22 January 2025
EMA/COMP/31017/2025
Human Medicines Division

Committee for Orphan Medicinal Products (COMP): Work Plan 2025

Adopted by the Committee on 22 January 2025

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The activities outlined in the COMP work plan for 2025 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2024-2026.

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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 improve the quality of submissions, thus increasing the chance of positive outcomes of designation procedures.
- Ensure consistency, transparency and quality 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 generation of real-world evidence (RWE) in orphan designation decision making and the principles for conduct of real-world data (RWD) studies and rapid analytics to support the Committee.

Activities in 2025:

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.
- Collaboration with the cross-committee Patient Experience Data initiative.
- Publish and communicate on the conclusions from the work done in 2023 and 2024.

COMP topic leader: Frauke Naumann-Winter

Other Committee participants:

Member	Name	Member State or affiliation
Chair	Tim Leest	Belgium
Member	Jana Mazelova	Czechia
Member	Inês Alves	Patient representative
Member	Brigitte Schwarzer-Daum	Austria
Member	Joao Rocha	Portugal
Member	Darius Matusevicius	Sweden
Member	Elisabeth Rook	Netherlands

- Work on the flexibility in the definition of orphan conditions to be more in line with innovative scientific development.
- Review the orphan conditions in the setting of treatment modalities like treatment in "solid organ transplant", "haematopoietic stem cell transplantation", and conditions resulting from invasive/surgical procedures.

COMP topic leader: Evangelia Yannaki

Other Committee participants:

Member	Name	Member State or affiliation
Chair	Tim Leest	Belgium
Vice-Chair	Frauke Naumann-Winter	Germany
Member	Zsofia Gyulai	Hungary

- 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 2025.

COMP topic leader: TBC

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

- Continue the conduct of RWD studies when appropriate to support COMP decision-making thanks to identification of potential use cases:
  - Provide expert input in support of the development of guidance on use of RWE for regulatory purposes;
  - 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 RWD sources for disease epidemiology and drug utilisation studies.

COMP topic leader: Frauke Naumann Winter

COMP participants: will depend on the case chosen.

Member	Name	Member State or affiliation
Member	Karri Penttila	Finland
Member	Julian Isla	Patient representative
Member	Maria Elisabeth Kalland	Norway
Member	Inês Alves	Patient representative
Member	Enrico Costa	Italy
Member	Judit Molnar	Nominated by EC
Member	Fernando Méndez-Hermida	Nominated by EC

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 COMP and the Committee for Medicinal Products for Human Use (CHMP) in view of appropriate consistency of opinions, exchange of expertise and information.

- To maintain and ensure evidence standards in view of methods used to support significant benefit claims. Specifically, to explore feasibility of adequate indirect comparisons in the field of rare diseases.

Activities in 2025

COMP activities to achieve the objectives set for this area:

- Contribute to an analysis and scoping exercise to initiate the work towards the development of guidance on indirect comparisons.
- Explore feasibility of adequate indirect comparisons in the field of rare diseases and when they should be used in the context of significant benefit.
- Explore collaboration and knowledge sharing with HTA bodies on the assessment of indirect comparisons.
- Review assessments of indirect treatment comparisons by the COMP and develop guidance for applicants for submission of such analyses.

COMP topic leader: Maria Elisabeth Kalland

Other Committee participants:

Member	Name	Member State or affiliation
Chair	Tim Leest	Belgium
Vice-Chair	Frauke Naumann-Winter	Germany
Member	Elisabeth Rook	Netherlands
Member	Joao Rocha	Portugal
Member	Karri Penttila	Finland
Member	Mariette Driessens	Patient representative
Member	Maria Elisabeth Kalland	Norway
Member	Enrico Costa	Italy

- Establishing the use of patient experience data for orphan medicines in regulatory purposes through a patient-validated methodology.
- Define criteria for when patient experts should be included in the assessment of orphan designations and orphan maintenance.
- Proactive use of CollaboRARE in maintenance procedures.
- Explore potential fit-for-purpose refinements in the CollaboRARE output.

COMP topic leader: Julian Isla

Other Committee participants:

Member/Alternate	Name	Member State or affiliation
Chair	Tim Leest	Belgium
Vice-Chair	Frauke Naumann-Winter	Germany
Member	Mariette Driessens	Patient representative
Member	Inês Alves	Patient representative
Member	Judit Molnar	Nominated by EC
Member	Fernando Méndez Hermida	Nominated by EC