Concerned Stakeholders Issue Call to Action on the Implementation of the EU Joint Clinical Assessment for ATMPs

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Without methodologies suited to ATMPs, the JCA will create a new barrier to patient access.

The new EU Joint Clinical Assessment (JCA) will become an additional barrier for patient access to transformative advanced therapies if the HTA Coordination Group does not modernize its approach to evaluating these medicines, warned 30 not-for-profit organisations, including patient groups, scientific societies, research foundations and medical institutes, in a Call to Action announced today.

The organisations urged all those involved in the JCA to recognize and use all types of available clinical evidence, including single-arm trials and real-world evidence, in the assessment of Advanced Therapy Medicinal Products (ATMPs), which include cell and gene therapies.

Recognising that randomised control trials are often unfeasible and unethical for ATMPs, which often target rare or ultra rare diseases, they called on the EU HTA Coordination Group to:

- Revise the JCA methodological guidance to avoid discrediting evidence from single-arm trials
- Take a more pragmatic approach to running JCAs of ATMPs by using real-world data to fill evidence gaps

The signatories of the Call to Action are working together to bring these issues to the attention of the European Commission, the Coordination Group and members at the national level, to ensure that the voices of patients and all stakeholders in the ATMP ecosystem are heard, and, more importantly, acted upon. The Call to Action was agreed following a workshop hosted by Cancer Patients Europe, the Alliance for Regenerative Medicine (ARM) and Fondazione Telethon.

Juan Ventura, Research & Patient Engagement Director, Cancer Patients Europe said:
"Increasing patient access has been a key driver for the current mandate of the European Commission. It would be extremely counterproductive if the JCA, with all its honorable intentions, becomes an obstacle for patients to access life-saving therapies. While a promising JCA would give faster access to medicines and reduce inequality of access across Europe for cancer patients, the current JCA methodologies for assessing clinical benefit to patients do not take into account the unique nature of ATMPs."

Paolo Morgese, VP, Public Affairs Europe, Alliance for Regenerative Medicine (ARM), said:

"By centralizing an assessment traditionally performed on a country-by-country basis, the Joint Clinical Assessment has the potential to reduce the time and costs of bringing new therapies to patients in the EU. Failing to recognize the value of single-arm trials and of real-world evidence when randomised controlled trials are not feasible introduces a new barrier for ATMPs and risks dramatically delaying patient access to transformative therapies. This opportunity to modernize the EU's approach to assessing innovative medicines cannot be missed."

Stefano Benvenuti, Head of Public Affairs, Fondazione Telethon said:
"The HTA Coordination Group has to appreciate the ethical dilemma associated with adopting a randomised controlled trial over a single arm trial when developing an ATMP for ultra-rare diseases, when a patient with no therapeutic alternative could be given a placebo. If EMA can grant marketing authorisation for some ATMPs on the basis of clinical efficacy based on single arm trials data surely the JCA can accept single arm trials with indirect comparisons, on the basis of very robust observational studies or registries."

Signatories to the Call to Action include: Active Citizenship Network (ACN); Alejandro Da Silva Foundation (Spain); Alliance for regenerative Medicine (ARM); Community Health Association (Romania); Canadian Organization for Rare Disorders (CORD); Cancer Patients Europe (CPE); CCRM Nordic; Cystic Fibrosis Europe (CF Europe); Dravet Syndrome Foundation (Spain); European Association for Haemophilia and Allied Disorders (EAHAD); European Associations of Urology (EAU); ELA International; European Brain Council (EBC); European Hematology Association (EHA); European Liver Patient Association (ELPA); European Multiple Sclerosis Platform (EMSP); European Society for Blood and Marrow Transplantation (EBMT); European Society for Paediatric Oncology (SIOPE); European Society of Gene & Cell Therapy (ESGCT); EveryLife Foundation for Rare Diseases; Fondazione Telethon Genéthon; Gynecological Cancer patients (Finland); International Patient Organisation for Primary Immunodeficiencies (IPOPI); International Society for Cell and Gene Therapy (ISCT); Italian Federation for Rare Disease (UNIAMO); Lymphoma Coalition Europe (LCE); Opie Jones Foundation; Pancreatic Cancer Europe (PCE); Partners for Patients (PFP) NGO; SMA Europe; Thalassaemia International Federation (TIF).

About the Alliance for Regenerative Medicine
The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. As a community, ARM builds the future of medicine by convening the sector, facilitating influential exchanges on policies and practices, and advancing the narrative with data and analysis. We actively engage key stakeholders to enable the development of advanced therapies and to modernize healthcare systems so that patients benefit from durable, potentially curative treatments. As the global voice of the sector, we represent more than 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations.

About Cancer Patients Europe
Cancer Patients Europe (CPE) represents and empowers the voices of cancer patients and cancer survivors, advocating for their needs and rights at every stage of their journey. Its commitment extends beyond simple advocacy; it actively works alongside policymakers, healthcare professionals, and other stakeholders to co-create policies that promote prevention, high-quality care, treatment, and support survivorship.

About Fondazione Telethon
Fondazione Telethon is a non-profit organisation recognized by the Italian Ministry of Education, Universities and Research. It was founded in 1990 in response to calls by people suffering from rare diseases. It manages fund raising activities and guarantees the necessary resources for its research projects. These projects are subjected to rigorous assessments on the part of an independent, international Scientific and Medical Committee.