EPTRI®

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

EPTRI Advanced Therapy Medicinal Products Thematic Research Platform

CVBF

EPTRI General Assembly & Scientific Meeting – Bologna – 14/03/2025

Advanced Therapy Medicinal Products (ATMPs)

European Medicines Agency (EMA)

- **The EMA defines ATMPs** as medicinal products that are based on genes, cells, or tissues and are used to treat serious or life-threatening conditions.

U.S. Food and Drug Administration (FDA)

- **The FDA describes ATMPs** as a category of biologics that use innovative cell or gene-based therapies to treat complex and often rare diseases.

Personalized medicine

ATMPs provide personalized treatments customized to a patient's genetic background, disease profile, or immune system.

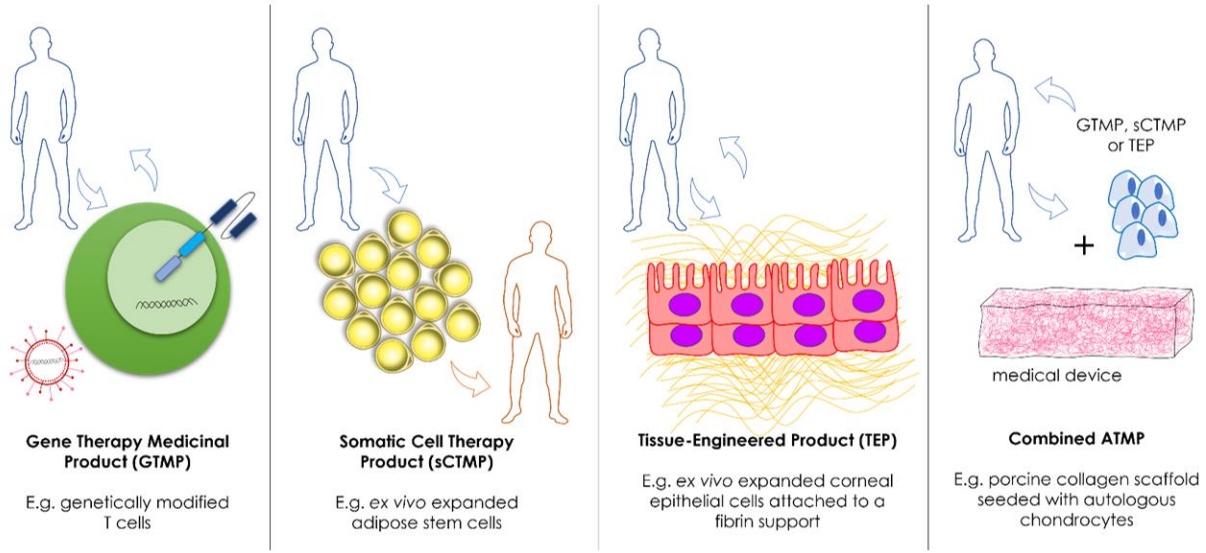
Regenerative medicine

ATMPs help regenerate or repair damaged tissues and organs, often using stem cells or bioengineered tissues.

Rare diseases

ATMPs offer potential breakthroughs for treating rare diseases with no cure.

e.g. SMA and Hemophilia



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propharma

Why ATMPs are relevant for paediatric patients?

