

Joint Statement on Hospital Exemption

The signatories of this statement are key stakeholders in the healthcare ecosystem at both the European Union and Member State level comprising of health care professionals' associations, patient organisations and national competent authorities for organs, tissues and cells.

Advanced therapy medicinal products (ATMPs) prepared under Hospital Exemption are the closest we have to personalised affordable therapies and are 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” [Article 2 of proposed Directive 2023/0132(COD); article 28 of current regulation].).

Since 2008, HE has ensured:

- access to safe and affordable innovative therapies through regulatory flexibility for the small scale developmental activities in hospitals, academic and other non-commercial settings under the scope of national competent authorities;
- the leveraging of our nonprofit activities into achieving a leadership position for our European centres in the ATMP field as most of ATMP therapeutic concepts and commercial treatments originate in academia;
- mitigation of the risks to accessing innovative and affordable therapies imposed by unsustainably high therapy prices; market withdrawals for commercial reasons; and the limited bargaining power of national governments, all of which have caused concerns at EU level.

We therefore welcome the general will to regulate and strengthen the HE use in the European Union through the systematic, structured and standardised collection of data on the use, safety and efficacy of ATMPs under HE including compliance with the Union requirements for good manufacturing practices (GMP), traceability and pharmacovigilance.

However our organisations would like to express collectively our deep concerns regarding certain changes in the original text of the European Commission on HE as proposed in the ENVI and ITRE reports¹. Hospital exemption is a vital part of a balanced regulatory framework that safeguards all interested parties bridging valuable innovation with sustainability of health systems. The proposed changes ignore the nonprofit nature of HE and the principles of research integrity regarding reproducibility and replicability of research findings:

- the use of HE cannot be linked to market considerations nor restricted in the presence of authorised commercial products, clinical trials, etc, given that its non profit use is prescribed by national unmet needs and the therapeutic importance, safety and efficacy of the ATMPs;
- simplistic definitions to delineate the boundary between routine and non routine production will cause the cessation of any academic research even when authorised commercial products are not available as no national competent authority approves products whose safety and quality are neither validated nor consistently reproducible as the original study.

Restricting the HE use will:

- reduce access to therapies with a knock-on effect on current levels of health outcomes, unattainable otherwise given the finite resources;
- jeopardise the role of our healthcare professionals as gatekeepers and health resource stewards;
- undermine existing national investments on enhancing safety and quality of ATMPs under HE;
- threaten the intersection of HE with the SoHO regulatory framework and those Substances of Human Origin (SoHO) regulated as ATMPs.

¹**Amendments 30 and 31;** DRAFT REPORT on the proposal for a directive of the European Parliament and of the Council on the Union code relating to medicinal products for human use, and repealing Directive 2001/83/EC and Directive 2009/35/EC (COM(2023)0192 – C9-0143/2023 – 2023/0132(COD)) ENVI Rapporteur: Pernille Weiss; 03.10.2023.

Amendments 44, 45, 178, 180, 181, 182, 183. AMENDMENTS 21 - 300 Draft opinion Henna Virkkunen (PE754.773v01-00) on the Proposal for a directive (COM(2023)0192 – C9-0143/2023 – 2023/0132(COD)). ITRE. 01.12.2023

Historically, HE has addressed gaps in access to therapies created by market changes - in 2021 seven out of nineteen ATMPs authorised by EMA were withdrawn for commercial reasons. More importantly HE has an enormous but untapped potential in addressing holistically, sustainably and timely complex rare diseases and other unmet needs such as rejection and limited availability of donor organs in transplantation. We ask that the curiosity, creativity and resourcefulness of our researchers will be acknowledged for these achievements rather than punished for their role in a competitive internal market out of speculative fears. The amendments in question are against universal access to therapies and human rights and as such they must be deleted. Our clinicians and researchers must and should be free to continue their work to deliver affordable innovative therapies for every European citizen.

Our collective recommendations will strengthen the non profit activities under HE and allow our healthcare professionals and researchers to contribute to the ambitious political vision for a healthier European Union:

1. Only efficacy, quality and safety criteria should govern the HE pathway, without restrictions either on time or on quantities as by definition the HE implementation is shaped by clinical considerations and the individual needs of Member States rather than market conditions.
2. The scope of HE must be clarified in the legislative text with explicit acknowledgment of the nonprofit nature of the development centres and their activities and that the nonprofit delivery of ATMP therapies under HE is irrespective of the availability of commercial treatment options.
3. The terms 'routine' and 'non-routine' should be removed from the legislative text: the former is scientifically unreasonable and the latter is unnecessary - the non-routine nature of ATMPs under HE is reflected sufficiently in the HE conditions e.g. custom-made product for individual patient, etc. [Recital 18 & Art. 2 (3)].
4. The validity period of HE should be maintained at current levels, otherwise it will undermine the right of patients to timely access to therapies.
5. The nonprofit activities and responsibilities of academic/noncommercial developers functioning under the HE must not be adversely affected by the central authorisation of other products.
6. A stronger coordinating role for the EMA in HE implementation through sufficient scientific/regulatory advice (whether HE or centralised authorisation procedure requirements) and in ensuring compliance with GMP principles at manufacturing level.
7. Leverage on the expertise of the EDQM to enhance the safety of SoHO derived ATMPs and protect both donors and recipients in the implementation of the [new SoHO Regulation](#).
8. The proposal of the European Commission to explore the potential of an adapted framework for certain ATMPs under HE must be retained in the legislative text as new concept models for academic centre ATMP development emerge attracting considerable national investment.
9. Patients, researchers, healthcare professionals, HTA bodies and industry should co-create a framework that integrates effectively HE in clinical practice for specific ATMPs across the EU.

We trust that the European Commission, the European Council and the European Parliament all have the public good at heart and that they will protect the interests of our European patients, our healthcare systems' sustainability and our potential to achieve cutting edge innovation.

The ethical, legal and financial implications of the proposed changes in the legislative text must be addressed responsibly during the trilogues.

We reiterate our commitment to help the EU institutions with our collective expertise and diverse perspectives.

CORE SOHO



[Common representation of Substances of Human Origin](#)
643638449840-70

[European Association for Clinical Pharmacology & Therapeutics](#)
401622952778-77



[European Association of Hospital Pharmacists](#)
82950919755-02



European Blood Alliance

[European Blood Alliance](#)
149855010621-40



[European Eye Bank Association](#)
840527028525-56



EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

[European Paediatric Translational Research Infrastructure](#)

EUROPEAN SOCIETY OF
GENE & CELL THERAPY



[European Society of Gene & Cell Therapy](#)



[European Society for Organ Transplantation](#)
344914634803-90



[European Society of Paediatric Oncology](#)
122803916413-09



ORGANIZACIÓN NACIONAL
DE TRASPLANTES

[National Transplant Organisation, Ministry of Health, Spain](#)



[Spanish Advanced Therapy Network](#)

This initiative is coordinated by



[European Alliance for Vision Research and Ophthalmology](#)
221589017973-83



[Lymphoma Coalition](#)
809500545743-75