EAHAD 2024

Review of the European Association for Haemophilia and Allied Disorders (EAHAD) Annual Congress 2024

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FRANKFURT, Germany, well known across Europe for its culture, education, and above all, bustling financial district, was home to the 17th Annual European Association for Haemophilia and Allied Disorders (EAHAD) Congress this year. The much anticipated event saw nearly 2,000 experts from around the world flock to the city of skyscrapers between 6th–9th February 2024 to present on the latest developments in the field, share information on current clinical research, and exchange ideas with other healthcare professionals, all to improve clinical care for patients with inherited or acquired bleeding disorders.

The congress featured an enthusiastic welcome from 2024 EAHAD Congress President, Wolfgang Miesbach, who discussed the ongoing innovation and education in haemophilia research which makes EAHAD unique. He praised the collaboration and cooperation of EAHAD and other scientific centres, before going on to discuss the latest projects being undertaken by the association, such as the EAHAD Gene Therapy Working Group, and a new webinar series organised by the Nurses Committee intended to educate on the role nurses can have in treating bleeding disorders. Miesbach then turned his attention to this year's congress, highlighting several of the illuminating sessions he looked forward to attending, before closing his speech with some recommendations for first-time visitors in the city, not least that they try some traditional food, namely *Grie Soß* and apple wine.

Later in the day, this year's EAHAD research grant reports and awards were presented. Ilja Oomen, Amsterdam University Medical Centre, the Netherlands, briefly presented her research entitled 'Getting a Grip on Tolerance', focusing on the dendritic cell response to FVIII immune complexes. The second research report came from David Stephenson, Canterbury Christ Church University, UK, for his work on identifying performance-based outcome measures of physical function in patients with haemophilia; and the third from Anna Wells, Hampshire Hospitals NHS Foundation Trust, Basingstoke, UK, who discussed post-traumatic stress symptoms and pain memories amongst patients with haemophilia. The second half of this session saw chairs Miesbach and Robert Klamroth present EAHAD lifetime achievement awards to Ulla Hedner, Novo Nordisk Haemophilia Foundation, for her lifelong commitment to haemophilia research, as well as her pioneering work on the use of recombinant coagulation factor VII; and Kate Khair, Haemnet, London, UK, for her extensive advocating for the role of nurses in haemophilia care.

The closing presentation saw Miesbach thank EAHAD President, Robert Klamroth, for his 2 years in the position, and all the work he has done for the association. Miesbach further expressed his gratitude for every individual who had a hand in organising the congress and making it such a roaring success. Former EAHAD President, Flora Peyvandi, gave a rousing speech in which she implored everyone listening to continue their hard work, research, and reporting on haemophilia.

Read on for our key insights into the 2024 EAHAD Congress, and watch out for next year's meeting, taking place in February 2025 in Milan, Italy.

Evaluation of Adherence to Prophylaxis Treatment in Haemophilia

NON-adherence to prophylactic treatment has significant implications for the development of arthropathies and mortality in patients with haemophilia. A recent study highlighting the importance of adhering to prophylaxis for haemophilia was presented at the 17th Annual Congress of the EAHAD, which took place in Frankfurt, Germany, from the 6th–9th February 2024.

A total of 95 patients with haemophilia A and B were included in the retrospective study, of whom 62 had severe haemophilia, and 33 had moderate haemophilia. Among these individuals, 77 (92.50%) were on secondary/tertiary prophylaxis regimens, and 18 (57.89%) were on primary prophylaxis. Only 23 patients on secondary/tertiary prophylaxis adhered to regular prophylaxis, with 54 patients reporting irregular adherence. Among patients on primary prophylaxis, 12 maintained a regular regimen, and six had irregular adherence.

Irregular adherence to haemophilia prophylaxis can increase the risk of bleeding and musculoskeletal complications. The study reported a significant proportion of patients on secondary/tertiary prophylaxis (54 out of 77) following an irregular treatment pattern; however, a higher proportion of patients on primary prophylaxis (12 out of 18) reported regular adherence.