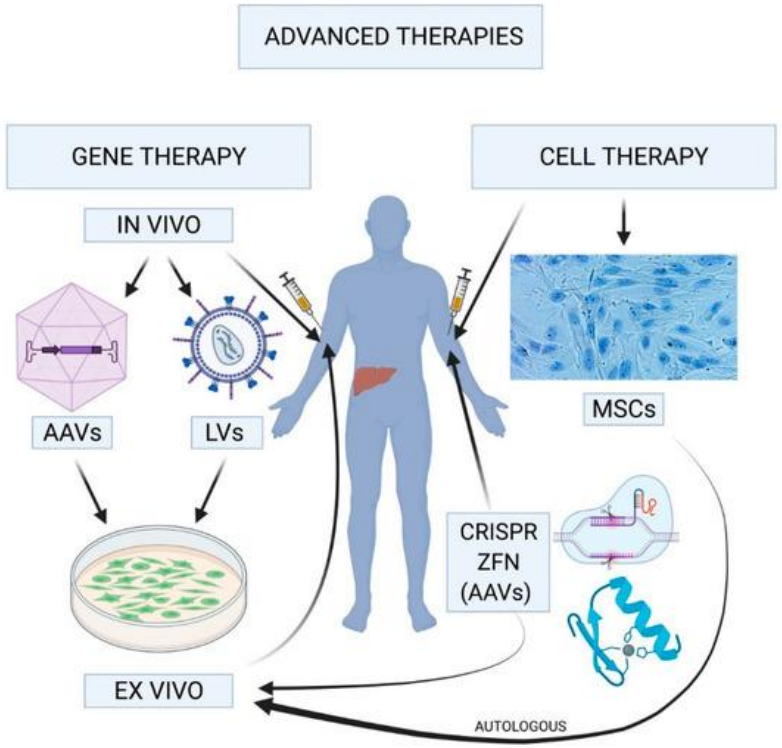
ATMPs are particularly relevant for pediatric patients due to their potential to treat **monogenic rare diseases** that manifest themselves in the **fetal or neonatal period**, where conventional treatments are often lacking or palliative.

Targeting Monogenic Rare Diseases

- Many severe pediatric diseases are caused by single-gene mutations (monogenic disorders), making them ideal candidates for gene therapy or cell-based treatments.
- ATMPs offer the potential for curative treatments rather than just symptom management.

Targeting early-onset, life-threatening conditions

- Many rare diseases have fetal or neonatal onset, leading to rapid disease progression.
- Early gene or cell therapy intervention can prevent irreversible damage and improve long-term outcomes.



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ATMPs and paediatric population

ATMPs and paediatric population

ATMPs are increasingly relevant to treat and cure paediatric diseases.

Of the 18 ATMPs currently licensed, 10 include or are dedicated to pediatric patients.

ATMPs and small target population size

However, the small target population size limits the potential return on investment leading to a market failure or lack of for-profit investments in development

No remove

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Challenges for developing ATMPs

Scientific & Technological

- Complexity of Technologies
- Biological Individual Variability
- Sustainability and Scalability
- Long-term Monitoring

Regulatory and Normative

- Ethical and Regulatory complexity
- GLP toxicology studies
- GMP manufacturing designed for small molecule medicinal products

Implications for Patient Safety

- Lack of GMP, GLP, or GCP (pediatric) standards
- Long-term issues as juvenile animal models are insufficient for evaluating treatment outcomes.

Challenges for developing ATMPs

Marketing obstacles

- Development changes implies small size populations
- Individualised Treatments resulting in high cost

Health Technology Assessment (HTA)

- HTA processes may struggle to assess the overall impact of these therapies on patients and healthcare systems, due to gaps in long-term safety & efficacy data.

Manufacturing cost reduction

- Reducing high costs through automation.
- Platform standardization for genetic therapies.

IP protection

- Cost of IP protection
- Identity definition *copycat?*

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Challenges for developing ATMPs in the paediatric population

Financial Challenges

- High Development Costs
- Business Model and Patient Access

Marketing challenges

- **How to commercialize pediatric ATMPs with a limited target population w/o increasing the single treatment cost to unsustainable levels?**
- **Role for non-profit development by academic institution ?**
- **Open license for non-profit or generic products ?**

