

Empowering Pediatric Drug Development: CDISC standards and ICH guidelines for Clinical trials with Little Participants

2023

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November 2023
DS05: Phuse EU Connect 2023

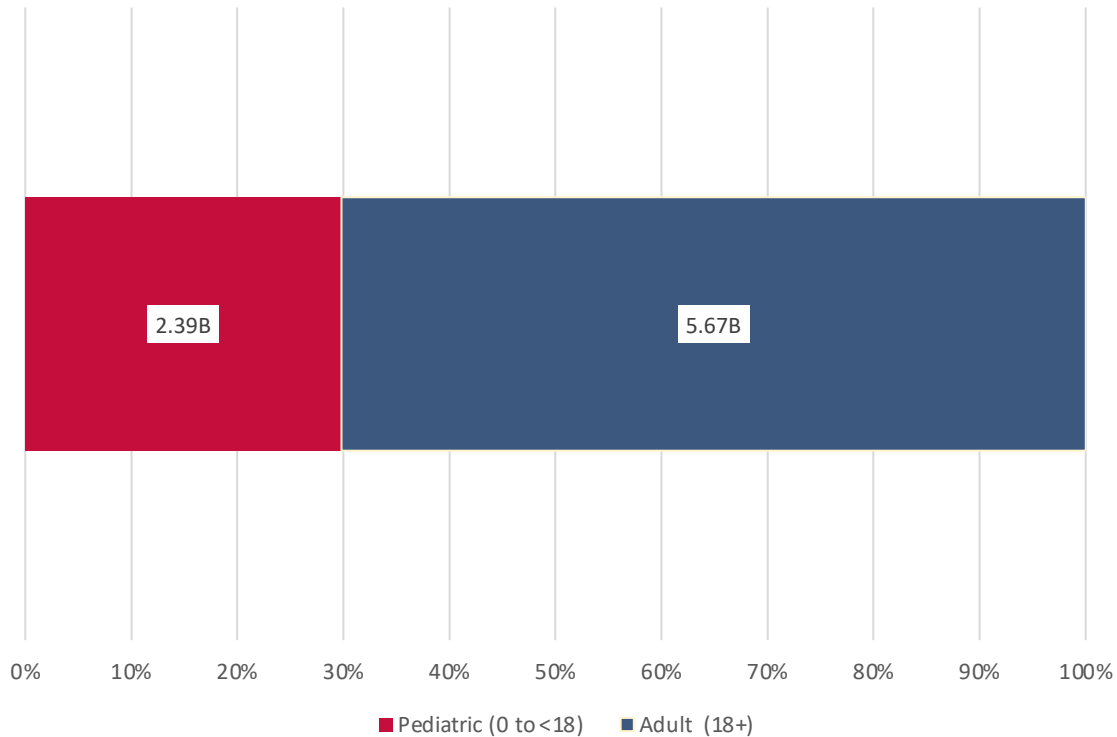
Disclaimer

While we journey through this presentation, please note that the viewpoints articulated herein are the author's own. They do not necessarily reflect the official stance of the organization the author represents. We all value diverse perspectives, and this presentation is one such perspective, offered for thoughtful consideration.

