



Non-Factor Therapies: Reflections on Current Clinical Practice

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The 17th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) took place on 6th–9th February 2024 in Frankfurt, Germany. In a comprehensive and interactive session, healthcare experts and patients gathered to discuss current approaches in clinical practice. The session was chaired by Jan Blatny, University Hospital Brno, Czechia; and Niamh O'Connell, The National Coagulation Centre, St James's Hospital, Dublin, Ireland.

ALLIED HEALTHCARE PROFESSIONALS' DAY

Nanda Uitslager, University Medical Center Utrecht, the Netherlands, opened the session by providing an overview of the Allied Healthcare Professions (AHP) Day. Comprising nurses, physiotherapists, and psychologists, this event was a collaborative effort to celebrate and recognise the remarkable work of the healthcare workforce, as well as highlight current challenges in haemophilia treatment. A joint midday session on non-factor therapies was held, with the aim to better understand the experiences of healthcare professionals (HCP) and patients with this line of treatment.

Common blood disorders, such as haemophilia, are caused by a lack of integral clotting proteins like Factor VIII and Factor IX in haemophilia A and B, respectively. Factor replacement therapy combats this by replenishing the clotting agent in question, through intravenous infusion. Whilst the safety and efficacy has proven successful, there is a considerable associated treatment and disease burden, impacting the patient's quality of life, and prompting HCPs to call for treatment alternatives. The bleeding rate, for example, remains high for those receiving prophylaxis, and intravenous drug

administration several times a week can be demanding on patients.

Conversely, non-replacement therapies target the coagulation cascade, boosting the haemostatic potential by mimicking coagulation factors, or by reducing naturally occurring inhibitors, known as factor mimetics and rebalancing agents, respectively. As administration is given subcutaneously, the treatment burden is also reduced. Differences in treatment and disease burden between factor versus non-factor-based replacement therapy was discussed in-depth within the AHP joint session.

SETTING THE SCENE: THE DIFFERENCE BETWEEN CHOOSING FOR YOURSELF AND RECOMMENDING

Ilaria Cutica, University of Milan, Italy, highlighted the unique challenges in making treatment decisions for oneself, and on behalf of someone else, and provided comprehensive guidance for each circumstance. For both caregivers and HCPs, she emphasised the importance of information, with greater treatment knowledge corresponding to improved engagement, trust, and communication from the patient.

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The influence of personal factors, such as beliefs, desires, cognitive bias, and risk perception in decision-making, was subsequently explored. When seeking to recognise potential conflicts of interest, Cutica explained that one might not necessarily agree with a patient's values or beliefs, but it is paramount that these factors are acknowledged, and incorporated within the decision-making process.

"Caregiver figures often exhibit greater risk perception than paediatric patients."

Finally, she drew on shared decision-making, which HCPs use when making decisions on behalf of a patient. In this collaborative approach, the treatment options are firstly relayed to the patients, with potential adverse risks and reported successes listed. A discussion then follows, in which any patient queries or questions are addressed. As noted by Cutica, the latter stage also serves as an opportunity for the HCP to better understand the patient as a person, allowing the provision of more tailored recommendations.

PANEL DISCUSSION ON TREATMENT CHOICES: MULTIDISCIPLINARY SUPPORT FOR DECISION-MAKING

A panel session followed, in which questions were posed to the audience, voted on, and the answers discussed amongst the panellists. The panel consisted of patient David Flanagan; Marie Katzerova, University Children's Hospital, Brno; Niamh O'Connell; Mary Kavanagh, Children's Health Ireland (CHI), Crumlin, Ireland; Ilaria Cutica; and Jan Blatny.

The first question focused on the decision-making process, and whether it changes when the perspective shifts from that of patient to caregiver. With 69% of the votes, 'No' was the predominant answer. Providing a patient and HCP perspective, respectively, Flanagan and O'Connell agreed that caregiver figures often exhibit greater risk perception than paediatric patients, acknowledging the natural worry parents may feel for their children. Offering a nurse's viewpoint, Kavanagh stated that the requirement for information may be higher, as the caregiver adopts a responsibility to understand all risks on behalf of the child.





The precise role of HCPs within the decision-making process was then questioned. A majority (85%) felt it was 'to describe the treatment options and discuss the potential benefits and risks before coming to an agreed decision', a view shared amongst the panellists. Few agreed with the remaining statements, suggesting leaving the decision entirely to the patient, or providing a direct recommendation. Opening the discussion further, the panellists explored several scenarios, such as a conflict of interest between caregiver and patient, and providing treatment information when limited literature exists.

Finally, with recent advances in treatment options for patients with haemophilia, the importance of the multidisciplinary approach was reviewed. This aims to optimise patient management and outcomes by supplying patients with a range of medical personnel, such as haematologists, physiotherapists, psychosocial support, dental care, and surgery.

In agreement with the majority (76%), Flanagan felt that multidisciplinary treatment was more important in haemophilia treatment, raising the point that as the availability and quality of treatment options improve, patients may live longer, and thus require wider holistic care as new, unforeseen complications may arise.

CONCLUSION

With greater implementation of non-factor replacement therapy in clinical practice for bleeding disorders, it is ever more necessary for reflections like this to occur. The discussion called for continued collaboration between patients and HCPs, and multidisciplinary care for haemophilia. The panel offered a comprehensive view of the considerations at play when making medical decisions, highlighting risk perception, cognitive bias, and personal beliefs, amongst others. ●