Rare Disease Moonshot's contribution to the Consultation on the EU Biotech Act

Introduction

The Rare Disease Moonshot welcomes the European Commission's ambition to develop a comprehensive EU Biotech Act. This initiative represents a timely opportunity to unlock the full potential of biotechnology as a strategic driver of health innovation, economic resilience, and scientific leadership in Europe. With biotechnology at the forefront of medical breakthroughs, green and digital transitions, and industrial competitiveness, the EU Biotech Act must aim to define a bold, forward-looking framework that empowers Europe's biotech community to deliver transformative impact.

The Rare Disease Moonshot is a multi-stakeholder initiative that brings together public and private actors in rare diseases, an area where biotechnology offers unparalleled promise. Rare diseases affect 30 million people in the EU, and over 80% have a genetic origin. Biotech innovation, including gene therapies, biologics, and molecular diagnostics, is central to delivering solutions. Yet, Europe's biotech potential remains underleveraged due to regulatory complexity, fragmented support structures, and limited pathways for scale-up and deployment.

The EU Biotech Act should be designed not as a patchwork of corrections, but as a forward-looking framework with structural enablers. It should reduce administrative barriers, simplify regulatory pathways, improve the interface between research and application, and ensure sustainable funding and production environments. Importantly, it must support innovation (and innovators) from end to end: from discovery to clinical validation, to manufacturing and patient access—particularly in areas of high societal need.

For the EU Biotech Act to deliver on this promise, it must:

- Establish a coherent policy architecture that supports biotechnology across its full lifecycle.
- Enable early-stage innovators through targeted support services and advisory mechanisms.
- Secure long-term public and private investment tailored to the biotech risk profile.
- Build and maintain data infrastructure to retain innovation in Europe.
- Align regulatory frameworks and health system incentives to foster adoption of biotech solutions.

The Rare Disease Moonshot is ready to contribute to this vision. Based on insights from our work across EU research, regulatory and policy initiatives, we put forward the following six recommendations for the EU Biotech Act.

Deliver regulatory simplification and flexibility, not layering

The EU Biotech Act should go beyond the scope of revising existing legislation and instead introduce a strategically focused framework that removes structural barriers to biotech development across sectors. Rather than layering or amending overlapping legal frameworks, it should address persistent horizontal challenges by promoting true regulatory simplification. This includes the introduction of user-friendly, harmonised guidance materials; standardised templates for regulatory submissions; fit-for-purpose, risk-based timelines; and experimentation spaces such as regulatory sandbox pilots to test innovative biotech approaches in a controlled, supervised manner.

Moreover, for advanced technologies such as ATMPs and other complex biologics, the EU Biotech Act should enable the creation of specialised, science-driven regulatory pathways that offer greater flexibility for data generation and evaluation. These pathways should include provisions for iterative data collection, adaptive licensing approaches, and stronger coordination mechanisms across the European Medicines Agency (EMA), Health Technology Assessment (HTA) bodies, and national competent authorities. Such frameworks should be fully aligned with scientific progress and patient need, providing biotech developers with regulatory clarity and reducing the time and cost to bring promising therapies to market.

Create hands-on, accessible support for biotech innovators and scale deployment across Europe

A dedicated EU biotech innovation interface is needed—providing early guidance, regulatory navigation, partnership facilitation, and access to legal and financial expertise. This should include a new Regulatory Science Platform and a Biotech Innovation Desk to serve as a coordinated entry point for biotech innovators, connecting them efficiently with relevant EU instruments, funding programmes, and regulatory bodies.

Notably, the European Commission has launched a new Biotech and Biomanufacturing Hub, as a key deliverable of its 2024 Strategy to boost biotechnology and biomanufacturing, to support innovative companies in scaling up their science-based solutions. While this initiative holds significant promise, it requires stronger communication, visibility, and outreach to biotech stakeholders across Member States. Innovators, particularly start-ups and small enterprises, need clearer guidance on how to access the Hub's services, how it links to other EU mechanisms, and what concrete support it offers in terms of technical, regulatory, and market readiness. To fully realise its potential, the Hub should be equipped not only with expert advisory services and access to facilities, but also with a structured framework for mentoring, matchmaking with investors, and tailored acceleration tracks. The Commission could complement this effort with funded training and coaching schemes to strengthen the capacity of biotech founders and leadership teams. These actions will ensure the Hub becomes an integral, widely used resource for biotech scale-up and translation across the EU.

