



**European Alliance
for Transformative
Therapies**



Charter of the MEP Interest Group for Transformative Therapies (TRANSFORM)

EU COOPERATION TO ENABLE SAFE AND TIMELY
ACCESS TO ADVANCED THERAPY MEDICINAL PRODUCTS

#MEPsforATMPs



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Key to legislation / initiative icons

GPL	General Pharmaceuticals Legislation	OMP & PAE	Orphan Medicinal Products & Paediatric Regulations	SoHO	Standards of quality and safety for Substances of Human Origin Regulation	EHDS	European Health Data Space Regulation	HTA	EU Health Technology Assessment Regulation
CBHD	Cross-Border Healthcare Directive	CTR	Clinical Trials Regulation	RD	Rare Disease Action Plan	HoEu	Horizon Europe	EU4H	EU4Health

FOREWORD FROM THE TRANSFORM MEP INTEREST GROUP CHAIR

Dear all,

As Members of the European Parliament, one of our mandates is to deliver policies that promote the health and wellbeing of EU citizens. The TRANSFORM MEP Interest Group is committed to realising the full potential of lifesaving and life-transforming therapies like ATMPs. Our aim, with support from the experts in the multi-stakeholder TRANSFORM Alliance, is to develop and act on evidence-based policy recommendations to enable safe and timely patient access to these new therapies, whilst ensuring the sustainability of healthcare systems.

The launch of our MEP Charter comes at an important juncture for healthcare policy in Europe. The EU is launching a holistic review of the pharmaceutical legislative environment (including for example the General Pharmaceutical Legislation and Orphan Medicinal Products & Paediatric Regulations) and reassessing how R&D is incentivized, therapies are assessed and classified, and patients' data is used. Our Charter Recommendations are designed to inform colleagues in the forthcoming legislative discussions, so that we can ensure that the EU access and regulatory framework is fit for cell and gene therapies and meets patients' needs. Patients and their perspectives are - and must always be - at the centre of our decision making.

There are increasing numbers of transformative therapies, both on the market and in development, so consideration needs to be given to new approaches to ensure sustainable access to patients. We see that the regulatory system is currently not fit for the therapies of tomorrow; the complex regulatory environment must balance safety and speed proportionately. Incentivizing companies to undertake clinical trials in Europe also plays a role in enabling earlier patient access.

Healthcare systems are still organised to cover chronic treatments with reoccurring payments rather than payments for potentially one-off therapies. Effective cross border cooperation is needed, as well as funding and training for the collection and assessment of real-world data. New payment models can address the challenges of upfront costs for potentially one-time treatments.

This Charter sets out 7 Recommendations to drive change across the full lifecycle of ATMPs. We must:

1. Create a dynamic, patient-centric innovation ecosystem to address areas of medical need;
2. Ensure regulatory requirements remain appropriate for the development of ATMPs;
3. Enable the use of real-world evidence to address uncertainties for ATMPs;
4. Realize an EU HTA system that supports timely patient access to ATMPs;
5. Share best practices and recommendations on newborn screening, testing and diagnostics;
6. Promote new access pathways to support sustainability of healthcare systems; and
7. Improve infrastructure and enable cross-border patient access to transformative therapies.

These Recommendations must be viewed holistically, as each is equally important in driving change to benefit patients across Europe. As a product of TRANSFORM's unique connection between cross-party Members of the European Parliament and the diverse multi-stakeholder Alliance of experts, we trust the Charter will be recognized as a well-timed consensus position on the complex issues around accessibility, affordability and availability of cell and gene therapies, and attract wide support.

