ERDERA European Rare Diseases Research Alliance

ERDERA POLICY BRIEF

EUROPEAN BIOTECH ACT

EXECUTIVE SUMMARY

Europe has world-class scientific research but continues to underperform in translating discoveries into biotech innovation. The proposed European Biotech Act[1] is a critical opportunity to close the gap and strengthen Europe's global competitiveness. Rare diseases are a strategic test case. They depend heavily on advanced biotechnologies and face acute unmet medical needs. At the same time, Europe's fragmented clinical, regulatory, and data ecosystems disproportionately hinder progress in this field.

The European Rare Diseases Research Alliance (ERDERA), together with the European Reference Networks (ERNs), provides the missing operational layer required to implement the European Biotech Act across the full innovation pathway —from discovery to patient access

1 Proposal for a regulation of the European Parliament and of the Council on establishing a framework of measures for strengthening Union's biotechnology and biomanufacturing sectors particularly in the area of health and amending Regulations (EC) No 178/2002, (EC) No 1394/2007, (EU) No 536/2014, (EU)2019/6, (EU)2024/795 and (EU) 2024/1938 (European Biotech Act): https://www.europarl.europa.eu/RegData/docs_autres_institutions/commission_europeenne/com/2025/1022/COM_COM(2025)1022_EN.pdf.

ERDERA and the broader rare disease ecosystem already operate infrastructures, networks, and expertise that align closely with the Biotech Act's objectives. ERDERA provides the platform both to serve the needs of the rare disease research and patient community and ensure the Biotech Act is effective, future-proof and impactful. By coordinating the capacities of multiple public and private stakeholders, ERDERA provides many of the capabilities the Act aims to build: pan-European clinical networks, trial-ready cohorts, interoperable data infrastructures, and translational expertise. The ERDERA research and innovation community works at the forefront of high-performance technologies such as gene therapies, RNA-based treatments, nanomedicine, and advanced therapy medicinal products (ATMPs).

Insights from ERDERA's Policy Think Tank nevertheless highlight critical gaps in the proposed Biotech Act, including insufficient recognition of existing infrastructures, an overly narrow definition of biotechnology, persistent translational bottlenecks, underdeveloped data frameworks, limited linkage between multinational trials and patient access, and insufficient attention to skills shortages.

To deliver impact, the Biotech Act must not inadvertently enable duplication of existing efforts but build on the combined ERDERA–ERN ecosystem as its operational backbone

WHY RARE DISEASES MATTER FOR THE EUROPEAN BIOTECH ACT

Rare diseases highlight both Europe's strengths and its structural weakness, which remains the translation of scientific discoveries into biotech innovation. They represent a significant opportunity to bridge this translational gap and provide sustainable competitive advantage across Europe.

Indeed, rare diseases are uniquely suited as test cases because of:

- High reliance on biotech innovation (gene therapy, RNA tools, ATMPs)
- Clear and measurable outcomes in diagnosis and treatment
- Structural exposure to fragmentation across clinical trials, data and regulatory systems

Rare diseases are not a niche issue, as they affect an estimated 446 million people globally, with profound health and societal impact.

Delivering impact in this field requires connecting all stages of the innovation journey —from early discovery to patient access. This is precisely where the combined strengths of ERDERA and ERNs become critical

THE STRUCTURAL GAP IN THE BIOTECH ACT

The Biotech Act establishes ambitious instruments—accelerators, centres of excellence, data infrastructures, and clinical trial reforms. However, a structural gap emerges: **the Act defines instruments but does not sufficiently anchor them in existing operational infrastructures.**

The risk is that new tools are created without fully leveraging mature European ecosystems such as ERDERA and ERNs, leading to duplication, fragmentation, and slower implementation.

