Preparing European health systems for Advanced Therapy Medicinal Products (ATMPs)

European Alliance for Transformative Therapies

ATMPs are medicines based on genes, tissues or cells.

They offer groundbreaking new opportunities for the treatment of disease and injury.

Despite health being a national competency, there are EU-level regulatory and access hurdles that hinder safe and timely ATMPs delivery to patients.

Collaboration between Member States and European level discussions can significantly impact patient access to ATMPs in Europe.

TRANSFORM is a multi-stakeholder Alliance working to improve timely and safe access to ATMPs in the EU.

TRANSFORM develops and shares evidence-based recommendations to facilitate access to ATMPs.We connect and foster effective dialogue between stakeholders and policymakers to ensure the unique characteristics of ATMPs are understood and considered in policy-making.

In 2022, TRANSFORM developed a Charter split into 7 main policy asks. The recommendations cover the entire lifecycle of ATMPs (from research to administration) to support safe and timely patient access to them.

The Charter was supported by Members of the European Parliament of the 2019-2024 mandate and launched in the European Parliament.

The EU possesses many tools that can be leveraged to improve the development, authorisation, access, and delivery of ATMPs.

The EU has many regulations in place impacting the ATMP environment. TRANSFORM has suggestions to further improve these in line with our mission to improve patient access to ATMPs.

For more information, visit transformalliance.eu

What can the EU do to support Member States' healthcare systems and facilitate safe and timely access to ATMPs?

- **Include the patient and caregiver perspective** throughout ATMPs' development pathway (from early research to value assessment)
- **Have a collaborative research and development ecosystem** where stakeholders (patients, healthcare professionals, research organisations, and industry):
  - Set research priorities
  - Identify effective incentives to promote innovation in areas of medical need
- **Full implementation and adherence to the Clinical Trials Regulation in all Member States** to increase Europe's attractiveness for innovation and development
- **Ensure that the General Pharmaceutical Legislation (GPL) is future-proof** and can support the development and authorisation of highly innovative technologies, like ATMPs, so that patients can benefit from timely access to these therapies

- **Extend the role of European Reference Networks (ERNs)** so they can support Member States' best practices and set up simple and standardised national and cross-border pathways
- **Strengthen national contact points** for more harmonisation and better access to information
- **Allocate financing** for cross-border travel expenses for ATMP treatments

- **Allow for earlier and enhanced EMA scientific and regulatory support**
- **Create a flexible regulatory framework** that can adapt to future innovations in health
- **Ensure the EMA has the appropriate resources** to fulfill its wide range of responsibilities

- **Ensure effective cross-border cooperation** for timely and effective access to ATMPs in centres of excellence
- **Leverage learnings** from regional collaborations
- **Support national governments** to share learnings on novel payment models and funding approaches for ATMPs

- **Collect real-world data** with the involvement of healthcare professionals and patients to address the uncertainties on the long-term safety and effectiveness of ATMPs
- **Ensure alignment between regulators, health technology assessment (HTA) bodies, and payers** on requirements for real-world evidence generation and use

- **Develop EU guidelines** on newborn screening, genetic testing, and diagnostics, which can be included in a potential European action plan on rare diseases
- **The ERN Expert Platform for Newborn Screening** could support this
- **Position the EU as the central point** for information sharing on good practices from existing national programmes on newborn screening and genetic testing

- **Ensure a fit-for-purpose system to support the effective implementation of the HTA Regulation** starting in **2025** when the first assessments for ATMPs become mandatory
- **Conduct early Joint Scientific Consultations (JSCs) in a multistakeholder dialogue** so the advice given can support evidence-generation plans
- **Avoid duplication** between EU and national assessments and/or requirements
- **Conduct assessments efficiently** with methodologies that value ATMP specificities