Realising the Potential of Transformative Therapies

A European Pharmaceutical Strategy that Enables Safe and Timely Patient Access to Cell & Gene Therapies

Event hosted by the European Alliance forTransformative Therapies (TRANSFORM) MEP Interest Group

European Alliance for Transformative Therapies

30 June 2021 | Virtual event

MEMORANDUM

#MEPsforATMPs

WELCOME REMARKS

Alessandra Moretti (S&D, IT), MEP Co-Chair

Medical and clinical research have made tremendous advances in the field of innovative and personalised medicine in the last few years. Once the added value of innovative treatments is demonstrated, we need to make sure they benefit patients. TRANSFORM will work to ensure the regulatory framework is fit-for-purpose, enabling safe and equitable access. In this legislative term, we can further develop the capacity of the EU in public health and to protect our citizens.

PANEL I

Launch of TRANSFORM Recommendations: Challenges and Opportunities for Transformative Therapies

Link to Recommendations

Simone Boselli, Public Affairs Director, EURORDIS

The challenges for ATMPs concern assessment, affordability, availability, and access. The TRANSFORM Alliance Recommendations build on our work at EURORDIS where we have identified: Enabling the use of real-world data through the creation of the European Health Data Space, taking the input of people living with rare diseases, **(Recommendation 3)**; Improved infrastructure and the ability for patients to access these treatments across borders - the ERNs having a valuable role to play **(Recommendation 4)**. Sharing best practices and recommendations on genetic testing and diagnostics **(Recommendation 6)**, with greater coordination of newborn screening in Europe so that families of a newborn with a rare disease are not left uncertain regarding the diagnosis, care and treatment, wherever they are in the European Union.

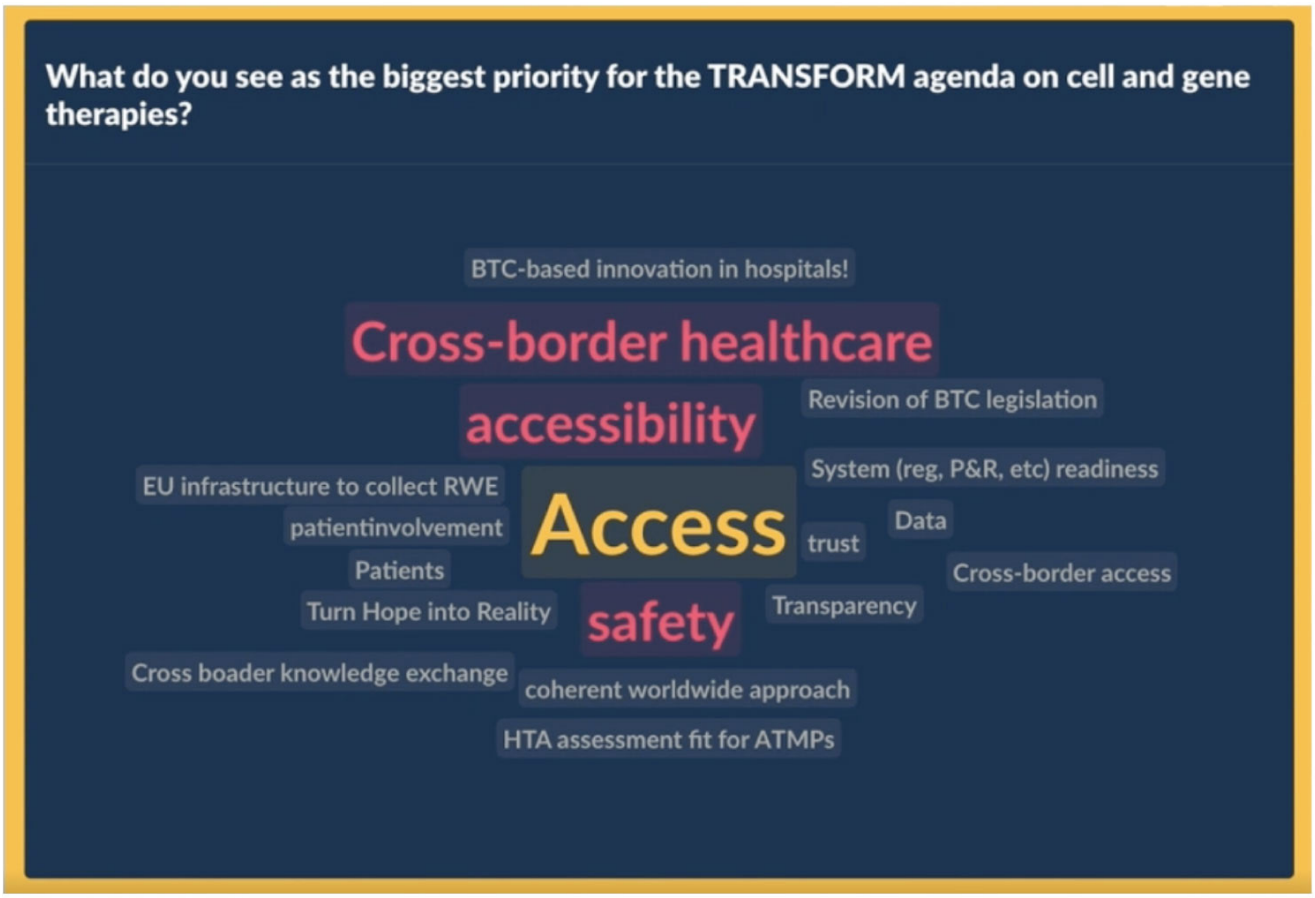
Dr Pauline Meij, Head of the Cell and Gene Therapy Facility, Leiden University Medical Center

