


# Position on the Pharmaceutical Package

Working document, updated as of October 2023

European Alliance for Transformative Therapies

#MEPsforATMPs

# Introduction

**The EU Pharmaceutical Package[1] provides a once-in-a-generation opportunity to deliver a regulatory and legislative framework capable of enabling safe, efficacious, and timely patient access to Advanced Therapy Medicinal Products (ATMPs), as well as enabling a dynamic and competitive research and development environment in the EU. It is important that the legislation is future-proof and can attract and support the development and authorisation of highly innovative technologies (like ATMPs) in the EU so that patients can benefit from these potentially life-changing therapies.**

The public health challenge to meet the needs of underserved populations when it comes to therapeutic options (particularly within rare diseases and paediatrics) is well recognised. ATMPs have the potential to contribute to addressing this unmet need and treat not only people living with a rare disease, but also those living with chronic conditions by targeting the underlying cause(s).[2]

The Pharmaceutical Package aims to enable equal access, promote innovation, and foster competitiveness. TRANSFORM believes it is important that policy change is realised across the lifecycle of ATMPs, supporting translational research, clinical trials, regulatory review, marketing authorisation, access and post-marketing monitoring and pharmacovigilance. Moreover, the innovation ecosystem is complex, and non-legislative and collaborative approaches are also needed to address many of the current patient access challenges.

More investment and multistakeholder collaborative approaches, combined with targeted incentives, are needed in basic and translational research and development. This is particularly critical to support efforts to address the 95% of rare diseases that are without dedicated therapeutic options. Moreover, due to the complexity and specificities of ATMPs, to enable patient access there is a need for improved access models. Effective cross border co-operation is needed to unlock greater accessibility to these treatments, supported by investment in infrastructure across the EU for the delivery of innovative therapies. Moreover, a predictable and coherent regulatory system should encourage more clinical trials to be run in the EU, facilitating earlier access for patients (including through compassionate use programmes) to these therapies, and building more clinical expertise in the EU.[3]

The Pharmaceutical Package will shape, and it is a part of, the wider healthcare ecosystem. Recognising that this review will only be able to address specific elements of the pharmaceutical ecosystem, the revision should form part of a comprehensive approach to support the health, strategic security and scientific objectives of the wider European Health Union. The implementation of the EU HTA Regulation[4], the Directive on Patients'

[1] Encompassing the General Pharmaceutical Legislation, and the OMP and Paediatric Regulations, published by the European Commission on 26 April 2023

[2] Horgan D, Metspalu A, Ouillade MC, Athanasiou D, Pasi J, Adjali O, Harrison P, Hermans C, Codacci-Pisanelli G, Koeva J, Szucs T, Cursaru V, Belina I, Bernini C, Zhuang S, McMahon S, Toncheva D, Thum T. Propelling Healthcare with Advanced Therapy Medicinal Products: A Policy Discussion. Biomed Hub. 2020 Dec 3;5(3):130-152. doi: 10.1159/000511678. PMID: 33987187; PMCID: PMC8101061.

[3] The proportion of global clinical trials activity taking place in Europe has decreased over the past decade. In 2020, Europe's share was 19.3%, compared to a 25.6% average share over the last ten-years. See https://www.clinicaltrialsarena.com/clinical-trials-analysis/europe-accounts-for-19-3-share-of-global-clinical-trial-activity-in-2020/

[4] Regulation (EU) 2021/2282 of the European Parliament and of the Council of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU, https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:32021R2282

Rights in Cross-Border Healthcare[5], the proposed Regulation on Substances of Human Origin (SoHO)[6] and the proposed Regulation for a European Health Data Space (EHDS)[7], Europe's Beating Cancer Plan (EBCP) and EU Cancer Mission are all relevant in this regard and should present opportunities for synergies with the Pharmaceutical Package. Consideration needs to be given to these wider implications of the Pharmaceutical Package and the correlation between EU and national legislation.

