



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

9 June 2023
EMA/COMP/234403/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 13-15 June 2023

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

13 June 2023, 08:30-19:30, virtual meeting

14 June 2023, 08:30-19:30, virtual meeting

15 June 2023, 08:30-17:00, virtual 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 13-15 June 2023. See May 2023 COMP minutes (to be published post June 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 13-15 June 2023.

1.3. Adoption of the minutes

COMP minutes for 15-17 May 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000130058

Treatment of congenital diaphragmatic hernia

Action: For adoption, Oral explanation to be held on 13 June 2023 at 09:45

2.1.2. - EMA/OD/0000116655

Treatment of patients with light chain (AL) amyloidosis

Action: For adoption, Oral explanation to be held on 13 June 2023 at 11:30

2.1.3. - EMA/OD/0000119470

Treatment of osteosarcoma

Action: For adoption, Oral explanation to be held on 13 June 2023 at 13:45

2.1.4. - EMA/OD/0000131821

Treatment of nontuberculous mycobacterial pulmonary disease (NTM-PD)

Action: For adoption, Oral explanation to be held on 13 June 2023 at 15:30

2.1.5. - EMA/OD/0000125383

Treatment of cryopyrin associated periodic syndromes

Action: For information

Note: Withdrawal request received on 26 May 2023.

[2.1.6. - EMA/OD/0000124780](#)

Treatment of pre-eclampsia

Action: For information

Note: Withdrawal request received on 30 May 2023.

[2.1.7. - EMA/OD/0000130960](#)

Treatment of acute lymphoblastic leukaemia

Action: For adoption, Oral explanation to be held on 14 June 2023 at 15:30

[2.1.8. - EMA/OD/0000124931](#)

Treatment of primary biliary cholangitis

Action: For adoption, Oral explanation to be held on 14 June 2023 at 17:00

2.2. For discussion / preparation for an opinion

[2.2.1. - EMA/OD/0000077804](#)

Treatment of non-traumatic spontaneous intracerebral haemorrhage

Action: For discussion/adoption

[2.2.2. - EMA/OD/0000101423](#)

Treatment of solid organ transplant

Action: For discussion/adoption

[2.2.3. - EMA/OD/0000126381](#)

Treatment in allogeneic haematopoietic stem cell transplantation

Action: For discussion/adoption

[2.2.4. - EMA/OD/0000126406](#)

Treatment of congenital myasthenic syndromes

Action: For discussion/adoption

[2.2.5. - EMA/OD/0000129401](#)

Treatment of Leigh syndrome

Action: For discussion/adoption

2.2.6. - EMA/OD/0000131231

Treatment of malaria

Action: For discussion/adoption

2.2.7. - EMA/OD/0000131315

Treatment of myelofibrosis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000131435

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For discussion/adoption

2.2.9. - EMA/OD/0000133388

Treatment of inclusion body myositis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000133659

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.11. - EMA/OD/0000134159

Treatment of tuberculosis

Action: For discussion/adoption

2.2.12. - EMA/OD/0000134174

Treatment of malignant mesothelioma

Action: For discussion/adoption

2.2.13. - EMA/OD/0000134246

Treatment of Alport syndrome

Action: For discussion/adoption

2.2.14. - EMA/OD/0000134260

Treatment of amyotrophic lateral sclerosis (ALS)

Action: For discussion/adoption

2.2.15. - EMA/OD/0000134297

Treatment of sarcoidosis

Action: For discussion/adoption

2.2.16. - EMA/OD/0000134554

Treatment of neurofibromatosis type 1 (NF1)

Action: For discussion/adoption

2.2.17. - EMA/OD/0000134584

Treatment in haematopoietic stem cell transplantation

Action: For discussion/adoption

2.2.18. - EMA/OD/0000134682

Treatment of ovarian cancer

Action: For discussion/adoption

2.2.19. - EMA/OD/0000135073

Treatment of primary ciliary dyskinesia

Action: For discussion/adoption

2.2.20. - EMA/OD/0000135225

Treatment of spinal muscular atrophy

Action: For discussion/adoption

2.2.21. - EMA/OD/0000135270

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.22. - EMA/OD/0000135309

Treatment of glioma

Action: For discussion/adoption

2.2.23. - EMA/OD/0000135389

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.24. - EMA/OD/0000135426

Treatment in hematopoietic stem cell transplantation

Action: For discussion/adoption

2.2.25. - EMA/OD/0000135452

Treatment of primary membranous nephropathy

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

None

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 13-15 June 2023 COMP meeting

2.7. Evaluation on-going

25 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 Duchenne muscular dystrophy

Action: For adoption

3.1.2. -

Treatment of hypoparathyroidism

Action: For adoption

3.1.3. -

Treatment of sickle cell disease

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

None

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

4.2.1. - talquetamab - EMEA/H/C/005864/0000, EU/3/21/2486, EMA/OD/0000126657

Accelerated assessment

Janssen - Cilag International; Treatment of multiple myeloma

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

4.2.2. - cedazuridine, decitabine - EMEA/H/C/005823/0000, EU/3/21/2548, EMA/OD/0000141337

Otsuka Pharmaceutical Netherlands B.V.; Treatment of acute myeloid leukaemia

Action: For discussion/adoption

4.2.3. Bylvay - odevixibat - EMEA/H/C/004691/II/0011, EU/3/12/1040, EMA/OD/0000123138

Albireo AB; Treatment of Alagille syndrome

CHMP Rapporteur: Johann Lodewijk Hillege

Action: For discussion/adoption

4.2.4. - tislelizumab - EMEA/H/C/005919/0000, EU/3/20/2357, EMA/OD/0000129253

Novartis Europharm Limited; Treatment of oesophageal cancer

Action: For discussion/adoption

4.2.5. - epcoritamab - EMEA/H/C/005985/0000, EU/3/22/2581, EMA/OD/0000104478

Abbvie Deutschland GmbH & Co. KG; Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

4.3. Appeal

4.3.1. Jaypirca - pirtobrutinib - EMEA/H/C/005863, EU/3/21/2450

Eli Lilly Nederland B.V.; Treatment of mantle cell lymphoma

Action: For information, request for SAG, appointment of appeal rapporteur

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. Prevymis – letermovir - 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:

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

None

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 9 June 2023 at 14:00

Document tabled:

PAWG draft agenda for 9 June 2023 meeting

7.1.5. COMP Decisions Database

Action: For discussion

Document tabled:

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 May 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)

Action: For information

Document(s) tabled:

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. Discussion on Orphan Maintenance Assessment Reports (OMARs)

Action: For discussion

8.2. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:

Q2-2023 Update of the Business Pipeline report for the human scientific committees

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/