CALL TO ACTION ON TRANSFORMATIVE THERAPIES IN EUROPE

European Alliance for Transformative Therapies

The European Alliance for Transformative Therapies is calling for the integration of key priorities and considerations for cell and gene therapies during the implementation of the Pharmaceutical Strategy for Europe and other relevant EU healthcare initiatives. The Alliance is currently developing a Consensus Statement in collaboration with patient advocates, medical experts and industry representatives to identify key areas for action to promote rapid patient access to potentially life-saving cell and gene therapies. The following six points represent a Call to Action to European decision-makers which will be further detailed in the Consensus Statement.

Reinforce a competitive clinical trials framework in Europe for cell and gene therapies by:

- Facilitating effective discussions between national authorities and stakeholders involved in clinical trials of advanced therapeutic medicinal products (ATMP) to reduce regulatory burden and safeguard Europe's innovative competitiveness.
- Reducing burdens on clinical trials of ATMPs containing or consisting of Genetically Modified Organisms (GMOs) by exempting them from GMO requirements.

Disseminate best practices on innovative payment models that can support national governments to increase patient access to transformative therapies by:

- Identifying where innovative payment models have already been successfully implemented securing access to these therapies.
- Sharing information on innovative payment models such as outcome-based agreements which can play an important role in providing payers with the needed confidence to invest in transformative therapies.

Enhance the infrastructure for the provision of cell and gene therapies in Europe through:

- Launching funding opportunities within existing programmes, such as Horizon Europe or the European Regional Development Fund, to adapt infrastructures to enable the provision of cell and gene therapies.
- Setting-up or tailoring European funding programmes to promote cross-disciplinary and patient-centred education for healthcare professionals on cell and gene therapies.

Enable cross-border patient access to transformative therapies by:

- Addressing shortcomings (e.g. upfront payments) of the Cross-Border Healthcare Directive in its foreseen evaluation.
- Addressing the challenges of the S2 Regulations[1] (such as the variability of timeline approvals between countries), which negatively impact patient access.

Promote the development of appropriate Health Technology Assessments (HTA) by:

- Calling on Member States to re-assess their current HTA methods to ensure that they are appropriate for the specificities of cell and gene therapies – including for rare diseases – and consider new methods for evidence generation and assessment.
- Increasing stakeholder collaboration, including patient groups, to agree on core data requirements and support policy action to produce high-quality data including Real World Evidence, a critical factor for the approval of cell and gene therapies.
- Strengthening and harmonising patient registries for cell and gene therapies through effective policies.

Promote a modern innovation ecosystem with effective incentives by:

- Recognising the progress of transformative therapies in the rare disease space and further strengthening incentives that may increase innovation for the benefit of European patients.

[1] EC Social Security Regulations 883/2004 and 987/2009

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