EPTRI®

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

ATMP development infrastructure for paediatrics

CVBF

EPTRI GA – Bari – 18/07/2024

Advanced Therapy Medicinal Products (ATMPs) and Pediatrics

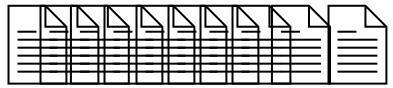
ATMPs are increasingly relevant to treat and cure pediatric diseases. Of the 18 ATMPs currently licensed, 10 include or are dedicated to pediatric patients.

Moreover, these therapies are often a single shot cure for monogenic inherited disease with no satisfactory current treatment.

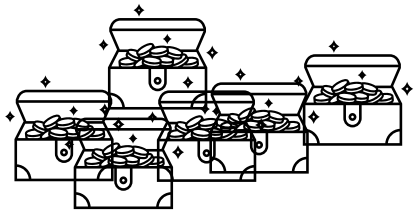
However, the small target population size limits the potential return on investment leading to a market failure or lack of for-profit investments in development.

What is done to remedy this?

Academic institution entering ATMPS development (450k references in the last 10 years)



Increased funding for innovation including phase I/II clinical studies from the EU programs and NIH.



Finally. remedies for academic institutions regulatory challenges are in place or proposed.

Translational Initiatives

Europe: A proposed European network of GMP manufacturing centers to deliver CAR T cell products for pediatric cancer multisite trials.

Spain: The Hospital Clínic de Barcelona manufactures and treats patients with ARI-001 CAR T cells under the **Hospital Exemption** national approval pathway.

UK and Europe: The AGORA (Access to Gene Therapies for Rare Disease Initiative) to explore creation of an independent, sustainable and not-for-profit entity to support marketing authorization, delivery and access to therapies that are not commercially sustainable.

Italy: Fondazione Telethon, an Italian biomedical charity focused on genetic diseases, is testing the feasibility of becoming a non-profit marketing authorization

Technical and regulatory challenges

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

- Regulatory challenges
  - The applications of OGM rules
  - Obligatory GLP toxicology studies
  - Obligatory GMP manufacturing for medicinal products

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

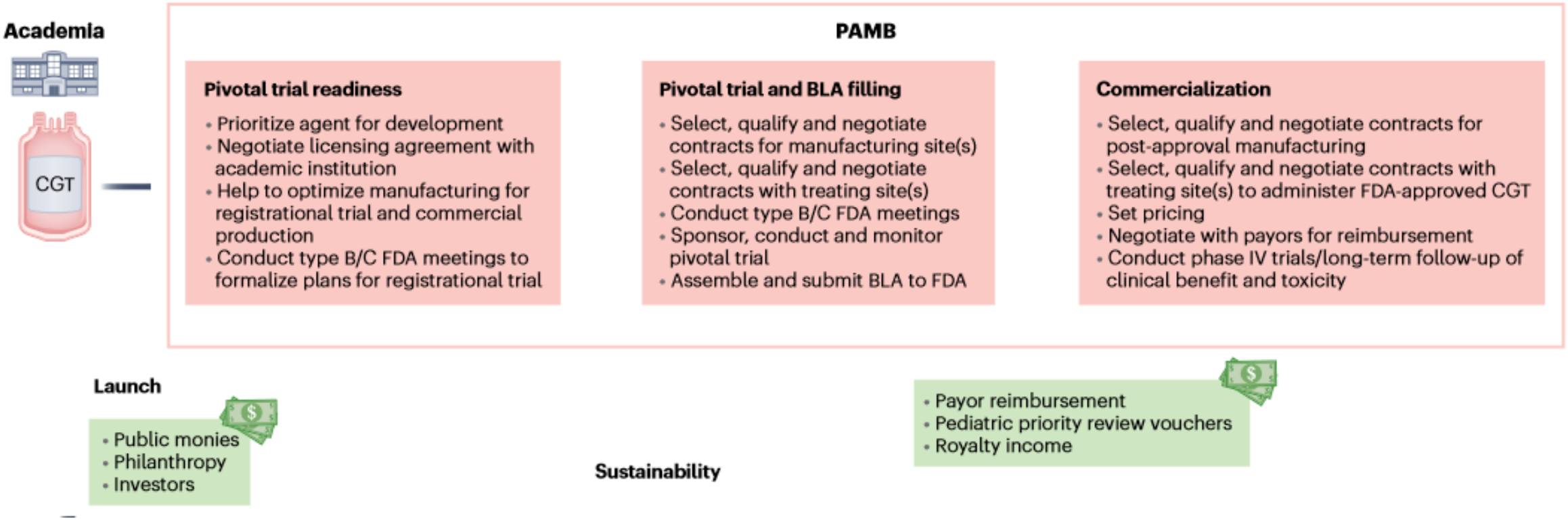
Marketing challenges

- Strategic partnering within the academic/hospitals ecosystem
- Leveraging accelerated approval and cost recovery pathways
- Establishing licensing practices that protect IP
- Life cycle of a pediatric CGT developed by the PAMB
- Funding the long-term

How to commercialize pediatric ATMPs with a limited target population w/o increasing the single treatment cost to unsustainable levels?

How to meet these challenges ?

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.

EPTRI®
European Paediatric Translational Research Infrastructure

What role for EPTRI?

- **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
- **Dedicated TRP**
  - Institutions with relevant technical capability for the manufacturing and testing of ATMPs
  - Action: identify the partner institution
- **Networking with EU and international organizations**
  - Mapping and contacting existing organizations dedicated to ATMP

The end or the beginning?

Thank you