07 October 2024
EMA/COMP/438930/2024
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 08-10 October 2024

Chair: Tim Leest – Vice-Chair: Frauke Naumann-Winter

08 October 2024, 08:30-19:30, virtual meeting room

09 October 2024, 08:30-19:30, virtual meeting room

10 October 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 08-10 October 2024. See October 2024 COMP minutes (to be published post November 2024 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 08-10 October 2024.

1.3. Adoption of the minutes

COMP minutes for 10-12 September 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000179978

Treatment of ATTR amyloidosis

Action: For adoption, Oral explanation to be held on 08 October 2024 at 11:00

2.1.2. - EMA/OD/0000175157

Treatment of idiopathic pulmonary fibrosis

Action: For adoption, Oral explanation to be held on 08 October 2024 at 14:00

2.1.3. - EMA/OD/0000175107

Treatment of glioma

Action: For adoption, Oral explanation to be held on 09 October 2024 at 14:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000175548

Treatment of focal segmental glomerulosclerosis

Action: For discussion/adoption

2.2.2. - EMA/OD/0000175842

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.3. - EMA/OD/0000177828

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.4. - EMA/OD/0000178220

Treatment of systemic sclerosis

Action: For discussion/adoption

2.2.5. - EMA/OD/0000178363

Treatment of spinal cord injury

Action: For discussion/adoption

2.2.6. - EMA/OD/0000179368

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.7. - EMA/OD/0000180034

Treatment of fragile X syndrome

Action: For discussion/adoption

2.2.8. - EMA/OD/0000180913

Treatment of non-syndromic inherited retinal dystrophies with a rod-dominant phenotype

Action: For discussion/adoption

2.2.9. - EMA/OD/0000182883

Treatment of AL amyloidosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000182940

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.11. - EMA/OD/0000183952

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.12. - EMA/OD/0000222144

Treatment in solid organ transplantation

Action: For discussion/adoption

2.2.13. - EMA/OD/0000222236

Treatment of immunoglobulin A nephropathy

Action: For discussion/adoption

2.2.14. - EMA/OD/0000222362

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

2.2.15. - EMA/OD/0000222517

Treatment of autoimmune haemolytic anaemia

Action: For discussion/adoption

2.2.16. - EMA/OD/0000222707

Treatment of Dercum disease

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 08-10 October 2024 COMP meeting

2.7. Evaluation on-going

1 application 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 hyperphenylalaninemia

Action: For adoption

3.1.2. -

Treatment of acute myeloid leukaemia

Action: For adoption

3.1.3. -

Treatment of hereditary angioedema

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. Elahere - mirvetuximab soravtansine - EMEA/H/C/005036, EU/3/15/1458, EMA/OD/0000178274

Abbvie Deutschland GmbH & Co. KG; Treatment of ovarian cancer

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

4.1.2. Hympavzi - marstacimab - EMEA/H/C/006240

Pfizer Europe MA EEIG

a) Treatment of haemophilia B, EU/3/23/2866, EMA/OD/0000179102

Action: For adoption, Oral explanation to be held on 08 October 2024 at 16:15

b) Treatment of haemophilia A, EU/3/16/1752, EMA/OD/0000179103

Action: For adoption

4.1.3. Hetronifly - serplulimab - EMEA/H/C/006170, EU/3/22/2731, EMA/OD/0000155775

Henlius Europe GmbH; Treatment of small cell lung cancer

Action: For adoption, Oral explanation to be held on 09 October 2024 at 11:30

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

4.2.1. - eplontersen - EMEA/H/C/006295, EU/3/23/2828, EMA/OD/0000177780

AstraZeneca AB; Treatment of transthyretin-mediated amyloidosis

Action: For discussion/adoption

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

5.3. None 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

Update on the SRLM meeting to be held in Budapest on 29-30 October 2024

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 04 October 2024 at 14:30

PAWG draft agenda for 04 October 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

None

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. Innovation Task Force (ITF) meetings

Upcoming ITF meetings

Action: For discussion

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

7.7.1. Draft COMP Work Plan for 2025

COMP Chair: Tim Leest

Action: For discussion

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

Q3/2024 Update of the Business Pipeline report for the human scientific committees

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/