20 February 2025
EMA/COMP/60685/2025 Corr.1[1]
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 21-23 January 2025

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

21 January 2025, 13:00-19:30, room 2A

22 January 2025, 08:30-19:30, room 2A

23 January 2025, 08:30-15:00, 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).

[1] Removal of internal reference in section 3

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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 21-23 January 2025. See January 2025 COMP minutes (to be published post February 2025 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 21-23 January 2025.

1.3. Adoption of the minutes

COMP minutes for 03-05 December 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000230201

Treatment of limb-girdle muscular dystrophy

Action: For adoption

2.1.2. - EMA/OD/0000228469

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 21 January 2025 at 14:00

2.1.3. - EMA/OD/0000230149

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

Action: For adoption, Oral explanation to be held on 22 January 2025 at 09:00

2.1.4. - EMA/OD/0000228739

Treatment of primary sclerosing cholangitis

Action: For adoption, Oral explanation to be held on 22 January 2025 at 11:00

2.1.5. - EMA/OD/0000231115

Treatment of Duchenne muscular dystrophy

Action: For information

Note: Withdrawal received on 16 December 2024.

2.1.6. - EMA/OD/0000225792

Treatment of mucosal melanoma

Action: For information

Note: Withdrawal received on 16 December 2024.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000223873

Treatment of hyperphenylalaninemia

Action: For discussion/adoption

2.2.2. - EMA/OD/0000226861

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.3. - EMA/OD/0000230735

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.4. - EMA/OD/0000231617

Treatment of Olmsted syndrome

Action: For discussion/adoption

2.2.5. - EMA/OD/0000232365

Treatment of bradykinin-mediated angioedema

Action: For discussion/adoption

2.2.6. - EMA/OD/0000233191

Treatment of arrhythmogenic cardiomyopathy caused by pathogenic mutations in the *PKP2* gene

Action: For discussion/adoption

2.2.7. - EMA/OD/0000233467

Treatment of adenosine deaminase 2 deficiency (DADA2)

Action: For discussion/adoption

2.2.8. - EMA/OD/0000233979

Treatment of Rett syndrome

Action: For discussion/adoption

2.2.9. - EMA/OD/0000234998

Treatment of acromegaly

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

None

2.7. Evaluation on-going

None

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of nephrotic syndrome

Action: For adoption

3.1.2. -

Treatment of Gaucher disease

Action: For adoption

3.1.3. -

Treatment of Berardinelli-Seip syndrome (congenital generalised lipodystrophy)

Action: For adoption

3.1.4. -

Treatment of pancreatic cancer

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. Rytelo - imetelstat - EMEA/H/C/006105, EU/3/20/2305, EMA/OD/0000225798

Geron Netherlands B.V.; Treatment of myelodysplastic syndromes

Action: For adoption, Oral explanation to be held on 21 January 2025 at 15:30

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

4.2.1. - vorasidenib hemicitrate hemihydrate - EMEA/H/C/006284, EU/3/22/2737, EMA/OD/0000159477

Les Laboratoires Servier; Treatment of glioma

Action: For discussion/adoption

4.2.2. Fabhalta - iptacopan hydrochloride - EMEA/H/C/005764/II/0001, EU/3/18/2104, EMA/OD/0000224423

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Action: For discussion/adoption

4.2.3. Vyvgart - efgartigimod alfa - EMEA/H/C/005849/II/0020, EU/3/21/2555, EMA/OD/0000222245

Argenx; Treatment of chronic inflammatory demyelinating polyneuropathy

CHMP Rapporteur: Thalia Marie Estrup Blicher; CHMP Co-Rapporteur: Alexandre Moreau

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

None

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

Scope: Update on the SRLM meeting to be held during the upcoming Polish Presidency

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 17 January 2025 at 10:00

PAWG draft agenda for 17 January 2025 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

Scope: Upcoming ITF meetings

Action: For discussion

7.3.3. Methodology Working Party

Scope: Call for volunteers - Q&A on indirect comparisons

Action: For discussion

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Scope: Exchange of views with European Commission on Pharmaceutical Legislation Reform

Action: For discussion

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Scope: EMA / FDA collaboration and introduction to Liaison program

Action: For information

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 adoption

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2025

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Patient engagement methodologies

Action: For information

8.2. EMA business Pipeline activity

Scope: Q4-2024 Update of the Business Pipeline report for the human scientific committees

Action: For information

8.3. Aspects of the orphan regulation Article 8(2)

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/