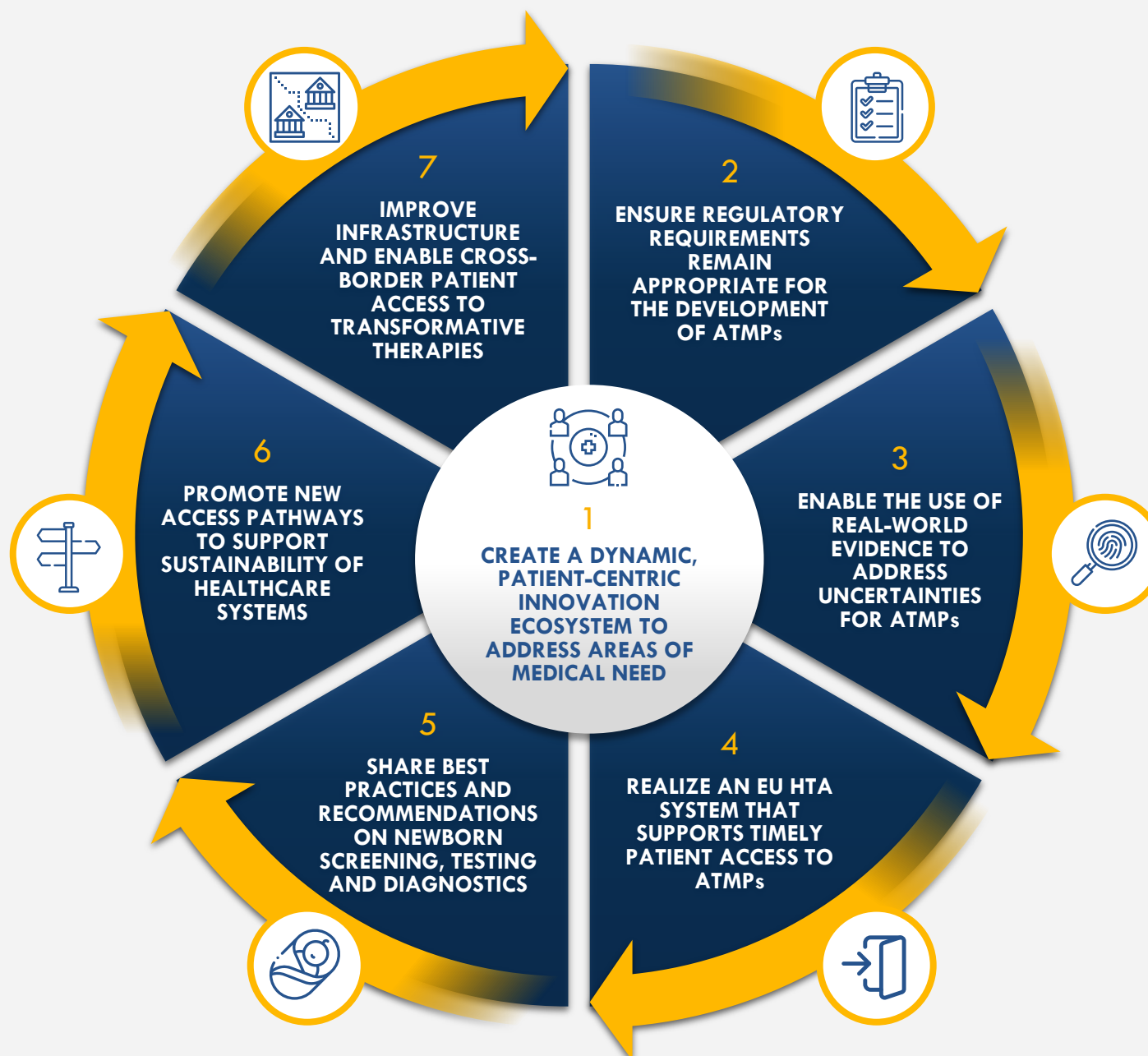
As TRANSFORM's MEP Chair, I call on colleagues in the European Parliament and the other European institutions to take these Recommendations on board in their consideration of the forthcoming legislative reviews, to build a healthcare system that is fit for the future, for all patients in Europe.

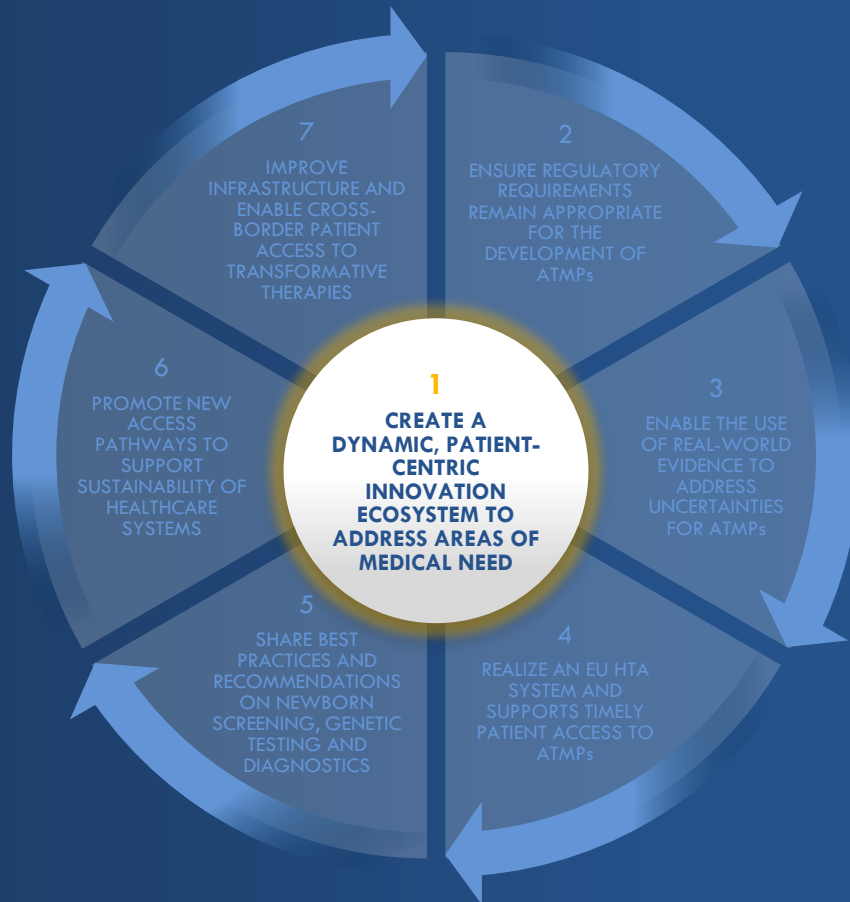


ONDŘEJ KNOTEK
MEMBER OF THE EUROPEAN PARLIAMENT



TRANSFORM Recommendations





POLICY ASKS

1.1

Ensure that the patient and caregiver perspective is included throughout the development pathway of ATMPs - i.e. conduct of clinical trials, evidence generation and value assessment - with transparent feedback on how input is factored into decision making. **Provide funding for cross-disciplinary education** to equip patients, caregivers and healthcare professionals with the knowledge to contribute to technical discussions around transformative therapies.

