



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

29 September 2023
EMA/COMP/412052/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 03-05 October 2023

Chair: Violeta Stoyanova-Beninska – Vice-Chair: Armando Magrelli

03 October 2023, 09:00-19:30, room 2A

04 October 2023, 08:30-19:30, room 2A

05 October 2023, 08:30-17:00, room 2A

Health and safety information

In accordance with the Agency's health and safety policy, delegates are to be briefed on health, safety and emergency information and procedures prior to the start of the meeting.

Disclaimers

Some of the information contained in this agenda is considered commercially confidential or sensitive and therefore not disclosed. With regard to intended therapeutic indications or procedure scopes listed against products, it must be noted that these may not reflect the full wording proposed by applicants and may also vary during the course of the review. Additional details on some of these procedures will be published in the COMP meeting reports once the procedures are finalised.

Of note, this agenda is a working document primarily designed for COMP members and the work the Committee undertakes.

Note on access to documents

Some documents mentioned in the agenda cannot be released at present following a request for access to documents within the framework of Regulation (EC) No 1049/2001 as they are subject to on-going procedures for which a final decision has not yet been adopted. They will become public when adopted or considered public according to the principles stated in the Agency policy on access to documents (EMA/127362/2006).

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1. Introduction

1.1. Welcome and declarations of interest of members and experts

Pre-meeting list of participants and restrictions in relation to declarations of interests applicable to the items of the agenda for the COMP plenary session to be held 03-05 October 2023. See October 2023 COMP minutes (to be published post November 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 03-05 October 2023.

1.3. Adoption of the minutes

COMP minutes for 05-07 September 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000143999

Treatment of ovarian cancer

Action: For adoption

2.1.2. - EMA/OD/0000128462

Treatment of Alport syndrome

Action: For adoption, Oral explanation to be held on 03 October 2023 at 09:45

2.1.3. - EMA/OD/0000139099

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 03 October 2023 at 15:30

2.1.4. - EMA/OD/0000143251

Treatment of Prader-Willi syndrome

Action: For adoption, Oral explanation to be held on 05 October 2023 at 09:00

2.1.5. - EMA/OD/0000137539

Treatment of narcolepsy

Action: For adoption, Oral explanation to be held on 04 October 2023 at 16:15

2.1.6. - EMA/OD/0000140934

Treatment of pulmonary arterial hypertension

Action: For information

Note: Withdrawal request received on 20 September 2023.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000119656

Treatment of Gaucher's disease

Action: For discussion/adoption

2.2.2. - EMA/OD/0000140338

Treatment of medium-chain acyl-coenzyme A dehydrogenase deficiency

Action: For discussion/adoption

2.2.3. - EMA/OD/0000142281

Treatment of ovarian cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000142974

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.5. - EMA/OD/0000142982

Treatment of moderate to severe traumatic brain injury

Action: For discussion/adoption

2.2.6. - EMA/OD/0000143407

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.7. - EMA/OD/0000143789

Treatment of hereditary angioedema

Action: For discussion/adoption

2.2.8. - EMA/OD/0000144266

Treatment of Menkes disease

Action: For discussion/adoption

2.2.9. - EMA/OD/0000144510

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000145792

Treatment of haemophilia B

Action: For discussion/adoption

2.2.11. - EMA/OD/0000146696

Treatment of pancreatic cancer

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

2.5.1. -

Treatment of patients with light chain (AL) amyloidosis

Action: For adoption, Oral explanation to be held on 04 October 2023 at 14:15

2.6. Nominations

2.6.1. New applications for orphan medicinal product designation - Appointment of COMP rapporteurs

Action: For adoption

Document(s) tabled:

OMPD applications - appointment of rapporteurs at the 03-05 October 2023 COMP meeting

2.7. Evaluation on-going

19 applications for orphan designation will not be discussed as evaluation is ongoing.

Action: For information

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of soft tissue sarcoma

Action: For adoption

3.1.2. -

Treatment of tuberous sclerosis

Action: For adoption

4. Review of orphan designation for orphan medicinal products at time of initial marketing authorisation

4.1. Orphan designated products for which CHMP opinions have been adopted

4.1.1. Zilbrysq - zilucoplan - EMEA/H/C/005450/0000, EU/3/22/2650, EMA/OD/0000120845

UCB Pharma S.A.; Treatment of myasthenia gravis

Action: For adoption, Oral explanation to be held on 03 October 2023 at 12:00

4.1.2. Yorvipath – teriparatide - EMEA/H/C/005934, EU/3/20/2350, EMA/OD/0000140073

Ascendis Pharma Bone Diseases A/S; Treatment of hypoparathyroidism

Action: For adoption

4.1.3. Finlee – dabrafenib - EMEA/H/C/005885/0000, EU/3/20/2372, EMA/OD/0000134197

Novartis Europharm Limited; Treatment of glioma

Action: For discussion/adoption

4.2. Orphan designated products for discussion prior to adoption of CHMP opinion

4.2.1. Albrioz – sodium phenylbutyrate / ursodoxicoltaurine - EMEA/H/C/005901, EU/3/20/2284, EMA/OD/0000096503

Amylyx Pharmaceuticals EMEA; Treatment of amyotrophic lateral sclerosis

Action: For information

4.2.2. – rezafungin - EMEA/H/C/005900/0000, EU/3/20/2385, EMA/OD/0000140230

Mundipharma GmbH; Treatment of invasive candidiasis

Action: For discussion/adoption

4.2.3. – vamorolone - EMEA/H/C/005679/0000, EU/3/14/1309, EMA/OD/0000141144

Santhera Pharmaceuticals (Deutschland) GmbH; Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