Good quality data across the lifecycle of a product will also be a driver to deliver on the promise of these transformative therapies and ensure patients have access to them. The generation, analysis, and use of real-world evidence (RWE) across the full lifecycle of ATMPs is important to overcome the uncertainties arising from evidence gaps at the time of market launch and confirmation of long-term safety, efficacy and utilisation. The proposed EHDS, published by the European Commission in May 2022, is an important complement to meet the objectives of the revised Pharmaceutical Package. It offers the opportunity to leverage secondary health data use and share RWE (see TRANSFORM's MEP Charter for our position on the EHDS)[8] to better inform decision making (i.e., market authorisation, access, pricing and reimbursement)

Outlined below are TRANSFORM Alliance Members' consensus positions in response the European Commission's legislative proposals on the Pharmaceutical Package. These positions aim to drive positive change across the full lifecycle of ATMPs, hence they are listed according to their place in the lifecycle of a therapy, from research through to patient access, and must be viewed holistically.

**This position paper was originally published on 1 June 2023, and was updated in October 2023 with a new position on unmet medical need. As discussions on the proposed legislation continue to progress, TRANSFORM's positions may evolve over time. This document will be updated accordingly.**

**THE PHARMACEUTICAL PACKAGE AIMS TO ENABLE ACCESS, PROMOTE INNOVATION, AND FOSTER COMPETITIVENESS. TRANSFORM BELIEVES IT IS IMPORTANT THAT POLICY CHANGE IS REALISED ACROSS THE LIFECYCLE OF ATMPS, SUPPORTING TRANSLATIONAL RESEARCH, CLINICAL TRIALS, REGULATORY REVIEW, MARKETING AUTHORISATION, ACCESS AND POST-MARKETING MONITORING AND PHARMACOVIGILANCE.**

[5] 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/ALL/?uri=celex%3A32011L0024
[6] Proposal for a REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL on standards of quality and safety for substances of human origin intended for human application and repealing Directives 2002/98/EC and 2004/23/EC
[7] Proposal for a Regulation of the European Parliament and of the Council on the European Health Data Space, COM/2022/197 final, https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX%3A52022PC0197
[8] https://transformalliance.eu/charter/

# Research

**TRANSFORM CALLS FOR the Pharmaceutical Package to include predictable, targeted incentives for the development of new innovative therapies,** to ensure that the EU remains competitive internationally, and attractive to all ATMP developers to invest in research and development for the benefit of EU patients.

**TRANSFORM CALLS FOR the prioritisation of patient-centred, multi-stakeholder collaborative approaches to identify research priorities to foster the development of ATMPs.** The EU must have the ambition and resources to be a global leader in health innovation, offering a fertile environment for the research and development of ATMPs.

- **TRANSFORM CALLS FOR Horizon Europe funding to be allocated to promote patient education and involvement** and empower patient organisations to actively participate in the identification of research priorities and clinical trial design in the ATMP space.[9] This funding should also be allocated to other relevant stakeholders, like clinicians and academia, who can promote education and involvement from patients, and extend the participation of healthcare professionals across the ATMP life cycle. Indeed, in the rare disease field in particular healthcare professionals should be equipped with a better understanding of ATMPs for instance with regard to their uses, the necessary infrastructure for their delivery, and access pathways for patients.

**TRANSFORM CALLS FOR measures to encourage translational research that is fit for use in regulatory settings**, particularly supporting academia with the necessary resources and expertise to translate pre-clinical research into clinical research.

- In particular, **TRANSFORM CALLS FOR partnerships between universities, research centres, hospitals, and industry to be enabled in the legislation**, as well as fostered in non-legislative policies (e.g. cross-stakeholder knowledge exchange and capacity building), to translate innovation into new patient treatments.

[9] Patients are experts in their own condition, and patient advocates/organisations can therefore (for example) determine relevant endpoints in trials and identify areas of unmet need where further research can be focused. For individual patients, it is important that they understand the regulatory and scientific landscape, so that they can give informed consent to treatment with ATMPs.


# Clinical Development

**TRANSFORM WELCOMES the proposal to streamline and harmonise the process under the GMO regulation for submission and assessment of an Environmental Risk Assessment (ERA) for ATMPs.**[10] Centralising the assessment of ERAs in the EMA's Committee for Medicinal Products for Human Use (CHMP) at the time of marketing authorisation applications is a first positive step towards the reduction of unnecessary regulatory burden for ATMP developers. This will help create more opportunities for EU patients to benefit from innovative (and in some cases multi-site) ATMP clinical trials across Member States, and faster access to innovative therapies[11].

