



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

17 June 2024
EMA/COMP/252180/2024
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 18-20 June 2024

Chair: Violeta Stoyanova-Beninska

18 June 2024, 09:00-19:30, room 2A

19 June 2024, 08:30-19:30, room 2A

20 June 2024, 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 18-20 June 2024. See June 2024 COMP minutes (to be published post July 2024 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 18-20 June 2024.

1.3. Adoption of the minutes

COMP minutes for 21-23 May 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - [EMA/OD/0000164215](#)

Treatment of myasthenia gravis

Action: For information

Note: Withdrawal request received on 5 June 2024.

2.1.2. - [EMA/OD/0000139776](#)

Prevention of T-cell engaging immunotherapy induced cytokine release syndrome

Action: For adoption, Oral explanation to be held on 18 June 2024 at 11:30

2.1.3. - [EMA/OD/0000164923](#)

Treatment of Duchenne muscular dystrophy

Action: For information

Note: Withdrawal request received on 3 June 2024.

2.1.4. - [EMA/OD/0000169022](#)

Treatment of symptomatic obstructive hypertrophic cardiomyopathy

Action: For information

Note: Withdrawal request received on 6 June 2024.

2.1.5. - [EMA/OD/0000164949](#)

Treatment of retinitis pigmentosa

Action: For adoption, Oral explanation to be held on 18 June 2024 at 14:00

2.1.6. - [EMA/OD/0000163852](#)

Treatment of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 19 June 2024 at 11:30

2.1.7. - [EMA/OD/0000169732](#)

Treatment of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 19 June 2024 at 11:30

2.1.8. - [EMA/OD/0000167882](#)

Treatment of variegate porphyria

Action: For adoption, Oral explanation to be held on 20 June 2024 at 08:45

2.1.9. - [EMA/OD/0000169065](#)

Treatment of eosinophilic esophagitis

Action: For information

Note: Withdrawal request received on 3 June 2024.

2.1.10. - [EMA/OD/0000162927](#)

Treatment of small cell lung cancer

Action: For information

Note: Withdrawal request received on 29 May 2024.

2.1.11. - [EMA/OD/0000169553](#)

Treatment of thrombotic thrombocytopenic purpura

Action: For adoption, Oral explanation to be held on 20 June 2024 at 11:00

2.2. For discussion / preparation for an opinion

2.2.1. - [EMA/OD/0000159992](#)

Treatment of acute lymphoblastic leukaemia

Action: For discussion/adoption

2.2.2. - [EMA/OD/0000164952](#)

Treatment of complex regional pain syndrome

Action: For discussion/adoption

2.2.3. - [EMA/OD/0000165577](#)

Treatment of Fabry disease

Action: For discussion/adoption

2.2.4. - [EMA/OD/0000166679](#)

Treatment of primary central nervous system lymphoma (PCNSL)

Action: For discussion/adoption

2.2.5. - [EMA/OD/0000166750](#)

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.6. - [EMA/OD/0000167609](#)

Treatment of thrombotic thrombocytopenic purpura

Action: For discussion/adoption

2.2.7. - [EMA/OD/0000168590](#)

Treatment of mitochondrial neurogastrointestinal encephalomyopathy

Action: For discussion/adoption

2.2.8. - [EMA/OD/0000169543](#)

Treatment of chronic pancreatitis

Action: For discussion/adoption

2.2.9. - [EMA/OD/0000169884](#)

Treatment of spinocerebellar ataxia

Action: For discussion/adoption

2.2.10. - [EMA/OD/0000171666](#)

Treatment of alpha-1 antitrypsin deficiency

Action: For discussion/adoption

2.2.11. - [EMA/OD/0000171870](#)

Treatment of Prader-Willi syndrome

Action: For discussion/adoption

2.2.12. - [EMA/OD/0000172086](#)

Treatment of punctate palmoplantar keratoderma

Action: For discussion/adoption

2.2.13. - [EMA/OD/0000172230](#)

Treatment of Wolfram syndrome

Action: For discussion/adoption

2.2.14. - [EMA/OD/0000172445](#)

Treatment of peripheral T cell lymphoma

Action: For discussion/adoption

2.2.15. - [EMA/OD/0000172743](#)

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.16. - [EMA/OD/0000172857](#)

Treatment of Friedreich ataxia

Action: For discussion/adoption

2.2.17. - [EMA/OD/0000173341](#)

Treatment of Creutzfeldt-Jakob disease

Action: For discussion/adoption

2.2.18. - [EMA/OD/0000173345](#)

Treatment of AL amyloidosis

Action: For discussion/adoption

2.2.19. - [EMA/OD/0000173347](#)

