfa prophylaxis. • **Results:** Median ABR was 0.24; 45% had zero bleeds and 71% zero spontaneous bleeds. Median dose was 52.6 IU/kg every 3.3 days. No inhibitors, thrombotic events, or serious adverse events occurred. • **Conclusions:** Simoctocog alfa delivered excellent real-world bleed protection with a favorable safety profile, supporting effective individualized FVIII prophylaxis.

Notable Presentations At EAHAD 2026
Gene Therapy, RNA, and Novel Molecular Therapeutics (1/3)

Date	Title	Author	Summary
06 Feb 2026	Durable Bleed Protection for all Bleeding Subtypes and Favorable Safety Over 5 Years (end-of-study) in the Phase 3 HOPE-B Trial for Persons With Severe or Moderately Severe Haemophilia B	F. W. G. Leebeek	• **Introduction:** Gene therapy aims to provide durable bleed control and eliminate prophylaxis in hemophilia B. Etranacogene dezaparvovec is the first approved FIX gene therapy with long-term efficacy data. • **Methodology:** HOPE-B followed 54 adult males with severe/moderately severe hemophilia B for 5 years post–gene transfer, assessing FIX-treated ABR, FIX activity, consumption, and safety versus lead-in prophylaxis. • **Results:** Endogenous FIX activity was sustained (~36 IU/dL at year 5). Mean FIX-treated ABR fell from 3.61 to 1.25 and remained <1 annually. FIX use dropped 96%, with 94% off prophylaxis. No late hepatotoxicity or oncogenicity occurred. • **Conclusions:** Etranacogene dezaparvovec delivered durable hemostasis, major prophylaxis independence, and sustained safety through 5 years.
06 Feb 2026	Characterization of ALT Elevations During the 5-Year GENEr8-1 Trial of Valoctocogene Roxaparvovec Gene Transfer for Severe Hemophilia A	R. Klamroth	• **Introduction:** Valoctocogene roxaparvovec is the first approved AAV5-based gene therapy for severe hemophilia A, with ALT elevations representing the most common safety signal requiring long-term characterization. • **Methodology:** GENEr8-1 followed 134 adults for 5 years post–single infusion (6×10^{13} vg/kg), centrally monitoring ALT elevations, severity, duration, and glucocorticoid use. • **Results:** ALT elevations peaked in year 1 (78%), were mostly mild/transient, declined thereafter, and none >5×ULN occurred after year 2. Glucocorticoid use largely ceased beyond year 2. • **Conclusions:** ALT elevations were early, manageable, and diminished over time, supporting a stable long-term hepatic safety profile.

Notable Presentations At EAHAD 2026
Gene Therapy, RNA, and Novel Molecular Therapeutics (2/3)

Date	Title	Author	Summary
All days	Bayesian Re-Analysis of Gene Therapy Trials in Hemophilia A: Giroctocogene vs Valoctocogene	M. R, Lopez4	• **Introduction:** Gene therapy for hemophilia A is evolving, with valoctocogene roxaparvovec (VR) approved and giroctogene fitelparvovec (GF) approaching approval. Direct comparative data are lacking. • **Methodology:** Indirect Bayesian reanalysis of published Phase 3 outcomes using binomial models to compare efficacy, bleeding, and safety, enabling probability-based interpretation. • **Results:** GF showed superior liver safety (99.7% probability of less corticosteroid use). VR demonstrated stronger bleeding protection, including lower spontaneous bleeding and reduced anticoagulation risk. FVIII >5% favored GF modestly but was inconclusive. • **Conclusions:** Bayesian analysis highlights a trade-off: GF offers hepatic safety advantages, while VR provides superior bleeding control, supporting individualized gene therapy selection.
All days	Normalization of Global Coagulation Potential in NXT007-treated Patients With Hemophilia A in a Phase 1/2 Clinical Study	Y. Nakajima	• **Introduction:** NXT007 is a next-generation bispecific antibody engineered to achieve near-normal coagulation in hemophilia A, potentially exceeding emicizumab's hemostatic ceiling. • **Methodology:** Coagulation potential was assessed in 12 PwHA from a phase 1/2 trial switching from FVIII or emicizumab to NXT007, using thromboelastometry and thrombin generation assays, benchmarked against healthy controls. • **Results:** NXT007 increased coagulation in a dose-dependent manner. Higher-dose cohorts achieved coagulation profiles comparable to healthy individuals, irrespective of prior emicizumab exposure. • **Conclusions:** NXT007 demonstrated near-normalization of coagulation in PwHA, supporting its potential as a transformative non-factor therapy.

Notable Presentations At EAHAD 2026
Gene Therapy, RNA, and Novel Molecular Therapeutics (3/3)

Date	Title	Author	Summary
All days	Establishing an Efficient Clinical Trial Ecosystem for Hemophilia Gene Therapy	M. Abu-Riash	• **Introduction:** Gene therapy trials in hemophilia are complex and resource-intensive, requiring robust institutional infrastructure to ensure efficient execution alongside regulatory and data integrity demands. • **Methodology:** A centralized Clinical Research Department (CRD) was established in 2020, implementing standardized SOPs, coordinated stakeholder workflows, and digital tracking, applied across three hemophilia gene therapy trials (2022–2025). • **Results:** The CRD framework streamlined trial start-up, regulatory turnaround, and enrollment timelines while maintaining compliance and data quality across all studies. • **Conclusions:** Dedicated, process-driven clinical trial infrastructure is critical to successfully delivering complex gene therapy programs in hemophilia.