However, **TRANSFORM CALLS FOR the EU to further consider risk-based circumstances in which a complete derogation from the GMO Regulation for therapeutic ATMPs may be appropriate.** The legislative proposal to exempt ATMPs being used for compassionate use from the need to complete an ERA is noted.[12] TRANSFORM therefore encourages the Commission to consider other cases where risks for the environment are negligible and are outweighed by the patient benefit in receiving an ATMP, meaning a derogation from the ERA requirement is proportionate.

**TRANSFORM WELCOMES the EMA's mandate to establish a consultation mechanism for early dialogue with authorities and bodies active along the lifecycle of medicinal products** (e.g., regulatory agencies and payers) to enable better clinical trials design and the collection of relevant data, for more streamlined authorisation, pricing and reimbursement, with the ultimate goal of achieving faster access for patients.

- Further, **TRANSFORM WELCOMES the proposal to extend this consultation mechanism to consumers, patients, healthcare professionals, industry, associations representing payers and other stakeholders, where relevant.** TRANSFORM encourages the EMA to use this power to ensure that the right stakeholders are involved throughout the ATMP development process at the appropriate time. Coordination should be promoted and duplication avoided, whilst maintaining payers' and regulators' separate remits.

[10] The Commission proposal is to transfer the ERA from national GMO authorities to the EMA's CHMP and streamline the GMO assessment for clinical trial applications across Member States (Article 177 of proposed Regulation).
[11] In the current system, cell and gene therapies containing or consisting of GMOs must conform to complex rules, especially when conducting clinical trials, which are not primarily intended for medicinal products. These rules vary greatly across the Member States and lead to divergent decisions depending on the Member State. Such complexity, and the associated formal assessment, leads to delays in patients' access to ATMPs and can act as a deterrent for clinical trials to be conducted in Europe.
[12] Recital 54 of the proposed Regulation

# Regulatory Approval & Oversight

**TRANSFORM WELCOMES the strengthening of the patient voice in the regulatory process with the introduction of patient representatives on the Committee for Medicinal Products for Human Use (CHMP).** The involvement of patient representatives in the decision-making process around marketing authorisation applications is paramount. Involvement should be enabled for all patients and their caregivers[13], as they are the ones living with their condition, and can provide unique insights and data around their impact, the therapeutic effect of a therapy, and its impact on their quality of life. Ultimately, this input will contribute to the goal of ensuring faster access to therapies that will make a difference for patients and their lives.

**TRANSFORM WELCOMES the proposal to streamline the regulatory framework and restructure the EMA for more efficient governance and provision of expertise.** It is proposed that the CHMP and the Pharmacovigilance Risk Assessment Committee (PRAC) assume new and stronger principal roles. The EMA will be able to create scientific working parties and scientific advisory groups that can advise the CHMP and PRAC.

- **TRANSFORM CALLS FOR the CHMP to leverage expertise built up within the (proposed to-be disbanded) Committee for Advanced Therapies (CAT) when it comes to, for example, scientific advice, classification, generation of ATMP specific guidelines and environmental risk assessment of ATMPs.** Retaining and deploying this expertise is necessary due to the very specific and complex nature of ATMPs compared to other medicinal products.
- **TRANSFORM CALLS FOR continued transparency regarding how opinions and decisions by the CHMP and Working Parties in relation to product marketing authorisation are made**, to ensure clarity and predictability around EMA decision-making. An explanation of the Committees' and Working Parties' scopes and competences should be made publicly available.
- **TRANSFORM CALLS FOR the establishment of a Working Party on ATMPs, leveraging the (proposed to be disbanded) CAT's expertise on this topic.** It would consolidate and drive regulatory science on the classification, scientific guidelines, certification, and other scientific and regulatory activity needed of these innovative technologies. Such a Working Party would also support the EMA to engage closely with the proposed SoHO Coordination Board on borderline products to ensure consistency and science-based decisions on ATMPs and borderline products. Patient representatives, as well as expert clinicians and academia, must be included as members of this Working Party.

[13] Particular attention should be given to populations that are unable to express an opinion, such as minors and incapacitated individuals, meaning the inclusion of their carer representatives is important.

