EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

8 February 2021
EMA/COMP/559551/2021
Human Medicines Division

COMP work plan 2021

Adopted on 21 January 2021

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The activities outlined in the COMP work plan for 2021 have been agreed taking into consideration the Agency's prioritisation set forth in the EMA multi-annual work programme 2021-2023.

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

Activities in 2021:

- 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	Vice-Chair
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	Elisabeth Rook	Netherlands
Member	Frauke Naumann-Winter	Germany

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

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- 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).

- Condition-specific discussions, list of conditions according to current working practice of the committee (e.g. solid tumours, haematological disorders, eye disease).

COMP topic leader: Ingeborg Barišić

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	Chair
Member	Elisabeth Penninga	Denmark
Member	Giuseppe Capovilla	Nominated by EC
Member	Tim Leest	Belgium

- Deliver a conclusion on what constitutes a satisfactory method of treatment as a working definition for the purposes of the work of the COMP. Publish the conclusions in a peer-review journal.

  COMP topic leader: Frauke Naumann-Winter

  Other Committee participants:

Member/Alternate	Name	Member State
Member	Armando Magrelli	Vice-Chair
Member	Eva Malikova	Slovakia
Member	Brigitte Schwarzer-Daum	Austria
Member	Darius Matusevicius	Sweden
Member	Violeta Stoyanova-Beninska	Chair
Member	Giuseppe Capovilla	Nominated by EC
Member	Maria Elisabeth Kalland	Norway

- 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

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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	Angelo Loris Brunetta	Patient representative

- Agree key principles for a pilot of real-world data analytics. A proof of concept study to inform the pilot could be performed in 2021
  - Identify use cases where the evidence from real word data can support the scientific assessment (e.g. supporting the evaluation of MCPC).

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

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 2021

- 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.

COMP topic leader: Elisabeth Rook

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Other Committee participants:

Member/Alternate	Name	Member State
Member	Dinah Duarte	Portugal
Member	Armando Magrelli	Vice-Chair

- Optimise the working practice in the COMP-CAT working group.

  COMP topic leader: Armando Magrelli

  Other Committee participants:

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

2.2. Inspections and compliance

Not applicable

2.3. Partners and stakeholders

2.3.1. Interactions with partners

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 2021

- COMP WG on Orphan Regulation

  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	Angelo Loris Brunetta	Patient representative
Member	Armando Magrelli	Vice-Chair
Member	Maria Elisabeth Kalland	Norway

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2.4. Data management support

Not applicable

2.5. Process improvements

No planned activities

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