22 January 2020
EMA/COMP/478696/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP): Work Plan 2020

Adopted by the Committee on 22 January 2020

Table of Contents

The activities outlined in the work plan for 2020 have been agreed taking into consideration that activities are gradually reinstated following a phase of business continuity.

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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.

Activities in 2020

- Consolidate the responses from a survey sent to external stakeholders (including payers and HTA bodies) on the utility and content of the Orphan Maintenance Assessment Report (OMAR). Present the survey results to COMP, discuss proposals and implement changes if deemed necessary.

  COMP topic leader: Pauline Evers

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Armando Magrelli	Italy
Member	Zsofia Gyulai	Hungary
Member	Maria Elisabeth Kalland	Norway

- Create a new guidance, and decide on the best format of this, to replace the "COMP recommendation on elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation, EMEA/COMP/436/01", taking into account the Commission Notice (2016/C 424/03), the updated Points to consider on estimation of prevalence (EMA/COMP/436/01 Rev. 1) and principles identified by the COMP when assessing criteria for orphan designation (at designation and at marketing authorisation).

  COMP topic leader: Julian Isla

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Julian Isla	Patient representative
Member	Elisabeth Rook	Netherlands
Member	Frauke Naumann-Winter	Germany
Member	Bruno Sepodes	Nominated by EC

- Organise and execute a workshop with stakeholders with specific focus on early development.

  COMP topic leader: Violeta Stoyanova-Beninska

  Other Committee participants:

Member	Name	Member State
Member	Elisabeth Rook	Netherlands
Member	Frauke Naumann-Winter	Germany
Member	Darius Matusevicius	Sweden
Member	Elisabeth Penninga	Denmark
Member	Armando Magrelli	Italy

- Sessions to be organised if needed during COMP plenary covering updates on the on-line working tools (IRIS) for handling the procedures. Evaluation of the use of IRIS by COMP members based on a short survey provided by the EMA project team.

  COMP topic leader: Armando Magrelli

- Continue the work on defining conditions in the context of the orphan regulation:

  - Build on the outcome of the workshop held in 2016 and use the workshop report from 2017.
  - Review the criteria based on mechanism of action (MoA). Review if the condition would be different if the MoA of the product was taken into account as opposed to if it would not.
  - Review the criteria based on the genetic background of the condition versus phenotypic presentation, in the context of recent disease classifications. Tissue agnostic indications and umbrella conditions should be considered.
  - Review the condition designated in solid cancers vs haematology, based on WHO classification, and identify how the definitions of the conditions can be harmonised.

  COMP topic leader: Ingeborg Barišić

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Darius Matusevicius	Sweden
Member	Armando Magrelli	Italy
Member	Frauke Naumann-Winter	Germany
Member	Elisabeth Rook	Netherlands
Chair	Violeta Stoyanova-Beninska	Netherlands
Member	Elisabeth Penninga	Denmark
Member	Giuseppe Capovilla	Nominated by EC

- Work on defining what would constitute a satisfactory method of treatment and come up with working recommendations for the COMP. Review specifically the following scenarios:

  - Old products with limited efficacy;
  - Broad indication (e.g. chemotherapies and regiments in oncology);
  - Medicinal products not in use even though approved;
  - Non-pharmacological methods.

COMP topic leader: Frauke Naumann-Winter

Other Committee participants:

Member/Alternate	Name	Member State
Member	Armando Magrelli	Italy
Member	Eva Malikova	Slovakia
Member	Brigitte Schwarzer-Daum	Austria
Member	Darius Matusevicius	Sweden
Member	Giuseppe Capovilla	Nominated by EC

- Review conditions with high number of designations (e.g. pancreatic cancer, glioma) and a low number of products approved. Investigate the possible causes/reasons why products have failed to reach the market. Report back to COMP plenary.

  COMP topic leader: Violeta Stoyanova-Beninska

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Armando Magrelli	Italy
Member	Dinko Vitezic	Croatia
Member	Gloria Maria Palomo Carrasco	Spain
Member	Eva Malikova	Slovakia
Member	Frauke Naumann-Winter	Germany
Member	Lenka Kovarova	Czech Republic
Member	Dinah Duarte	Portugal

- Create and maintain a list of principle 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	Frauke Naumann-Winter	Germany
Member	Armando Magrelli	Italy
Member	Bruno Sepodes	Nominated by EC
Member	Lyubina R. Todorova	Bulgaria
Member	Darius Matusevicius	Sweden

- Review and update the training for new COMP members. Create a new slide set and practice cases. Introduce mentorship for new members.

  COMP topic leader: Armando Magrelli

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.
- Establish a COMP-CAT working group to optimise the interaction and output of the two Committees in assessment of orphan ATMPs.

Activities in 2020

- 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.
- EMA to support collaboration between CHMP-COMP through provision of relevant information to CHMP and COMP Chairs on on-going applications.
- Evaluation of CHMP-COMP interaction process based on experience from 2020.

  COMP topic leader: Bruno Sepodes

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Dinah Duarte	Portugal
Member	Frauke Naumann-Winter	Germany
Member	Armando Magrelli	Italy

- Set up with the CAT, a COMP-CAT working group; agree on its scope, frequency of meetings and start meeting. Clear deliveries expected to be provided and agreed between committees.

  COMP topic leader: Armando Magrelli

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Karri Pentilla	Finland
Member	Lyubina R. Todorova	Bulgaria
Member	Maria Elisabeth Kalland	Norway
Member	Olimpia Neagu	Romania

2.2. Partners, stakeholders and transparency

2.2.1. Interactions with partners

Key objectives

- Support EC on follow-up activities to the orphan study in view of implementing recommendation from the study or address concerns.

Activities in 2020

- Follow-up on the findings from the EC study on 'Evaluation of the legislation on medicines for children and rare diseases (medicines for special populations)'.

  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	Armando Magrelli	Italy