**TRANSFORM WELCOMES the proposal's intention to allow for earlier and enhanced EMA scientific and regulatory support.** The scope of which therapies can benefit from this support should be sufficiently broad to include ATMPs, as highly innovative technologies. This builds on the learnings from the implementation of the PRIME scheme[14] and the rolling reviews during the COVID-19 pandemic, which can potentially enable transformative therapies to reach patients quicker. However, the current proposal offers this support to those medicinal products likely to offer an 'exceptional therapeutic advancement', which is not clearly defined, and thereby risks excluding transformative therapies like ATMPs which have in the past been the preferred beneficiaries of PRIME designations.[15] Undefined concepts such as 'exceptional therapeutic advancement' must be further clarified in the proposal.

**TRANSFORM WELCOMES the proposal for protocol assistance and research support from the EMA for orphan medicinal products.** As currently most of the authorised ATMPs have orphan indications, such measures will ensure that developers of ATMPs treating rare diseases and regulators can align on necessary evidence packages, clinical trial designs, and evidence pathways to avoid unnecessary delays. This support is particularly important for smaller companies, start-ups, and developers that are not-for-profit or based in academic institutions, who may lack either adequate resources or sufficient expertise to navigate the regulatory requirements independently.

**TRANSFORM CALLS for the eligibility of the PRIME scheme to remain sufficiently flexible to allow for innovative ATMPs to be considered.** Allowing the EMA to establish wider selection criteria for therapies to undergo the PRIME scheme, where they are 'promising medicinal products', is an opportunity for more ATMP developers, with a range of different applications for cell and gene therapies, to benefit from early, iterative dialogues and support. Flexibility in PRIME eligibility would also help to address the geographic imbalance where a number of innovative therapies are developed in the United States and may obtain Breakthrough Therapy designation from the US FDA , but are ineligible for PRIME. Increased enrolment in the EMA's PRIME scheme could promote more EU-centred clinical trials, which ultimately benefit EU patients who access them.

- **TRANSFORM CALLS FOR patients and their carers, clinicians, sponsors, developers and academia to be involved in the multistakeholder consultation mechanisms in determining criteria for defining 'promising medicinal products'** in discussions between the EMA, Member States and the European Commission, to ensure the benefits of the PRIME scheme are available to the broadest group of innovative treatment options and benefit patient access.

In general terms, **TRANSFORM WELCOMES the idea of the proposed 'regulatory sandbox approach'**, by which new technologies can be assessed and approved by the EMA, initially on a temporary basis. This could be an important step by the EMA and European Commission to adapt to the new ways in which highly innovative technologies are developed, and evidence is generated. As an example, TRANSFORM would encourage use of the regulatory sandbox to test out new types of real-world data and real-world evidence for ATMPs, to assess how useful they are in informing regulatory decision making.

[14] The PRIME (Priority Medicines) scheme is a scheme launched by the EMA in 2020 to enhance support for the development of medicines for patients whose disease cannot be treated or who need better treatment options to help them live healthier lives.
[15] ATMPs are potentially life-changing, as they oftentimes seek to address the underlying cause of a condition. For chronic diseases, ATMPs may offer significant added benefit beyond traditional medications/interventions. In the area of rare diseases, where 95% of most complex and challenging rare diseases do not currently have a dedicated treatment option, ATMPs may offer a therapy where none currently exists.

- **TRANSFORM CALLS FOR the regulatory sandbox's scope to be extended to include, for instance, methodologies for drug development.** Methodologies, particularly for clinical trials or data collection, can be unique in the case of ATMPs, meaning that the use of the sandbox for validating such methodologies may be appropriate.
- At the same time, **TRANSFORM CALLS FOR appropriate conditions for use of the regulatory sandbox to be put in place.** A regulatory sandbox should be used in exceptional circumstances, where the current regulatory framework is inappropriate, and it should not lead to a tiered regulatory framework for different types of developers. TRANSFORM Alliance members would welcome the opportunity to collaborate on defining the parameters of the regulatory sandbox with the EMA, to ensure it balances patient safety with innovation in regulatory processes.

**TRANSFORM CALLS on the EU to ensure that the EMA has the appropriate resources** to appropriately fulfil its wide range of responsibilities outlined above, especially as those have been expanded under the Health Union. This should include opportunities for additional training, especially in the case of regulatory science for novel and innovative therapies such as ATMPs.

