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EMA/PDCO/710956/2015
Procedure Management and Committee Support Division

PDCO work plan 2016

adopted by the Committee on 29 January 2016

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1. Evaluation activities for human medicines

1.1. Pre-authorisation activities

1.1.1. Paediatric procedures

Key objectives

- Facilitate and encourage early dialogue between applicants and the European Medicines Agency (EMA)/Paediatric Committee (PDCO) with a view to defining the scope and strategy of paediatric investigation plans (PIPs) and to identifying paediatric needs.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Establish 'Early Interaction' on Paediatric Development taking into account the outcome of the pilot phase launched in 2015 and address interdependencies with the PRIME scheme.

  PDCO topic leader: Dirk Mentzer

1.1.2. Addressing the needs of special populations

Key objectives

- Ensure that the needs of the paediatric population are systematically considered in the medicinal products development, assessment and monitoring of their use:
  - Support the continuity of the paediatric safety and efficacy assessment throughout the lifecycle of medicines;
  - Facilitate seamless provision of relevant information from the regulatory assessment to Health-Technology-Assessment bodies (HTA) for relative effectiveness assessments.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- By reinforcing cooperation with CHMP, to facilitate knowledge transfer from PIP decisions and with a view to utilising the best available scientific expertise in the Network, to identify PDCO experts to support scientific evaluation of medicines of paediatric interest.

  PDCO topic leader: Dirk Mentzer, Koenraad Norga

- Explore opportunities to open a dialogue with HTA organisations for parallel discussion on paediatric clinical trials.

  PDCO topic leader: Dirk Mentzer, Koenraad Norga

1.1.3. To improve the availability of medicinal product specifically authorised for children (facilitate patients access to medicines)

Key objectives

- Fostering the cooperation with the European Commission (EC) to ensure greater input into the research agenda in paediatric areas taking into account the objectives outlined in the Horizon 2020 strategy;
- Contribute to the finalisation of the 10-year report to the EC on the implementation of the Paediatric Regulation. Identify activities to increase compliance and results.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Provide input in the draft 10-year report to the EC on the implementation of the Paediatric Regulation;

  PDCO topic leader: Koenraad Norga
- Compile a list of medicinal products where further studies are needed for the safe use in the paediatric population (i.e. Inventory of Paediatric Therapeutic Needs) for the selected disease area: respiratory diseases;

  PDCO topic leader: Ninna Gullberg
- Provide recommendations to the EC on priority areas for research in paediatrics, in line with the objectives outlined in the Horizon 2020 strategy;

  PDCO topic leader: Tsveta Schyns-Liharska

1.1.4. Parallel Scientific Advice Working Party (SAWP) / HTA scientific advice

SAWP/HTA scientific advice offers an option to drug developers wishing to construct a drug development programme that is able to address the different needs of regulators, health technology appraisals and reimbursement considerations in the most efficient manner possible.

Key objectives

- Provide input to SAWP on parallel SAWP/HTA advice of paediatric interest with a view to ensure that health technology appraisals and reimbursement aspects are also specifically considered for drug development in paediatric populations.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Continue to participate in early dialogue with HTAs through existing procedures.

  PDCO topic leader: Karl-Heinz Huemer

1.1.5. Scientific Advice for paediatric medicines

Key objectives

- Consolidate SAWP-PDCO cooperation process and increase transparency of PDCO input in paediatric scientific advice;
- Strengthen collaboration with SAWP on provision of scientific advice throughout the lifecycle of the product.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Review experience gained with coordination process with CHMP-SAWP for scientific advice/protocol assistance applications with potential paediatric development in order to identify opportunities for improvement;
- To optimise and strengthen PDCO scientific memory in Scientific Advice procedures of paediatric interest, to ensure a consistent scientific approach within therapeutic areas;
- Increase transparency on PDCO involvement in scientific advice procedures;
- Contribute to implementation of procedure for non-imposed Post-authorisation Safety Studies (PASS) through the SAWP.

PDCO topic leader: Karl-Heinz Huemer

1.2. Other specialised areas and activities

1.2.1. Extrapolation

The extrapolation regulatory framework is important for informed and efficient drug development and hence relevant to CHMP-SAWP and PDCO. Use of structured approaches to evidence synthesis and inference will increase in importance for decision making at CHMP.

Key objectives

- To develop a regulatory framework for extrapolation across age groups, supporting informed and efficient drug development.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Publish the EMA reflection paper on extrapolation of efficacy and safety in medicine development across age groups;
- Develop a regulatory framework supporting innovative approaches for extrapolation concepts;
- Hold a workshop with relevant stakeholders on extrapolation across age groups;
- Develop a guideline on extrapolation;
- Draft a paper to summarise progress and to suggest new areas of guidance / training on the use of extrapolation methodology for paediatric development.