GPL

OMP
& PAE

HoEu

1.2

Stimulate a collaborative R&D ecosystem for ATMPs, with patients, healthcare providers, universities, research centres and industry involved in identifying **research priorities and effective incentives** to promote innovation in areas of patient medical need.

GPL

OMP
& PAE

EU4H

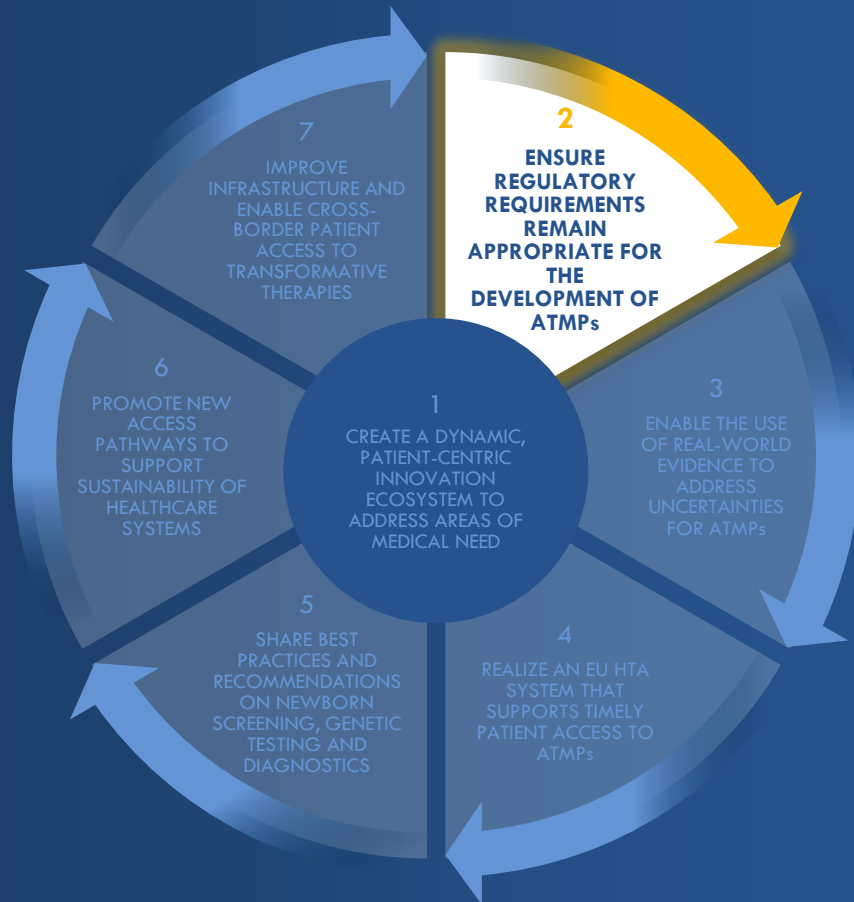
1.3

Ensure that the EU remains attractive for the development of ATMPs, so that patients benefit from early access to innovation. Promote a broad understanding of medical needs that captures the evolving nature of technological advances, and **ensure that the Clinical Trial Regulation is fully implemented and adhered to** in all Member States, avoiding the introduction of additional national requirements for clinical trials outside of the Regulation.

GPL

OMP
& PAE

CTR



POLICY ASKS

2.1

Implement, via the proposed Regulation on Substances of Human Origin (SoHO), consistent and harmonised definitions for **classification criteria of blood, tissues and cells (BTC)**¹ and ATMPs, to avoid divergent interpretations and decisions across Member States and ensure a level playing field for all stakeholders.

GPL

SoHO

2.2

Uphold high safety and efficacy standards for ATMPs, with equivalent regulatory oversight for ATMPs authorised under the centralised procedure and ATMPs manufactured under the Hospital Exemption in a clinical setting. Introduce EU regulatory guidelines on quality with equivalent **standards for use of the Hospital Exemption**, accompanied by **European registries** to record its use, giving greater transparency for patients, regulators and healthcare professionals.

GPL

¹ Also referred to as Substances of Human Origin (SoHO)



POLICY ASKS

3.1

Ensure the European Health Data Space enables the secondary use of data and sharing of real-world evidence in the full lifecycle of ATMPs. It should develop interoperability standards for health data sets, thereby facilitating pooling of data to increase reliability.²

EHDS

3.2

Facilitate better and earlier healthcare professional and patient involvement in the collection of real-world data to address uncertainty on the long term effectiveness and safety and thus improve patient and health system outcomes. Invest in the infrastructure needed to create and **harmonise data collected in patient registries** to enhance monitoring of outcomes over the **longer-term** for patients treated with ATMPs.

GPL

CTR

EHDS

3.3

Establish greater alignment between regulators, HTA bodies and payers on evidence generation requirements and use of real-world evidence throughout the development pathway.

