MIND THE GAP!

How to Pave the Way to Access to Authorised ATMPs for Patients?

Event hosted by the MEP Interest Group on Transformative Therapies

31 March 2022

#MEPsforATMPs

European Alliance for Transformative Therapies

EVENT REPORT

Executive Summary

On 31 March 2022, the TRANSFORM MEP Interest Group held an online event with the TRANSFORM Alliance and other selected guests, representing patients, clinicians, research institutes, academia, payers, HTA bodies, pharmaceutical industry and EU institutions.

The event launched the TRANSFORM Alliance's Policy Asks on Access to Authorised ATMPs. These Policy Asks represent the current thinking of the TRANSFORM Alliance as of March 2022, and will be further refined and developed in light of external policy developments, culminating in a final and integrated set of Asks in a Charter to be launched in October 2022.

During the event, solutions to improve access to authorised ATMPs were discussed in both panel and workshop sessions. Attendees spoke of the need to adapt the Health Technology Assessment (HTA) process to ATMPs, and the need for more early, iterative dialogues that include all stakeholders (including patients and clinicians) to support the use of real-world evidence (RWE) to reduce uncertainties inherent in ATMPs. Outcomes-based payment models were presented as the 'gold standard' solution for reducing the upfront cost for ATMPs, but also reducing uncertainty of the long-term effect of a therapy. The importance of newborn screening and genetic testing was also raised.

Speakers noted that political will among Member States for more EU-aligned solutions to access challenges for ATMPs will be key to success. There was recognition that policymakers must ensure the EU remains attractive to developers – for example, by ensuring the access pathway for these innovative therapies is more streamlined and predictable.

Opening speeches

Co-creating optimal solutions for equitable and sustainable access to ATMPs

Alexandra Moretti MEP (S&D, IT), Co-Chair of the TRANSFORM MEP Interest Group, highlighted:

- The benefits offered by ATMPs are enormous, but there is concern that the EU does not have the tools to look at the impact and benefit of ATMPs on healthcare systems, such as their savings and value.
- We should not be afraid to acknowledge that ATMPs are expensive, and this is challenging particularly for smaller countries. The EU has collective purchasing power, and there should be more transparency over the cost of developing therapies and of the negotiations by national governments with developers.
- At the same time, decision makers need to foster a better environment for research in the EU.

Opportunities and challenges in rewarding innovation in Europe

Alexander Natz, Secretary-General of EUCOPE, highlighted:

- It is vital that developers can bring ATMPs to Europe.
- The existing ATMP legislation is fit for purpose; rather, stakeholders need to work with national payer systems to ensure access to ATMPs.
- ATMPs are one-time therapies, with one-time upfront costs. This can overburden healthcare systems, but the solution is the use of real-world evidence (RWE). RWE can remove uncertainty from the system, and the European Health Data Space (EHDS) presents an opportunity to support the use of RWE.
- At the national level, we need to be using outcomes-based payment models. These payment models should be seen as positive because developers are committing to the performance of their own products.

Legislative solutions to patients' challenges in accessing ATMPs

Radka Maxová MEP (S&D, Czechia), TRANSFORM Member, highlighted:

- Now is an opportune moment for the European Parliament to address legislation that touches on ATMPs.
- For rare diseases, access issues are caused by a lack of information and the burden of upfront payment for therapies.
- This year, MEP Maxová co-authored a written Parliamentary question, asking if the European Commission intends to include the introduction of universal new-born screening in the revision of the OMP Regulation, and asking whether it will develop an action plan for rare diseases by 2023.[1]

Panel Discussion – Perspectives on Rearising Safe and Timely Patient Access to Authorised ATMPs in Europe

Jo De Cock, Administrateur Général Honoraire, INAMI-RIZIV
Simone Boselli, Public Affairs Director, EURORDIS-Rare Disease Europe
Dr Wim Goettsch, Special Advisor HTA, Dutch National Health Care Institute (ZIN)
Oswald Bentinck, Head of Market Access EMEA, Novartis Gene Therapies
Billy Kelleher MEP (Renew Europe, Ireland), Member of the TRANSFORM MEP Interest Group

- Panelists spoke of the cooperative response from the EU during the COVID-19 pandemic, and how this shows that Europe can do great things together. There was also alignment among speakers on the importance of extending early dialogues between competent authorities, HTA officials, patients and ATMP developers.
- **Billy Kelleher** called for the EU to play a more central role in approving and assessing ATMPs, but also possibly in procurement/negotiation for reimbursement. He felt that political will for the EU to be more involved is there, in the case of rare diseases. He also called for EU-wide newborn screening and genetic testing programmes, and a coordinated approach to feeding data from such programmes into the EHDS. MEP Kelleher supports the sharing of best practice/templates of innovative payment models among Member States. Moreover, he suggested that an EU fund for ATMPs could help speed up assessment/provide certainty for ATMP developers in Europe.
- **Jo De Cock** called for clear evidence generation plans to be developed for ATMPs, to support the use of RWE by payers. He called for collaboration on putting these plans together among payers and industry via early dialogues. He also agreed that there needs to be more awareness of the rationale and reasons behind the cost of ATMPs, as highlighted by Novartis.