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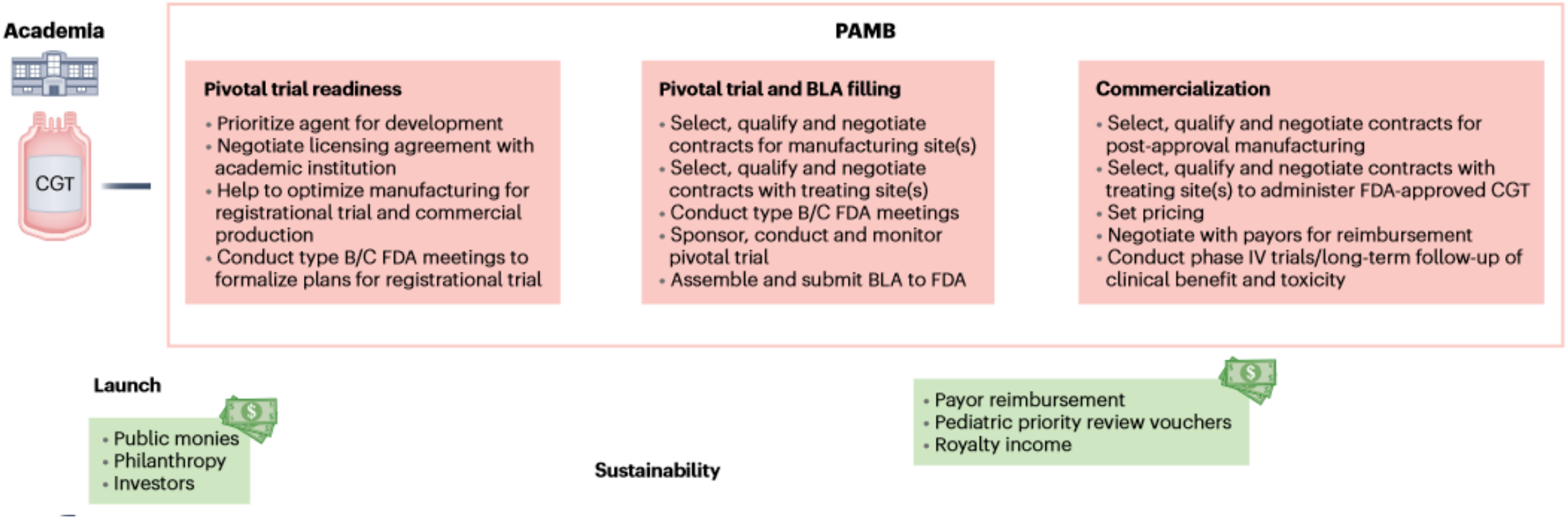
How to meet these challenges ? USA proposal

A Pediatric Advanced Medicines Biotech



Mackall, C. L. *et al.* Enhancing pediatric access to cell and gene therapies. *Nat Med* 1–11 (2024) doi:10.1038/s41591-024-03035-1.

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What role for EPTRI?

1. Centralised Services

- Many of the proposed PAMB activities are of supporting nature, including advice (preclinical and clinical) and networking. **Action: Creation of an expert group to provide the required services**

