



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

20 January 2022
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Human Medicines Division

Committee for Orphan Medicinal Products: Work Plan 2022

Adopted by the Committee on 20 January 2022

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The activities outlined in the COMP work plan for 2022 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2022-2024.

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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 details 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 in orphan marketing authorisation decision making and the principles for future piloting of rapid RWE analytics to support the committee.

Activities in 2022:

- Discuss the results from the OMAR survey, discuss follow-up actions, proposals and implement changes if deemed necessary.

COMP topic leader: Pauline Evers

Other Committee participants:

Member/Alternate	Name	Member State
Member	Armando Magrelli	Vice-Chair
Member	Zsofia Gyulai	Hungary
Member	Maria Elisabeth Kalland	Norway
Member	Olimpia Neagu	Romania

- Update on EMA website with a new interview with the COMP chair.

COMP topic leader: Violeta Stoyanova

- Continue the work on defining conditions in the context of the orphan regulation:
 - Build on the outcome of the workshops held in 2016 and 2017 together with the discussions during the SRLM under the Croatian and the German presidency (February 2020 and September 2020).
 - Focus in 2022 on eye disorders and a workshop dedicated to this topic.
 - European Commissions involvement in scientific development and discussions.

COMP topic leader: Tim Leest

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	
Member	Elisabeth Penninga	Denmark
Member	Giuseppe Capovilla	Nominated by EC
Member	Robert Nistico	Malta
Member	Tim Leest	Belgium
Member	Ingeborg Barsic	Nominated by EC

- Maintain a list of principal decisions made by COMP on the criteria for designation and maintenance. The list will be a repository of critical decisions and a way to maintain consistency in the decision making.

COMP topic leader: Geraldine O'Dea

Other Committee participants:

Member/Alternate	Name	Member State
Member	Elisabeth Rook	Netherlands
Member	Armando Magrelli	Vice-Chair
Member	Lyubina R. Todorova	Bulgaria
Member	Darius Matusevicius	Sweden
Member	Eva Malikova	Slovakia
Chair	Violeta Stoyanova-Beninska	

- Agree key principles for a pilot of real-world data analytics.
 - Finalise the proof of concept and start a pilot to provide real-world evidence services to support COMP decision-making;
 - Provide expert input in the implementation of the recommendations from the HMA/EMA Big Data Steering Group;

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

- Prevalence of haematological conditions (NHL), COMP topic lead: Frauke Naumann Winter

Member/Alternate	Name	Member State
Member	Maria Elisabeth Kalland	Norway
Member	Karri Penttila	Finland
Member	Elisabeth Johanne Rook	Netherlands
Member	Enrico Costa	Italy

- Identify challenges of non-clinical in vivo data in order to support advanced and innovative therapies within the context of medical plausibility at the time of an initial orphan designation.
- Publish the results in a peer reviewed journal, COMP topic lead: 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
Member	Dinah Duarte	Portugal
Chair	Violeta Stoyanova-Beninska	
Member	Eva Malikova	Slovakia
Member	Maria Elisabeth Kalland	Norway
Member	Brigitte Schwarzer-Daum	Austria
Member	Zsofia Gyulai	Hungary
Member	Enrico Costa	Italy
Member	Dinko Vitezic	Croatia

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.

Activities in 2022

- 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 and 2021.

COMP topic leader: Elisabeth Rook

Other Committee participants:

Member/Alternate	Name	Member State
Member	Dinah Duarte	Portugal
Member	Brigitte Schwarzer-Daum	Austria
Member	Armando Magrelli	Vice-Chair
Member	Olimpia Neagu	Romania

2.2. Partners and stakeholders

2.2.1. Revision of the pharmaceutical legislation

Key objectives

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

Activities in 2022

- Provide feedback to EC on the Staff Working Document as needed and the legislative proposal.

COMP topic leader: Violeta Stoyanova-Beninska

Other Committee participants:

Member	Name	Member State
Member	Dinah Duarte	Portugal
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	Enrico Costa	Italy