EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 December 2022
EMA/PDCO/867297/2022
Human Medicines Division

Paediatric Committee (PDCO): Work Plan 2023

Adopted by the Committee on 16 December 2022

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The activities outlined in the PDCO work plan for 2023 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 2023

PDCO activities to achieve the objectives set for this area:

- Multi-stakeholder paediatric oncology strategy forum PI3K/AKT/mTOR Pathway Inhibitors in children and adolescents in Q1 2023; and on CDK 2/4/6/9 inhibitors in Q3 2023.
- 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; pharmacotherapeutic interventions to improve outcome in asphyxiated neonates undergoing therapeutic hypothermia; Type I diabetes).

PDCO topic leader: Fernando de Andres Trelles (Psychiatry), Dina Apele-Freimane (Neonates), Carine de Beaufort (Type I diabetes)

- Development of the guideline on neonatal therapeutics.

PDCO topic leader: Dina Apele-Freimane

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 2023

- Conduct the pilot on RWE studies including through DARWIN EU® to support PDCO decision-making including identification of use cases where the evidence from real word data can support the scientific assessment.
- Provide expert input to a review of the experience gained with RWE studies conducted across the regulatory network to support regulatory decision making.
- Conduct a RWE interrogation and analysis for at least one paediatric condition, the results of which can be used in assessment of new PIP submissions.
- Provide expert input in support of the development of guidance on use of RWE for regulatory purpose.

PDCO topic leader: Sylvie Benchetrit, Tomasz Grybek

1.1.3. Stepwise PIP

Key objectives

- To develop the stepwise PIP concept.

Activities in 2023

- Publish guidance on the pilot phase for the stepwise PIP.
- Publish updated key elements form to be used for both stepwise and conventional PIPs.
- Start the pilot phase.
- Test the feasibility of processes.

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 2023

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 to clinical study initiations.

PDCO topic leader: Anette Solli Karlsen, Sabine Scherer

2.1.2. Collaboration with HTA bodies

Key objectives

- To improve dialogue between EMA/PDCO and HTA bodies and facilitate mutual understanding especially taking into account the increased use of extrapolation within PIPs.

Activities in 2023

PDCO activities to achieve the objectives set for this area:

- Publish a document reflecting on practical considerations related to the use of extrapolation from a regulatory and HTA perspective; linked to the priority activity reflected in the joint workplan of EUnetHTA21 and EMA.

PDCO topic leader: Siri Wang, Sylvie Benchetrit

2.1.3. 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 2023

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

2.1.4. 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 2023

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 and collect 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.

2.1.5. 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 2023

PDCO activities to achieve the objectives set for this area:

- Re-discuss the current approach to come to a more structured way of involvement.
- PDCO rapporteurs and the PDCO member from the same NCA as CHMP Rapporteur to follow CHMP procedures aiming at paediatric indications at the time of MAA.
- PDCO rapporteurs and the PDCO member from the same NCA as CHMP Rapporteur to provide feedback to the PDCO/CHMP on issues identified during assessment of paediatric data.

PDCO topic leader: Siri Wang, Sabine Scherer

2.2. Partners and stakeholders

2.2.1. Interactions with partners

Key objectives

- To support EC activities on the review of the paediatric regulation and to ensure that the experience and consolidated proposal of the PDCO are captured.

Activities in 2023

- Provide recommendations to EC on the new paediatric regulation, as needed. Work with the EC on implementing the legislation.

PDCO topic leader: Brian Aylward, Sylvie Benchetrit

2.2.2. Collaboration with stakeholders

Key objectives

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

Activities in 2023

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.

PDCO topic leader: Sabine Scherer