The innovation ecosystem needs to be improved by stimulating translational research between universities, research centres and industry, to create beneficial partnerships and a collaborative approach to define high unmet medical needs **(Recommendation 1)**. Appropriate regulatory requirements need to be defined, and this can be done in a number of ways: firstly, we should reinforce more patient-oriented design, planning and conduct of clinical trials, for which the Clinical Trials Regulation can be a first step.

Secondly, requirements for therapies that contain or consist of GMOs should be reviewed. The environmental assessments should be simplified and harmonised across European core countries. Thirdly, a consistent and harmonised classification of different product groups is needed, including harmonisation of the criteria for blood, cells and tissues, ATMPs, and medical devices. Fourthly, the hospital exemption should be used as a bridge between different studies during product development, or for products that are not suitable for marketing authorisation; however, its application should be harmonised, setting a minimum of standards. A registry should be developed to record information and achieve transparency on ATMP use under the hospital exemption **(Recommendation 2).**

Dr Alexander Natz, Secretary-General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

A number of new cell and gene therapies have come to the market recently, which are potentially life-changing. However, they bring challenges given the complexity to administer them and the expertise needed. The legislation and cross-border healthcare framework doesn't necessarily have to be changed, but it needs to function through better collaboration with policymakers and payers. The European Reference Networks in rare diseases need to be connected with national contact points to speed up patient access **(Recommendation 4)**. Innovative payment models – such as outcomes based guarantees – should be in place that can support national governments and spread costs for bringing cell and gene therapies to the market **(Recommendation 5).** Finally, we need to close evidence gaps through the use real-world data once the product has been put onto the market **(Recommendation 3).**



Results from a live poll taken during the event on 30 June

PANEL II

The Way Ahead: What is Needed to Solve the Challenges?

