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European Confederation of Pharmaceutical Entrepreneurs AISBL

The journey of ATMPs from early development to access in EU: challenges and opportunities

SME perspective

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EUCOPE, an EU association…

…representing mid-sized innovative companies at national and EU levels

2600+

mid-sized innovative companies

EMIG · IML · BPI · BIO DEUTSCHLAND · france biotech (medtech | biotech) · Bio Biotechnology Innovation Organization

Innovative, mid-sized pharma and biotech companies many in the field of rare diseases

Founded in 2008 in Brussels with **10** member companies

150 **members in 2022**

Recognised stakeholder by EMA, HMA, European Commission and European Parliament

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http://www.eucope.org/en/eucope-members/

Focus on EU Advocacy, Regulatory and Market Access

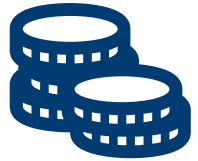
Need for a new paradigm

Rethinking healthcare models

Current reimbursement models focus on managing symptoms

Cannot fully capture value of cell and gene therapies on patients and society at large

Payer concern about translating clinical trial outcomes to real life situations and how to value long-term outcomes



Tension between long-term savings and upfront cost

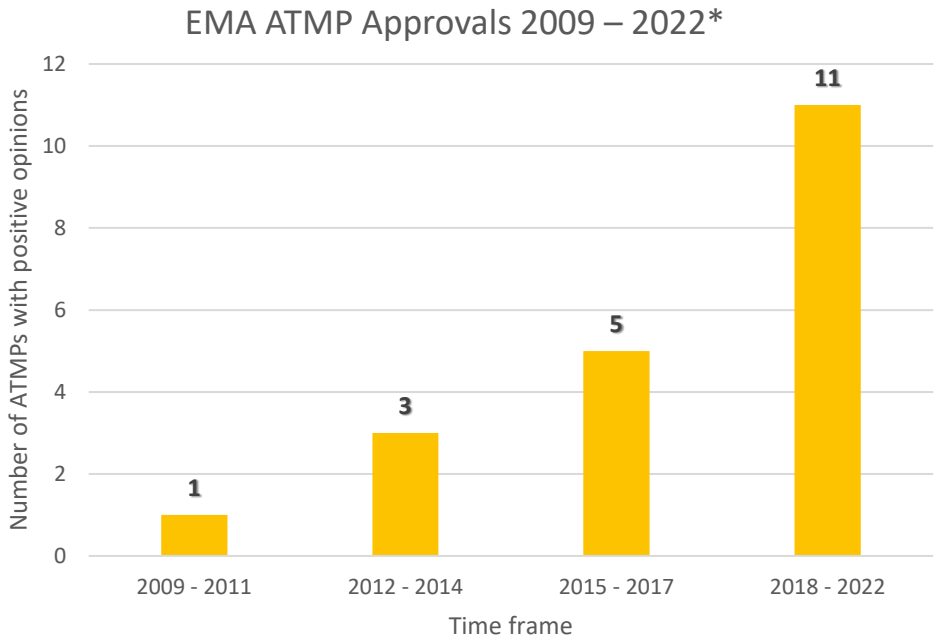
Need ***a collaborative approach to funding/reimbursement/ payment mechanisms*** which could ensure appropriate pricing & risk sharing schemes, where adequate incentives for research and innovation are in place

ATMP Pipeline

EMA has approved **25 ATMPs** since 2009, **11 of which since 2018*** and several more are being assessed this year

Number of ATMPs being approved and authorized on the European market **will grow and accelerate** which could put pressure on healthcare systems to adapt

- Approximately 2,400 clinical trials globally
- Former FDA Commissioner predicted that the FDA would approve between 10 and 20 cell and gene therapies per year by 2025



* Figures as of June 2022

Evolving European policy environment

Access and the EU

Equal access **Affordability** **Availability**

are major priorities for the **current European Commission** which are undertaking the most significant review of the pharmaceutical legislation in over 2 decades.

Key revisions include:

- General Pharmaceutical Legislation
- Orphan Medicinal Products (OMP) Regulation
- The SoHO Regulation

EU's regulatory and incentive framework will **shape national pricing discussions**

Impact of the Pharmaceutical Package

Selected examples

ATMP Regulation is not being re-opened, but key changes coming to the ATMP space which will **impact investment decisions**.

Several important changes were also made pertaining to ‘future-proofing’ the regulatory ecosystem

GMO provisions – Commission introduced exemptions from the deliberate release directive which will facilitate clinical trials

EMA/CAT – EMA structure is being updated with fewer Committees, and expertise retained through Working Parties. ENVI voted to to enshrine an ATMP **Scientific Working Party** in the legislation

Active Substance and Additional Quality Master Files – European Parliament has asked to expand scope to cover biological starting materials explicitly

Hospital Exemption – Become a major political lightning-rod

- Discussions about its use for cost containment
- Text being discussed that aims to provide more clarity as to when it is appropriate
- Expanded data collection requirements and harmonisation of standards expected

Platform Technologies – European Parliament has proposed the creation of a dedicated Master File