4.2.4. – elranatamab - EMEA/H/C/005908, EU/3/21/2471, EMA/OD/0000147440

Pfizer Europe MA EEIG; Treatment of multiple myeloma

Action: For discussion/adoption

4.2.5. – pegzilarginase - EMEA/H/C/005484, EU/3/16/1701, EMA/OD/0000140263

Immedica Pharma AB; Treatment of hyperargininemia

Action: For discussion/adoption

4.3. Appeal

4.3.1. Bylvay - odevixibat - EMEA/H/C/004691/II/0011, EU/3/12/1040, EMA/OD/0000152080

Albireo AB; Treatment of Alagille syndrome

CHMP Rapporteur: Johann Lodewijk Hillege

Action: For adoption, Oral explanation to be held on 03 October 2023 at 14:00

4.4. On-going procedures

Action: For information

Document(s) tabled:

Review of orphan designation for OMP for MA - On-going procedures

4.5. Orphan Maintenance Reports

Action: For information

5. Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Prevydis – Ietermovir - EMEA/H/C/004536/II/0033/G, EU/3/11/849, EMA/OD/0000133054

Merck Sharp & Dohme B.V.; Prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk

CHMP Rapporteur: Filip Josephson; CHMP Co-Rapporteur: Aaron Sosa Mejia

Action: For discussion/adoption

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:

Review of orphan designation for OMP for MA extension - On-going procedures

6. Application of Article 8(2) of the Orphan Regulation

None

7. Organisational, regulatory and methodological matters

7.1. Mandate and organisation of the COMP

7.1.1. COMP membership

Action: For information

7.1.2. Vote by proxy

Action: For information

7.1.3. Strategic Review & Learning meetings

SRLM meeting in Madrid under the Spanish Presidency of the Council of the EU

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 02 October 2023

Document tabled:

PAWG draft agenda for 02 October 2023 meeting

7.1.5. COMP Decisions Database

Action: For discussion

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

PRIME eligibility requests - list of adopted outcomes September 2023

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

None

7.3.2. Upcoming ITF meetings

Action: For discussion

Upcoming ITF meetings

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

None

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

7.5.3. Therapeutic Goods Administration (TGA), Australia

None

7.5.4. Health Canada

None

7.6. **Contacts of the COMP with external parties and interaction with the Interested Parties to the Committee**

None

7.7. **COMP work plan**

None

7.8. **Planning and reporting**

7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2023

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. **Any other business**

8.1. **Quarterly update on Real World Evidence, including DARWIN EU®**

Action: For discussion

8.2. **Nomination of COMP representative for ENCePP Steering Group 2024-2026**

Action: For information

9. **Explanatory notes**

The notes below give a brief explanation of the main sections and headings in the COMP agenda and should be read in conjunction with the agenda or the minutes.

Abbreviations / Acronyms

CHMP: Committee for Medicinal Product for Human Use

COMP: Committee for Orphan Medicinal Products

EC: European Commission

OD: Orphan Designation

PA: Protocol Assistance

PDCO: Paediatric Committee

PRAC: Pharmacovigilance and Risk Assessment Committee

SA: Scientific Advice

SAWP: Scientific Advice Working Party

Orphan Designation *(section 2 Applications for orphan medicinal product designation)*

The orphan designation is the appellation given to certain medicinal products under development that are intended to diagnose, prevent or treat rare conditions when they meet a pre-defined set of criteria foreseen in the legislation. Medicinal products which get the orphan status benefit from several incentives (fee reductions for regulatory procedures (including protocol assistance), national incentives for research and development, 10-year market exclusivity) aiming at stimulating the development and availability of treatments for patients suffering from rare diseases.

Orphan Designations are granted by Decisions of the European Commission based on opinions from the COMP. Orphan designated medicinal products are entered in the Community Register of Orphan Medicinal Products.

Protocol Assistance *(section 3 Requests for protocol assistance with significant benefit question)*

The protocol assistance is the help provided by the Agency to the sponsor of an orphan medicinal product, on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of the medicinal product in view of the submission of an application for marketing authorisation.

Sponsor

Any legal or physical person, established in the Community, seeking to obtain or having obtained the designation of a medicinal product as an orphan medicinal product.

Maintenance of Orphan Designation *(section 4 Review of orphan designation for orphan medicinal products for marketing authorisation).*

At the time of marketing authorisation, the COMP will check if all criteria for orphan designation are still met. The designated orphan medicinal product should be removed from the Community Register of Orphan Medicinal Products if it is established that the criteria laid down in the legislation are no longer met.

More detailed information on the above terms can be found on the EMA website:

www.ema.europa.eu/