Dolors Montserrat, MEP Rapporteur on the European Parliament Own-Initiative Report on the Pharmaceutical Strategy for Europe

Patients need to be at the centre of health policies and involved at every stage of the care pathway. It is vital to support a competitive and innovative European pharmaceutical industry while ensuring sustainability of the national healthcare systems and protecting the supply chain. It is crucial to increase the EU's resilience and crisis preparedness by preventing shortages, sharing information, and ensuring constant dialogue among stakeholders. The EU needs to continue working with the WHO to standardize effective, safe and sustainable regulatory frameworks for innovative medicines. SMEs have a big role to play in this context as they can innovate quickly and foster new emerging forms of manufacturing that could speed up production times, reduce costs, and facilitate greater access. The EU pharmaceutical legislation needs to be adapted to cutting-edge products, scientific advances and technological transformation – we cannot lag behind.

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Dr Ana Hidalgo-Simon, Head of Advanced Therapies, European Medicines Agency (EMA)

Gene therapies, somatic cell therapies and tissue engineered products are now available to patients. Some products were approved, brought to market, but are no longer available for commercial reasons. These therapies need to be made sustainable and affordable for everyone. Europe is doing well on a global basis, with one of the highest number of approved therapies on the market. However, many clinical trials are still being conducted in the US and Asia rather than in Europe. The time between approval and launch (when it actually gets to patients) is longer than for most products and work needs to be done on global harmonisation. It is important to only put on the market the products with a proven evidence of a positive benefit risk, to ensure the safety of ATMPs. The TRANSFORM multi-stakeholder group can be a phenomenal forum for stakeholders to work together to enable patient access to these new, innovative therapies.

Dr Gaetano Guglielmi, Deputy Director General for Health Research and Innovation, Italian Ministry of Health

Transformative therapies can dramatically change the lives of patients suffering from cancer or a rare disease. However, the high costs of such therapies could create the risk of selection of a type of patient, which is unethical. Connecting academia, policymakers, research institutions and manufacturers is important to discuss how to reduce development costs and accelerate production of these therapies for timely access.

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Joint Q&A session

Panels I & II, with participation of TRANSFORM Alliance members, Dr Giovanni Migliaccio (EPTRI), Prof Marcela Votruba (EU EYE), Dr Carmelo Rizzari (SIOP Europe), Delphine Heenen (CCI-Europe)

What does a genuine patient-centred ecosystem look like?

Simone Boselli: It means taking a patient perspective from the beginning and through the lifecycle of the development of therapies, particularly when it comes to defining the unmet needs. The patient experience and outcomes count.

Dr Pauline Meij: The translational research pathway needs to be improved, silos broken down and optimal collaboration defined. The TRANSFORM Alliance can play an important role in defining these collaborations, putting the patients at the centre and recognising everyone's expertise.

Dr Alexander Natz: It's critical to get the patients' voice throughout the lifecycle of a product, right from the development phase through to reimbursement, and including in the health technology assessment process.

Dr Ana Hidalgo-Simon: We need well-educated, committed patients who are willing to work with us; that's a lot to ask, so patients need support and funding.

What is the role of real-world evidence (RWE) in the European Health Data Space?

Dr Alexander Natz: RWE has a key role to play in pricing and reimbursement processes, especially when limited data is available such as for rare diseases. Industry is committed to generate RWE to support payers in their decisions, but the request for RWE needs to be reasonable and has to be discussed at EU level through collaboration with the national payers and the EMA.

Dr Ana Hidalgo-Simon: For ATMPs, RWE is needed at all stages: development, market authorisation and post-marketing monitoring. It has the potential to help developers fill in the gaps and reduce uncertainty.

Dr Carmelo Rizzari: For many rare diseases, randomized clinical trials are not possible or are limited, so RWE becomes more important. Short-, medium- and long-term safety and efficacy of treatments should be monitored, in particular for paediatric patients.

Are patients aware of the potential of these therapies? Do they have concerns?

Simone Boselli: Patients – and parents of children with rare diseases – are quickly able to determine the benefits and risks of these therapies, but still have concerns relating to safety. Patient input is not always followed up appropriately. Moreover, these treatments are potentially transformative and represent a hope, but they are sometimes communicated as curative.

