Realising the Potential of Transformative Therapies

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

On 30 June 2021, the TRANSFORM MEP Interest Group – spearheaded by its Co-Chairs **Alessandra Moretti** (S&D, Italy) and **Ondrej Knotek** (Renew Europe, Czechia) – held an online event which brought together over 150 representatives representing patients, clinicians, research institutes, pharmaceutical industry and EU institutions.

The **first panel** launched the TRANSFORM Recommendations which were presented by **Simone Boselli**, Public Affairs Director at EURORDIS, **Dr Pauline Meij**, Head of the Cell and Gene Therapy Facility of the Leiden University Medical Center; and by **Dr Alexander Natz**, Secretary-General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE). The six priorities for action identified by TRANSFORM are: 1) Create a patient-centric, **innovation ecosystem** to address unmet medical needs; 2) Safeguard patient access by defining appropriate **regulatory requirements for ATMPs**; 3) Enable the use of **real-world patient data** through the creation of a European Health Data Space; 4) Improve infrastructure and enable **cross-border patient access** to transformative therapies; 5) Increase patient access and ensure sustainability of healthcare systems through **innovative payment models**; and 6) Share best practices and recommendations on **genetic testing and diagnostics.**

In the **second panel**, our speakers reflected on what is needed to realise the potential of these 'Advanced Therapy Medicinal Products' (ATMPs). **Dolors Montserrat**, MEP Rapporteur on the European Parliament Own-Initiative Report on the Pharmaceutical Strategy, said that 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. Patients need to be involved at every stage of the care pathway. **Dr Ana Hidalgo-Simon**, Head of Advanced Therapies at the European Medicines Agency, outlined that Europe has one of the highest number of approved therapies on the market, however a challenge is that many clinical trials are still being conducted in the US and Asia rather than in Europe. She reiterated the importance of ensuring the safety profile of ATMPs and of having a proven positive benefit risk. **Dr Gaetano Guglielmi**, Deputy Director General for Health Research and Innovation at the Italian Ministry of Health, stressed the importance of reducing development costs and accelerating production for timely patient access to these transformative therapies through greater collaboration between academia, policy-makers, research institutions and manufacturers.

There was a clear message that for these transformative therapies to be accessible and safe for patients across the EU, policy change is needed and health systems need to adapt. Greater stakeholder collaboration and cross-country co-operation is required to find actionable solutions, underpinned by a fit for purpose regulatory framework, so that these valuable treatments can improve patient's lives.

The Mission of the TRANSFORM MEP Interest Group – supported by the expertise of the TRANSFORM multi-stakeholder Alliance – is to foster dialogue and find consensus on the critical issues which need to be addressed.

The next meeting of the TRANSFORM MEP Interest Group will take place on **9 November 2021 (afternoon)** and will focus on how EU collaboration and cross-border co-operation can support equal access to transformative therapies for patients across the EU. SAVE THE DATE - information to follow.