European Alliance
for Transformative
Therapies

From Breakthrough to Access: Delivering ATMP Value through EU Cooperation

Informal roundtable on ATMP access – success stories, bottlenecks and opportunities for coordinated action

27 October 2025

Summary Event Report

Held under the Chatham House rule

Executive Summary

On 27 October 2025, **MEP Carlo Ciccioli** (ECR, Italy) hosted an online round table for TRANSFORM, the European Alliance for Transformative Therapies (TRANSFORM), bringing together close to 40 Alliance Members, including patients, healthcare professionals, academics and industry.

The objective of the discussion was to hear from Italian stakeholders on their national ecosystem and identify opportunities for coordinated action to boost Europe's ability to develop and deliver medical technologies such as Advanced Therapy Medicinal Products (ATMPs).

Key messages of the discussion include:

- The EU needs to create stable and predictable frameworks that will attract investment and embed real-world evidence into ATMP assessment.
- The narrative around ATMPs needs to change. These breakthrough therapies need to be recognised as long-term investments that deliver lasting healthcare and economic value and not be seen as short-term cost.
- To improve access, we need to foster collaboration across all stakeholders and build an EU-wide specialised network to accelerate innovation and overall progress.
- Outcome- and instalment-based payment models, and a better use of EU accounting rules are crucial in incentivising innovation.
- There is a strong need to develop new assessment tools for ATMPs, to ensure the unique characteristics of ATMPs are duly recognised.
- Patients must be at the centre of the ecosystem and consulted throughout the lifecycle of ATMPs. The EU also must ensure that the concept of "unmet medical need" truly leaves no one behind.
- New and flexible production models, such as hospital-based and point-of-care manufacturing, combined with investment into existing and new infrastructure can improve the entire ecosystem.

Participants agreed that EU legislations, such as the General Pharmaceutical Legislation or EU Biotech Acts, were an opportunity to build a world-class ATMP ecosystem in the EU. Building such an ecosystem is critical to improve patient access and boost EU life sciences competitveness. The time to act is now.

The work of the TRANSFORM Secretariat is enabled by funding from the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) and its members BioMarin, Bristol Myers Squibb, CSL Behring, Gilead Sciences, Medac, Miltenyi Biomedicine, Orchard Therapeutics, PTC Therapeutics, Regeneron, Santen and Vertex.

Introduction

Patients in the EU face barriers in accessing ATMPs, largely due to a fragmented regulatory and appraisal system, as well as challenges with integration into national healthcare systems. ATMPs and the life science sector more broadly have become EU strategic priorities. The coming years represent a turning point for Europe's ability to leverage its scientific excellence into life-changing therapies.

The Draghi report on EU competitiveness highlights a particular weakness in the pharmaceutical sector, especiallly in the area of advanced therapies. It estimates the global market value of ATMPs at €8 billion, but notes that Europe is falling behind. Contributing factors include lower capital investments, siloed research, and a slow, fragmented regulatory landscape. Notably, the report points out that obtaining marketing authorisation in the EU takes, on average, 100 days longer than in the US. Current EU legislative initiatives offer a chance to address some of these shortcomings.

The ongoing revision of the **General Pharmaceutical Legislation** (GPL), and the announced **EU Biotech Acts** aim to modernise the framework and contribute to a vibrant ecosystem. Discussions around the next **Multiannual Financial Framework** (MFF) present an opportunity to channel more funding toward the development and adoption of ATMPs and advanced biotechnologies more broadly.

Participants stressed **the need for predictable, patient centered regulation and dedicated funding to support innovation and competitiveness in life sciences**. While the revision of the GPL marks a step forward, persistent challenges – such as regulatory fragmentation and a lack of support for translational research – continue to hold the sector back. The proposed EU Biotech Acts represents a critical opportunity to address these gaps, mobilise venture capital, and better support the translation of research into clinical application.

Challenges and opportunities in advancing ATMPs in Europe

Learning from Italy

The discussions showed how Italy effectively utilises initiatives to overcome the challenges affecting access, and research and development of ATMPs. By **recognising ATMPs as long-term investments instead of short-term costs,** Italy has demonstrated to the rest of the EU that these therapies can present a return on investment and createa more sustainable environment for the development and access to ATMPs.[1] The effectiveness of this change in narrative should be seen as a call to the EU to learn from Italy's approach, as ATMPs are essential in strengthening Europe's competitiveness.