Treatment adherence can be influenced by many clinical and psychosocial factors, such as a family history of bleeding and a lack of social support; therefore, the individualisation of treatment must be considered, to meet the specific needs of each patient. Factors such as the quality of venous access, frequency of infusions, patients' life routines, associated costs, and the potential development of arthropathies, must be taken into account to improve adherence, preserve joint health, and ensure a better quality of life for patients with haemophilia. ●

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Haematuria Management in Paediatric Congenital Haemophilia A

PAEDIATRIC patients diagnosed with haemophilia with inhibitors can encounter a rare but serious complication whilst undergoing treatment with bypassing agents, in the form of an inhibitory antibody to factor VIII (FVIII). Bypassing agents are also used to prevent bleeds in this patient group. Those with haemophilia frequently experience haematuria, which can significantly worsen their quality of life.

Lead study author Murat Sokar, Paediatric Hematology and Oncology, Dicle University Medical Faculty, Diyarbakır, Türkiye, and colleagues, presented a case of a 14-year-old male at this year's EAHAD Congress, held in Frankfurt, Germany, in February.

The patient in question, who had been diagnosed with severe haemophilia A (FVIII level 1) with inhibitor at the age of 8 months, presented with asymptomatic macroscopic haematuria. He was currently on episodic therapy and had an FVIII inhibitor titre of 9.4 BU. His vital signs were stable. The patient underwent radiologic imaging of his abdomen and ultrasonography of his pelvis. A microthrombus was found in his bladder.

He was started on hyperhydration and was placed on complete bed rest, as well as receiving two doses of activated prothrombin complex concentrates (aPCC). On Day 2, once he had received four doses of aPCC in total, his haematuria persisted. Doctors discontinued aPCC, and started the patient on a treatment of recombinant FVIIa at 90 µg/kg−1 per dose; this treatment was continued every 6 hours, for 4 days. Four days later, after 19 doses of recombinant FVIIa, the patient's haematuria was rectified, and a bladder ultrasound demonstrated normal results. The FVIIa treatment was discontinued, as was hyperhydration. After discharge, the patient continued to take aPCC 3 days per week, as a secondary prophylaxis. No bleeding and normal kidney functions have ensued, and the patient is receiving follow-up care in an outpatient setting.

> **"Macroscopic haematuria is a significant problem for patients with haemophilia."**

The development of FVIII inhibitors is persistently the most serious complication of congenital haemophilia A management. Despite haematuria being widely considered a benign complaint, the literature demonstrates that macroscopic haematuria is a significant problem for patients with haemophilia. Sokar and colleagues stress that more studies are needed to determine the correct management of haematuria with possible renal dysfunction. ●

Musculoskeletal Ultrasound in Haemophilia and Sports

MUSCULOSKELETAL ultrasound is an effective method for monitoring intra-articular and intra-muscular haemorrhage, which allows patients with haemophilia to safely return to sports activities. The new research was presented at the 17th Annual Congress of the EAHAD, which took place in Frankfurt, Germany, from 6th–9th February 2024.

The study examined a group of children, adolescents, and young adults (4–27 years) with haemophilia who play sports recreationally, under supervision. Basketball players were found to suffer the most from injuries, despite prophylactic treatment, and one patient with mild haemophilia A developed acute compartment syndrome, after receiving an impact while playing soccer. Patients who swam did not present significant haemorrhages. The most severe muscular injuries occurred from direct impact, while a minority were caused by overload.

The authors found musculoskeletal ultrasound to be the ideal method for evaluating intra-articular bleeding, as well as peri-articular or muscular soft tissue haemorrhage. As a fast, economical, and radiation-free method, it allowed the team to assess the size of patient haematomas, and their relationship with the bone plane, to avoid the formation of myositis ossificans. It also enabled monitoring of haematoma size reduction until complete healing of the injury, for a safe return to usual sports activity. Furthermore, musculoskeletal ultrasound allowed the detection of uncommon bleeding manifestations in acute compartment syndrome, which can lead to irreversible tissue damage, as well as small haematomas, not detectable in physical examinations, and interstitial haemorrhage.

The team emphasised the usefulness of musculoskeletal ultrasound for monitoring the health of patients with haemophilia who play sports, minimising the risk of relapse, avoiding new injuries, and allowing safe re-incorporation into sports practice. They also highlighted the importance of working in an interdisciplinary team to provide the most efficient diagnostic methods and improve patient support.

