EHC
Empowering the bleeding disorders community

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EHC Position Paper on EU Biotech Act

The European Haemophilia Consortium (EHC), representing people living with bleeding disorders across Europe, welcomes the European Union's (EU) initiative to develop a comprehensive EU Biotech Act aimed at strengthening Europe's capacity for biomedical innovation. Breakthroughs in biotechnology, including gene therapy, advanced therapies, and precision medicine, are transforming the treatment landscape for rare conditions such as bleeding disorders. Ensuring that the EU remains an attractive environment for research, development, and patient access is therefore of critical importance to our community.

To fully realise the potential of the EU Biotech Act for people with bleeding disorders, the EHC calls for particular attention to three key areas: meaningful patient involvement throughout the innovation lifecycle, the effective use of real-world evidence, and the facilitation of cross-border clinical trials.

1. Ensuring Meaningful Patient Involvement

Patients and patient organisations provide unique insights into disease burden, treatment priorities, and acceptable risk-benefit trade-offs. Their early and meaningful involvement is essential to ensure that biotechnology innovation delivers real value for patients.

The EHC recommends that the EU Biotech Act:

- Establish mechanisms to involve patient organisations systematically in the design of research programmes, clinical trials, and regulatory discussions.
- Support capacity-building initiatives that enable patient organisations to participate effectively in scientific and regulatory processes.
- Encourage the co-creation of research priorities between policymakers, researchers, industry, and patient communities.

2. Strengthening the Role of Real-World Evidence (RWE)

For bleeding disorders, traditional clinical trial data are often limited due to small patient populations and evolving treatment paradigms. Real-world evidence, generated through patient registries, post-marketing studies, and routine clinical data, plays a critical role in understanding long-term safety, effectiveness, and quality-of-life outcomes.

The EHC recommends that the EU Biotech Act:

- Promote the systematic collection and use of high-quality real-world data, including through interoperable European registries and digital health infrastructures.
- Support harmonised methodologies and standards for the generation and use of RWE across regulatory, health technology assessment (HTA), and reimbursement processes.

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- Encourage collaboration between regulators, clinicians, patient organisations, and industry to ensure that real-world data reflect outcomes that matter to patients.

3. Facilitating Cross-Border Clinical Trials

People with rare bleeding disorders are often dispersed across multiple countries, making multinational collaboration essential for conducting robust clinical research. The EHC therefore emphasises the importance of simplifying and accelerating cross-border clinical trials within the EU.

The EU Biotech Act should:

- Build on existing frameworks to streamline regulatory and administrative requirements for multinational clinical trials.
- Support the harmonisation of ethics review processes and administrative procedures across Member States.
- Invest in infrastructure that enables cross-border trial participation.

The EU Biotech Act represents a significant opportunity to strengthen Europe's leadership in biotechnology while improving health outcomes for people living with bleeding disorders. By embedding meaningful patient involvement throughout the innovation ecosystem, prioritising the integration of real-world evidence, and enabling cross-border clinical research, the EU can ensure that scientific progress translates into tangible benefits for patients.

The EHC stands ready to collaborate with EU institutions, Member States, researchers, and industry partners to help build a patient-centred biotechnology framework that supports innovation, equity, and access across Europe.