European Parliament Perspective

Member of the European Parliament welcomed participants and voiced his strong support for TRANSFORM and its objectives. **While Europe excels in science, it lacks the ability to scale up** due to fragmented regulation, slow processes, and weak investments. To tackle this, he called for the strengthening of research, infrastructure, talent retention, and real-world data use, while fast tracking innovation, harmonising frameworks, and improving reimbursement of investment schemes and access across all Member States

The transformative value of ATMPs

ATMPs offer strong value for patients, healthcare systems, and society. They represent a chance for patients, changing how diseases are treated by targeting the root causes. By improving patient outcomes and reducing disease burden, **ATMPs can generate long-term savings to contribute to healthcare sytems while strenghtening Europe's life sciences ecosystem.** For the rare disease community, the medical journey is often long and uncertain, with diagnoses often taking several years. ATMPs change that narrative, offering

[1] In September 2025, the President of the Italian Senate's Health Commission, Sen. Franco Zaffini, announced the filing of a draft law establishing a dedicated national fund for Advanced Therapy Medicinal Products (ATMPs). The proposal provides for a €250 million allocation over five years to support R&D, strengthen domestic manufacturing capacity, and facilitate patient access through outcome-based reimbursement mechanisms and a dedicated funding pathway. Parliamentary discussion is expected to begin in 2026. See here.

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real, tangible hope. Significant advancements include CAR-T therapies, which have resulted in lasting remission for children with relapsed leukemia, and gene therapies that have recently achieved a breakthrough in treating Huntington's disease.

Redefining the narrative

ATMPs are not only clinical achievements, they are life-changing interventions that impact patients, their families, and entire communities.

Yet, despite their promise, too few ATMPs in the EU reach the people who need them. In some cases, therapies are withdrawn after approval – not due to clinical failure, but because of reimbursement hurdles, fragmented regulation, or lack of commercial viability.

ATMPs should be regarded as long-term investments, supported by payment schemes that allow the recovery of high development costs despite the limited number of patients eligible for these treatments. The Italian Pilot programme, which defines ATMPs as strategic assets, should here set a benchmark. It showed that successful treatment achieves a full return on investment in 5-7 years. This return may be even quicker if indirect costs are taken into account. This demonstrates the importance of looking beyond clinical endpoints to also consider individuals' capacity to resume academic or occupational activities and participate in social life. Now is the time to reconsider how we measure and define the success of advanced therapies.

Challenges for optimal ATMP uptake

Speakers agreed that Europe's regulatory and healthcare systems are not yet adapted to the realities of advanced therapies.

ATMPs are complex and costly to manufacture. Their production requires sterile environments and highly skilled operators, making production economically difficult for small or ultra-rare diseases. This creates a context where the application of **EU regulatory frameworks for medicines faces significant challenges when applied to ATMPs**. Fragmented national infrastructures and a lack of specialised treatment centres further limit patient access. Combined with a still limited expertise, the absence of fit-for-purpose assessment tools, and insufficient patient involvement means Europe runs the risk of missing out on breakthrough innovation.

Opportunities for change

The ongoing revision of the EU pharmaceutical legislation and the Biotech Acts offers an opportunity to modernise the existing frameworks.

To fully make use of this situation, the discussion showed that we need **complement our existing capacities** with more flexible production capacities, such as **point-of-care manufacturing**, while investing in **novel capacities**. We need to make use of regulatory sandboxes to test new ideas and fully leverage the flexibility under the Stability & Growth Pact to support patient access to ATMPs and the entire ecosystem. By incoporating real-world evidence and patient-reported outcomes into clinical trials, we could better show the value of advanced therapies. Italy's national Pilot Programme based on a Model for Enhancing Healthcare Innovation Accessibility elaborated by the Minitry of Health and which defines ATMPs as strategic assets, has here already shown clear benefits for healthcare systems.[2] Additionally, we now have the opportunity to **strengthen the cooperation** between authorities, industry, and patients together with better use of existing cross-border networks.

[2] The pilot programme was presented during the Press Conference on the 2025 Activities of the Parliamentary Intergroup for Sustainable Innovation in Healthcare held on 27th February.

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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: BioMarin, Bristol Myers Squibb, CSL Behring, Kite – a Gilead company, Medac, Miltenyi Biomedicine, Orchard Therapeutics, PTC Therapeutics, Regeneron, Santen and Vertex.

EHC – European Haemophilia Consortium

EPTRI – European Paediatric Translational Research Infrastructure

EURORDIS – Rare Diseases Europe

GBS-CIDP Foundation

IFCCA – International Federation of Crohn and Ulcerative Colitis Association

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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