



# Advanced Therapies in paediatrics

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# What is an Advanced Therapy ?

- In the EU is defined by the regulation 1394/2007
- Centralized procedure for MA is compulsory
- Dedicated Committee for Advanced Therapies (CAT) at the EMA

# What are the ATMP Advanced Therapies Medicinal Product

- They are defined as Cell Therapy Medicinal Products (CTMP); Gene Therapy Medicinal Products (GTMP), Tissue Engineering Products (TEP)

# Relevance for Paediatric population

- Research mainly driven by Academic Groups
- Especially active in marginal markets
  - Rare diseases
  - Paediatric diseases of genetic origin

# Gene therapy for ADA-SCID deficiency

- Developed by the Telethon foundation
- Rare paediatric disease
- Acquired by GSK, which paved the way to MA Strimvelis (2016)
- December 2017 - GSK announces to exit the field before a full reimbursement policy is defined

# Scope for a AT WG

- Diffusion of the knowledge about ATMP manufacturing in the Paediatric community
- Definition of the specific requirements for paediatric applications
- Focus on the research in human development underpinning the development of dedicated therapies
- Focus on the business model required for their application