- **TRANSFORM CALLS for the European Commission to provide additional resourcing for the PRIME scheme** to ensure the EMA can accommodate increased demand.

# Patient Access

**TRANSFORM WELCOMES the ambition of the Pharmaceutical Package to increase patient access to innovative treatments, enhance availability, and ensure medicines can always be supplied to patients across the EU.** With specific regard to ATMPs, while developers work to make these therapies available, important infrastructural, knowledge, and system barriers exist which mean ATMPs can often only be administered in Centres of Excellence, which are not present in every Member State, particularly in the case of rare diseases. Given ATMPs' complexity, the equipment, technical and medical expertise, volume and resources needed to administer and manage these treatments for patients, many ATMPs cannot be administered in all Member States and are necessarily clustered in given Centres. In these cases, where it is not possible to bring a therapy to the patient, the patient must travel to the technology.

To achieve optimal access for patients and reflect the specificities described above, **TRANSFORM CALLS FOR the identification and discussion of improved cross-border access pathways and new models for ATMPs to reach EU patients, coupled with engagement on the infrastructure required in Member States for delivery of these therapies.**[16]

- **TRANSFORM CALLS FOR early dialogue between stakeholders (including patients, industry, clinicians, academia, the EMA and national authorities) to identify which Member States possess the necessary infrastructure (e.g. Centres of Excellence) to deliver ATMPs to patients,** and to support the development of such infrastructure in other Member States across the EU. Such discussions should be coupled with consideration of different delivery models for innovative therapies.
- **TRANSFORM CALLS FOR the strengthening of cross-border healthcare rights for patients to allow access to ATMP Centres of Excellence for all EU patients regardless of where they live, as well as considering cross-border payment models.** It should be noted that the European Court of Auditors published a report in 2019 on the implementation of the Cross-border healthcare directive and recommended that access to cross-border treatment for rare disease patients should be significantly improved.[17]
- For rare diseases patients, **TRANSFORM CALLS FOR existing infrastructure like European Reference Networks to be better leveraged as a tool to enable patient access to therapies across borders.** Boosting existing structures that seek to unlock patient access to highly innovative technologies is essential to realising the ambition of greater availability of medicines across all

[16] https://ehcthinktank.eu/workstream/workstream-two/
[17] https://eca.europa.eu/en/publications?did=49945

Member States. Increased resourcing from the EU for FAIR, independent ERN registries would, for example, go some way to providing data that could identify patients for referral to an existing Centre of Excellence in the EU for treatment or a clinical trial, where appropriate. Increased EU support towards the ERNs efforts to issue guidelines and promote more equitable access to newborn screening, a crucial tool in detecting rare diseases at birth and enabling early access to treatment, would contribute significantly to improved patient access to innovative therapies.

**TRANSFORM WELCOMES the proposal to introduce clear reporting requirements on the use, safety and efficacy of products prepared under hospital exemption (HE) in Member States, but also calls for further precision to be given to the rules around HE.**

Currently, HE is intended for products 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. It should continue to be used on a non-routine basis for individual patients where no authorised treatments or clinical trials are available. Indeed, TRANSFORM considers HE has a legitimate role to play in areas of unmet need where ATMPs may be lifesaving (e.g. life-threatening or rare disease settings, like paediatric cancer) and there are no commercial treatment options available. Greater transparency and harmonisation around the interpretation and legitimate use of HE is essential to avoid deviations from the intended use and ensure ATMPs are not subject to a two-tier regulatory system, which risks undermining the EMA's central authorisation process (which ensures efficacy and patient safety) and on the attractiveness of the EU for investments in ATMPs manufacturing and R&D.