"Musculoskeletal ultrasound allowed the detection of uncommon bleeding manifestations in acute compartment syndrome."

The Value of MRI in Early Haemophilic Arthropathy Detection

NOVEL research presented at the 17th Annual Congress of the EAHAD, which took place in Frankfurt, Germany, between 6th–9th February 2024, explored the role of T1 3D fast field echo (FFE) MRI sequence in detecting early signs of joint arthropathy in patients with haemophilia.

Given the frequency of haemophilic arthropathy as a complication of haemophilia, Gehan Lofty Khalifa and Mohammed F. Amin, Minia University, Al Minya, Egypt, performed a cohort study to assess whether MRI was capable of detecting joint changes at an early stage and whether this correlated to patients' symptoms.

To do this, they enrolled 70 patients with haemophilia aged 2–18 years. All participants had a full clinical history taken, had affected target joints that were managed with either prophylactic or on-demand therapy protocols, and had an assessment of the musculoskeletal function of their joints using the Hemophilia Joint Health Score (HJHS; version 2.1). These functional assessments were evaluated by a haematologist and a physiotherapist, and the International Prophylaxis Study Group (IPSG) MRI scale was utilised to score the MRIs of examined joints.

The findings revealed a strongly positive correlation between the HJHS score and IPSG MRI scores for those with symptomatic joints ($p<0.001$). Conversely, no significant correlation between HJHS and IPSG MRI scores was identified for those with asymptomatic joints. Of note, the T1 3D FFE MRI sequence performed better in detecting hemosiderin and cartilage degeneration, markers of early haemophilic arthropathy, in asymptomatic joints than in symptomatic joints.

> **"The T1 3D FFE MRI sequence can be used to identify early haemophilic arthropathy changes."**

They concluded that MRI can identify pathology in asymptomatic joints with a normal clinical examination and that the T1 3D FFE MRI sequence can be used to identify early haemophilic arthropathy changes in such patients. This could allow for earlier intervention before progression to more advanced states. ●

New Insights Into Paediatric Diagnosis of von Willebrand Disease

DIAGNOSIS of von Willebrand disease (VWD) can be difficult, especially in populations of children with fewer haemostatic challenges. Using the FranceCoag, a French cohort of inherited bleeding disorders, a new study has emerged with results that highlight the importance of family screening. This investigation was presented at the 17th Annual Congress of the EAHAD, which took place in Frankfurt, Germany, from the 6th–9th February 2024.

Patients are usually diagnosed through three different features: bleeding symptoms, family history, or fortuitous discovery. The usefulness of this work is clear when the scarcity of published literature on diagnosing symptoms of VWD in children is considered.

Close to 14,000 patients diagnosed with an inherited bleeding disorder were included in the registry, using the multicentre cohort from FranceCoag. Participants were diagnosed with VWD between 2003–2022, whilst younger than 18 years of age. Diagnosing features, severe bleeding, and treatment requirements were all studied until participants reached 18 years. In the current study, 3,578 patients with VWD were taken from the overall cohort, and 1,054 were included in the investigation, 49% of whom were male. Eight percent (n=73) presented with a very severe VWD (von Willebrand Factor [VWF]: <5% or factor VIII: <5%), 39% (n=374) with severe VWD (VWF: 5–15% and factor VIII: >5%), and 54% (n=518) with moderate VWD (VWF: 16–40% and factor VIII: >5%). A total of 60% of the patients were diagnosed in the context of family history, 22% due to bleeding features, and 17% fortuitously. Overall, 68% did not necessitate treatment during follow-up. The median age at first treatment was 4.7 years, and this first treatment was non-substitutive in 17% of the patients (n=57). Meanwhile, substitutive VWF was used in 40% of the patients (n=135). Prophylaxis was required in only 24 patients, and just 20 severe bleedings were reported: nine intracranial haemorrhages, nine gastro-intestinal bleedings, and two uterine bleedings. The median age at the time of severe bleeding was 6 years.

The results from this study highlight the rarity of fortuitous discovery in paediatrics and underscore the importance of familial screening. This study will inform decision-making in practice, encouraging screening from a young age despite a lack of need for specific treatment from low severe bleeding occurence. ●

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