Early dialogues such as those between the European Medicines Agency (EMA) and ATMP developers should be extended to be more iterative and inclusive of the full range of stakeholders, i.e. HTA bodies, payers, academia, researchers and patients/caregivers.

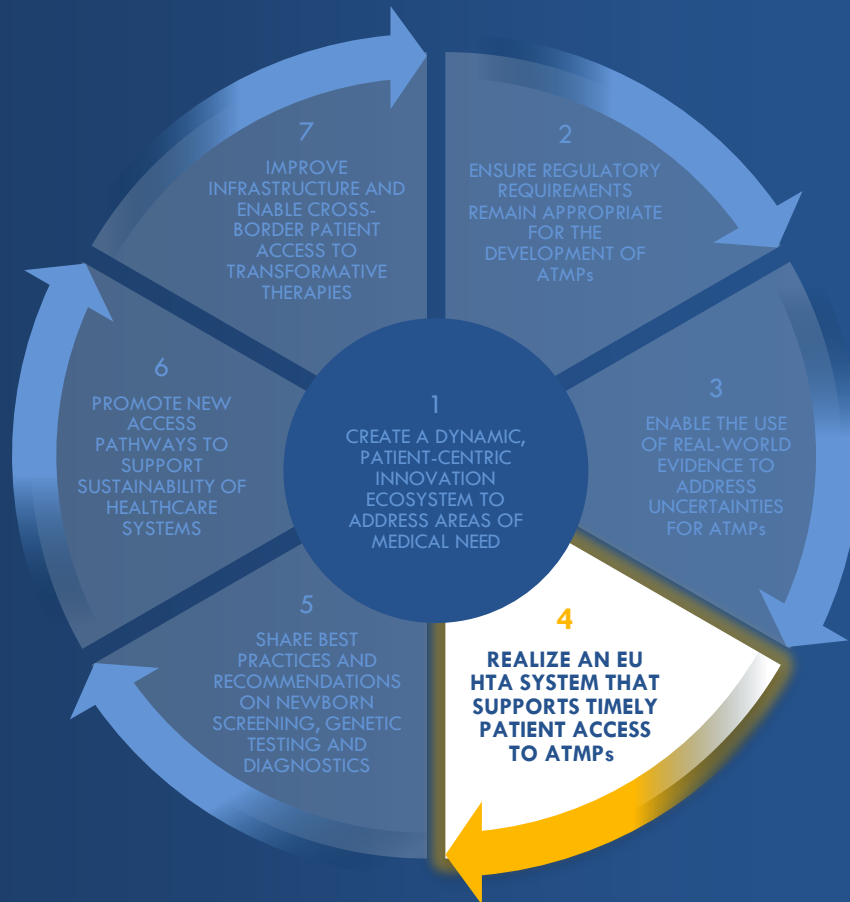
GPL

EHDS

3.4

Consider a dedicated “EU Fund for real-world evidence collection” for the establishment, operation and maintenance of real-world evidence registries, to support the generation and use of data and RWE for ATMPs from the point of (conditional) marketing authorisation.

² TRANSFORM welcomes **DARWIN EU**, which will strengthen EU-wide real-world evidence to support regulatory, HTA and healthcare professional decisions. It should be inclusive of all stakeholders. See [Data Analysis and Real World Interrogation Network \(DARWIN EU\)](#) | [European Medicines Agency \(europa.eu\)](#)

