



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 July 2024
EMA/COMP/298873/2024
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 16-18 July 2024

Chair: Violeta Stoyanova-Beninska

16 July 2024, 08:30-19:30, virtual meeting room

17 July 2024, 08:30-19:30, virtual meeting room

18 July 2024, 08:30-17:00, virtual meeting room

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 16-18 July 2024. See July 2024 COMP minutes (to be published post September 2024 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 16-18 July 2024.

1.3. Adoption of the minutes

COMP minutes for 18-20 June 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000159992

Treatment of acute lymphoblastic leukaemia

Action: For adoption, Oral explanation to be held on 16 July 2024 at 09:30

2.1.2. - EMA/OD/0000173345

Treatment of AL amyloidosis

Action: For adoption, Oral explanation to be held on 17 July 2024 at 15:30

2.1.3. - EMA/OD/0000173347

Treatment of ATTR amyloidosis

Action: For adoption, Oral explanation to be held on 17 July 2024 at 15:30

2.1.4. - EMA/OD/0000169543

Treatment of chronic pancreatitis

Action: For adoption, Oral explanation to be held on 18 July 2024 at 08:45

2.1.5. - EMA/OD/0000164952

Treatment of complex regional pain syndrome

Action: For adoption, Oral explanation to be held on 17 July 2024 at 17:15

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

Treatment of punctate palmoplantar keratoderma

Action: For adoption, Oral explanation to be held on 16 July 2024 at 16:30

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

Treatment of epidermolysis bullosa

Action: For adoption, Oral explanation to be held on 16 July 2024 at 14:30

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

Treatment of Fabry disease

Action: For adoption, Oral explanation to be held on 18 July 2024 at 11:00

2.2. For discussion / preparation for an opinion

2.2.1. - [EMA/OD/0000154786](#)

Prevention of arteriovenous access dysfunction in patients undergoing surgical creation of an arteriovenous fistula for haemodialysis

Action: For discussion/adoption

2.2.2. - [EMA/OD/0000155550](#)

Diagnosis of marginal zone lymphoma

Action: For discussion/adoption

2.2.3. - [EMA/OD/0000160667](#)

Treatment of anti-neutrophil cytoplasmic antibody (ANCA) – associated vasculitis (AAV)

Action: For discussion/adoption

2.2.4. - [EMA/OD/0000167672](#)

Treatment of small cell lung cancer

Action: For discussion/adoption

2.2.5. - [EMA/OD/0000168913](#)

Treatment of large-cell neuroendocrine carcinoma of the lung

Action: For discussion/adoption

2.2.6. - [EMA/OD/0000168919](#)

Treatment of extrapulmonary neuroendocrine carcinoma

Action: For discussion/adoption

2.2.7. - [EMA/OD/0000168961](#)

Treatment of fungal keratitis

Action: For discussion/adoption

2.2.8. - [EMA/OD/0000169888](#)

Treatment of Wiskott Aldrich syndrome (WAS)

Action: For discussion/adoption

2.2.9. - [EMA/OD/0000170073](#)

Prevention of acute liver failure (ALF)

Action: For discussion/adoption

2.2.10. - [EMA/OD/0000171663](#)

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.11. - [EMA/OD/0000171786](#)

Treatment of glutaric aciduria

Action: For discussion/adoption

2.2.12. - [EMA/OD/0000173551](#)

Treatment of Wilson's disease

Action: For discussion/adoption

2.2.13. - [EMA/OD/0000174349](#)

Treatment of complex regional pain syndrome

Action: For discussion/adoption

2.2.14. - [EMA/OD/0000174573](#)

Treatment of chondrosarcoma

Action: For discussion/adoption

2.2.15. - [EMA/OD/0000178304](#)

Treatment of pouchitis

Action: For discussion/adoption

2.2.16. - [EMA/OD/0000178496](#)

Treatment of inborn errors of primary bile acid synthesis

Action: For discussion/adoption

2.2.17. - [EMA/OD/0000178582](#)

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

2.2.18. - [EMA/OD/0000179126](#)

Treatment of familial chylomicronemia syndrome (FCS)

Action: For discussion/adoption

2.2.19. - [EMA/OD/0000179366](#)

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.20. - [EMA/OD/0000179561](#)

Treatment of cutaneous T-cell lymphoma (CTCL)

Action: For discussion/adoption

2.2.21. - [EMA/OD/0000183012](#)

Treatment of syndromic inherited retinal dystrophies of the rod-dominant phenotype

Action: For discussion/adoption

2.2.22. - [EMA/OD/0000171163](#)

Treatment of gastric cancer (including cancer of the gastro-oesophageal junction)

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 16-18 July 2024 COMP meeting

2.7. Evaluation on-going

None

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of *STXBP1* developmental and epileptic encephalopathy

Action: For adoption

3.1.2. -

Treatment of hereditary angioedema

Action: For adoption

3.1.3. -

Treatment of primary sclerosing cholangitis

Action: For adoption

3.1.4. -

Treatment of glioma

Action: For adoption

3.1.5. -

Treatment of Dravet syndrome

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. - odronextamab - EMEA/H/C/006215

Regeneron Ireland Designated Activity Company

a) Treatment of follicular lymphoma, EU/3/22/2649, EMA/OD/0000168564 **Action:** For adoption, Oral explanation to be held on 17 July 2024 at 09:00

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

Action: For adoption, Oral explanation to be held on 17 July 2024 at 09:00

4.1.2. 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 adoption, Oral explanation to be held on 17 July 2024 at 11:30

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

4.2.1. - elafibranor - EMEA/H/C/006231/0000, EU/3/19/2182, EMA/OD/0000173044

Ipsen Pharma; Treatment of primary biliary cholangitis

Action: For information

4.2.2. - 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, Oral explanation to be held on 17 July 2024 at 14:00

Document(s) from applicant: [link](#)

EMA documents: [link](#)

Notes: Status of the procedure at the CHMP: CHMP opinion is expected in July 2024.

4.3. Appeal

None

4.4. On-going procedures

Action: For information

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. Lutathera - lutetium (¹⁷⁷Lu) oxodotreotide - EMEA/H/C/004123/II/0052, EU/3/07/523

Advanced Accelerator; Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For discussion/adoption

5.2.2. Ngenla - somatogon - EMEA/H/C/005633/II/0016, EU/3/12/1087

Pfizer Europe MA EEIG; Treatment of Growth hormone deficiency

Action: For discussion/adoption

5.3. Appeal

None

5.4. On-going procedures

Action: For information

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 - Hungary

Update on the SRLM meeting to be held during the upcoming Hungarian Presidency

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 17 July 2024 at 12:50

PAWG draft agenda for 17 July 2024 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 June 2024

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 2024

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. EMA business Pipeline activity

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.

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/