CHANGER

CHALLENGES AND INNOVATIVE CHANGES IN RESEARCH ETHICS REVIEWS

Legal and regulatory considerations for patient access to gene therapies.

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What are gene therapies?

- Modify, replace, or introduce genetic material
- Used to treat or prevent disease
- Highly innovative therapies
- Scientific, regulatory, and ethical challenges

Advanced therapies medicinal products (ATMP's)

They include:

- Gene therapies
- Somatic cell therapies
- Tissue-engineered products

Why do they have a special regulation?

- Genetic material
- Living cells
- Complex manufacturing processes

The Centralized Market Authorization

European Medicines Agency (EMA)

Scientific evaluation at EU level
Assessment of:

- Safety
- Efficacy
- Quality

Committee for Advanced Therapies (CAT)

Advantage:

✓ One evaluation for all Member States

Two key regulations in the EU

1. The ATMP Regulation (Regulation (EC) No 1394/2007). The ATMP Regulation acts as a lex specialis (a specialized law), meaning it overrides general EU pharmaceutical legislation regarding advanced therapies unless otherwise specified.
2. The Centralized Authorization Procedure (Regulation (EC) No 726/2004). The centralized procedure requires pharmaceutical companies to submit a single Marketing Authorization Application (MAA) to the EMA

Accelerated access mechanisms

Goal:

Earlier access to innovative therapies

Main pathways:

- Accelerated Assessment (Regulation (EC) No 726/2004 (Art. 14(9))
- Conditional Marketing Authorization (Regulation (EC) No 507/2006)
- PRIME Scheme (EMA initiative (2016))

Balance:

Patient access ↔ Safety & efficacy

Access Barriers

Regulatory approval ≠ Patient access

Main barriers:

- High cost
- Geographical disparities
- Fragmented healthcare systems

Alternative Access Pathways

Two key pathways:

1. Hospital Exemption (HE) governed under Article 28(2) of the ATMP Regulation. It allows hospitals to prepare and provide certain gene therapies on a case-by-case basis without full EMA authorization, provided they comply with national requirements.
2. Compassionate Use Program (CUP) governed under Article 83 of the regulation that establishes the EMA's centralized procedure. It allows patients with serious or life-threatening conditions to receive therapies that are still under investigation, particularly when they cannot participate in clinical trials.

Purpose:
Provide access outside standard authorization pathway

Limitations of the current system

Three key challenges:

1. Fragmented implementation
2. Affordability
3. Approval does not guarantee access

EU authorization

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Equal patient access

National strategies for access

Different countries, different approaches

⚠ Remaining barriers

* Hospital funding
* Specialist shortages
* Patient costs

National strategies matter, but healthcare system capacity matters even more.

Rare diseases and orphan drugs

Orphan Medicines Regulation (Regulation EC n° 141/2000)

Benefits:

- Market exclusivity
- Reduced fees
- Funding incentives

Ethical tension:

Encouraging innovation vs. Ensuring affordable access

Conclusion

EU Regulatory Framework

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Supports innovation

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Approves gene therapies

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Guarantees patient access

Remaining challenges:

- Affordability
- Geographical disparities
- National differences in implementation

How do we balance innovation with equitable access?

Thank you!