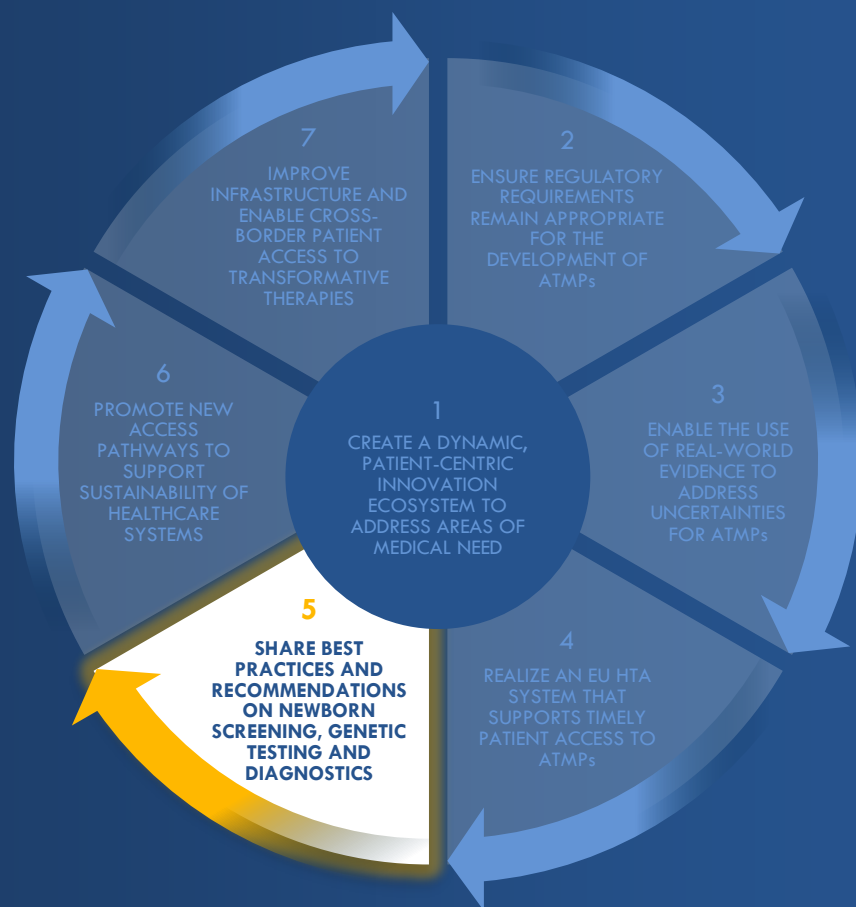


4.1

POLICY ASKS

Ensure effective implementation of the EU HTA Regulation, to ensure timely and efficient evaluation of, and patient access to, innovative therapies when first assessments become mandatory in 2025 for ATMPs. **Joint Scientific Consultations should be conducted early, in multi-stakeholder** (e.g. patients, industry, regulators, academia) **dialogue, so that advice can influence evidence generation plans**, and the opportunity for advice should be offered to all developers that will undergo a Joint Clinical Assessment at EU level. Given the highly specialised nature of ATMPs, assessments should be conducted as efficiently as possible using appropriate methodologies which recognise the specificities of ATMPs. Duplication between EU and national assessments and/or requirements should be avoided.

HTA



5.1

POLICY ASKS

Develop EU guidelines on newborn screening, genetic testing and diagnostics, to be included in the proposed European action plan on rare diseases. The **ERN Expert Platform for Newborn Screening** would be well placed to support this, and to position the EU as the central point for information sharing on good practices from existing national programmes.³

RD

³ See further Screen4Rare's Call to Action June 2022 <https://screen4rare.org/calltoaction/>, <https://screen4rare.org/ern-expert-platform/>



POLICY ASKS

6.1

Optimize and develop new access pathways, ensure effective cross-border cooperation to enable timely and effective patient access to ATMPs by patients in multiple centres of excellence.⁴
Establish a pathway for ATMPs that can be launched and delivered through a number of designated centres in the EU, alongside with adequate support for patients and their families.

CBHD

6.2

Leverage learnings, both the opportunities and challenges, from regional collaborations (e.g. Benelux-AI, FINOSE) to promote access to ATMPs for patients across countries.⁵

6.3

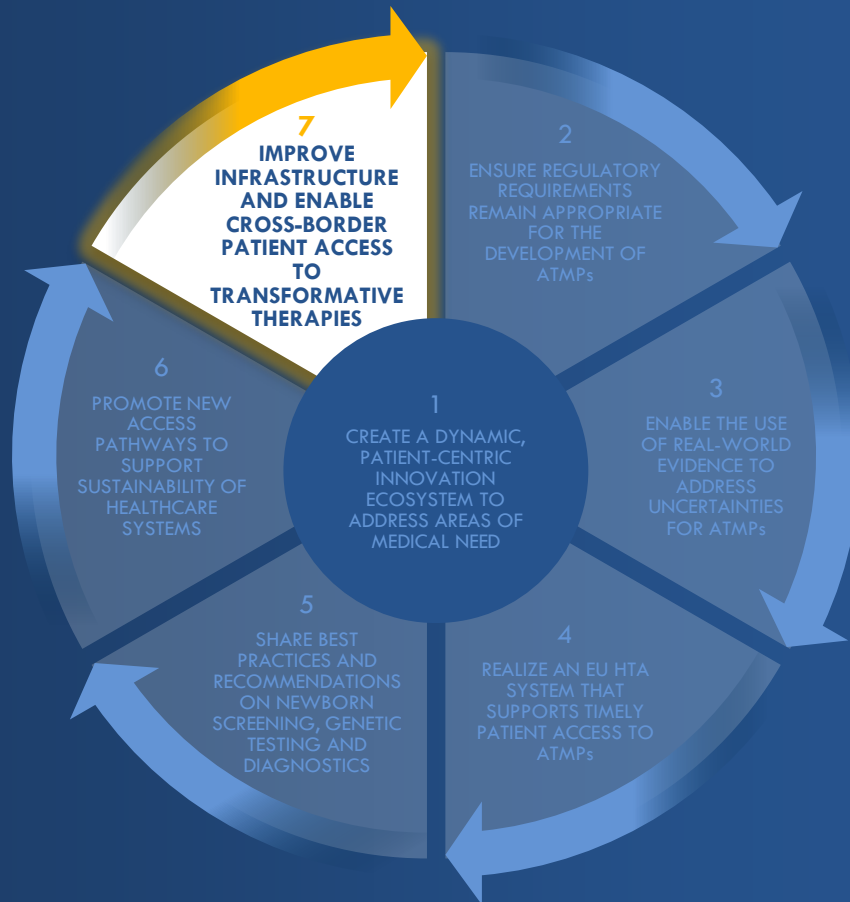
Support national governments to **share learnings on novel payment models and funding approaches for ATMPs**, including pay-for-performance and annuity-based payment models, outcomes-based agreements, and risk sharing agreements. Consideration should be given to differentiated pricing to better align medicines' prices with countries' ability to pay.