Key Industry Sponsored Sessions Information

EAHAD 2025 Key Industry Sponsored Sessions Information (2/2)

Date	Sponsor	Title
04 Feb 2026	Takeda	BEYOND CONSENSUS: A SCIENTIFIC DEBATE ON PROPHYLAXIS IN VWD
04 Feb 2026	Pfizer	Advancing haemophilia care through lived experiences: exploring anti-TFPI approaches
04 Feb 2026	Roche	Advancing Haemophilia A Care: Exploring Current and Future Horizons of FVIII mimetics
04 Feb 2026	CSL	DURABILITY OF GENE THERAPY IN HAEMOPHILIA: BRIDGING TRIAL RESULTS WITH CLINICAL PRACTICE
04 Feb 2026	Hemab	The Unfinished Revolution
04 Feb 2026	Sobi	MOVING TOWARDS NORMALISED HAEMOSTASIS IN THE REAL WORLD
04 Feb 2026	Novo Nordisk	Refocusing on haemophilia B: Patient-centric approaches to using anti-TFPI

EAHAD 2025 Key Industry Sponsored Sessions Information (2/2)

Date	Sponsor	Title
04 Feb 2026	Kedrion	Raising the Bar in Hereditary Factor X Deficiency (HFCD): Personalized Strategies To Address Patient Needs
04 Feb 2026	Sanofi	Finding Haemostatic Balance Within the Evolving Treatment Landscape
04 Feb 2026	Octapharma	Tailored to the Patient, Driven by Data: Optimising Prophylaxis in VWD
04 Feb 2026	LFB	Haemophilia with inhibitors: Bridging evidence and experience

Noteworthy AI / ML presentations at EAHAD 2026

Themes from key AI / ML presentations at EAHAD 2026 (1/2)

- **AI and machine learning will play an increasingly pivotal role in personalizing treatment and improving diagnostic accuracy for hematology and bleeding disorders. From enhancing gene therapy protocols to analyzing social media data for patient insights, these technologies will drive more precise, efficient, and patient-centered care**

- Check out the key AI / ML themes at EAHAD 2026 below:

- **Generative AI for Hemophilia Management:**

  - Generative AI will be employed to enhance diagnostic accuracy and treatment planning in hematology, with a specific focus on improving gene therapy protocols under clinical oversight

- **AI in Social Listening for Hemophilia:**

  - AI-driven analysis of social media will explore physical activity constraints in young hemophilia patients, revealing psychosocial factors like stigma and the need for integrated clinical and social support

Themes from key AI / ML presentations at EAHAD 2026 (2/2)

- **AI in Physician Perspectives on Hemophilia Treatments:**
  - AI-driven tools will support data collection and analysis of physician preferences for rebalancing agents, focusing on patient-centered care, convenience, and efficacy in the evolving hemophilia treatment landscape
- **AI for Standardization in Fibrinolysis Diagnostics:**
  - AI systems will aid in addressing the lack of standardization in fibrinolysis assays, ensuring more reliable and consistent diagnostic protocols across European laboratories
- **AI Integration in Genetic Testing for Platelet Disorders:**
  - AI tools will be integral to the diagnostic process for disorders like Hermansky–Pudlak syndrome, facilitating the integration of genetic findings with functional platelet assays to improve variant pathogenicity assessments

Noteworthy AI / ML presentations at EAHAD 2026

Notable Presentations At EAHAD 2026
AI / ML (1/3)

Date	Title	Author	Summary
All days	Artificial Intelligence and Gene Therapy: About the First Patient to Receive Etranacogene Dezaparvovec in Spain	M. C. Suarez	• **Introduction**: Generative AI is increasingly positioned as a transformative tool in haematology, with potential to improve diagnostic accuracy, treatment planning, and knowledge dissemination, while requiring rigorous clinical oversight. • **Methodology:** An ultra-specialized, multilingual LLM-based chatbot using retrieval-augmented generation (RAG) was developed to support hematologists managing gene therapy (GT) patients, integrating expert-curated literature, real-time analytics, and secure text/voice interaction. • **Results:** In real-world testing during early GT implementation in Spain, chatbot recommendations for follow-up and clinical management fully aligned with established center protocols and published evidence. The system enabled real-time laboratory benchmarking, longitudinal interpretation, and scenario anticipation without observed clinical discordance. • **Conclusions:** Ultra-specialized AI chatbots demonstrated accuracy, reproducibility, and clinical reliability, supporting GT patient selection, follow-up, and continuous expert knowledge translation under strict data-protection frameworks.
All days	Navigating the Challenges: Understanding the Impact of Haemophilia on Physical Activity as Expressed through Social Media	R. Nagra	• **Introduction**: This study examined how haemophilia influences physical activity participation in the UK, with particular focus on adolescents and young adults, using real-world patient and caregiver perspectives from social media. • **Methodology:** A 36-month retrospective social listening analysis (2021–2024) captured public UK posts via TalkWalker and Infegy. AI-driven thematic analysis (CoLoop) and netnography were applied, with a focused subanalysis of individuals aged 13–25. • **Results:** From 47,239 posts, 839 were relevant; 164 reflected the younger cohort. Physical activity discussions were more frequent among youth (26% vs 17%). Patients promoted active lifestyles, favoring low-impact sports, while parental and HCP caution sometimes limited participation. Social isolation and stigma were prominent concerns. • **Conclusions:** Haemophilia significantly constrains physical and social activity in young patients, underscoring the need for integrated clinical, educational, and psychosocial support.