- Introduction
- Regulatory Requirements
- ICH E11 & CDISC TAUG
- Conclusion

Pediatric Drug Development today

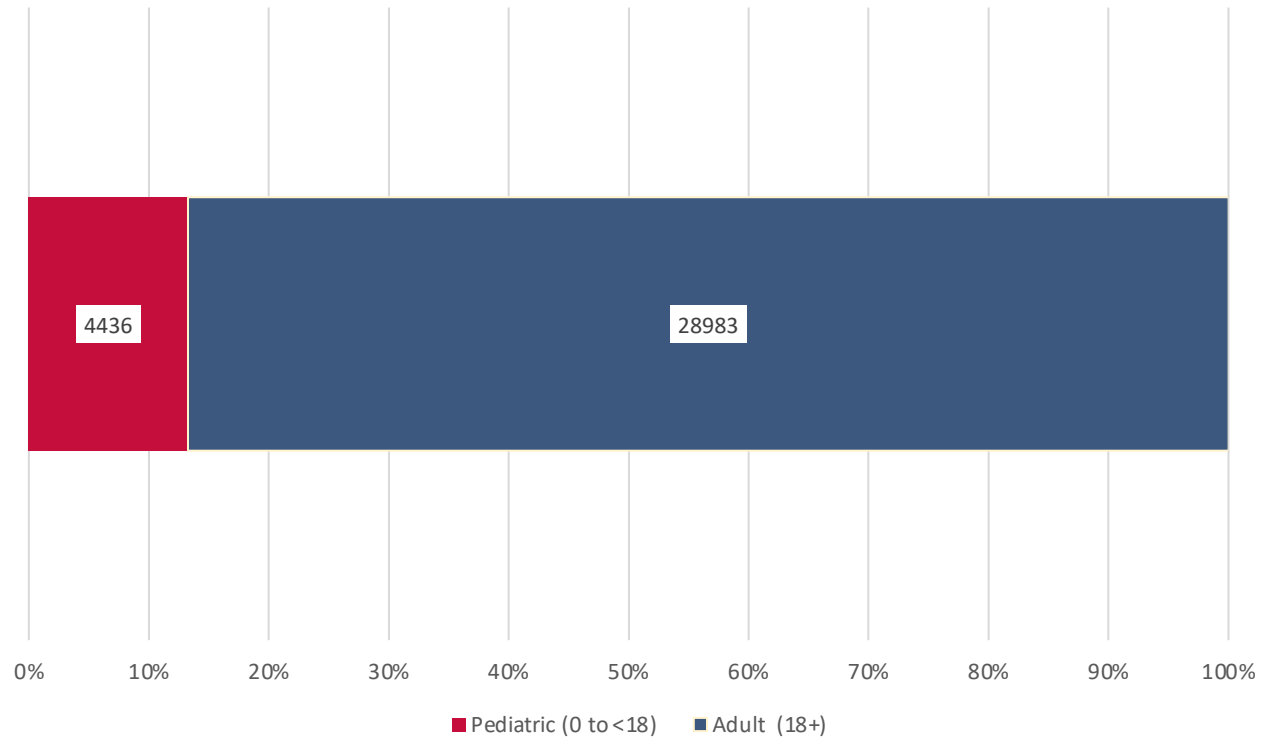
World Population*



Source : <https://population.un.org/>

*Data as of Oct-2023