OMP
& PAE

GPL

⁴ Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare: <https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A02011L0024-20140101>

⁵ Benelux-AI has successfully led coordination efforts on Horizon Scanning and HTA, but discussions were less successful on the pricing & reimbursement of therapies. Other voluntary collaborations are increasingly inactive, like Valletta and the Visegrad Group.




7.1

POLICY ASKS

Create a multi-stakeholder platform, involving EU Member States, the European Commission, European Parliament, industry, patient representatives, physicians and healthcare insurers and payers, to **develop concrete, operable, transparent mechanisms to facilitate access to cross-border healthcare**, based on current EU cross-border healthcare related legislation.

CBHD



Further Actions for Change

FURTHER ACTIONS FOR CHANGE

SUPPORTING THE CHARTER RECOMMENDATIONS

RECOMMENDATION 1: CREATE A DYNAMIC, PATIENT-CENTRIC INNOVATION ECOSYSTEM TO ADDRESS AREAS OF NEED

- The views of patients/caregivers should be reflected throughout the development pathway: from using patient-orientated design, planning and conduct of clinical trials, to inclusion in discussions around post-marketing real-world data collection. Existing initiatives to better involve patients should be leveraged, including Young Persons Advisory Groups (YPAG).ⁱ
- Translationalⁱⁱ research should be stimulated in the EU to ensure that universities, research centres, patients and industry can create the necessary partnerships to convert innovation into new, transformative treatments. Actions could include (i) promoting and sustaining international research collaboration through IRDIRC and other consortiums, (ii) investing in pre-competitive infrastructures like European Reference Networks and Clinical Research Networks, and (iii) optimizing resources through strategic investments and incentivization of sharing research assets and data through, e.g., the upcoming European Partnership on Rare Diseases.ⁱⁱⁱ
- Given innovative therapies' complexities, EU-level support should be provided for education and training on ATMPs for patients, their caregivers, patient representatives and healthcare professionals. Organisations like EUPATI, SIOP Europe and the TEDDY Network are working to achieve this. The same applies for healthcare professionals, who act as a vital link in facilitating access and understanding of ATMPs.

RECOMMENDATION 2: ENSURE REGULATORY REQUIREMENTS REMAIN APPROPRIATE FOR THE DEVELOPMENT OF ATMPs

- The EMA, and its expert body the Committee on Advanced Therapies (CAT) must be involved as more than an observer in the proposed SoHO Coordination Board (as currently provided under Article 67 of the proposed SoHO Regulation), given the CAT's extensive experience advising on the classification of ATMPs. Where appropriate, decisions of the CAT should take precedence over the SoHO Coordination Board. This would bolster consistent classification of SoHO and ATMPs across the bloc.

ⁱ For example, see the TEDDY Network: <https://www.teddynetwork.net/ypag/>

ⁱⁱ Research aimed at translating (i.e. converting) results in basic research into results that directly benefit humans.

ⁱⁱⁱ For further suggestions, see for example (i) EURORDIS' Rare 2030 Recommendations http://download2.eurordis.org/rare2030/Rare2030_recommendations.pdf, and (ii) the EPTRI Manifesto on Paediatric Research: <http://icri2021.ca/wp-content/uploads/2021/06/paediatric-research-manifesto-ICRI-v2.pdf>

- Harmonized minimum standards for the Hospital Exemption should be introduced to ensure an efficient, clear, transparent and streamlined approach across EU Member States.
- ATMPs should be exempted from, and/or the Commission should simplify, the environmental assessment for clinical trials with cell and gene therapies under GMO legislation.

RECOMMENDATION 3: ENABLE THE USE OF REAL-WORLD EVIDENCE TO ADDRESS UNCERTAINTIES FOR ATMPs

- Continue efforts to build flexibility in the EMA's standards to allow for other forms of data generation, in addition to single arm trials, and increase acceptance of RWE in the regulatory pathway. This is important for ATMPs due to their nature, as well as practical and ethical limitations that mean randomised control trials are not always a feasible approach to generate robust clinical evidence (e.g. lack of comparators for clinical and economic evaluation, limited treatment centres to administer investigational products, ethical considerations of not providing a transformative treatment to a patient while their condition deteriorates).
- The European Health Data Space should be used as an opportunity to reconcile the fragmented and differing interpretations of EU General Data Protection Regulation (GDPR) rules across Member States, including those related to real-world data and the legal basis for data sharing in scientific research in the public interest.
- A multi-stakeholder learning network on Real-World Evidence generation and its use in innovative payment models should be supported, with a mandate to develop common guidelines for using RWE in regulatory decision-making.^{iv}

RECOMMENDATION 4: REALIZE AN EU HTA SYSTEM THAT SUPPORTS TIMELY PATIENT ACCESS TO ATMPs

- Develop methodologies for joint clinical assessments (JCA) of ATMPs, with flexibility in evidence requirements.

