20 February 202521 February 2025
EMA/COMP/529687/2024 Corr.1[1]
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 03-05 December 2024

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

03 December 2024, 08:30-19:30, virtual meeting room

04 December 2024, 08:30-19:30, virtual meeting room

05 December 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).

[1] Removal of internal reference in section 3

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Table of contents

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 December 2024. See December 2024 COMP minutes (to be published post January 2025 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 03-05 December 2024.

1.3. Adoption of the minutes

COMP minutes for 05-07 November 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000226832

Treatment of chronic pancreatitis

Action: For adoption, Oral explanation to be held on 04 December 2024 at 14:00

2.1.2. - EMA/OD/0000227928

Treatment of glioma

Action: For adoption, Oral explanation to be held on 03 December 2024 at 09:30

2.1.3. - EMA/OD/0000223853

Treatment of primary biliary cholangitis (PBC)

Action: For adoption, Oral explanation to be held on 04 December 2024 at 11:00

2.1.4. - EMA/OD/0000226506

Treatment of inherited retinal dystrophy due to defects in the *RPE65* gene

Action: For adoption, Oral explanation to be held on 04 December 2024 at 09:00

2.1.5. - EMA/OD/0000224798

Treatment of congenital alpha-1 antitrypsin deficiency

Action: For adoption, Oral explanation to be held on 03 December 2024 at 15:15

2.1.6. - EMA/OD/0000223155

Treatment of pancreatic cancer

Action: For information

Note: Withdrawal received on 19 November 2024.

2.1.7. - EMA/OD/0000227422

Treatment of non-traumatic osteonecrosis

Action: For adoption

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000178930

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.2. - EMA/OD/0000179381

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.3. - EMA/OD/0000225145

Treatment of autoimmune haemolytic anaemia

Action: For discussion/adoption

2.2.4. - EMA/OD/0000225792

Treatment of mucosal melanoma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000228469

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.6. - EMA/OD/0000228739

Treatment of primary sclerosing cholangitis

Action: For discussion/adoption

2.2.7. - EMA/OD/0000230149

Treatment of functional cobalamin deficiency in genetic defects of intracellular cobalamin processing

Action: For discussion/adoption

2.2.8. - EMA/OD/0000230201

Treatment of limb-girdle muscular dystrophy

Action: For discussion/adoption

2.2.9. - EMA/OD/0000230429

Treatment of adult polyglucosan body disease

Action: For discussion/adoption

2.2.10. - EMA/OD/0000230573

Treatment of Cushing's syndrome

Action: For discussion/adoption

2.2.11. - EMA/OD/0000230712

Treatment of gastrointestinal stromal tumours

Action: For discussion/adoption

2.2.12. - EMA/OD/0000231115

Treatment of Duchenne muscular dystrophy

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 03-05 December 2024 COMP meeting

2.7. Evaluation on-going

2 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 hereditary angioedema

Action: For adoption

3.1.2. -

Treatment of glioma

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. - beremagene geperpavec - EMEA/H/C/006330, EU/3/18/2012, EMA/OD/0000233504

Krystal Biotech Netherlands B.V.; Treatment of epidermolysis bullosa

Action: For information

4.2.2. - garadacimab - EMEA/H/C/006116, EU/3/21/2532, EMA/OD/0000133460

CSL Behring GmbH; Treatment of hereditary angioedema

Action: For information

4.2.3. - seladelpar - EMEA/H/C/004692, EU/3/17/1930, EMA/OD/0000170646

Cymabay Ireland Limited; Treatment of primary biliary cholangitis

Action: For adoption, Oral explanation to be held on 04 December 2024 at 16:30

4.2.4. - acoramidis - EMEA/H/C/006333, EU/3/18/2081, EMA/OD/0000224696

BridgeBio Europe B.V.; Treatment of ATTR amyloidosis

Action: For adoption, Oral explanation to be held on 03 December 2024 at 16:30

4.2.5. Pemazyre - pemigatinib - EMEA/H/C/005266/II/0015, EU/3/19/2216, EMA/OD/0000167021

Incyte Biosciences Distribution B.V.; Treatment of myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2

CHMP Rapporteur: Alexandre Moreau; CHMP Co-Rapporteur: Janet Koenig**Action:** For information

4.2.6. - tiratricol - EMEA/H/C/005220, EU/3/17/1945, EMA/OD/0000168628

Rare Thyroid Therapeutics; Treatment of monocarboxylate transporter 8 (MCT8) deficiency

Action: For information

4.3. Appeal

4.3.1 Hetronifly - serplulimab - EMEA/H/C/006170, EU/3/22/2731, EMA/OD/0000237657

Henlius Europe GmbH; Treatment of small cell lung cancer

Action: For adoption, Oral explanation to be held on 03 December 2024 at 14:00

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. Blincyto - blinatumomab - EMEA/H/C/003731/II/0056, EU/3/09/650, EMA/OD/0000162410

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia

Action: For discussion/adoption

5.2.2. Amvuttra - vutrisiran - EMEA/H/C/005852/II/0015, EU/3/18/2026, EMA/OD/019/18

Alnylam Netherlands B.V; Treatment of transthyretin-mediated amyloidosis

CHMP Rapporteur: Janet Koenig; CHMP Co-Rapporteur: Fatima Ventura

Action: For discussion/adoption

5.2.3. Darzalex, daratumumab - EMEA/H/C/004077/II/0077, EU/3/13/1153, EMA/OD/038/13

Janssen Cilag International N.V.; Treatment of plasma cell myeloma

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

None

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 02 December 2024 at 08:30

PAWG draft agenda for 02 December 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

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

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. Implementation of new fee regulation (EU) 2024/568 from 1 January 2025

Action: For information

8.2. Feedback from HTA/EMA workshop on uncertainties

COMP member: Frauke Naumann-Winter

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/