

European Alliance for Transformative Therapies (TRANSFORM)

Position Paper on the EU Biotech Act I: *Strengthening Europe's framework for ATMPs*

TRANSFORM welcomes the European Commission's EU Biotech Act I as a timely and ambitious initiative to reinforce Europe's competitiveness, resilience and leadership in health biotechnology. The proposal provides a strong foundation to support innovation, investment and scale-up across the biotech ecosystem, including advanced therapy medicinal products (ATMPs).

However, given the scientific complexity, long development timelines and high upfront investment associated with ATMPs, TRANSFORM believes the proposal could be further strengthened in several key areas to fully deliver on its objectives.

A recurring challenge identified across stakeholders is the effective and consistent implementation of EU frameworks across Member States. **Therefore, greater emphasis should be placed on**

- **Strengthening implementation capacity across Member States,**
- **Reducing fragmentation through improved coordination,**
- **Ensuring that Europe's clinical, regulatory and data infrastructures are equipped to support the development and delivery of ATMPs at scale.**

This should also include facilitating the transition from academic research to medicinal product development, including by simplifying the environment for smaller developers and those emerging from academia, which play a critical role in early-stage innovation.

Addressing these gaps will be critical to translating Europe's scientific leadership into real-world patient access to transformative therapies.

Strengthening patient and stakeholder involvement

TRANSFORM calls for stronger provisions to embed meaningful patient involvement across the ATMP lifecycle, from research and clinical development to regulatory decision-making and access. The Act should foster structured multi-stakeholder dialogue, involving patients, clinicians, researchers, regulators, HTA bodies and payers. This approach is essential to ensure that biotech innovation remains patient-centred, clinically relevant and socially sustainable.

Accelerating and simplifying clinical trial authorisation

To be effective, **regulatory simplification should be accompanied by the necessary investments to ensure that authorities and clinical ecosystems have the capacity and resources required to meet the new timelines and coordination demands.** Streamlining and acceleration of clinical trial processes should therefore be matched with adequate resourcing,

expertise and infrastructure to ensure quality, patient safety and effective implementation across Member States. Indeed, **faster clinical trial approvals should not come at the expense of patient safety or high-quality ethical review.** Accelerated procedures will therefore require increased staffing and resources for hospitals and national authorities, to ensure they can meet these enhanced demands.

Member States should prioritise the implementation of the new rules and work closely with stakeholders to strengthen Europe's clinical trial ecosystem. Despite the progress made, capacity and knowledge gaps related to ATMPs persist across Member States, continuing to slow down clinical development and patient access to these life-changing therapies. This will be essential to address persistent fragmentation and inefficiencies that continue to delay trial initiation and discourage sponsors from conducting ATMP clinical trials in Europe.

In this context, **TRANSFORM underlines the importance of facilitating and actively enabling cross-border clinical trials across ATMPs,** given that patient populations are small and geographically dispersed across Member States.

Establishment of ATMP Centres of Excellence

TRANSFORM strongly supports the establishment of ATMP Centres of Excellence (CoEs) as a practical mechanism to strengthen Europe's capacity across the ATMP lifecycle. CoEs represent a tangible way to translate the objectives of the Biotech Act into operational capacity on the ground. As such, **CoEs should be interconnected to foster collaboration and knowledge sharing, improve clinical trial readiness and delivery, training and workforce development, and facilitate cross-border collaboration and patient access.** As such, the CoEs should also play a key role in fostering cross-therapeutic learnings and disease understanding.

CoEs should build on and integrate with existing specialised clinical networks and comprehensive care centres to ensure continuity of care and efficient use of existing expertise. In this context, TRANSFORM highlights the important role that European Reference Networks (ERNs) can play in supporting the ATMP CoEs. Leveraging the expertise, patient pathways and cross-border collaboration mechanisms of ERNs can help ensure that CoEs are closely connected to existing networks of clinical excellence and patient communities across the EU.

TRANSFORM also notes the relevance of the **EU Health Biotechnology Investment Pilot** foreseen by the Act as an important instrument to facilitate access to funding, de-risk innovation and support capacity-building across the ecosystem. This **should be complemented by strengthened and well-coordinated EU-level funding and support mechanisms to ensure that Member States can build capacity at a comparable pace and effectively implement the objectives of the Act.** Alignment between the Investment Pilot, CoEs and other support instruments will be essential to maximise impact and ensure coherence.

Finally, **CoEs should play a valuable role in supporting the generation and responsible use of real-world evidence (RWE), including through structured data collection and long-term patient follow-up in routine clinical practice.** Strengthening system capacity to generate high-quality RWE can support regulatory decision-making, post-authorisation learning and, where relevant, access and value discussions, while respecting data protection and governance requirements.