Notable Presentations At EAHAD 2026
AI / ML (2/3)

Date	Title	Author	Summary
All days	Immunogenicity Assessment of CFVIII with Generative AI	M. R, Lope	• **Introduction**: Inhibitor development remains the principal complication of FVIII replacement in haemophilia A, with uncertainty regarding the role of concentrate origin. Generative AI may enhance evidence synthesis and risk stratification. • **Methodology:** A systematic review (2015–2025) of 23 prospective studies included 4,847 PUPs and 8,392 PTPs. Claude 3.7 Sonnet supported data extraction and analyses, fully human-validated. Outcomes were analysed using meta-analysis and adjusted regression models. • **Results:** High-titer inhibitor risk was lowest with HEK-293 FVIII (5.2%) and pdFVIII (5.9%), higher with CHO (8.1%), and highest with BHK products (12.4%). BHK FVIII showed significantly increased risk versus pdFVIII and HEK-293. No SHL/EHL difference was observed in PTPs. • **Conclusions:** Human cell–derived FVIII demonstrates immunogenicity comparable to pdFVIII, supporting individualized product selection. AI-assisted analysis proved efficient when rigorously validated.
All days	Evolving Treatment Priorities in Hemophilia: Physician Perspectives on Convenience, Quality of Life, and the Emerging Role of Rebalancing Agents across the US and EU5	S. Hendry	• **Introduction**: As novel rebalancing agents enter haemophilia care, prophylaxis priorities are shifting beyond bleed prevention toward patient-centered outcomes, emphasizing quality of life, convenience, and shared decision-making. • **Methodology:** Physician perspectives were assessed using three 2025 Spherix Global Insights studies across the US and EU5 (n=265 hematologists). AI tools supported abstract drafting only; data collection and analysis were fully human-led. • **Results:** Across regions, treatment burden and daily-life convenience emerged as key drivers of therapy choice alongside efficacy. US physicians reported early reductions in complexity with fitusiran, while EU clinicians favored concizumab and marstacimab in inhibitor populations. Familiarity and satisfaction with rebalancing agents increased steadily. • **Conclusions:** Rebalancing agents are reshaping haemophilia management toward individualized, lifestyle-aligned care where convenience and patient voice define therapeutic success.

Notable Presentations At EAHAD 2026
AI / ML (3/3)

Date	Title	Author	Summary
All days	Evaluation of Fibrinolysis Testing Methods in Haemostasis and Thrombosis Laboratories: Results of a National Survey by the Spanish Society on Thrombosis and Haemostasis (SETH) Laboratory Working Group	J. M. Calvo Villas	• **Introduction**: Laboratory assessment of fibrinolysis remains heterogeneous and poorly standardised, limiting diagnostic confidence in haemostasis and thrombosis practice. • **Methodology:** A structured survey of 31 European laboratories (mainly Spain) assessed fibrinolysis assays, reagents, protocols, training, and challenges. Descriptive analyses were performed; AI was used only for language editing. • **Results:** Routine use focused on Clauss fibrinogen and D-dimer, while key fibrinolysis-specific assays (ECLT, tPA, fibrin monomers) were rarely performed. Only 25% reported standardised protocols. Training was infrequent, reagent access limited, and perceived assay reliability was moderate (6.6/10). • **Conclusions:** Marked variability and limited standardisation persist, underscoring the need for harmonised protocols, reference networks, and structured training to improve fibrinolysis diagnostics.
All days	Hermansky-Pudlak Syndrome Type 1: An Unusual Clinical Presentation	L. Díaz Ajenjo	• **Introduction**: Hermansky–Pudlak syndrome (HPS) is a genetically heterogeneous platelet disorder that may present with bleeding without overt albinism, leading to misdiagnosis as VWD. • **Methodology:** A 67-year-old woman with lifelong bleeding was reassessed using ISTH-BAT, comprehensive platelet function testing (LTA, flow cytometry), whole-mount electron microscopy, and targeted gene panel sequencing. • **Results:** Despite normal coagulation and VWD screening, platelet studies showed impaired aggregation and dense granule secretion. Electron microscopy confirmed markedly reduced dense granules. Sequencing identified two novel compound heterozygous HPS1 variants. • **Conclusions:** This case highlights atypical HPS1 presentation and underscores the necessity of integrating genetic findings with functional platelet assays to establish variant pathogenicity.

Strategic Insights and Strategy Development is our focus