Clinical Trials*

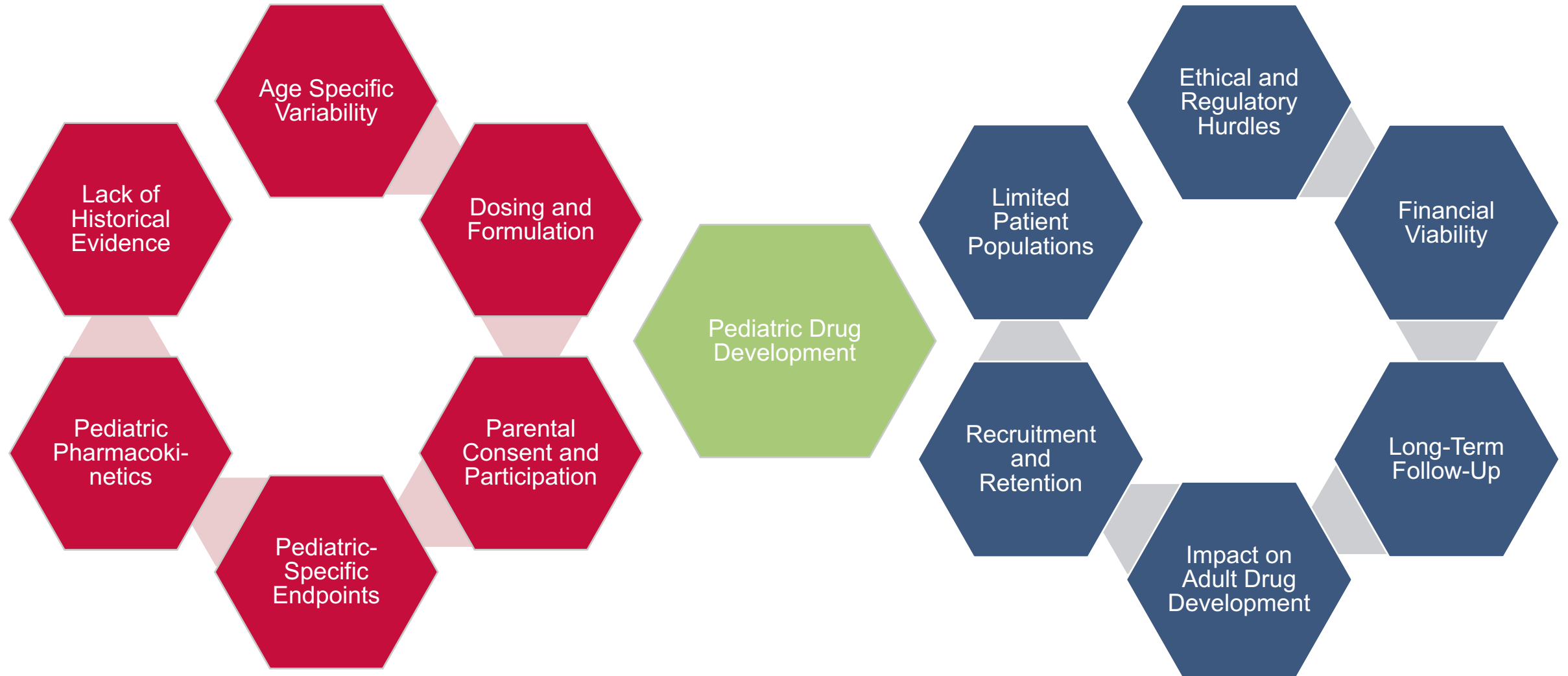


Source : <https://clinicaltrials.gov/>

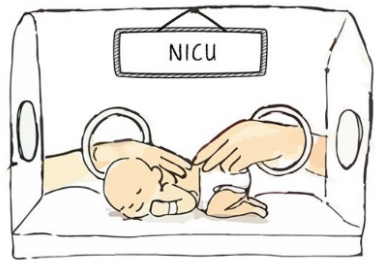
*Active interventional trials as of Oct-2023



Challenges around...