Key gaps identified include:

1. **Insufficient recognition of existing infrastructures:** ERDERA, alongside ERNs, already offer a mature, connected ecosystem for multi-country clinical research, data interoperability, and collaboration between academia, industry, and patients.
2. **Narrow definition of biotechnology,** excluding key modalities such as antisense oligonucleotides (ASOs), nanomedicine and AI-enabled therapies.
3. **Persistent translational bottlenecks,** with fragmentation across the innovation pipeline.
4. **Underdeveloped data frameworks,** lacking clear standards for interoperability and AI-readiness.
5. **Limited linkage between multinational trials and cross-border patient access.**
6. **Critical skills shortages across the biotech value chain.**

Without leveraging existing infrastructures, the Biotech Act risks building new instruments without the operational substrate required for their success

FROM BENCH TO BEDSIDE: ERDERA AND ERNs AS THE INTEGRATED INFRASTRUCTURE OF THE BIOTECH ACT

Complementary roles across the innovation pathway

ERNs and ERDERA represent two complementary and interdependent layers of the European innovation ecosystem:

- → **ERNs provide the clinical and care infrastructure:** patient pathways, specialised centres, registries, and cross-border clinical expertise
- → **ERDERA provides the research and innovation infrastructure:** translational coordination, data integration, regulatory support, and innovation acceleration

Together, they form a continuous, integrated system capable of supporting the full innovation lifecycle.

Breaking silos across the innovation lifecycle

The combined ERDERA–ERN ecosystem enables a seamless pathway:

1. **Discovery and Target Identification:** ERDERA coordinates research programmes and data resources, while ERNs provide access to patient populations, registries, and unmet clinical needs.
2. **Preclinical and translational research:** ERDERA's Acceleration Hub supports project development, regulatory guidance, and industry engagement, while ERNs ensure clinical relevance and early validation.
3. **Clinical development:** ERNs provide multi-country trial sites, patient recruitment, and harmonised expertise, while ERDERA coordinates trial design, regulatory alignment, and funding instruments.
4. **Regulatory and HTA integration:** ERDERA embeds regulatory science throughout the research lifecycle, while ERNs contribute real-world evidence and clinical expertise.
5. **Patient access and post-market evidence:** ERNs deliver care and therapies across Member States, while ERDERA supports data collection, outcomes measurement, and continuous innovation.

Together, ERDERA and ERNs transform a fragmented innovation landscape into a continuous pathway from discovery to patient access

LEVERAGING ERDERA AS AN IMPLEMENTATION PLATFORM FOR THE BIOTECH ACT

ERDERA already operationalises, at scale, many of the core objectives of the Biotech Act and should therefore be explicitly recognised as a strategic implementation platform, **in coordination with ERN clinical networks.**

It provides a fully integrated framework that connects all stages of the research and innovation pathway —from early discovery to multinational clinical trials— thereby addressing fragmentation across the translational pipeline.

Through its Acceleration Hub, ERDERA identifies high-potential projects, delivers tailored scientific and regulatory support, and links them to funding opportunities, **while leveraging ERN clinical expertise and patient networks.**

ERDERA also advances platform technologies such as ATMPs and other emerging modalities, while working with ERNs to ensure clinical deployment and patient access.

A defining feature of ERDERA is the integration of regulatory expertise throughout the research lifecycle. Its Expertise Service Hub ensures early alignment with regulatory requirements, while collaboration with the European Medicines Agency is embedded within its clinical development model.

The ERDERA Data Services Hub integrates and upgrades existing European data infrastructures, including ERN registries, towards interoperability, FAIRness, and regulatory-grade quality. This enables federated data analysis across Europe.

Education and training are embedded across all ERDERA activities, complementing ERN capacity-building efforts. Crucially, ERDERA is anchored at the national level through National Mirror Groups, ensuring translation into national health systems, while ERNs ensure cross-border clinical integration and delivery.

Taken together, ERDERA and ERNs already deliver the key ambitions of the Biotech Act: accelerating innovation, integrating regulatory pathways, leveraging existing infrastructures, and strengthening Europe's global competitiveness

