EMA
EUROPEAN MEDICINES AGENCY

COMP workplan 2026

Adopted by the committee on 22 January 2026

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Human Medicines Division

Contents

Introduction by the COMP Chair

Tim Leest

2026 will be a year of continuation, whereby the COMP aims to maintain the strong evidence-based scientific assessment of orphan designations and maintenances. Some of the main challenges that lay ahead are furthering the work on indirect comparisons, how to best employ and integrate these in the orphan medicines context, as well as initial scoping towards integrating new approach methodologies (NAMs), such as the 3R initiative, in the committee assessment modalities.

For specific activities, COMP collaborates with EURORDIS.

The activities outlined in this workplan have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme.

Workplan structure

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Evaluation activities for human medicines:

- Pre-authorisation activities

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Horizontal activities and other areas:

- Committees and working parties

1

Evaluation activities for human medicines

Pre-authorisation activities

Orphan designation 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.

Activities in 2026:
Review new approach methodologies (NAMs) used in orphan designations for 2025 and prospectively for 2026.
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.

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

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Horizontal activities and other areas

Committees and working parties

Key objectives:

- In the context of the reform of the pharmaceutical legislation, the COMP will ensure that learnings from the past years are collected and made available to future decision makers.

Activities in 2026:
Collaboration with the cross-committee Patient Experience Data initiative.
Article series capturing learnings of the Committee over the years.

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

Committees and working parties

Review the assessment of indirect treatment comparisons

Indirect comparisons are sometimes utilized to support regulatory claims and decision-making. Strengthening reporting standards to deliver reliable, interpretable evidence would reduce review burden and enable faster, more informed regulatory decisions across key assessments.

Key objectives:

- To maintain and ensure evidence standards for claims based on indirect comparisons, with a focus on reporting methods used to support significant benefit. Specifically, to assess the feasibility of adequate indirect comparisons used in orphan designation applications for products intended to manage rare diseases.

Activities in 2026:	
Collaborate with the Methodology Working Party (MWP) to review the potential impact of the new pharma legislation on the guidance.	
Explore feasibility of adequate indirect comparisons in the field of rare diseases and when they are to be used in the context of significant benefit.	
Collaborate with the Committee for Medicinal Products for Human Use (CHMP) and Methodology Working Party (MWP) to develop guidance for applicants for reporting on indirect comparisons in submissions.	CHMP
Explore collaboration and knowledge sharing with HTA bodies on the assessment of indirect comparisons.	

See Annex for details on the Lead(s)/Contributor(s) and key deliverables

Annex

Leads and contributors for the activities

Activity	Lead(s)	Contributor(s)
Review of past NAMs / Prospective scanning of future NAMs	Joao Rocha	Elisabeth Rook, Gloria Palomo Carrasco
Continue the conduct of and expert input in RWD studies to support COMP decision-making when appropriate	Frauke Naumann-Winter	Karri Penttila, Maria Elisabeth Kalland, Inês Alves, Enrico Costa, Judit Molnar, Fernando Méndez-Hermida, *other members depending on case specifics*
Cross-Committee Patient experience data contribution	Inês Alves	Tim Leest, Frauke Naumann-Winter, Mariette Driessens, Inês Alves, Judit Molnar, Fernando Méndez Hermida
Article series on learning of the COMP over the years	Elisabeth Rook	Frauke Naumann-Winter, Darius Matusevicius, Judit Molnar, Brigitte Schwarzer-Daum, Maria Elisabeth Kalland, Joao Rocha
Indirect comparisons: case review, guidance development and applicability scoping	Maria Elisabeth Kalland	Tim Leest, Frauke Naumann-Winter, Joao Rocha, Karri Penttila, Mariette Driessens, Enrico Costa

Main deliverables and achievements of 2025

Activity	Deliverables
Defining the requirements for major contribution to patient care (MCPC) at orphan designation as well as at marketing authorisation stage	• Activity concluded at the Strategic Review and Learning Meeting in Copenhagen.
Defining a suitable orphan condition for large B-cell lymphomas	• Agreement that the term large B-cell lymphomas (LBCL) can be used, with only minor impact on the prevalence in view of the rarity of the additional subtypes to be covered by the family term.
Real World Evidence	• Contributed to the DARWIN EU protocol for measuring the prevalence of selected cancers • Requested a DARWIN EU study in the prevalence of tetanus, contributed to report and made suggestions to improve future study usability.
Review of data on medical plausibility for applications on advanced and innovative therapies at the time of an initial orphan designations	• Navigating the orphan medicinal product designation: Evidence requirements for gene therapies in Europe – ScienceDirect.
Mapping the orphan designations for very rare conditions	• Completed; the IRIS database has been updated, and this is reflected in the information in the IRIS portal.
Contribute to an analysis and scoping exercise to initiate the work towards the development of guidance on indirect comparison	• Activity started with initial discussions, to be continued in 2026.
Patient Experience Data initiative	• Piloted the use of CollaboRARE, a tool for capturing important aspects of the disease for patients. Decision not to develop it further. • Contributed to the Reflection paper on patient experience data with ongoing public consultation.

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