Treatment of ATTR amyloidosis

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

OMPD applications - appointment of rapporteurs at the 18-20 June 2024 COMP meeting

2.7. Evaluation on-going

None

2.8. Ongoing procedures

2.8.1. -

Treatment of narcolepsy

Action: For adoption

2.8.2. -

Treatment of glioma

Action: For adoption

2.8.3. -

Treatment of acute myeloid leukaemia

Action: For adoption

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

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

3.1.1. Adzynma - apadamtase alfa - EMEA/H/C/006198, EU/3/08/588, EMA/OD/0000150694

Takeda Manufacturing Austria AG; Treatment of thrombotic thrombocytopenic purpura

Action: For adoption, Oral explanation to be held on 19 June 2024 at 15:00

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

3.2.1. - masitinib - EMEA/H/C/005897, EMA/OD/081/16, EMA/OD/0000125972

AB Science.; In combination with riluzole for the treatment of adult patients with amyotrophic lateral sclerosis

Action: For information

3.2.2. Tepkinly - epcoritamab - EMEA/H/C/005985/II/0001, EU/3/22/2634, EMA/OD/0000157895

Abbvie Deutschland GmbH & Co. KG; Treatment of follicular lymphoma

Action: For discussion/adoption

3.2.3. - sotatercept - EMEA/H/C/005647, EU/3/20/2369, EMA/OD/0000157014

Merck Sharp & Dohme B.V.; Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

3.2.4. - chimeric monoclonal antibody against claudin-18 splice variant 2 - EMEA/H/C/005868, EU/3/10/803, EMA/OD/0000166702

Astellas Pharma Europe B.V.; Treatment of gastric cancer

Action: For discussion/adoption

3.2.5. - odronextamab - EMEA/H/C/006215

Regeneron Ireland Designated Activity Company

a) Treatment of follicular lymphoma, EU/3/22/2649, EMA/OD/0000168564 **Action:** For discussion/adoption

b) Treatment of diffuse large B-cell lymphoma, EU/3/22/2656, EMA/OD/0000168574

Action: For discussion/adoption

3.2.6. - elafibranor - EMEA/H/C/006231, EU/3/19/2182, EMA/OD/0000173044

Ipsen Pharma; Treatment of primary biliary cholangitis

Action: For discussion/adoption

3.3. Appeal

None

3.4. On-going procedures

Action: For information

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

3.5. Orphan Maintenance Reports

Action: For information

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

4.1. After adoption of CHMP opinion

None

4.2. Prior to adoption of CHMP opinion

4.2.1. Darzalex – daratumumab – EMEA/H/C/004077/II/0072, EU/3/13/1153, EMA/OD/0000171125

Janssen Cilag International; Treatment of plasma cell myeloma

Action: For discussion/adoption

4.3. Appeal

None

4.4. On-going procedures

Action: For information

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

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

None

6. Organisational, regulatory and methodological matters

6.1. Mandate and organisation of the COMP

6.1.1. COMP membership

Action: For information

6.1.2. Vote by proxy

Action: For information

6.1.3. Strategic Review & Learning meetings

None

6.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 18 June 2024 at lunch time

PAWG draft agenda for 18 June 2024 meeting

6.1.5. COMP Decisions Database

Action: For discussion

6.1.6. Election of COMP Chairperson

Action: For adoption

6.2. Coordination with EMA Scientific Committees or CMDh-v

6.2.1. Recommendation on eligibility to PRIME – report

PRIME eligibility requests - list of adopted outcomes May 2024

6.2.2. Joint resolution on the principles of conduct within EMA Scientific Committees, WPs and CMDx

Action: For information

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

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

None

6.3.2. Innovation Task Force (ITF) meetings

Action: For discussion

Upcoming ITF meetings

6.3.3. Scientific Advice Working Party (SAWP): nomination of COMP member to SAWP

Action: For adoption

Election of Joint COMP-SAWP alternate member - call for nomination – Extended deadline
12 June 2024

6.4. Cooperation within the EU regulatory network

6.4.1. European Commission

None

6.5. Cooperation with International Regulators

6.5.1. Food and Drug Administration (FDA)

None

6.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

6.5.3. Therapeutic Goods Administration (TGA), Australia

None

6.5.4. Health Canada

None

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

None

6.7. COMP work plan

None

6.8. Planning and reporting

6.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2024

Action: For information

6.8.2. Overview of orphan marketing authorisations/applications

Action: For information

7. Any other business

7.1. Spinal muscular atrophy (SMA) registry report

Action: For discussion

8. 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.

For a list of acronyms and abbreviations, see:

[Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA's regulatory activities](#)

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

www.ema.europa.eu/