19 January 2023
EMA/COMP/48038/2023
Human Medicines Division

COMP work plan 2023

Adopted on 19 January 2023

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The activities outlined in the COMP 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. Designation and maintenance of orphan medicines

Key objectives

- Optimise the quality of initial orphan designation applications and maintenance by sharing COMP experience with stakeholders, with the objective to reduce failed orphan designation attempts and removals of orphan status at marketing authorisation.
- Ensure consistency, transparency, quality and detail of the grounds of opinions and orphan maintenance assessment reports given by the COMP at the time of designation and marketing authorisation.
- Explore cases and process options for real world evidence (RWE) in orphan designation decision making and the principles for future piloting of rapid RWE analytics to support the committee.

Activities in 2023:

- Defining the requirements for major contribution to patient care at orphan designation as well as at marketing authorisation stage.
- Draft a concept paper outlining the conclusions as guidance to sponsors.

  COMP topic leader: Other Committee participants:

Member/Alternate	Name	Member State
Member	Jana Mazelova	Czechia
Member	Frauke Naumann-Winter	Germany
Member	Brigitte Schwarzer-Daum	Austria
Member	Time Leest	Belgium
Member	Pauline Evers	Patient representative
Chair	Violeta Stoyanova-Beninska	Netherlands
Member (Vice Chair)	Armando Magrelli	Italy

- Implement a revised and optimised process for assessment at time of maintenance (marketing authorisation application and extension of indication), summary report collaboration and OMAR finalisations. Pilot the new process over 6 months and revise if needed after pilot phase completed.

  COMP topic leader: Pauline Evers Other Committee participants:

Member/Alternate	Name	Member State
Member	Maria Elisabeth Kalland	Norway
Member	Elisabeth Rook	Netherlands
Member	Olimpia Neagu	Romania

- Review information on the EMA website and propose improvements to the information available to sponsors if deemed needed.

  COMP topic leader: Violeta Stoyanova

- Work on the flexibility in the definition of orphan conditions to be more in line with innovative scientific development (for example the use of biomarker or tissue-agnostic therapies):
  - Publication on IRDs

  COMP topic leader: Tim Leest

  - Other disease areas

  COMP topic leader: will depend on the condition

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Darius Matusevicius	Sweden
Member	Armando Magrelli	Vice-Chair
Member	Frauke Naumann-Winter	Germany
Member	Elisabeth Rook	Netherlands
Chair	Violeta Stoyanova-Beninska	Netherlands
Member	Elisabeth Penninga	Denmark
Member	Giuseppe Capovilla	Nominated by EC
Member	Robert Nistico	Malta
Member	Tim Leest	Belgium
Member	Ingeborg Barsic	Nominated by EC

- Refine the COMP decisions repository collected by COMP on the criteria for designation and maintenance. The list is a repository of critical decisions, scientific memory and a way to support informed and well substantiated decision-making.

COMP topic leader: Frauke Naumann-Winter

- Continue the pilot of RWE studies to support COMP decision-making including identification of use cases.
  - Provide expert input from COMP to a review of the experience gained with RWE studies conducted 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;
  - Provide expert input in the implementation of the recommendations from the HMA/EMA Big Data Steering Group in accordance with the Big Data workplan deliverables for 2023;
  - Explore the use of RWE for the estimation of prevalence.

  COMP topic lead: Frauke Naumann Winter,

  COMP participants: will depend on the case chosen.

Member/Alternate	Name	Member State
Member	Eva Malikova	Slovakia
Member	Armando Magrelli	Vice-Chair
Member	Karri Penttila	Finland
Member	Julian Isla	Patient representative

Member/Alternate	Name	Member State
Member	Pauline Evers	Patient representative
Member	Maria Elisabeth Kalland	Norway
Member	Inês Alves	Patient representative
Member	Enrico Costa	Italy

- Review the data on medical plausibility for applications on advanced and innovative therapies at the time of an initial orphan designation:
  - Publish the first results in a peer reviewed journal, COMP topic lead: Gloria Maria Palomo Carrasco;
  - Follow up research on the CAR-T cell-based products and on the innovative therapies (i.e. siRNA, CRISPR cas, etc).

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Darius Matusevicius	Sweden
Member	Armando Magrelli	Vice-Chair
Member	Frauke Naumann-Winter	Germany
Chair	Violeta Stoyanova-Beninska	Netherlands
Member	Eva Malikova	Slovakia
Member	Maria Elisabeth Kalland	Norway
Member	Brigitte Schwarzer-Daum	Austria
Member	Zsofia Gyulai	Hungary
Member	Dinko Vitezic	Croatia
Memebr	Olimpia Neagu	Romania
Member	Enrico Costa	Italy
Member	Joao Rocha	Portugal

2. Horizontal activities and other areas

2.1. Committees and Working Parties

2.1.1. Additional objectives and activities

Key objectives

- To further develop the early interaction process between the CHMP and COMP in view of appropriate consistency of opinions, exchange of expertise and information.
- Revision of guideline on small clinical trials including indirect comparisons.

Activities in 2023

- COMP/CHMP members to liaise in case of rapporteurship or co-rapporteurship of the procedures requiring exchange of expertise and information, especially in issues where the different regulatory frameworks might pose a challenge.
- Evaluation of CHMP-COMP interaction process based on experience from 2020 - 2022.

COMP topic leader: Elisabeth Rook

Other Committee participants:

Member/Alternate	Name	Member State
Member	Brigitte Schwarzer-Daum	Austria
Member	Armando Magrelli	Vice-Chair
Member	Karri Penttila	Finland
Member	Maria Elisabeth Kalland	Norway
Member	Olimpia Neagu	Romania

- Review of indirect comparison methodology for demonstrating significant benefit in preparation of the revision of the guideline on trials in small populations.
- Systematic literature review on indirect comparison methods.
- Review of statistical methods for indirect comparisons used in the context of demonstrating significant benefit for orphan medicines and major therapeutical advantage in conditional marketing authorisation submitted to EMA from 2012 to 2022.

COMP topic leader: Frauke Naumann-Winter

Member/Alternate	Name	Member State
Member	Enrico Costa	Italy
Member	Maria Elisabeth Kalland	Norway
Member	Elisabeth Rook	Netherlands

2.2. Partners and stakeholders

2.2.1. Interactions with partners

Key objectives

Support EC activities on the review of the orphan regulation along with the pharmaceutical legislation, ensure that the experience and consolidated proposal of the COMP are captured.

Activities in 2023

- Provide expertise and recommendation to EC on the new orphan regulation as needed. Work with the EC on implementing the legislation.

COMP topic leader: Violeta Stoyanova-Beninska

Other Committee participants:

Member	Name	Member State
Member	Giuseppe Capovilla	Nominated by EC
Member	Frauke Naumann-Winter	Germany
Member	Elisabeth Rook	Netherlands
Member	Pauline Evers	Patient representative
Member	Ingeborg Barisic	Nominated by EC
Member	Armando Magrelli	Vice-Chair
Member	Maria Elisabeth Kalland	Norway

Member	Name	Member State
Member	Enrico Costa	Italy
Member	Brigitte Schwarzer-Daum	Austria
Member	Gloria Maria Palomo Carrasco	Spain