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Holistic approach to paediatric research - the industry perspective on challenges and opportunities

Authors: Lene Rose Arfelt, Tillmann Taube; Martine Dehlinger-Kremer

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Holistic approach to paediatric research - the industry perspective

- **The Paediatric Regulation has had a positive impact on paediatric drug development**
- **Timely completion of paediatric development programmes remains a challenge**
- **Topics to be considered to improve on definition, early start and completion of paediatric development programmes :**
    - Identification of paediatric needs, PIP planning, preparation and agreement
    - Alignment with different stakeholders (patients, academia, regulators, industry)
    - Conduct of paediatric studies, trial preparedness, feasibility

Identification of paediatric needs, PIP planning, preparation and agreement

Industry Challenges	Opportunity

• Select the right scientific approach to address paediatric needs:
  • Right indication
  • Right population
  • Right trial design / endpoints
	• Establish early dialogue with all stakeholders to agree on paediatric strategy/needs and to ensure the scientific viability of the PIP
	  • Implement early strategic interface (prior to draft PIP) between Enpr-EMA, PDCO and industry to discuss paediatric strategy
	• Ensure global alignment on scientific approach, incl. the selection of indication, population and endpoints

• PIP planning takes place early and is based on preliminary / limited data or assumptions	• Built paediatric development programmes and trials progressively (iterative PIP) to align with the overall product development and specify PIP details as data are emerging

Conduct of paediatric studies, trial preparedness, feasibility

Industry Challenges

- Practical issues with conduct of paediatric clinical trials (especially in rare paediatric diseases and paediatric oncology)
- Competitive paediatric studies in rare paediatric diseases (small population) with similar agents

Opportunities

- Improve trial preparedness:
  - Establish early dialogue with all stakeholders for alignment on paediatric needs and feasibility
  - Use and expand infrastructure (support networks) for paediatric clinical trial conduct
- Use innovative approaches (e.g., extrapolation) to reduce patient numbers, while increasing the usefulness of generated data
- Ensure international / global alignment and cooperation (e.g., further expand Enpr-EMA beyond Europe)

Conclusion – Mitigating Challenges By Creating Opportunities

Opportunities to improve on PIP planning and preparation, paediatric trial preparedness and timely completion of paediatric development programmes / PIPs:

- **Establish early dialogue and alignment** with all stakeholders
  Proposal: implement early strategic interface (prior to draft PIP) between Enpr-EMA, PDCO and industry to discuss paediatric strategy, paediatric needs, selection of paediatric indication, population and endpoints

- **Use and expand infrastructure (networks)** for the conduct of global paediatric development programmes
  Proposal: support networks and strengthen global alignment and cooperation by further expanding Enpr-EMA beyond Europe

- Use **innovative approaches** to reduce number of patients to be enrolled, while increasing the value of generated data
  Proposal: promote innovative approaches by close collaboration between Enpr-EMA and industry (consider separate Enpr-EMA workstream on the topic)