Pediatric sub-classes



Preterm and term neonates
0 to 27 days



Infants and toddlers
28 days to below 2 years



Children
2 years to below 12 years



Adolescents
12 years to below 18 years

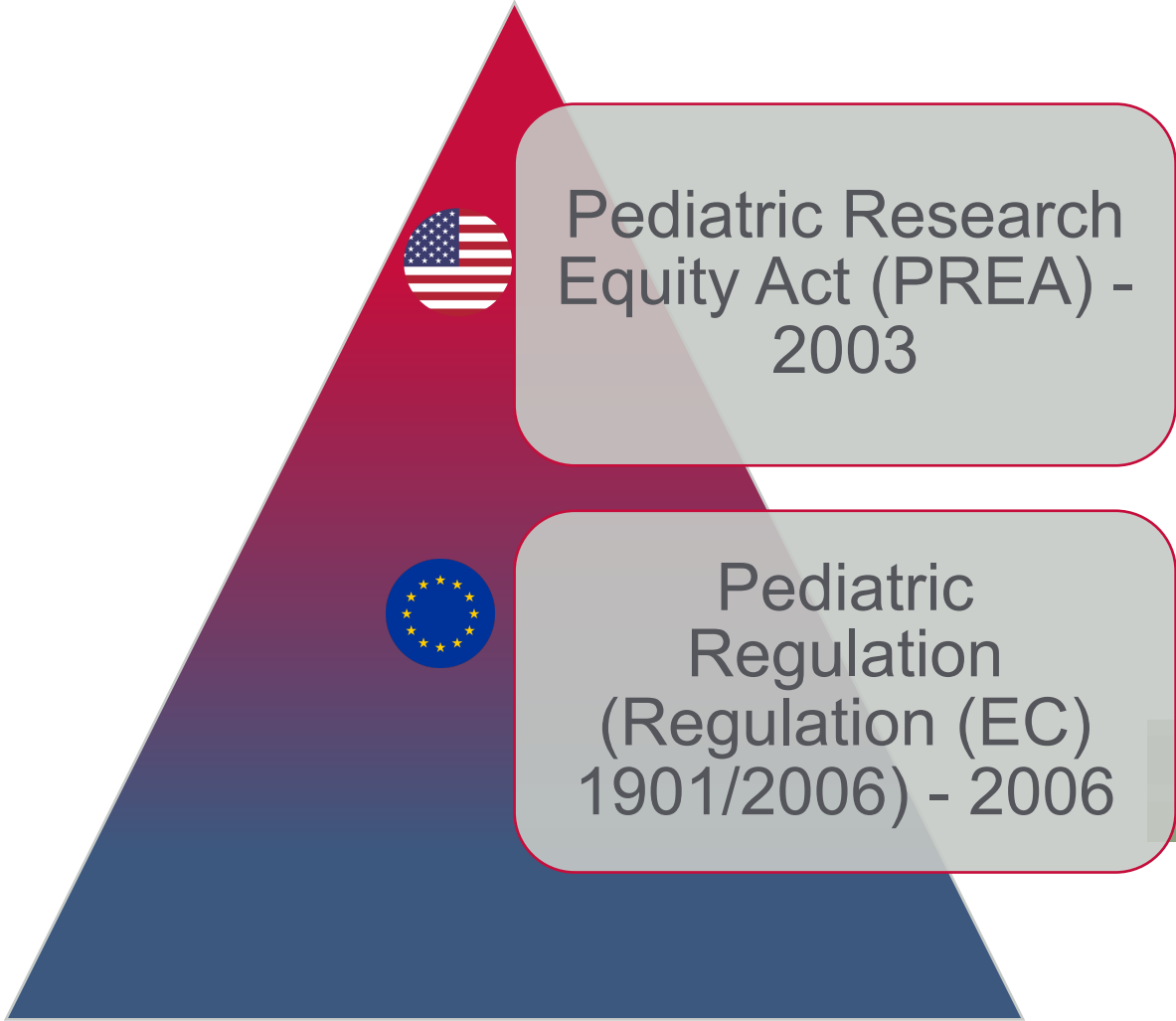
Tailoring Treatments for the future

Requires careful attention to ethical, scientific, and practical considerations.

Pediatric clinical trials are crucial for advancing medical knowledge and improving healthcare for Pediatric population.

Need for more Harmonization & Standardization.

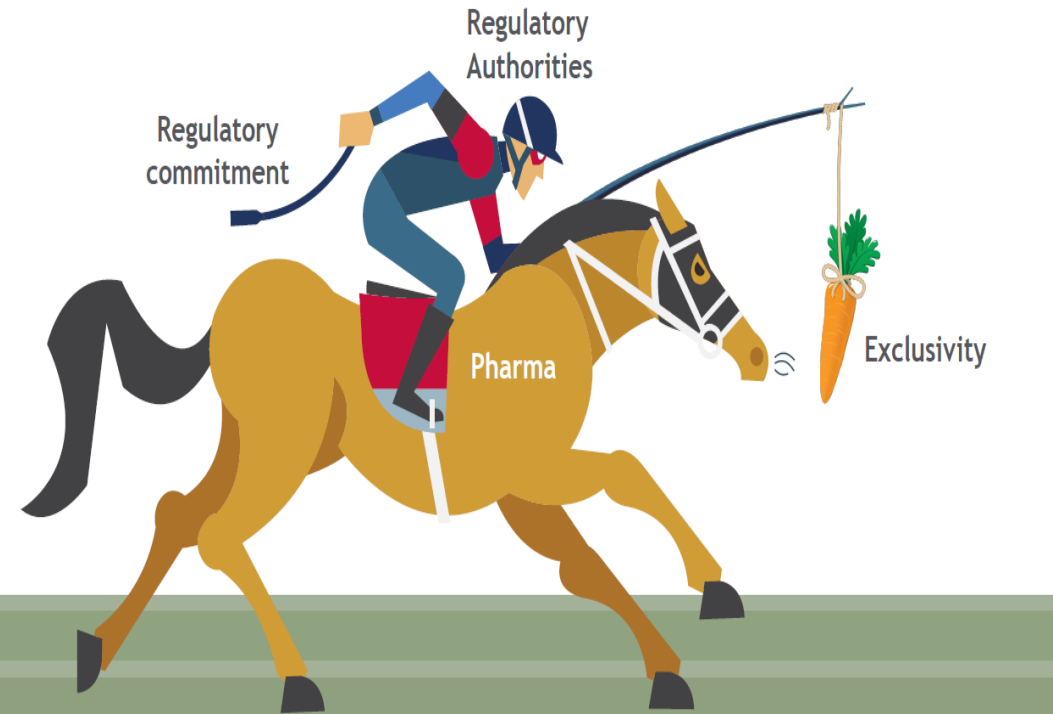
Navigating through regulatory requirements



