EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Mechanism of action based PIPs in paediatric oncology

EnprEMA annual meeting

Maria Sheean, Dominik Karres, 07 November 2025

Mechanism of action based PIPs in paediatric oncology

- Regulatory Framework Evolution: US Regulation (FDARA 2017)
  - Promote mechanism-of-action based pediatric drug development, independent of adult cancer indications.
- Pediatric cancers differ biologically from adult cancers
  - Adult data on activity most likely challenging to extrapolate to children
  - Limited patient populations
- Need for robust non-clinical data to balance (lack of) efficacy versus safety & support go/no go decision
  - Academic activities anticipating: EU ITCC P4 – US PIVOT
- Lack of regulatory framework, need for capacity building & structured assessment

Mechanism of action based PIPs in paediatric oncology

- Work in progress
  - Drafting Group of NcWP consisting mostly of non-clinical experts
  - Analysis of legacy PIPs
  - **Draft Concept Paper on proof-of-concept data supporting the development of anti-cancer products in paediatric patients**

Draft Concept Paper on proof-of-concept data supporting the development of anti-cancer products in paediatric patients

- Draft Concept Paper
  - Involvement of clinical experts
  - Consultation of relevant committees/groups + FDA
  - Public consultation  Q1 2026
  - Workshop  Q2-3 2026
- Reflection paper
  - Including a process for consultation of stakeholders, including academic experts/consortia and regulatory bodies like the FDA to support informed decision-making

Any questions?

Further information

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