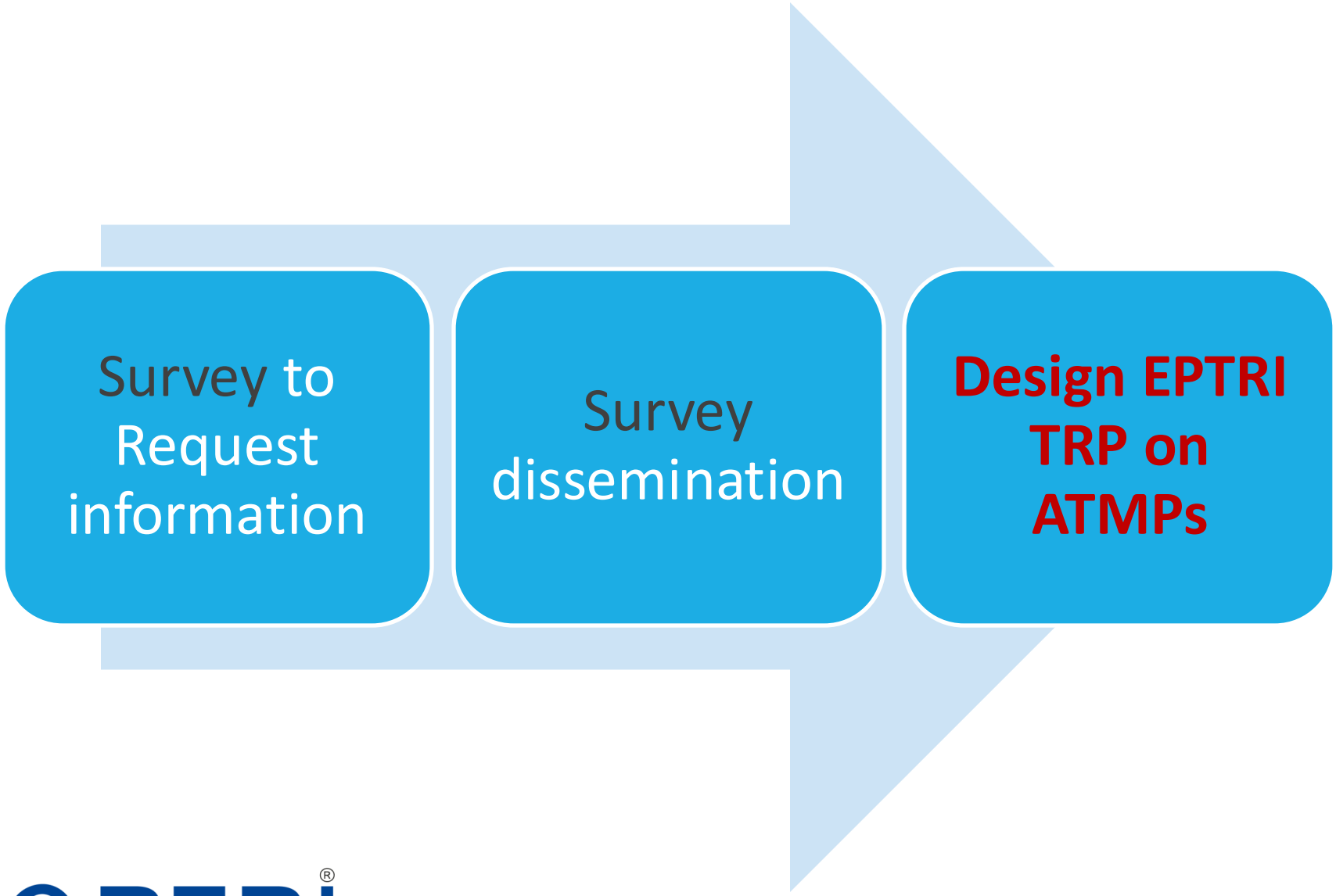
2. Dedicated EPTRI Thematic Research Platform

- Institutions with relevant technical capability for the manufacturing and testing of ATMPs
- **Action: identify the partner institution**

3. Networking with EU and international organizations

- Mapping and contacting existing organizations dedicated to ATMP
- Action: a concerted action for rare and ultra-rare non profitable treatments

Ongoing activities in EPTRI



AIMS of the Thematic Research platform on ATMPs

- ✓ Define the available Services
- ✓ Description, pricing and marketing
- ✓ Cooperative research/participation to calls for basic and applied research
- ✓ Network to enhance both the innovation and availability/access to innovative ATMPs

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Challenges for developing ATMPs

Scientific and Technological

- Complexity of Technologies
- Biological Variability
- Sustainability and Scalability
- Long-term Monitoring

Regulatory and Normative

- Lack of Clear Guidelines
- Ethical and Regulatory complexity
- OGM rules application
- GLP toxicology studies
- GMP manufacturing for medicinal products

Implications for Patient Safety

- Compromised Safety: caused by Lack of GMP, GLP, or GCP standards
- Long-term issues arise when juvenile animal models or regulatory measures are insufficient for evaluating treatment outcomes.

Manufacturing

- Reducing high costs through automation.
- Platform standardization for genetic therapies.

IP protection and licensing

- Cost of IP protection
- Open license for non-profit or generic products

Health Technology Assessment (HTA)

HTA processes may struggle to assess the overall impact of these therapies on patients and healthcare systems, creating gaps in long-term safety analysis.

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