PDCO topic leader: Dirk Mentzer

Other Committee participants:

Member/alternate	Name	Member State
PDCO alternate	Christoph Male	AT
PDCO alternate	Ine Skottheim Rusten	NO
PDCO alternate	Anna-Karin Hamberg	SE
PDCO member	Johannes Taminiau	NL
CHMP Chair	Tomas Salmonson	SE
CHMP Member	Robert Hemmings	UK
CHMP Member	Nela Vilceanu	RO
CHMP Member	Daniel Brasseur	BE
CHMP Member	Romaldas Mačiulaitis	LT
PRAC representative	Jolanta Gulbinovic	LT

1.2.2. Modelling and Simulation

Modelling and Simulation is important for informed and efficient drug development and hence relevant to PDCO and CHMP-SAWP. In addition, use of structured and quantitative approaches (described under the general term of Modelling and Simulation) to evidence synthesis and inference will increase in importance for decision making.

Key objectives

- Further integrate modelling and simulation expertise into the work of PDCO and ensure that methodological approaches are consistently applied by PDCO in coordination with the Modelling and Simulation Working Group (MSWG);
- Ensure that '*best practices*' are captured in existing or developing guidance documents, specifically:
  - PDCO decision making for which Modelling and Simulation approaches are commonly used or are seen as being particularly useful;
  - Methodological approaches to model building, verification, presentation and inference.

Activities in 2016

PDCO activities to achieve the objectives set for this area. These activities are run through MSWG:

- Development of guideline on extrapolation (in collaboration with Extrapolation Working Group);
- To set a system ensuring a harmonised handling of modelling and simulation methodologies in paediatric procedures at PDCO and relevant working groups;
- Draft a document at the end of the PDCO work plan cycle to summarise progress and to suggest new areas of guidance/training.

PDCO topic leader: Ine Skottheim Rusten

Other contributors:

Member/alternate	Name	Member State
CHMP Chair	Tomas Salmonson	SE
CHMP Member	Robert Hemmings	UK
PDCO member	Anna-Karin Hamberg	SE
PDCO member	Johannes Taminiau	NL

1.2.3. Development of Therapeutic Areas strategies

Key objectives

- To assess cluster applications where many medicinal products appear in the same condition in order to apply a consistent scientific assessment approach.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Expert meetings on therapeutic areas with high numbers of applications, for example: psoriasis.

  PDCO topic leader: Dirk Mentzer

1.2.4. Adaptive Pathways

Adaptive Pathways offers an option to drug developers wishing to construct a potentially more time-effective drug development programme by the early input from regulators, health technology assessment bodies and patients into a programme that optimises regulatory tools and the use of real world data to supplement clinical trials.

Key objectives

- To provide input to Adaptive Pathways pilot project for medicines of paediatric interest.

Activities in 2016

PDCO activities to achieve the objectives set for this area

- Contribute to pilot (phase II) initiative on adaptive pathways on products of paediatric interest.

  PDCO topic leader: Karl-Heinz Huemer

1.2.5. Ethical considerations

Children (minors / the paediatric population) are all vulnerable and special provisions for their protection when involved in research are necessary. The 'Ethical considerations for clinical trials on medicinal products conducted with the paediatric population' (2008) encompass a number of recommendations aiming to contribute to the promotion and protection of the dignity, the well-being and the rights of children. In discussing and doing research with children, stakeholders in Europe have learned steeply in the last years, and it is timely to reflect on the experience with a view to improve the usefulness of the document and eventually to further foster high-quality, ethical research.

Key objectives

- To collect, analyse and communicate the experience of the PDCO and of the EMA with respect to ethical questions and aspects concerning the design, conduct and analysis of paediatric clinical research;
- To provide input to the revision of the 'Ethical considerations for clinical trials on medicinal products conducted with the paediatric population';
- To appraise the state of affairs of discussing and considering ethical aspects in the procedures and in other works of PDCO and of EMA.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- To provide input, by February 2016, to the revision of the 'Ethical considerations for clinical trials on medicinal products conducted with the paediatric population'
- To engage in a dialogue with the Rapporteur / Member State (Netherlands) for this revision;
- To prepare for a public consultation on the revision.

PDCO topic leader: Carine de Beaufort, Christoph Male, Marek Migdal, Stefan Grosek, Tsveta Schyns-Liharska

2. Horizontal activities and other areas

2.1. Working parties

2.1.1. PRAC-PDCO working group

Key objectives

- To implement the mandate of collaboration, support and mode of input in the respective Committee.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Review of the experience gained with the use of the Standard EudraVigilance Paediatric Query to inform PDCO answers to PRAC questions on pharmacovigilance issues of paediatric interest.

  PDCO topic leader: Dirk Mentzer, Sylvie Benchetrit, Angeliki Siapkara

- Progress the use of the Organ maturation table.

