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PDCO update

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Including slides of Chrissi Pallidis, Paediatric Medicines Office, EMA, with thanks

An agency of the European Union



Disclaimer

The presenter does not have any conflict of interests.

Personal views are presented.

Stepwise PIP

- **2023 Pilot phase:** Launch Feb, Opinion key elements new proposal June, PIP scientific document update November
- **2024 Confirmed initiative:**
 - Published guidance*
 - PDCO procedures: 11 sPIP, 5 agreed opinions
- Therapeutic areas identified: oncology ; metabolic diseases, neurology, inflammatory (genetic disorders) ; cardiology ...

* [guidance-stepwise-pip-pilot_en.pdf \(europa.eu\)](#)

[Paediatric investigation plans: questions and answers | European Medicines Agency \(EMA\) \(europa.eu\)](#)

sPIP with an Opinion on a plan not yet fully defined

- In general, all **PIP measures** can be agreed upon at the time of **initial PIP application**
- **When crucial data are not yet available** to sufficiently define all the key elements (KEs) of the planned measure and by when → **PIP stepwise approach**
- **Minimum set of data** required initially → condition, age subsets, preliminary outline of planned studies and completion date, but defining them and by when
- The **sPIP will be modified** when relevant data available as agreed in milestones → **fully developed PIP at the end** (same details as for conventional PIP)
- **Paediatric development must not be delayed**

PDCO interactions

- **Expert groups :**
 - NcWG / MOESG
 - OncoWG / NeurologyWG / ImmunolyWG / ... -> guidelines
- **CTCG**
 - Adolescents inclusion recommendations 2 S 2023
 - Cases under analysis (safety)
- **CHMP**
- **SAWP**
 - Monthly alignment, concomittant submissions with PIPs to avoid

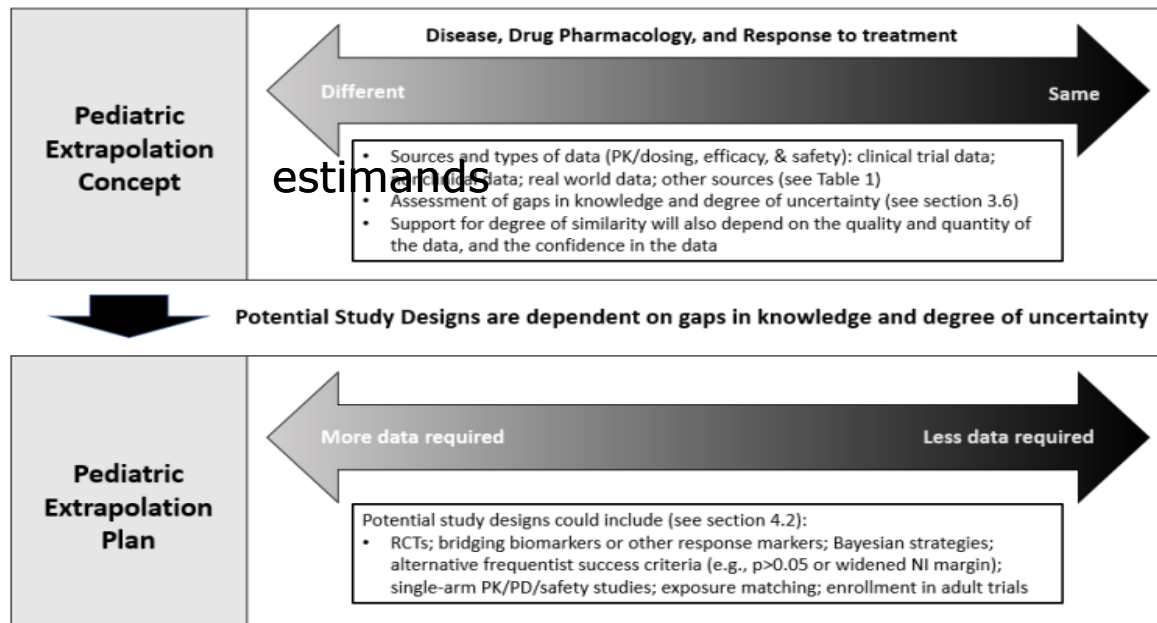
* [ICH guideline E11A on pediatric extrapolation - Scientific guideline | European Medicines Agency \(EMA\) \(europa.eu\)](#)

Extrapolation

- 2024 ICH international guideline E11A on pediatric extrapolation

- step 3 adopted
- Dose, PK, biomarker, MS
- Methodology (estimands)
- External data

1: Pediatric extrapolation as a continuum



Paediatric curriculum in EU

- Potential for any short session in EU for assessors?
- Updates / new information available on paediatrics in diseases, clinical endpoints and biomarkers, maturation and developmental aspects, pharmacometrics, formulation, ... might be welcomed

Questions?



Thank you for your attention!

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