- **TRANSFORM WELCOMES the proposed requirement for regular collection of data on the use, safety and efficacy of products prepared under the HE at Member State level, and the establishment of a central, publicly accessible registry (managed by the EMA) with the list of HE granted in all Member States and the indications for which they have been granted**. Such a registry should be regularly updated. This will ensure greater transparency on patient outcomes and will contribute to much needed harmonisation on the application of HE rules across the EU.
- In addition to this, **TRANSFORM CALLS FOR the Commission to specify in further detail the data to be collected at Member State level** in the implementing act called for in Article 2(7) of the proposed Directive, which should at a minimum include a description of HE use, patient population and the clinical and quality of life outcomes used to measure efficacy and safety.
- **TRANSFORM CALLS FOR a clear definition of the concept of 'non-routine' use to be included** in the proposed Directive by the co-legislators, and its harmonised implementation to be detailed in the implementing act called for in Article 2(7)(d) of the proposed Directive, as well as guidance on eligibility criteria and situations where the HE can be used, so as to ensure a consistent application and usage of the HE in Member States.


# Use of (Real-World) Data

**TRANSFORM WELCOMES the recognition that real-world data can play an important role in regulatory decision-making where it is appropriate.** Gathering evidence for ATMPs through traditional clinical trials can sometimes be challenging, given the uncertainty of their long-term effects (safety, efficacy, and costs) complexity of development, in the case of orphan diseases, small and disperse patient population sizes. Regulators should be empowered to make greater use of real-world data in assessing ATMPs and engaging in discussions with HTA bodies and other stakeholders (incl. patients, clinicians, academia, industry) during earlier dialogues on the acceptability of such evidence pathways.

**TRANSFORM CALLS FOR the Pharmaceutical Package to stipulate the need for systematic early (and continued) dialogues with healthcare professionals and patients around the collection of real-world data.** Academics, healthcare professionals, and patients and their carers should be fully involved and empowered to build the capability to generate real-world-data that would comply with regulatory and HTA requirements and provide evidence of standard of care treatment to evaluate new health technology. Furthermore, the patient perspective must be prioritised, including in relation to the ethical and practical considerations of data collection and use (such as registry design and secondary use of data).

# Cross-cutting concepts: Unmet Medical Need

**TRANSFORM RECOGNISES that significant unmet needs continue to exist and should be urgently addressed. The legislative proposal can play an important role in bringing new and innovative therapies to EU patients.** Any discussion of this concept is complex and must address the plurality of social, economic, regulatory, and medical factors that contribute to unmet needs among patients. In addition, it is important to recognise that what would be considered as a (high) unmet medical need varies from one disease to another, and understandings of unmet needs may evolve over time. The TRANSFORM Alliance is committed to continue engaging on this topic and providing a multi-stakeholder perspective.

**TRANSFORM CALLS FOR the proposed definitions of (high) unmet medical need in the proposed legislation to be adapted so that they are flexible enough to accommodate (i) the diversity of (high) unmet medical needs among different patient groups, and (ii) innovation in therapies over time.** A strict and narrow definition that is enshrined in legislation could overlook specific patient populations and hinder innovation, and not allow for the flexibility required by scientific and medical advances. Rather, a broad, common understanding of principles of (high) unmet medical need is more appropriate. We recommend the proposal should:

- **Set out the basis for broad principles to be applied when considering if a new therapy addresses a (high) unmet medical need.** These principles could include, among others: the absence of an authorised product for the disease in the EU, disease severity/burden of the disease, the adequacy of currently authorised treatments for the disease (i.e., their impact on quality of life, adverse reactions, their acute and long-term toxicity, and the burden of treatment/administration) and the novelty of the technology and/or mechanism of action of the new medicinal product.[18] When considering when a new therapy addresses (high) unmet medical need, the new therapy would need to meet at least one of these principles.

[18] For further discussion of understandings of (high) unmet medical need, see Vreman et al *Unmet Medical Need: An Introduction to Definitions and Stakeholder Perceptions*, Value in Health, Volume 22, Issue 11, 2019.

- **Require the production of detailed, science-based guidelines on determining if a medicinal product addresses a (high) unmet medical need for a specific patient population, based on the broad principles set out in the legislation.** These guidelines should be prepared and (periodically) updated in a multi-stakeholder context with input from the EMA and the compulsory involvement of patient organisations, clinicians, academia, researchers, developers, and other relevant stakeholders to discuss and refine understandings of unmet needs in given disease areas. These dialogues should be iterative over an appropriate period of time as the evidence base, and thus justification to address unmet needs, evolves.

# 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.

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The European Medicines Agency is an Observer to the Alliance.

ESGCT – European Society of Gene and Cell Therapy

reNEW Consortium – Stem Cell Medicine

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