Pediatric Research
Equity Act (PREA) -
2003

Pediatric
Regulation
(Regulation (EC)
1901/2006) - 2006

Ethics



Pediatric Investigation Plan & Pediatric Study Plan



PIP

Purpose: A Pediatric Investigation Plan (PIP) is a regulatory requirement in the EU for pharmaceutical companies developing medicinal products for pediatric population and is typically submitted early in the drug development process, often during the initial phases of clinical development before marketing authorization application.

Scope: PIPs are required for new drugs, biological products, or changes to existing products, including new indications, new route of administration and formulations, that are under development for diseases that also affect pediatric population.

Contents: A PIP includes details on the proposed pediatric studies, timelines, and measures to adapt the drug's formulation and dosing to suit different age groups within the pediatric population. The plan must be agreed upon by the EMA before marketing authorization.

Enforcement: Failure to comply with a PIP can result in regulatory consequences, such as delaying marketing approval or potential financial penalties.



PSP

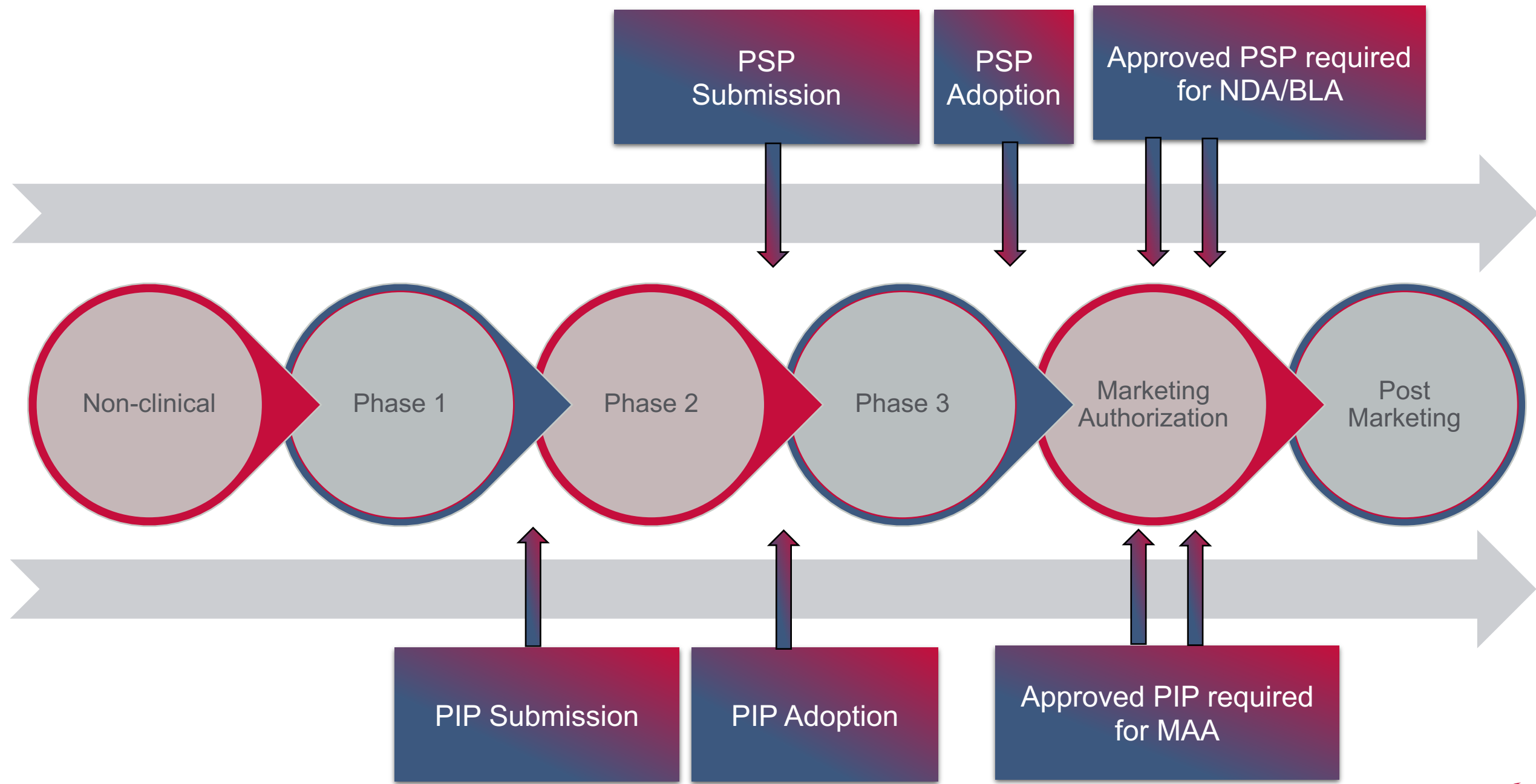
Purpose: Pediatric Study Plan (PSP) is a similar requirement in the United States regulated by the US FDA. PSP is required later in the development process, typically when a drug is already in the approval pipeline to ensure that pediatric medications undergo rigorous testing to establish their safety and efficacy.

