

**EAHAD-EHC Joint Statement on:
Promoting hub-and-spoke model for the treatment of haemophilia and rare bleeding
disorders using gene therapies**

For the past three decades, haemophilia A and B have been treated using FVIII and FIX replacement concentrates, and their treatment and care has been managed through comprehensive or standard haemophilia treatment centres.

We are now in a promising era of new treatment choices, which will soon include the first gene therapies for haemophilia patients.

The European Association for Haemophilia and Allied Disorders (EAHAD) and the European Haemophilia Consortium (EHC) welcome the long-awaited arrival of gene therapies and their potential to have a meaningful impact on patients' treatment, care and quality of life.

Gene therapies are new to this specific patient population as well as relatively new in the healthcare setting generally. It is therefore important that, in addition to both patients and clinicians being well-educated about the modalities of gene therapies, that these therapies also be prescribed and carefully monitored in expert centre hubs.

To ensure the safe introduction, use, monitoring and optimal learning regarding the delivery of gene therapies over time, EAHAD and EHC jointly call for all first-generation gene therapies to be managed using a hub-and-spoke model, as follows:

- Prescribed and managed exclusively by expert haemophilia comprehensive care centres (as the national hubs), and
- Monitored, by haemophilia treatment centres in close communication with the primary expert hub (as spokes linking into that hub).

These expert hubs should have previous experience with gene therapy trials and/or specialists who can ensure expertise in a timely manner in gene therapy research, education and monitoring, including laboratory monitoring. This will also maximise long-term benefits for gene therapy patients.

EAHAD and the EHC have previously recommended patients being treated with any novel therapies carry an identity card indicating information about their condition, treatment regimen, the haemophilia comprehensive care centre where their treatment is being overseen, and the name and telephone number of the contact person there, who can be reached 24 hours a day, 7 days a week. This should continue with gene therapies.

Any adverse events should be managed by both the expert 'hubs' as well as the treatment centre 'spokes' to provide the timeliest state-of-the-art treatment options to patients and maximise long-term benefits. All adverse events should be reported to a centralised reporting scheme and should include bleeding episodes.

It is the responsibility of all healthcare stakeholders to ensure the prescription, management and monitoring of gene therapies by expert hubs, at least initially, to ensure the optimal introduction, use and monitoring of these very different and complex new treatments.