^{iv} In 2021 the EMA adopted a *Guideline on registry-based studies*, but it is high level and should be further expanded to cover other types of RWE. See https://www.ema.europa.eu/en/documents/scientific-guideline/guideline-registry-based-studies_en-0.pdf

- All relevant stakeholders must be broadly involved in the EU HTA procedures provided under the HTA Regulation to ensure they are fit for purpose and improve time to access for patients.
- The Commission should develop and adopt EU-level guidelines setting acceptable and harmonised HTA review and approval timelines for ATMPs, to provide greater certainty across Member States for patients and developers.

RECOMMENDATION 5: SHARE BEST PRACTICES AND RECOMMENDATIONS ON NEWBORN SCREENING, GENETIC TESTING AND DIAGNOSTICS

- Whilst newborn screening, genetic testing and diagnostics policy remains a Member State competence, action at the EU-level should support the work developed by the ERN Expert Platform for Newborn Screening, which can provide trusted, high-quality information and best practice examples to support decision-making regarding newborn screening programmes at national level.^{v,vi}

RECOMMENDATION 6: PROMOTE NEW ACCESS PATHWAYS TO SUPPORT SUSTAINABILITY OF HEALTHCARE SYSTEMS

- Whilst there are multiple pathways that can be promoted to support access, a common EU access pathway for ATMPs should be established making use of cross-border healthcare rights, given that ATMPs may require specialised infrastructure and expertise to be delivered, so require a concentration of medical expertise in centres of excellence. For rare diseases in particular (including those in children), there are often conditions where it would not be viable to establish infrastructure and centres of excellence in every Member State to administer an ATMP given a small, dispersed patient population across the bloc. Moreover, given 59% of clinical trials in gene therapies globally are targeting prevalent diseases, future proofing access pathways for an increasing number of patients is important.^{vii}
- More should be done at EU-level to encourage cross-fertilisation of ideas and information sharing on how to achieve timely access for patients through innovative pricing & reimbursement approaches like pay for performance.
 - Find common agreement on core parameters for outcomes-based payment models for ATMPs.
 - Member States should learn from the achievements and shortcomings of existing national innovative medicines funds (e.g. in Scotland, England, Italy and the Netherlands) with specific regard to ATMPs.

^v <https://screen4rare.org/ern-expert-platform/>

^{vi} See further Screen4Rare's Call to Action June 2022 <https://screen4rare.org/calltoaction/>

^{vii} See ARM's annual report, pages 10-11: [Regenerative Medicine: Disrupting the Status Quo - Alliance for Regenerative Medicine \(alliancerm.org\)](https://alliancerm.org/)

^{viii} <https://ihsi-health.org/>

- Request the European Parliament's Panel for the Future of Science and Technology (STOA) to undertake an assessment of the possibilities to de-risk investments in ATMPs with the creation of a European access pathway. This could include, for example, examining where risk-sharing agreements have enabled timely patient access to ATMPs.
- The field of ATMPs is rapidly evolving, and it is important that those likely to reach the market are within the sight of healthcare decision-makers as early as possible. Horizon scanning at EU, Member State and regional level already exists but should be improved to enable healthcare resource planning to prepare for transformative therapies coming to the market, building on the International Horizon Scanning Initiative (IHSI).^{viii}

RECOMMENDATION 7: IMPROVE INFRASTRUCTURE AND ENABLE CROSS-BORDER PATIENT ACCESS TO TRANSFORMATIVE THERAPIES

- The role of European Reference Networks (ERNs) should be extended to support best practice across Member States in decision-making for cases where cross-border care may be necessary, with closer cooperation between the ERNs to set up simple, standardised national and cross-border care pathways.
- National Contact Points (NCPs) should be accessible and clearly visible to patients, to support the provision of information on cross-border treatment to patient. Guidelines on NCP's functioning should be developed by the Commission, in conjunction with other stakeholders, to create a harmonised, simplified and patient-friendly pathway to accessing information on cross-border care.^{ix}
- To remove the burden of upfront payment for patients accessing cross-border care, the existing mechanism of financial compensation between Member States as outlined in the Cross-Border Healthcare Directive (Article 9(5)) should be activated. The Commission should clarify, for the benefit of national experts, the complexity of the current legal situation deriving from the interaction between the Cross-Border Healthcare Directive and the Regulation on the coordination of social security systems.^x
- EU financing should be allocated for travel expenses and (if applicable) translation services for patients and their family/legal representatives where they must travel long distances to access ATMPs.

^{ix} See further EP Resolution on 12 February 2019 on the implementation of the Cross Border Healthcare Directive: <https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A52019IP0083>

^x Regulation 883/2004 on the coordination of social security systems: <https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=OJ%3AL%3A2004%3A166%3ATOQ>



