EUCCT

The EU Biotech Act: What Patients Need to Know

1 What is the EU Biotech Act?

The EU Biotech Act is a proposal from the European Commission to improve how Europe supports biotechnology, medicines development, and clinical trials. It aims to make Europe a more attractive place for clinical research by:

- speeding up clinical trial approvals,
- reducing unnecessary bureaucracy,
- and improving how countries work together.

While the Act is mostly driven by economic and industrial competitiveness goals, when comparing Europe to other world regions, it creates an ***important opportunity for patients—especially those with life-threatening rare diseases and cancers, where research can be accessible to more patients when countries cooperate.***

2 What is Critical and What Does It Mean for Patients?

Here is what matters most - especially for patients with serious or life-threatening illness due to rare diseases, including rare cancers, or childhood diseases:

- ***Faster approvals → faster access to clinical research***

Today, clinical trials often start slowly because approvals take too long. The Act aims to shorten these timelines —important for patient communities where time is critical.

- ***Stronger cooperation across Europe***

Some diseases affect only a small number of people in each country. Clinical research is accessible to more patients suffering from these diseases when countries collaborate better.

The Act tries to address disconnection in the EU by improving cross-country cooperation. ***However, it does not yet ensure that patients can easily join trials in another EU country — and this is where advocacy is needed.***

3

What the Biotech Act Does Not Address — and Why It Matters

Despite many positive elements, the Act:

- ***does not provide a legal framework for cross-border participation in clinical trials,***
- mentions rare diseases only briefly,
- focuses mainly on competitiveness and innovation rather than patient access,
- and may risk not delivering ***better equity and access to trials for patients.***

This is why a patient-driven message is needed: ***cross-border access is important for patients and should be based on unmet medical needs — not on cost or convenience.***

* Proposal for a Regulation to establish measures to strengthen the Union’s biotechnology and biomanufacturing sectors (European Biotech Act)

4

Why Cross-Border Trial Access Is More Essential for Some Diseases than Others?

For many rare conditions, including rare cancers:

- no single country has enough patients to run a meaningful trial,
- expertise is concentrated in a few specialised centres, often in larger and economically stronger countries
- early access to a trial can be lifesaving.

Cross-border access enables:

- participation when no trial exists in a patient's home country, fairer access to new treatments,
- stronger, faster research outcomes.

Without clear rules, cross-border access will remain inconsistent, unpredictable, and dependent on individual stakeholders, e.g. hospitals or ethics committees, decisions.

5

Where Improvements to the Biotech Act Could Strengthen Patient Access

Centres of Excellence: A promising starting point

In Article 6, the Act ***supports cross-border access*** to Centres of Excellence for advanced therapies (such as cell and gene therapies). These principles—access to clinical settings and cross-border availability—***could serve as a model for rare diseases and rare cancers, where similar centres already exist, independent of the type of innovative medicine under reaearch.*** But currently, this Article applies only to advanced therapies, not to all types of innovative medicines.

We suggest that Article 6 be expanded to include clinical trials for other types of medicines targeting rare and/or life threatening diseases and enable cross-border access for patients to those clinical trials.

EUCCT

6

Where could Amendments to the Clinical Trial Regulation Strengthen Patient Access?

In addition to the Biotech Act itself, patient advocacy groups can propose changes to the ***Clinical Trials Regulation (CTR)***, which the Biotech Act reopens. Potential scope for amendments to the CTR might include:

Article 50 (Suitability of Trial Sites):
The facilities where the clinical trial is to be conducted shall be suitable for the conduct of the clinical trial in compliance with the requirements of this Regulation.

This could mention that when suitable clinical trial sites do not exist in the patient's home country, provisions for safe and coordinated cross-border access for patients to a suitable trial facility should be considered.

7

Clear Call to Action for Patient Organisations

Patient organisations—especially in rare diseases, rare cancers and childhood diseases—have a unique opportunity to ensure patient needs are included while the law is still being shaped.

What patient groups should advocate for:

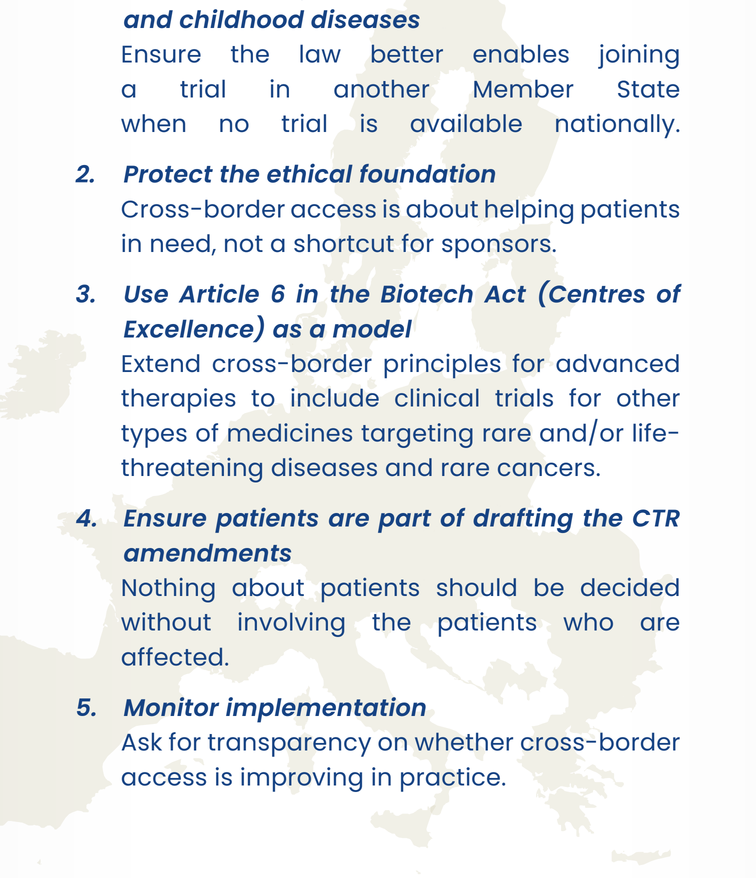
1. ***Make cross-border participation easier for life threatening or rare diseases, rare cancers and childhood diseases***
   Ensure the law better enables joining a trial in another Member State when no trial is available nationally.

2. ***Protect the ethical foundation***
   Cross-border access is about helping patients in need, not a shortcut for sponsors.

3. ***Use Article 6 in the Biotech Act (Centres of Excellence) as a model***
   Extend cross-border principles for advanced therapies to include clinical trials for other types of medicines targeting rare and/or life-threatening diseases and rare cancers.

4. ***Ensure patients are part of drafting the CTR amendments***
   Nothing about patients should be decided without involving the patients who are affected.

5. ***Monitor implementation***
   Ask for transparency on whether cross-border access is improving in practice.



8

Final Message for Patient Societies

This is a critical moment.
The EU is redesigning how clinical trials will work for the next decade. If patient organisations speak up now, we can ensure:

- cross-border trial access is part of the new legal framework,
- research for rare and/or life-threatening diseases, including rare cancers and childhood diseases, gains stronger support,
- centres of excellence are strengthened, additional ones are built up and used effectively,
- and patient voices shape the future of innovation.

If patients remain silent, decisions may be made without considering their needs.

Now is the time to make your voice heard.