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Hospital Exemption

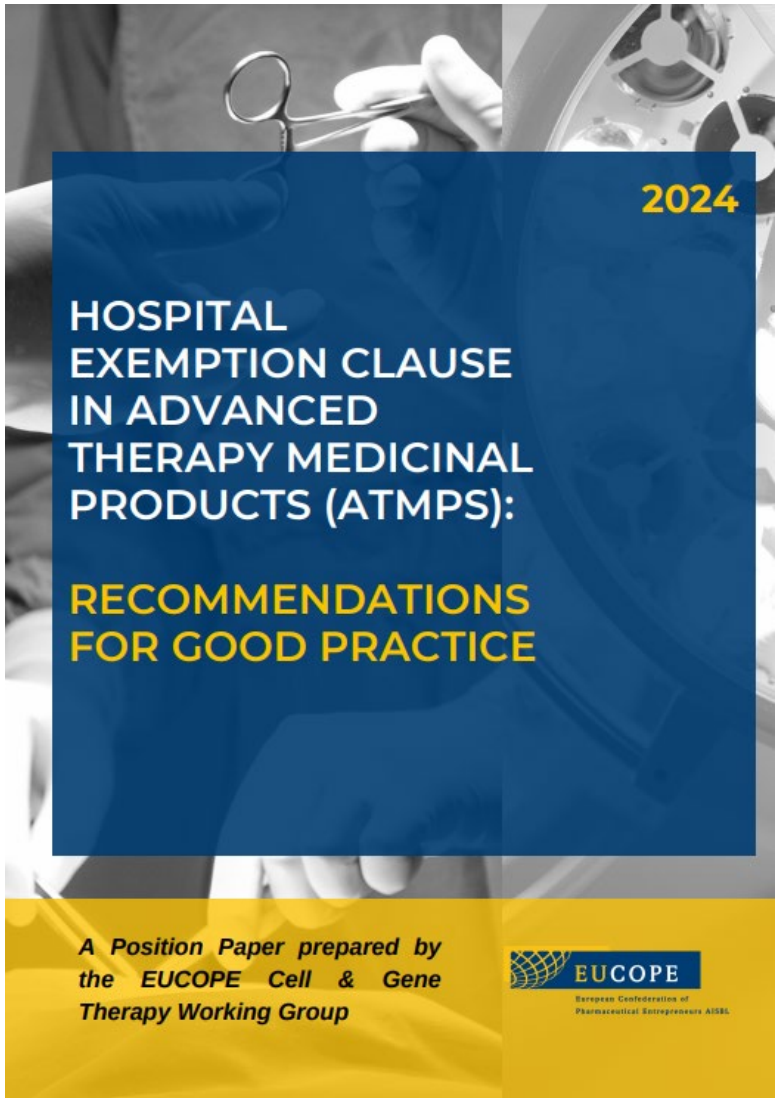
Current Debate

- European Commission has proposed an updated interpretation of Hospital Exemption
- Concept has become a political lightning rod
- Clarity about the role of Hospital Exemption is needed moving forward
- **Dispelling myths**: Industry acknowledges the importance of Hospital Exemption and support its continued use under clearly defined exceptional circumstances in addressing unmet needs and facilitating access for patients in EU countries where no centrally authorised ATMPs are accessible

Hospital Exemption

EUCOPE's reflections

- Important to stress when and where Hospital Exemption can and should be used
  - **Non-routine definition** – clear and predictable guidelines, highlighting its use is appropriate where there is:
    - No ongoing clinical trial or compassionate use programme with an ATMP in the Member States for the same indication for which a patient would be eligible
    - Demonstrated that there is no centrally approved ATMP available for the specific indication in the Member State for which the patient is eligible
  - Hospital Exemption should remain the remit of National Competent Authorities
    - Benchmark set at EU level but sufficiently flexible to adapt to national requirements
- Support initiatives to increase data collection requirements
- Continue to and learn from the EMA pilot to support academics to become developers



https://www.eucope.org/wp-content/uploads/2022/03/eucope-he-position-paper-final-15042024.pdf

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Changes *BEYOND* Pharmaceutical package

ATMP landscape will be shaped by a number of other initiatives

- **EU competitiveness:** New European political environment is stressing the importance of competitiveness, as seen in the Draghi report which explicitly called for ATMP centres of excellence.
- **Potential European Biotechnology Act**: The new European Commission is expected to publish a Biotechnology Act or strategy which will have major implications for ATMPs and manufacturing
- **Additional EU Regulations**: European Health Data Space, SoHO Regulations, and the Critical Medicines Act will shape the ATMP landscape
- **Manufacturing and Hospital capacity**: Number of ATMPs is growing, the ability to meet the manufacturing capacity and healthcare sustainability questions will inform the direction of ATMP investment
- **Role of future technologies**: How Europe responds to new technologies, including Platform Technologies will determine how the EU is positioned

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Alternative Payment Models

A range of solutions being put forward by various stakeholders

- Essential to consider the **unique characteristics** of novel therapies such as ATMPs
- ATMPs require **specialized centres** with specific infrastructure and training
- Many ATMPs are currently being developed by **small and mid-sized companies**


Outcomes-based payments


Annuity payments


Risk-sharing models


Cross-border access

ATMPs should be seen as different from traditional and chronic therapies when discussing value and reimbursement

QUESTIONS

COMMENTS

ANSWERS