18 January 2024
EMA/COMP/31131/2024
Human Medicines Division

Committee for Orphan Medicinal Products (COMP): Work Plan 2024

Adopted by the Committee on 18 January 2024

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The activities outlined in the COMP 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. 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 2024:

COMP activities to achieve the objectives set for this area:

- Defining the requirements for major contribution to patient care (MCPC) at orphan designation as well as at marketing authorisation stage.
- Publish and communicate on the conclusions from the work done in 2023.

COMP topic leader: Frauke Naumann-Winter

Other Committee participants:

Member/Alternate	Name	Member State
Member	Jana Mazelova	Czechia
Member	Inês Alves	Patient representative
Member	Brigitte Schwarzer-Daum	Austria
Member	Tim Leest	Belgium
Member	Pauline Evers	Patient representative
Chair	Violeta Stoyanova-Beninska	Netherlands
Member	Armando Magrelli	Vice-Chair

- Work on the flexibility in the definition of orphan conditions to be more in line with innovative scientific development.
- Emerging discussion on gene independent medicinal products and defining conditions for the purpose of orphan designation. Agreement on a suitable way to currently designate conditions for these type of products. This activity will be subject to new submissions for such products in the first half of 2024.

COMP topic leader: Tim Leest

Other Committee participants: pending rapporteurship of relevant procedures, and therapeutic area expertise.

- Defining a suitable orphan condition for large B-cell lymphomas based on the World Health Organization (WHO) Classification 5th edition. Discuss with PDCO and oncology working party. COMP to agree on which terminology to use for future orphan designation.

COMP topic leader: Elisabeth Johanne Rook

Other Committee participants:

Member/Alternate	Name	Member State
Member	Maria Elisabeth Kalland	Norway
Member	Karri Penttila	Finland
Member	Bozenna Dembowska-Baginska	Poland
Member	Frauke Naumann-Winter	Germany
Member	Evangelia Yannaki	Greece

- 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.
- Review to identify major principle decisions and impact on 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 work plan deliverables for 2024;
  - Explore the use of real-world data (RWD) sources for disease epidemiology.

COMP topic leader: 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	Pauline Evers	Patient representative
Member	Maria Elisabeth Kalland	Norway
Member	Inês Alves	Patient representative
Member	Enrico Costa	Italy
Member	Judit Molnar	Nominated by EC

- Review the data on medical plausibility for applications on advanced and innovative therapies at the time of an initial orphan designation:

- Continue the research with focus on the remaining innovative products i.e. siRNA, CRISPR-Cas, etc.;
- Publish results in a peer reviewed journal.

COMP topic leader: Gloria Maria Palomo Carrasco

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
Member	Olimpia Neagu	Romania
Member	Enrico Costa	Italy
Member	Joao Rocha	Portugal
Member	Judit Molnar	Nominated by EC
Member	Evangelia Yannaki	Greece

- Mapping the orphan designations for very rare conditions.

COMP topic leader: Enrico Costa

Other Committee participants:

Member/Alternate	Name	Member State
Chair	Violeta Stoyanova-Beninska	Netherlands
Member	Tim Leest	Belgium
Member	Judit Molnar	Nominated by EC
Member	Joao Rocha	Portugal
Member	Elisabeth Rook	Netherlands
Member	Gloria Maria Palomo Carrasco	Spain
Member	Brigitte Schwarzer-Daum	Austria
Member	Frauke Naumann-Winter	Germany
Member	Elisabeth Penninga	Denmark
Member	Eva Malikova	Slovakia
Member	Darius Matusevicius	Sweden
Member	Armando Magrelli	Vice-Chair
Member	Zsofia Gyulai	Hungary
Member	Ingeborg Barisic	Nominated by EC
Member	Inês Alves	Patient representative

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.
- To have a guidance addressing the issues of indirect comparisons, either through revision of small population guideline or a dedicated Q&A.

Activities in 2024

COMP activities to achieve the objectives set for this area:

- Summarise the conclusions from the work done in 2023 on the procedures requiring exchange of expertise and information, especially in issues where the different regulatory frameworks might pose a challenge.
- Propose changes if identified in the collaboration between the Committees.

COMP topic leader: Elisabeth Rook

Other Committee participants: NA

- Collaboration with the Patient Experience Data initiative.
- Establishing the use of patient experience data for orphan medicines in regulatory purposes through a patient-validated methodology.

COMP topic leader: Julian Isla

Other Committee participants: NA