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Human Medicines Division

Paediatric Committee (PDCO): Work Plan 2024

Adopted by the Committee on 15 December 2023

Table of Content

1. Evaluation activities for human medicines	2
1.1. Pre-authorisation activities	2
1.1.1. Development of Therapeutic Areas strategies.....	2
1.1.2. Real world evidence	2
1.1.3. Stepwise PIP.....	3
2. Horizontal activities and other areas	4
2.1. Committees and Working parties.....	4
2.1.1. Collaboration with CTCG	4
2.1.2. Collaboration with HMPC.....	4
2.1.3. Collaboration with Patients' and Consumers' working party	4
2.1.4. PDCO involvement in PIP-related CHMP procedures	5
Key objectives	5
2.1.5. Collaboration with stakeholders	5

The activities outlined in the PDCO work plan for 2024 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2023-2025.

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1. Evaluation activities for human medicines

1.1. Pre-authorisation activities

1.1.1. Development of Therapeutic Areas strategies

Key objectives

- Define strategies on how to approach PIPs for identified therapeutic areas or certain paediatric developments.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- Multi-stakeholder paediatric oncology strategy forum on brain tumours in children and adolescents in Q1 2024 and on anti GD2 antibodies in Q3 2024.
- Publication of the outcome of above workshops.

PDCO topic leader: Sylvie Benchetrit

- C4C (Conect4children) activities on future topics (e.g. paediatric psychiatry - unmet need in symptom of irritability).

PDCO topic leader: Sylvie Benchetrit

- Development of the guideline on neonatal therapeutics.

PDCO topic leader: Dina Apele-Freimane

- To explore the mode of action approach for PIPs on non-oncology medicines.

PDCO topic leader: Sylvie Benchetrit, Sabine Scherer

- Consolidate experience of regulatory science in support of development of fixed-dose combination products for treatment and prevention of HIV.

PDCO topic leader: Maria Fernandez Cortizo

- Retrospective review project on the regulatory efficiency of the weight of evidence (WoE) approach in ICH (International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use) guideline S11.

PDCO topic leader: Karen Van Malderen

- Update of the paediatric section of the obesity guideline.

PDCO topic leaders: Carine de Beaufort, Helena Fonseca

1.1.2. Real world evidence

Key objectives

- To identify how real-world evidence (RWE) can support paediatric development and promote its use.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- Continue the pilot on RWE studies including through DARWIN EU to support PDCO decision-making including identification of use cases where the evidence from real world data can support the scientific assessment. Provide expert input to a review of the experience gained with real-world data (RWD) studies conducted (as part of the pilot) across the regulatory network to support regulatory decision making.
- Provide expert input in support of the development of guidance on use of RWE for regulatory purpose.
- Define a framework for use of RWD/RWE in support of extrapolation of efficacy data to the paediatric population.

PDCO topic leader: Sylvie Benchetrit, Tomasz Grybek

1.1.3. Stepwise PIP

Key objectives

- To develop the stepwise PIP concept.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- Review the experience from the pilot scheme.
- Review the experience from the new key elements form (KEF) and scientific document also focusing on how extrapolation is reflected in these documents.

PDCO topic leader: Sabine Scherer, Siri Wang, Sylvie Benchetrit

2. Horizontal activities and other areas

2.1. Committees and Working parties

2.1.1. Collaboration with CTCG

Key objectives

- In line with the EMA/EC Action plan, to improve dialogue between EMA/PDCO and clinical trial assessors and facilitate mutual understanding of the interplay between assessment of PIPs and of clinical trials.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- Strengthen dialogue between PDCO and CTCG and establish the process for future interactions.
- Elaborate on criteria for joint adolescent-adult early phase clinical trials.
- Discuss specific PIP cases, particularly related to identified dependencies required prior clinical study initiations.

PDCO topic leader: Anette Solli Karlsen, Sabine Scherer

2.1.2. Collaboration with HMPC

Key objectives

- To improve the interaction between PDCO and HMPC to provide input to HMPC if needed / requested on matters relevant to paediatric medicines.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- Strengthen dialogue between PDCO and HMPC and establish the process for future interactions.

PDCO topic leader: Peter Sisovsky

2.1.3. Collaboration with Patients' and Consumers' working party

Key objectives

- To improve the interaction between PDCO and Patients' and Consumers' working party (PCWP) to provide input to PCWP on matters relevant to paediatric medicines.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- To contribute to the elaboration of a reflection paper to provide advice on the best EU approach to generate, collect and analyse patient experience data.
- Explore how to better reflect in the assessment the way that patient experience data is assessed and the rationale for acceptance/exclusion for benefit/risk decision-making.

PDCO topic leader: Johannes Taminiau

2.1.4. PDCO involvement in PIP-related CHMP procedures

Key objectives

- To follow CHMP procedures on PIP studies, to provide input to CHMP if needed / requested and to regularly inform the PDCO about critical issues identified by CHMP during assessment of paediatric data especially in cases where issues with the studies included in the PIP opinion were identified that require further discussion on PDCO's approach.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- Review of experience from 2023.
- Learnings on CHMP assessment on the use of extrapolation.

PDCO topic leader: Siri Wang, Sabine Scherer, Sara Vennberg (extrapolation)

2.1.5. Collaboration with stakeholders

Key objectives

- To increase interactions with stakeholders (including patients/public) and raise awareness of the work by the PDCO.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- To explore ways to improve communication on topics discussed during PDCO meetings, raise awareness on the work of PDCO and inform relevant stakeholders about important topics including new paediatric medicines.
- To explore ways on when, how and to what extent Young Persons Advisory Groups (YPAGs) could be involved in PIP procedures.

PDCO topic leader: Sabine Scherer

Key objectives

- To contribute to the update of the paediatric curriculum of the EU Network Training Centre (EU NTC) curricula.

Activities in 2024

PDCO activities to achieve the objectives set for this area:

- Analysis of learning needs and update of the paediatric curriculum taking into account new learning needs.

PDCO topic leader: Sylvie Benchetrit, Sara Vennberg, Francesca Rocchi