Joint Statement on the Hospital Exemption for ATMPs

The signatories of this Joint Statement are key stakeholders in the healthcare ecosystem at both the European Union and Member State level, comprising research centres, health care professionals' associations and patient organisations.

Our Joint Statement asks that the EU decision-makers uphold legislation that supports equitable, need-driven development of therapies. The original purpose of Hospital Exemption (HE) for Advanced Therapy Medicinal Products (ATMPs) must be retained and remain unlimited in the revised legislation.

Since the adoption of the ATMP Regulation in 2007, HE has served as the key enabler for timely response to unmet medical needs through the nonprofit research activities of academic and hospital-based innovators. The HaDEA Study on the Hospital Exemption shows clearly that ATMPs under Hospital Exemption (HE-ATMPs) are the closest we have to personalised, affordable and equitable innovative therapies. We therefore welcome the strengthening of the development of HE-ATMPs with new provisions to increase accountability and trust, such as the annual data collection of HE-ATMPs. We applaud in particular the EMA support to not-for-profit ATMP developers. Most therapeutic concepts and commercial treatments emerged from hospitals, academic, and other non-commercial settings. The regulatory support serves as recognition of such contribution to the therapeutic progress of Europe and it is a fundamental condition for strengthening Europe's research and innovation ecosystem.

The legislative text must be however further balanced, fair and clear of ambiguities with administrative processes proportionate to the risk and scale of academic and clinical research. The current text unintentionally restricts the HE implementation, ultimately compromising:

- access to therapies, particularly for rare diseases with impact on current levels of health outcomes;
- European initiatives, e.g. PRECISEU; national strategic investments in infrastructure e.g. Genomic Medicine Sweden and eventually the efficiency and cost-effectiveness of our European ATMP ecosystem;
- the leadership position of our European centres in the ATMP field.

We urge the co-legislators to integrate the following critical points for a pragmatic, uniform, and ethical regulatory framework that truly allows fair access to quality and safe treatments at the lowest cost possible:

1. **Regulatory clarity and primacy of GMP:** Unequivocally confirm the GMP-equivalent standards as the definitive benchmark for HE-ATMP quality and safety and provide clarifications on equivalency to prevent interpretations at national level.
2. **A dedicated pathway for manufacturing under HE:** Grant a clear derogation for non-profit HE-ATMPs from the rules for commercial decentralized manufacturing (Articles 26a, 142-154, 160-161) to prevent different interpretations at national level.
3. **Remove the '*non-routine*' term:** Ideally delete 'non-routine' from Article 2. The term is redundant for a custom-made product such as HE-ATMPs and incoherent with GMP requirements which demand procedures to be well-established. Above all we collectively call that any attempt to delineate 'non-routine' must not limit the right of any patient to therapies.
4. **Fit-for-purpose evidence:** HE authorization should require minimum efficacy evidence with the understanding that the level of required evidence will depend on the specific case. The HE must not be made a mandatory step towards a marketing authorisation application but remain as alternative pathway to ATMP products to address unmet medical needs for indications which are not commercially viable.
5. **Cross-border exchange of HE-ATMPs**: Introducing cross-border exchange of HE-ATMPs put patient needs first, particularly of those who are clinically unfit to travel e.g. transplant cases or sufferers of rare conditions. Such exchange should be further strengthening as complementary to the right to cross-border health, and more importantly fully aligned with the scope of the European Reference Networks where expertise travels, not the patients.

We trust that the European Commission, the European Council, and the European Parliament will consider our collective key concerns. On our part, we continue to reiterate our commitment to help the EU institutions and agencies with our collective expertise and diverse perspectives.

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