Europe must also address the persistent leakage of biotech innovation abroad by creating the conditions necessary for full deployment and scale-up within the EU with dedicated support for transforming regional biotech clusters into globally competitive hubs. By embedding these hubs within the broader EU biotech infrastructure, the EU Biotech Act can ensure that breakthrough innovations are not only developed but also produced and commercialised in Europe.

Strengthen and diversify biotech financing tools

Financing remains one of the greatest unmet needs in EU biotech, particularly for SMEs, start-ups, and public-private partnerships operating in high-risk and high-need areas such as rare diseases. The EU Biotech Act should establish a dedicated European Biotech Innovation Fund, combining grants, convertible loans, and milestone-based payments to support early-stage development and translation. This proposal addresses the persistent funding gap that hampers the progression of biotech innovations from research to market—especially in fields with limited commercial returns but high societal impact.

This fund should be designed to complement and extend existing EU instruments, such as the Innovation Fund—which finances high-risk, large-scale clean tech—and the EIC Accelerator, which supports deep-tech SMEs and start-ups. Drawing from these models, the Biotech Innovation Fund should be tailored to the unique needs of biotechnology: including longer development timelines, high regulatory and scientific complexity, dependency on public infrastructure, and the lack of immediate return on investment. Such an instrument should offer flexible, risk-tolerant financial support that can catalyse both public and private co-investment, ensuring that promising innovations are not lost due to undercapitalisation. Its design should consider modular financing stages, performance-linked disbursement, and equity conversion options to adapt to diverse business models and maturity levels. Financial instruments should be structured to reward not only commercial viability but also public health and societal value, ensuring that biotech innovations addressing unmet needs receive the support required to achieve scale and sustainability.

Align incentives and expand regulatory support to link innovation to access

Europe must link upstream R&D support with downstream market-shaping and regulatory instruments to create a truly enabling environment for biotechnology development and deployment. The EU Biotech Act should foster demand for biotech solutions by introducing targeted procurement frameworks, health system integration pilots, and the inclusion of biotech products in joint clinical assessments and value-based reimbursement schemes. These tools are critical for translating scientific progress into real-world impact, particularly for high-need public health priorities.

Complementary push and pull mechanisms—such as early-stage funding, milestone-based investments, outcome-based pricing, and preferential procurement—are especially important in areas like rare diseases and advanced therapies, where traditional market signals are weak. These mechanisms help de-risk private investment and signal sustained public sector commitment, thereby accelerating the path from innovation to patient access. A coordinated and balanced mix of incentives can stimulate investment, support health system readiness, and reinforce Europe's capacity to lead in delivering cutting-edge biotech solutions.

At the same time, regulatory support must keep pace with scientific advancement. The EU Biotech Act should introduce a specialised pathway for ATMPs and other complex biologics that offers early scientific advice, protocol harmonisation, and flexible evaluation standards across jurisdictions. These

efforts should be supported by investments in trial infrastructure, bioanalysis platforms, and adaptive data systems.

Make PPPs a central delivery tool and resource them accordingly

Public-private partnerships must be embedded as structural enablers of biotech innovation, not project-by-project exceptions. The Biotech Act should include long-term operational funding for PPPs that deliver on EU health and competitiveness goals. This includes creating a European PPP Competence Centre hosted in ERDERA to support partnership design, IP frameworks, regulatory co-development, and stakeholder alignment.

Our recommendations

To ensure the EU Biotech Act delivers on its full potential, we recommend the following priority actions, aligned with the needs of the biotech ecosystem:

- **Define a forward-looking EU Biotech Act anchored in strategic enablers.** The Act should go beyond legislative adjustments to establish a coherent framework that proactively supports biotechnology development, deployment, and scale-up across sectors.
- **Simplify and modernise regulatory pathways for biotech innovation.** This includes flexible approval mechanisms for ATMPs and complex biologics, regulatory sandboxes, harmonised guidance documents, and coordinated scientific advice to accelerate clinical development and authorisation.
- **Equip biotech innovators with hands-on, integrated support mechanisms.** The EU should strengthen the visibility and functionality of the Biotech and Biomanufacturing Hub, establish a Biotech Innovation Interface, and provide advisory, legal, regulatory, and acceleration services tailored to SMEs and start-ups.
- **Enable deployment through investment in biotech infrastructure.** EU funding should prioritise the development of regional biotech clusters to secure industrial resilience and strategic autonomy.
- **Introduce a dedicated European Biotech Innovation Fund.** Designed to complement existing instruments like the Innovation Fund and EIC Accelerator, it should provide financing to support early-stage and translational biotech, especially in high-need, low-return areas like rare diseases.
- **Align innovation incentives with market access through coordinated push and pull tools** to reward biotech solutions for their societal value and patient impact.