POLICY RECOMMENDATIONS

1. **Recognise the ERDERA-ERN ecosystem as the operational backbone of the Biotech Act**. Embed both ERDERA and ERNs into the Act's governance structures and implementation mechanisms, ensuring they are recognised within the European Health Biotechnology Steering Group; the identification and selection of strategic projects; and in the implementation of ATMP Centres of Excellence.
2. **Future-proof the definition of biotechnology**. Expand the scope to include RNA-based therapies, nanomedicine, and AI-enabled technologies.
3. **Reduce translational bottlenecks.** Promote early regulatory engagement, align incentives across stakeholders, support continuous collaboration between research, clinical, and industrial actors, and create "connecting" instruments and funding which enable fluid transition between different stages of the innovation journey.
4. **Strengthen data infrastructures.** Support development of interoperable, federated, AI-ready data frameworks and standards (e.g. ORPHA codes for rare diseases), integrating ERDERA data services and ERN registries.
5. **Promote equitable multinational clinical trials.** Leverage ERNs for trial coordination and ERDERA for trial readiness and support, ensuring access across Member States and beyond.
6. **Establish a pan-European skills and training initiative.** Build on ERDERA and ERN training programmes to address critical workforce gaps.
7. **Strengthen investment conditions for EU biotech.** Leverage ERDERA infrastructures and ERN clinical networks to de-risk development and improve capital attraction.

CONCLUSION

The success of the European Biotech Act will depend not only on new instruments, but on its ability to leverage existing European infrastructures.

ERDERA and the European Reference Networks together provide a comprehensive, integrated ecosystem spanning the full pathway from research to care. They offer the EU an immediate and scalable platform to operationalise the Biotech Act's ambitions.

Policymakers should position this combined ecosystem as an execution tool for the Biotech Act. By doing so, the EU can deliver meaningful improvements in patient outcomes, strengthen its translational capacity, and establish rare diseases as a flagship success of the European biotech ecosystem.

BIOTECH ACT INSTRUMENT

	What it requires to function	ERN Contribution (Clinical & Care Layer)	ERDERA Contribution (Research & Innovation Layer)	Combined impact (Bench → Bedside)
Development Accelerators	Translational research sites, GMP capacity, access to patient cohorts	Network of specialised centres, access to patients, clinical validation environments	Network of translational research sites linked to GMP facilities in multiple EU countries; Acceleration Hub identifying projects, linking to funding, supporting translation	Seamless transition from discovery to clinical validation
Centres of Excellence (ATMPs)	Integrated research, manufacturing, clinical delivery	Hospitals delivering advanced therapies, cross-border care pathways	Standardisation, regulatory streamlining, support for existing (e.g. in vivo gene therapies) and emerging modalities (e.g. ASOs)	Faster and safer deployment of advanced therapies
AI Testing Environments	High-quality, interoperable datasets and validation cohorts	ERN registries, clinical datasets, phenotype–genotype expertise	Data Services Hub enabling FAIR, federated, AI-ready data infrastructures and models	Reliable AI-driven diagnostics and innovation at EU scale
Data Quality Accelerator	Harmonised, high-quality, interoperable health data	Existing rare disease registries and clinical data sources	Integration, standardisation, interoperability and regulatory-grade data readiness	EU-wide data ecosystem supporting research, regulation, and care
Multinational Clinical Trials	Multi-country sites, patient recruitment, harmonised protocols	Cross-border patient pathways, trial-ready cohorts, clinical expertise	Trial coordination, regulatory integration, centralised funding instruments	Faster, more efficient trials accessible across Member States
Regulatory Sandboxes & Support	Early regulatory engagement and adaptive pathways	Clinical expertise, real-world evidence, patient-centred outcomes	Embedded regulatory science through all stages of research, EMA alignment and collaboration from early stages	Reduced time-to-approval and increased predictability
Biotech Investment & Strategic Projects	De-risked pipelines, clinical validation, scalable platforms	Clinical infrastructure for validation and delivery across EU	Project identification, matchmaking, support for public–private partnerships	Increased investor confidence and EU biotech competitiveness
Skills & Capacity Building	Trained workforce across biotech value chain	Clinical training environments, specialised expertise	Training programmes, capacity building in regulatory science, data, innovation and patient partherships	Sustainable European talent pipeline
Patient Access & Uptake	Delivery systems, outcome tracking, real-world evidence	Cross-border care delivery through ERNs, long-term follow-up	Data collection, outcomes analysis, national implementation via NMGs	Equitable access and continuous learning health systems

ERDERA

European Rare Diseases
Research Alliance

Co-funded by
the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595. Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.