EAHAD-EHA-EHC Joint Support Statement on the EMA draft Guideline for the clinical requirements for non-replacement therapy in haemophilia A and B

For the last thirty years, haemophilia A and B patients have been treated using FVIII and FIX replacement concentrates. These therapies are well known to both clinicians and patients.

We are now entering a new era in the treatment of haemophilia and rare bleeding disorders. Novel, non-replacement therapies have been approved and are becoming available, which may have a significant impact on the clinical care and quality of life of patients.

The European Medicines Agency (EMA) launched in October 2023 a public consultation on their draft guideline on the clinical requirements for non-replacement therapy in haemophilia A and B. The end of the consultation and deadline for comments was on 30 April 2024.

The European Association for Haemophilia and Allied Disorders (EAHAD), the European Hematology Association (EHA), and the European Haemophilia Consortium (EHC) support EMA's efforts on drafting the guideline which aims to provide both applicants and regulators with much needed harmonised marketing authorisation requirements for applications of non-replacement therapies for haemophilia A and B.

We welcome all novel therapies that have a meaningful impact on patients' treatment, care and quality of life.

Since these treatments function completely differently than standard factor replacement therapies, we emphasise that marketing authorisation harmonisation, general design principle definition, ability to compare with previous treatment options, posology optimisation, safety, efficacy and post-marketing data collection are crucially important.

The draft EMA guideline touches upon all these issues in a comprehensive way.

EAHAD, EHA, and EHC reiterate the importance of education and vigilance of all healthcare stakeholders on these novel treatments. It is our responsibility to remain involved and ensure the best possible safe introduction and use of these innovative and complex new treatments.

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