Annexes

ANNEX I

DEFINITIONS

- **“Advanced Therapy Medicinal Products” (ATMPs):** medicines for human use that include gene therapy, cell therapy and tissue-based therapy. They offer ground-breaking new opportunities for the treatment of disease and injury.
- **“DARWIN EU”:** DARWIN stands for “Data Analysis and Real World Interrogation Network”; coordinated by the European Medicines Agency, it is a federated network of data, expertise and services that supports better decision-making throughout the product lifecycle by generating reliable evidence from real world healthcare data.
- **“European Health Data Space” (EHDS):** a single market for the sharing of health data; the proposed Regulation on the European Health Data Space sets out rules, common standards, infrastructures and a governance framework for the use of electronic health data for healthcare, research, innovation and policy making.
- **“European Reference Networks” (ERNs):** established under the Cross-Border Healthcare Directive, they are virtual networks involving healthcare providers across Europe. They aim to tackle complex or rare diseases and conditions that require highly specialised treatment and a concentration of knowledge and resources.
- **“Hospital Exemption” (HE):** Any advanced therapy medicinal product, as defined in Regulation (EC) No 1394/2007, which is prepared on a non-routine basis according to specific quality standards, and used within the same Member State in a hospital under the exclusive professional responsibility of a medical practitioner, in order to comply with an individual medical prescription for a custom-made product for an individual patient. Manufacturing of these products shall be authorised by the competent authority of the Member State (Article 28 of Regulation (EC) 1394/2007).
- **“Health Technology Assessment” (HTA):** is a scientific evidence-based multidisciplinary process that summarises information about the medical, economic, social and ethical issues related to the use of a health technology, including ATMPs. Rules are laid out in the [Regulation \(EU\) 2021/2282](#) on health technology assessment and amending Directive 2011/24/EU, published 15 December 2021.
- **“Joint Clinical Assessment” (JCA):** is a tool in the context of the EU HTA Regulation to assess relative effectiveness and safety of a medicinal product compared to pre-existing medicines already on the market, conducted at the EU level. Its purpose is to evaluate what added value a medicine or a medicinal product will bring to patients. Under the EU HTA Regulation, a JCA is not binding on Member States, but must be taken into consideration in their national HTA decision-making.
- **“Joint Scientific Consultation” (JSC):** discussions between developers and HTA agencies and regulators (EMA), whereby information is exchanged on development plans for therapies. The objective is to help generate optimal and robust evidence that satisfies the needs of both regulators and HTA bodies, and facilitate patient access to new medicines.
- **“National Contact Points” (NCPs):** under the Cross-Border Healthcare Directive, all EU/EEA Member States are obliged to designate one or more NCPs which are assigned to provide patients with information on all aspects of cross-border healthcare (including information concerning available care (providers and institutions), costs and quality of care, method of reimbursement, steps in the event of a dispute, accessibility of facilities for people with disabilities).
- **“Real-world data” (RWD):** routinely collected data relating to patient health status or the delivery of health care from a variety of sources other than traditional clinical trials.
- **“Real-world evidence” (RWE):** information derived from analysis of real-world data.

ANNEX II

LINKS TO RELEVANT LEGISLATIVE FILES

ATMP

Regulation on Advanced Therapy Medicinal Products

[[Regulation \(EC\) 1394/2007](#) of 13 November 2007]

CBHD

Directive on the application of patients' rights in cross-border healthcare

[[Directive 2011/24/EU](#) of March 2011 / [Study supporting the evaluation of the Directive 2011/24/EU](#) published on 13 May 2022]

CTR

Regulation on **Clinical Trials** on medical products for human use

[Regulation (EU) 536/2014 of 16 April 2014]

EHDS

Proposal for a Regulation on the **European Health Data Space**

[[COM\(2022\) 197](#), published on 3 May 2022]

GPL

Revision of the EU **General Pharmaceuticals Legislation**

[planned for Q4 2022; [Roadmap](#)]

HTA

Implementation of the Regulation on **Health Technology Assessment**

[[Regulation \(EU\) 2021/2282](#) of 15 December 2021]

OMP & PAE

Revision of the Orphan Medicinal Products and Paediatric Regulations [planned for Q4 2022;

[Roadmap](#)]

RD

Proposed European **Rare Disease** Action Plan [See [Rare2030 Foresight Study](#)]

SoHO

Proposal for a Regulation on standards of quality and safety for substances of human original intended for human application and repealing Directives 2002/98/EC and 2004/23/EC, COM/2022/338 [[COM\(2022\) 338](#), published on 14 July 2022]



THE TRANSFORM MEP INTEREST GROUP

The TRANSFORM MEP Interest Group is an informal group that brings together cross-party Members of the European Parliament (MEPs) who are committed to ensuring that innovative and life-changing therapies can reach patients in a timely manner across Europe.

Chair



Ondřej Knotek
RE, Czechia

Why an MEP Interest Group on Transformative Therapies?

The European Parliament can play a key role in helping tackle the issues that prevent or delay access to treatments for patients by raising awareness and rethinking the current policy and legislative framework. MEPs can ensure that transformative therapies are considered as a priority in the European agenda, highlighting the potential that these novel therapies can offer to patients, resulting in better health outcomes as well as societal benefits.

What are the main activities of the MEP Interest Group?

The TRANSFORM MEP Interest Group hosts meetings to inform, educate and promote dialogue with decision-makers to shape the policy and legislative framework to accommodate these transformative therapies for the benefit of patients.

Members



Claudia Gamon
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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 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.

The work of the TRANSFORM Secretariat is enabled by funding from EUCOPE and its members, BioMarin, CSL Behring, Kite – a Gilead company, Miltenyi Biomedicine, Novartis, Novo Nordisk, PTC Therapeutics, Santen and Vertex Pharmaceuticals.

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

