Advanced Therapy Medicinal Products (ATMPs) and their groundbreaking potential

European Alliance for Transformative Therapies

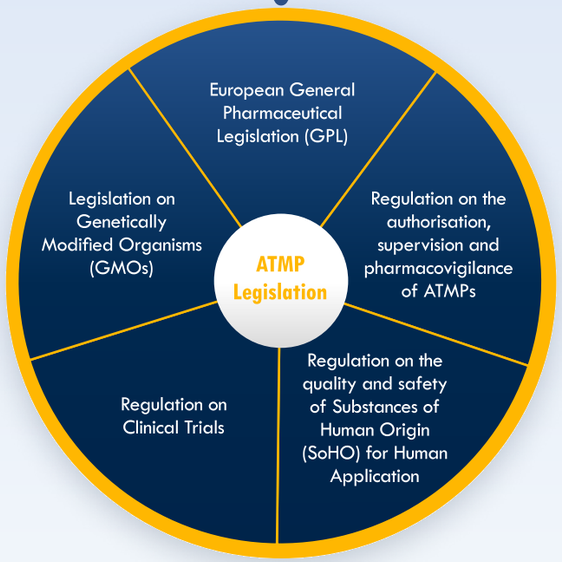
What are ATMPs?

In the EU, there are 3 categories:

ATMPs are innovative, biological medicines that significantly differ from chemical-based medicines and face many challenges in their development and clinical application.

Regulatory framework for ATMPs

Because of their complexity ATMPs are regulated by several pieces of European legislation.



Gene Therapy

Human genes define our physical and biological traits but also give instructions to build proteins and maintain normal cell functions.

When one of these genes is missing or does not work properly, it can cause disease.

Gene therapies use genetic material to treat or prevent disease. Most gene therapies seek to limit the impact of a faulty gene by modifying, replacing or inactivating specific genetic material. Gene editing is a type of gene therapy, which enables scientists to make changes to DNA within a gene.

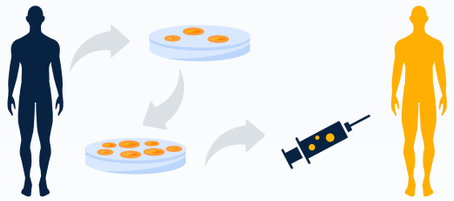
Somatic Cell Therapy

Cell therapy centres around replacing or regenerating human cells, tissues or organs to restore the function of damaged or lost cells/organs.

Cell therapy can be either allogeneic (produced from a donor) or autologous (utilises the patient's own cells).

Allogeneic cell therapies derive from a source other than the patient, such as a healthy donor. In this case, the manufacturing process is not individualised, and this cell therapy can be produced at scale and distributed worldwide.

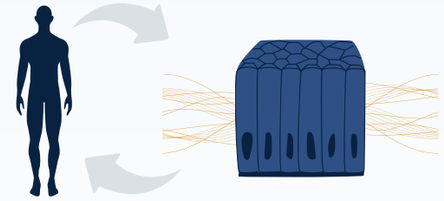
Autologous cell therapy derives from the same person who receives the treatment and is, therefore, an individualised treatment that needs to be produced close to the patient.



Tissue Engineering

Tissue engineering combines a scaffold, i.e., a structure on which tissue, cells and other active molecules are grown and then placed onto a person's functional body tissue.

This process is used to repair, regenerate or replace human tissues or organs.



Approved ATMPs in the EU

→ **7** out of 26 withdrew or did not renew their marketing authorisation showing clear difficulties in bringing these therapies to patients.

These therapies are used in a variety of medical areas, such as:

- **Nervous system**
- **Ophthalmology**
- **Neuromuscular system**
- **Blood and bone marrow cancers**
- **Inflammatory diseases**

Hundreds are currently being developed in other disease areas including those listed above and cardiovascular diseases and endocrine or metabolic disorders.

About TRANSFORM

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

TRANSFORM aims to facilitate discussions between stakeholders and policy-makers to:

- collaborate in developing and disseminating evidence-based recommendations to enable patients safe access to cell and gene therapy.
- Inform MEPs about the specificities of these transformative therapies to shape policy.
- Prepare healthcare systems for the adoption of these therapies.

In its Charter supported by Members of the European Parliament of the 2019-2024 mandate, TRANSFORM identified recommendations in 7 policy areas to streamline ATMP development and enable patient access.

Find out more about TRANSFORM at transformalliance.eu

TRANSFORM takes a holistic, lifecycle approach

to address the existing difficulties and challenges in the ecosystem

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Challenges		TRANSFORM proposed solutions
ATMPs are at the forefront of innovation, offering treatment options for diseases that currently have few or no effective treatment options. ATMPs face several challenges in their clinical development due to the nature of the product, the limited availability of non-clinical data and their mode of administration. The gold standard of a controlled, randomised, clinical trial may not be feasible or ethically justified for all indications, particularly in life-threatening diseases, where there is no satisfactory standard of care. This makes it harder to generate data for licensing and reimbursement and to showcase these medicines' full potential.	**Research & Clinical Development**	• Ensure that the Clinical Trials Regulation is fully implemented and adhered to in all Member States. • Allow for early dialogue with authorities and bodies active along the lifecycle of medicinal products (e.g. regulatory agencies and payers) to enable better clinical trial design and collection of relevant data. • Consider risk-based circumstances in which a complete derogation from the GMO legislation for therapeutic ATMPs may be appropriate.
ATMPs' specificity requires adapted regulatory and pharmacovigilance standards. To this end, ATMPs require an appropriate regulatory framework that balances benefits and risks while considering the specificities of these innovative technologies.	**Regulatory Approval & Oversight**	• Strengthen the patient voice in the regulatory process by introducing a patient representative on the EMA Committee for Medicinal Products for Human Use. • Allow for earlier and enhanced EMA scientific and regulatory support. • Support early dialogue, such as through the PRIME scheme. • Enable alternative regulatory approaches, such as use of the 'Regulatory Sandbox' approach proposed in the Pharmaceutical Package.
ATMPs are personalised and innovative medicines with complex manufacturing processes that include specific standards for collecting starting material, product storage, and administration, resulting in high up-front costs. When the European Medicines Agency (EMA) approves a medicine, it does not automatically mean patients can access it. Member States conduct their own value assessment and pricing & reimbursement discussions with the therapy developer. Due to the type of target diseases, the inherent complexity of these products, and their accelerated developments, less comprehensive clinical data might be generated. This needs to be taken into account by regulatory bodies and health authorities when assessing the value of these therapies.	**Value Assessment**	• Support the uptake of innovative payment models like risk-sharing agreements and annuity-based models between payers and innovators to balance up-front costs and the uncertainty of effects duration. • Enable the use of real-world evidence to address uncertainties for ATMPs, for example with greater alignment between regulators, HTA bodies and payers on evidence generation requirements and earlier involvement of patients and clinicians in these discussions.
ATMPs are potentially one-off treatments with lasting therapeutic effect, often administered in state of the art medical centres. Unfortunately, not all Member States have the medical infrastructure and trained personnel for the preparation, administration and follow-up of ATMPs.	**Access**	• Invest in system capacity and training for these innovative therapies across the EU. • Remove barriers for patients to travel to centres of excellence to access ATMPs, for example by removing administrative and economic burdens that hinder cross-border access. • Leverage existing specialised infrastructure such as European Reference Networks (ERNs).

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