[1] See https://www.europarl.europa.eu/doceo/document/E-9-2022-000365_EN.html

- **Simone Boselli** stressed the need to look at access challenges within the wider ecosystem. In addition, he called for Europe to be strategically appealing to ATMP developers otherwise, there is a risk that ATMP developers may leave Europe, undermining access.
- **Wim Goettsch** said national HTA authorities need to strike a balance between added value for the individual patient and the overall healthcare system. They must identify those ATMPs that are really good and have the right data sitting behind them. The EHDS needs to provide a system whereby historical data can be accessed for these purposes. The DARWIN initiative that is coordinated by EMA may provide an excellent start to achieve this.
- **Oswald Bentinck** called for stakeholders to challenge their perceptions of value and sustainability for healthcare products such as ATMPs; people may initially think ATMPs are the 'most expensive' drugs, but they are comparing the prices of one-time therapies to those of chronic drugs that may be taken over many years – by contrast, ATMPs are usually a one-time administration providing long-term benefit that could potentially last a lifetime. Mr Bentinck noted that 9 out of 10 pipeline therapies do not make it to the market, so we need to take this into consideration in the wider debate on price. The current system is not incompatible with ATMPs, it needs to evolve. He called for acceptance of the long-term benefit of innovative therapies with the security of payback if they do not work via outcomes-based payment models. Moreover, he would like to see one set of rules for RWE generation in the EU.

Workshop – TRANSFORM's commitment to ensure safe and timely patient access to ATMPs in Europe

Note this workshop was held under Chatham House rules.

Alliance Members (including Prof. Gilles Vassal, Dr Androulla Eleftheriou, Thomas Bols, Dr Stuart Faulkner, Dr Giovanni Migliaccio, Dimitrios Athanasiou) highlighted the following points:

- The importance of joint clinical assessments under the EU HTA Regulation, and the need to develop tailored evidence standards for ATMPs.
- The importance of early iterative dialogues, with countries sharing best practice on market access approaches for ATMPs.
- The need to ensure business models for ATMPs that are commercially viable.
- The need for education of patients and clinicians on ATMPs, especially when ATMPs are due to launch in a given therapeutic area, to ensure the community is ready to 'receive' them. Education for competent authorities was also seen as vital to ensure regulators understand the benefits that ATMPs can bring for patient communities.

Conclusions and next steps – Aligning on a TRANSFORM position in the context of the Pharmaceutical Strategy for Europe

Ondřej Knotek (Renew Europe, Czechia), Co-Chair of the TRANSFORM MEP Interest Group, highlighted:

- The key takeaway from all the discussions and interventions is that stakeholders are ready for EU-wide solutions to improve access to authorised ATMPs.
- The key highlights for MEP Knotek are calls for better horizon scanning, the use of RWE and leveraging the EHDS to improve access.
- TRANSFORM is a crucial forum to discuss, among experts and policy makers, the steps needed to ensure all patients have access to transformative therapies at the forefront of medical innovation.
- The next event of the TRANSFORM MEP Interest Group will be hosted on 16 June and focus on the EU regulatory framework for ATMPs.

- In mid-October, the MEP Interest Group is looking forward to hosting an in-person event in the European Parliament, to launch of an MEP Charter outlining the key Policy Asks across the two strands of work on Access and Regulatory.

About the European Alliance for Transformative Therapies (TRANSFORM)

We invite you to join the TRANSFORM Community on Twitter and LinkedIn and keep an eye on the website to continue this important discussion online and to stay updated about the TRANSFORM MEP Interest Group and Alliance of experts' 2022 plans.

If you did not attend the event or if you wish to re-watch the discussion, you can access the recording here:

https://transformalliance.eu/event/european-alliance-for-transformative-therapies-transform-mep-interest-group-discussion/

You can read the TRANSFORM Policy Asks on Access to Authorised ATMPs launched on 31 March 2022 here:

https://transformalliance.eu/wp-content/uploads/2022/03/TRANSFORM-Policy-Asks-for-Access-to-Authorised-ATMPs.pdf

You can read the TRANSFORM MEP Interest Group Policy Asks on Cross-Border Healthcare launched on 10 November 2021 here:

https://transformalliance.eu/wp-content/uploads/2021/11/TRANSFORM-MEP-Interest-Group-Five-Asks-on-Cross-border-Access-to-ATMPs-1.pdf

You can read the TRANSFORM Recommendations for Actions in the context of the Pharmaceutical Strategy for Europe launched in June 2021 here:

https://transformalliance.eu/wp-content/uploads/2021/06/TRANSFORM-Recommendations-Version1.0_June2021-1.pdf

The European Alliance for Transformative Therapies (TRANSFORM) is a multi-stakeholder Alliance that connects Members of the European Parliament (MEPs) and policy-makers 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 sustainability of healthcare systems.

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The European Medicines Agency is an Observer to the Alliance.

EFNA – European Federation of Neurological Associations

RI – Retina International

WDO – World Duchenne Organization

IPOPI – International Patient Organisation for Primary Immunodeficiencies

TIF – Thalassaemia International Federation

EHC – European Haemophilia Consortium

EURORDIS – Rare Diseases Europe

EPTRI – European Paediatric Translational Research Infrastructure

CCI Europe – Childhood Cancer International Europe

SIOP Europe – the European Society for Paediatric Oncology

EAHAD – European Association for Haemophilia and Allied Disorders

EU EYE – European Alliance for Vision Research and Ophthalmology

EUCOPE – European Confederation of Pharmaceutical Entrepreneurs