Delphine Heenen: Transformative therapies such as CAR-Ts, present one of the greatest hope for the treatment of young patients with cancer in a way that is less toxic than conventional procedures. However, there is a risk of deepening the unbalance between Western and Eastern Europe regarding cure rates and access to these innovative treatments

Prof Marcela Votruba: These new therapies are potentially revolutionary against eye diseases. However, patients need opportunities to be educated, supported and empowered in their choices. Moreover, we will need to make them aware that not all patients can be treated because of the stage of their disease. This means developing realistic natural history evidence for each disease, and appropriately managing expectations.

Simone Boselli: At EURORDIS we empower patient organisations with winter and summer schools that train patient experts. There is a need to structure support in terms of funding and opportunities for patients to learn and be engaged.

What are the key challenges in bringing these innovative therapies to market?

Dr Alexander Natz: Almost all the ATMPs launched in recent years have offered innovative payment models and outcome-based guarantees. It is important to discuss the outcome parameters that payers want.

Dr Giovanni Migliaccio: We have to move towards a model where the final product price is based on the cost of goods not on the cost of development. A centralised system is needed, with a minimum price for an innovative product based on a fixed outcome for a specific disease.

Dr Ana Hidalgo-Simon: We have to find a middle ground; developers need incentives and help, but we also need these therapies to be sustainable in the long-run.

Dr Pauline Meij: We need to look at alternative payment models and how the product is assessed in the registration process and by the HTAs. A lot of development is done by academia all the parties need to sit at the table to find solutions.

What are the challenges around infrastructure and the cross-border framework?

Simone Boselli: Our recent surveys clearly show the patients are willing to travel to another country if their therapy is available. A holistic and strategic view on how to make use of these therapies is needed at EU level. This means increasing cooperation and funding for translational research.

Dr Alexander Natz: We have to work with a cross-border framework because not every country will have a specialised centre for specific rare diseases. More education is needed and each country needs to take full responsibility for patient access.

Dr Pauline Meij: Transparency and collaboration.

Dr Ana Hidalgo-Simon: These therapies need to be given in trained, specialised centres. Especially when we are talking about orphan drugs, it doesn't make sense to expect them in every hospital.

CLOSING REMARKS

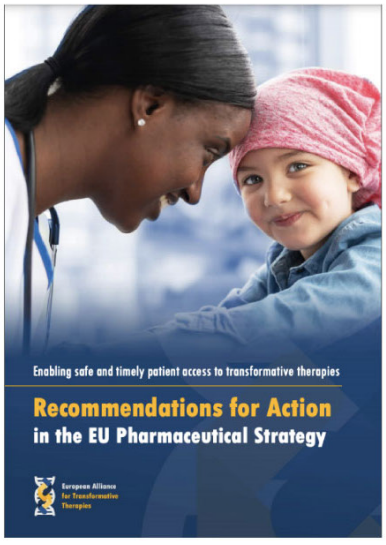
Ondřej Knotek (RE, CZ), MEP Co-Chair

This debate makes clear that EU and national policymakers need to cooperate in addressing and implementing measures to improve patients' lives. There are bottlenecks and challenges that need to be addressed. In particular: adapt the existing framework to also cover cell and gene therapies; harmonize best practices; develop a common approach among all stakeholders to cross-border cooperation and access to treatments; stimulate research and development of infrastructure through new funding or other financial instruments; ensure sustainability and access to therapies through innovative payment models; and address the data gap through real-world data.

The TRANSFORM MEP Interest Group – supported by the TRANSFORM Alliance experts – will work to find consensus on actionable solutions so that patients can benefit from the innovation in a safe and timely way.

The next debate in the Autumn planned on the 9th November will focus on how EU collaboration and cross border co-operation can support equal access to transformative therapies for all patients across the EU. More information will follow.

Read the TRANSFORM Recommendations



Watch the event recording

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 sustainability of healthcare systems.

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