  PDCO topic leader: Johannes Taminau

2.1.2. Paediatric non-clinical aspects

Key objectives

- Contribute to a global non-clinical strategy to support paediatric studies;
- Evaluate the value of juvenile toxicity studies in the areas of oncology and the central nervous system (CNS);
- Evaluate and identify non-clinical models to support neonatal drug development (safety and efficacy);
- To contribute to marketing authorization strategy about inclusion of paediatric data in public information;
- To evaluate the need for pre-clinical studies to support paediatric developments of Scientific Advice procedures, as deemed necessary by the SAWP;
- Providing training to PDCO members on non-clinical aspects to support clinical development of medicines for paediatric use.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Collaboration with and participation in international working groups on non-clinical safety studies for special paediatric populations, such as neonatology or in case of first in children studies;
- Contribution to relevant International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH) guidelines, such as ICH S11 'Nonclinical Safety Testing in Support of Development of Paediatric Medicines';
- Review of outcomes and significance of juvenile toxicity studies included in PIPs in the areas of oncology and the central nervous system (CNS);
- To evaluate and identify non-clinical models to support neonatal drug development (safety and efficacy);
- Review the interpretation and inclusion into public information of results of juvenile toxicity studies;
- Collaboration/support of relevant SAWP procedures, if deemed necessary by SAWP and if Non-clinical WG (NcWG) resources allow;
- Conduct a training for PDCO members on non-clinical aspects to support clinical development of medicines for paediatric use.

  PDCO topic leader: Jacqueline Carleer

2.1.3. Formulation working group

Key objectives

- Successfully implement the FWG mandate.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Contribute to the revision of the guideline on 'Excipients in the label and package leaflet of medicinal products for human use' (CPMP/463/00).

  PDCO topic leader: Brian Aylward

2.1.4. Neonatology working group

Key objectives

- To reflect in the current regulatory guidance the recent progress in science and experience acquired by PDCO in evaluating PIPs;
- To actively work with stakeholders in the neonatology field to leverage their experience on questions related to clinical studies of investigational medicinal products for neonates.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- Draft a concept paper for the revision of the neonatal guideline by end of 2016;

- Continue working collaboration with the International Neo Consortium, in specific areas (e.g. neonatal seizures, bronchopulmonary dysplasia, neonatal pharmacology).

PDCO topic leader: Dina Apele-Freimane

Member/alternate	Name	Member State
PDCO member	Ninna Gullberg	SE
PDCO member	Irja Lutsar	EE
PDCO Chair	Dirk.Mentzer	SE
PDCO alternate	Ine Skottheim Rusten	NO
PDCO member	Marek Migdal	PL
PDCO member	Siri Wang	NO
PDCO member	Stefan Grosek	SI
PDCO member	Sylvie Benchetrit	FR

2.2. Partners and stakeholders

2.2.1. Strengthen dialogue with all stakeholders

Key objectives

- Strengthen dialogue with external stakeholders (Industry, Academia, patients, Healthcare professionals (HCP)) and Internal stakeholders (CHMP, PRAC, SAWP, Committee for Orphan Medicinal Products (COMP), other);
- Consolidate cooperation with international regulators (e.g. U.S. Food & Drug Administration (FDA)) for potential harmonisation of procedures;
- Streamline regulators' support for medicines for children with a rare or very rare disease;
- Provide drug developers clear and compatible guidance with considerations specific to global product development of paediatric medicines.

Activities in 2016

PDCO activities to achieve the objectives set for this area:

- In coordination with Patients' and Consumers' Working Party (PCWP) and Healthcare Professionals Working Party (HCPWG), to establish process for involvement of young people to provide feedback on paediatric medicines and paediatric clinical trial development to the PDCO members;
- To increasingly use European network of paediatric research at the EMA (Enpr-EMA) and its working groups as platform for discussion among academia and industry with the PDCO.

  PDCO topic leader: Angeliki Siapkara, Christoph Male
- Develop with the FDA regulatory science approaches for paediatric diseases (including rare diseases), including finalise joint guidance document for Gaucher disease.

  PDCO topic leader: Dirk Mentzer
- Establish a list of PDCO Interested Parties to promote the possibility for stakeholders organisations to participate in a direct dialogue with PDCO;
- Organise ad-hoc PDCO meetings with Interested Parties.

PDCO topic leader: Koenraad Norga, Sylvie Benchetrit, Angeliki Siapkara.

- To coordinate and provide the European regulatory network expertise in the development of the addendum to the current E11 guidance.

  PDCO topic leader: Dirk Mentzer

- In coordination with CHMP and COMP to participate in a CHMP-PDCO-COMP work group to contribute to development of internal reflection paper on the concepts and experience of assessment of 'significant benefit'.

  PDCO topic leader: Tsveta Schyns-Liharska, Paolo Rossi, Koenraad Norga