Scope: PSPs are typically required for drugs and biological products if sponsor is planning to submit a marketing application for a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration.

Contents: A PSP outlines the pediatric studies that the sponsor plans to conduct, along with their timelines and details. The plan is submitted to the U.S. Food and Drug Administration (FDA) as part of the drug approval process.

Enforcement: Non-compliance with PREA requirements can lead to regulatory consequences, such as delayed approval of the drug or the imposition of post-marketing pediatric study requirements.





Streamlining Pediatric Clinical Trials Through Guidelines



Importance of Harmonization & Standardization...

Provides guidance for the design, conduct, & evaluation of clinical trials involving pediatric participants

ICH E11



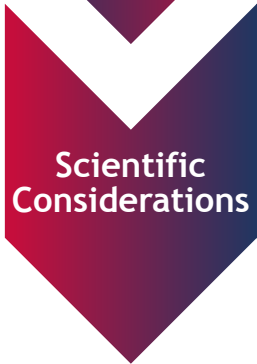
CDISC
TAUG

Elaborate the utilization of **CDISC** standards for the representation of data relevant to pediatric research

ICH E11: Significance in Pediatric Drug Development



- Emphasising the ethical considerations involved in pediatric research, emphasizing the need for informed consent, assent, and the role of ethics committees in reviewing pediatric trial protocols.





Data Collection and Analysis

- Discusses the collection, analysis, and presentation of data from pediatric clinical trials. It provides guidance on statistical methods and data handling to assess the efficacy and safety of the investigational medicinal product.

Extrapolation

- Acknowledges the importance of extrapolating data from adult trials to support pediatric development when feasible and scientifically justified. This approach helps reduce the need for unnecessary pediatric trials.

Formulation and Dosage

- Encourages pharmaceutical companies to develop age-appropriate formulations and dosage forms for pediatric patients. This is critical to ensure accurate dosing and safety in this population.

Post-Approval Considerations

- The guideline also discusses post-approval commitments and the importance of ongoing monitoring of pediatric drug use, including the need for long-term follow-up and the consideration of labelling updates based on new pediatric data.

Pediatrics User Guide (TAUG)



Summary...

- ❖ Pediatric drug development requires a comprehensive approach that considers ethical, scientific, & practical factors which will eventually improve healthcare outcomes globally of future generation.
- ❖ ICH E11 and the CDISC Pediatric User Guide provide essential guidance and standards for optimizing pediatric drug development.
- ❖ Integration of these principles into clinical trial design, data collection, analysis, and submission processes is crucial.
- ❖ Benefits include accelerated approvals, enhanced cross-trial comparisons, cost reduction, and fostering international collaboration in pediatric drug development.

REFERENCES

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<https://www.cdisc.org/>

<https://www.ema.europa.eu/en/human-regulatory/research-development/paediatric-medicines/paediatric-investigation-plans>

<https://www.scendea.com/regulatory-aspects-of-paediatric-drug-development>



ACKNOWLEDGMENTS

The author would like to thank Caroline Shi, Kulin Varma and Susanne Crowe for their support and for providing their valuable input for this paper. The author would also like to acknowledge the Gilead Clinical Data Science leadership team for their support in continuous learning and participation in conferences.



Thank You

Your comments and questions are valued
and encouraged.

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