



Position Paper

Access to treatment for patients with blood disorders

Access to treatment is an essential element of the human right to health, as enshrined in article 25 of the EU Charter of Fundamental Rights. However, this still represents an issue for many EU citizens, including patients affected by blood disorders.

Multiple barriers arise with regard to access to treatment: an effective treatment may not have been developed yet, or a treatment is available but inaccessible because of limited coverage, shortages or unaffordable prices. In recent years, the financial and economic crises and consequent policy responses have exacerbated these challenges, generating debate about how to ensure good quality healthcare while maintaining financially sustainable health systems.

The European Union (EU) has a key role to play in helping Member States implement health policies and reforms that positively affect access to treatment. In particular, the European Hematology Association (EHA) invites the European Commission and Member States to address the following issues:

1. IC PerMed Roadmap for personalized medicine should include identified priorities in hematology
2. Medicine shortages represent a real threat to patients
3. Long-term support for the European Reference Network in Rare Hematological Diseases (EuroBloodNet) is essential



1. IC PerMed Roadmap for personalized medicine should include identified priorities in hematology

Personalized medicine is one of the most promising and innovative areas of health research and provision. Hematology has already successfully developed personalized medicine strategies and has managed to increase cure rates. Discoveries in hematology personalized medicine research have found applications in other specialties such as oncology and cardiology. Yet, to make good on its promise, research into personalized medicine needs to be supported, and access for patients with blood disorders improved.

The **International Consortium for Personalised Medicine (IC PerMed)** is currently drafting a roadmap with actionable research targets needed to progress in this area. EHA calls for this document to include the recommendations of the EHA Roadmap for European Hematology Research, which describes the state of the art in the field and presents a consensus on the research priorities for the next 5-10 years, including in the realm of personalized medicine.

EHA calls upon the IC PerMed consortium to include in its Roadmap for personalized medicine the unmet needs identified by the EHA Roadmap for European Hematology Research.

2. Medicine shortages represent a real threat to patients

Reports on drug shortages in Europe suggest that the problem is real and growing and that these shortages can have an alarming impact on healthcare costs, patient safety and clinical outcomes. The fact that treatments for blood cancers often involve complex combined therapies makes them particularly vulnerable to shortages. In some Member States shortages are already affecting the daily practice of hematologists when they have to take patients off therapies mid-treatment, choose less effective alternative therapies, or go for 'grey zone' medicines or biosimilars. Sometimes these alternatives are of (much) lower quality than the recommended treatment. Moreover, inexperience with alternative products can increase the risk of errors in dosages or administration.

To be able to curb shortages effectively, there is a need for Europe-wide coordination to alert and exchange information on shortages. EHA invites the European Commission to facilitate exchange of experiences and good practices among Member States by creating an EU-wide information system. EHA also calls upon the national competent authorities to allow hematologists to report the occurrence of shortages.

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3. Long-term support for the European Reference Network in Rare Hematological Diseases (EuroBloodNet) is essential

EHA believes ERNs have a great potential to address common challenges faced by professionals when diagnosing and providing highly specialized healthcare for complex rare diseases in the field of hematology. For this purpose, EHA positioned itself at the forefront of this initiative and endorsed a unique proposal for a **European Reference Network in Rare Hematological Diseases (EuroBloodNet)**, encompassing both non-malignant and malignant hematology.

The EuroBloodNet ERN, approved in December 2016, will enable patients to access quality care which might otherwise not be available to them. For this initiative to be successful, the European Commission and Member States should commit to making this ERN a long-term political priority and guarantee its financial sustainability by establishing appropriate funding mechanisms.

EHA calls upon the European Commission and Member States to ensure long-term political and financial support for the ERN in Rare Hematological Diseases (EuroBloodNet).

About EHA

The European Hematology Association (EHA) is a not-for-profit membership organization that promotes excellence in patient care, research and education in hematology. EHA's Annual Congress is attended by some 10,000 delegates. The association also publishes *Haematologica*, Europe's primary hematology journal. Being the leading hematology organization in Europe, EHA represents the interests of the discipline, its clinicians and its scientists in Europe.

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