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Mechanism of action-based PIPs in paediatric oncology and beyond

16th Industry stakeholder platform on research and development support

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Reform of the EU pharmaceutical legislation- Paediatrics

It covers medicines for human use throughout their lifecycle, from development support to safety monitoring.

Key areas include:

- support to **innovation**
- the evaluation of medicines and structure of **EMA's scientific committees**,
- **paediatric** and **rare disease** medicines,
- medicine **shortages**, and
- **environmental sustainability**.

Changes to the **Paediatric Investigation Plan concept and processes**

Introduction of the **Mechanism of Action (MoA) concept** in waiver discussions

- Reform of the EU pharmaceutical legislation (europa.eu) - 26 April 2023
- https://www.ema.europa.eu/en/about-us/what-we-do/reform-eu-pharmaceutical-legislation

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

- Concept Paper
  - Public consultation finished on 30 June 2026
  - **Workshop** on advancing proof-of-concept data in paediatric oncology drug development:

Save the date: 21 September 2026, 13:00-16:00 CET

- Reflection paper
  - Including reflections on the methodology of assessment and the process for consultation of stakeholders, including academic experts/consortia and regulatory bodies like the FDA to support informed decision-making

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16 February 2026
EMA/32831/2026
Non-clinical Working Party (NcWP)

Concept paper on the development of a reflection paper on proof-of-concept data to support the development of anti-cancer medicinal products in paediatric patients

Agreed by Non-clinical Working Party	5 February 2026
Adopted by PDCO	27 February 2026
Adopted by CHMP for release for consultation	16 February 2026
Start of public consultation	13 March 2026
End of consultation (deadline for comments)	30 June 2026

Comments should be provided using this EUSurvey form. For any technical issues, please contact the EUSurvey Support.

Keywords	mechanism of action, paediatric oncology, non-clinical, weight of evidence, proof of concept

Short Communications – keeping stakeholders informed

Two short communication publications will be submitted imminently to a Peer-review journal:

- **Translating non-clinical proof-of-concept into paediatric development decisions**: the case of HER2-targeting antibody-drug conjugates
- **Integrating non-clinical and clinical evidence for prioritisation** in paediatric oncology drug development: regulatory experience with PARP inhibitors

MoA concept – learnings from oncology

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Domains	Challenge	Need	Action
1. Vision & Education	Understanding background to MoA-based (regulatory) expectations across stakeholders	Develop shared understanding incl. individual responsibilities towards delivery	Publications*, Educational programs (eg ALADDIN), Conferences
2. Ecosystem & Infrastructure	Understanding (academic) research landscape - Need for data and resource access initiatives	Develop knowledge gap analysis – able to support future research investments Develop mechanism to share learnings	ITCC, ITCC-P4, ACCELERATE (Paediatric Strategy Forums), Academic sponsored platform trial**
3. Scientific content & decision making	Need for structured approach to scientific MoA based development discussions across decision makers	Develop scientific principles, convene stakeholders and decision makers, to enable evidence-based R&D discussions on feasible priority MoA driven developments	Concept paper on development of reflection paper Short communications Facilitation framework proposal***

* Implementation of mechanism of action biology-driven early drug development for children with cancer Eur J Cancer. 2016 Jul:62:124-31
ACCELERATE - Five years accelerating cancer drug development for children and adolescents . 2022 May:166:145-164. doi: 10.1016/j.ejca.2022.01.033
European regulatory strategy for supporting childhood cancer therapy developments. 2022 Dec:177:25-29. doi: 10.1016/j.ejca.2022.09.025. Epub 2022 Oct 6.
** https://mrctcenter.org/platform-oncology/
***https://www.ema.europa.eu/en/documents/presentation/presentation-reflections-concept-facilitation-framework-dkarres_en.pdf

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Next aims: the workshop – register and submit questions!

- to present the outcomes following the public consultation on the **Concept Paper**;
- to gather **stakeholder feedback** on key questions, data needs, and perceived challenges to practical application, with a view to further strengthen the evidence-base supporting paediatric oncology development;
- to leverage learnings from oncology and discuss challenges in mechanism-of-action based paediatric developments **beyond oncology**, and what is needed to support such developments as foreseen under the new EU Pharmaceutical Legislation across other therapeutic areas.

https://www.ema.europa.eu/en/events/workshop-advancing-proof-concept-data-paediatric-oncology-drug-development

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Thank you

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