In this context, greater support for real-world data collection and registries, including at European level, would benefit both developers and health systems and enable better-informed decision-making across the lifecycle, pre- and post-marketing authorisation. It is particularly important to ensure robust governance frameworks and to recognise the role of independent, clinician-led registries in generating high-quality, sustainable and trusted evidence across the ATMP lifecycle.

Creation of an EU Health Biotechnology Support Network

TRANSFORM welcomes the proposal to establish and coordinate an EU Health Biotechnology Support Network, bringing together expertise across regulators, developers, clinicians, researchers and patients. **The Network should also include healthcare professionals involved in the preparation, handling and delivery of ATMPs, such as hospital pharmacists, reflecting the multidisciplinary nature of these therapies.**

The Network should play an important role in facilitating the exchange of best practices on ATMP preparation, handling, administration and long-term patient management, as well as supporting the development of operational standards and guidance for clinical centres. A well-designed network could support developers navigating complex regulatory pathways, promote best practice exchange across Member States, and reduce fragmentation in implementation.

Streamlining and simplifying regulatory frameworks

TRANSFORM supports the Act's ambition to reduce duplication and improve coherence across interlinked regulatory frameworks affecting biotechnology and ATMPs. Efforts to improve alignment between pharmaceutical, clinical trial, GMO, medical device regulations, as well as relevant data governance frameworks are particularly welcome. **Ensuring that these frameworks operate in consistent and mutually reinforcing way will be critical to provide legal certainty and a predictable environment, ultimately supporting Europe's competitiveness in global R&D.** In addition, expanding the use of regulatory sandboxes at EU and national level is also a positive signal. Regulatory sandboxes can provide flexibility, legal certainty and regulatory learning for innovative therapies that do not fit existing pathways, while maintaining high standards of patient safety and quality.

A proportionate approach to environmental risk assessments for ATMP clinical trials

Finally, **TRANSFORM supports the proposal to exempt sponsors of ATMP clinical trials from routine environmental risk assessments (ERA) where science has demonstrated no risk for the environment.** While maintaining robust biosafety and risk assessment standards where scientifically justified, this change will help Europe be more attractive for R&D for ATMPs across the entire ecosystem, strengthen Europe's role as a scientific leader. ATMPs are manufactured and tested in highly controlled clinical settings, under strict biosafety protocols, and involve organisms that, in many cases, do not persist in the environment. Considering other regions, such as the US, do not mandate an environmental risk assessment at clinical stage, the current rules only contributing to moving R&D and clinical trials outside Europe. **A proportionate, science-based exemption as proposed would reduce administrative burden, accelerate trial initiation and improve the overall efficiency of ATMP development in the EU.**

About the European Alliance for Transformative Therapies (TRANSFORM)

The European Alliance for Transformative Therapies (TRANSFORM) is a multi-stakeholder alliance that connects Members of the European Parliament (MEPs) and policymakers with patient groups, medical experts and associations, scientists, researchers, industry actors, networks and other relevant stakeholders. TRANSFORM aims to foster effective dialogue and provide evidence-based policy recommendations to enable safe and timely patient access to cell and gene therapies, whilst ensuring the sustainability of healthcare systems.

TRANSFORM members include:

- **CCI Europe – Childhood Cancer International Europe**
- **EAHAD – European Association for Haemophilia and Allied Disorders**
- **EAHP – European Association of Hospital Pharmacists**
- **EFNA – European Federation of Neurological Associations**
- **ESGCT – European Society of Gene and Cell Therapy**
- **EUCOPE and its members: AOP Health, CSL Behring, Medac, Miltenyi Biomedicine, Orchard Therapeutics, PTC Therapeutics, Regeneron and Santen.**
- **EHC – European Haemophilia Consortium**
- **EPTRI – European Paediatric Translational Research Infrastructure**
- **EURORDIS – Rare Diseases Europe**
- **GBS-CIDP Foundation International**
- **IFCCA – International Federation of Crohn’s & Ulcerative Colitis Associations**
- **IPOPI – International Patient Organisation for Primary Immunodeficiencies**
- **Lymphoma Coalition**
- **reNEW Consortium Stem Cell Medicine**
- **RI – Retina International**
- **SIOP Europe – the European Society for Paediatric Oncology**
- **TIF – Thalassaemia International Federation**
- **WDO – World Duchenne Organization**

The European Medicines Agency (EMA), the European Haematology Association (EHA) and the European Cancer Organisation (ECO) are Observers.

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