12 February 2024
EMA/COMP/37898/2024
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 13-15 February 2024

Chair: Violeta Stoyanova-Beninska – Vice-Chair: Armando Magrelli

13 February 2024, 09:00-19:30, room 2A

14 February 2024, 08:30-19:30, room 2A

15 February 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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000
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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 13-15 February 2024. See February 2024 COMP minutes (to be published post March 2024 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 13-15 February 2024.

1.3. Adoption of the minutes

COMP minutes for 16-18 January 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000146222

Treatment of Berardinelli-Seip syndrome (congenital generalised lipodystrophy)

Action: For adoption, Oral explanation to be held on 13 February 2024 at 11:00

2.1.2. - EMA/OD/0000147895

Treatment of Lawrence syndrome (acquired generalised lipodystrophy)

Action: For adoption, Oral explanation to be held on 13 February 2024 at 11:00

2.1.3. - EMA/OD/0000156633

Treatment of soft tissue sarcoma

Action: For information

Note: Withdrawal request received on 24 January 2024.

2.1.4. - EMA/OD/0000150709

Treatment of pilonidal disease

Action: For information

Note: Withdrawal request received on 2 February 2024.

2.1.5. - EMA/OD/0000142006

Treatment of mesothelioma

Action: For adoption, Oral explanation to be held on 14 February 2024 at 11:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000152958

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000155985

Treatment of traumatic spinal cord injury

Action: For discussion/adoption

2.2.3. - EMA/OD/0000157446

Treatment of fragile X syndrome

Action: For discussion/adoption

2.2.4. - EMA/OD/0000158128

Treatment of Becker muscular dystrophy (BMD)

Action: For discussion/adoption

2.2.5. - EMA/OD/0000158137

Treatment of Duchenne muscular dystrophy (DMD)

Action: For discussion/adoption

2.2.6. - EMA/OD/0000158813

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.7. - EMA/OD/0000158981

Treatment of congenital pseudarthrosis of long bones

Action: For discussion/adoption

2.2.8. - EMA/OD/0000159738

Treatment of ataxia-oculomotor apraxia-4

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 13-15 February 2024 COMP meeting

2.7. Evaluation on-going

4 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 Fabry disease

Action: For adoption

3.1.2. -

Treatment of sickle cell disease

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. - sparsentan - EMEA/H/C/005783, EU/3/20/2345, EMA/OD/0000110380

Vifor France; Treatment of primary IgA nephropathy

Action: For discussion/adoption

4.2.2. – danicopan - EMEA/H/C/005517, EU/3/17/1946, EMA/OD/0000136076

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion/adoption

4.2.3. – tofersen - EMEA/H/C/005493, EU/3/16/1732, EMA/OD/0000137554

Biogen Netherlands B.V; Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

4.2.4. – retifanlimab - EMEA/H/C/006194, EU/3/22/2743, EMA/OD/0000152395

Incyte Biosciences Distribution B.V.; Treatment of Merkel cell carcinoma

Action: For discussion/adoption

4.2.5. – iptacopan - EMA/H/C/005764, EU/3/20/2281, EMA/OD/0000141229

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion/adoption

4.2.6. – dantrolene sodium, hemiheptahydrate - EMEA/H/C/006009, EU/3/21/2443, EMA/OD/0000102465

Norgine B.V.; Treatment of malignant hyperthermia

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

5.1.1. Aspaveli – pegcetacoplan - EMEA/H/C/005553/II/0011, EU/3/17/1873, EMA/OD/0000140083

Swedish Orphan Biovitrum AB (publ); Treatment of paroxysmal nocturnal haemoglobinuria

CHMP Rapporteur: Alexandre Moreau; CHMP Co-Rapporteur: Selma Arapovic

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

5.2. Prior to adoption of CHMP opinion

5.2.1. Carvykti - ciltacabtagene autoleucel - EMEA/H/C/005095/II/0021, EU/3/20/2252, EMA/OD/0000141581

Janssen - Cilag International; Treatment of multiple myeloma

CHMP Rapporteur: Jan Mueller-Berghaus

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

SRLM meeting in Leuven under the Belgian Presidency of the Council of the EU

Action: For discussion

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 13 February 2024 at 13:00

PAWG draft agenda for 13 February 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 January 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)

Action: For information

Summary report of the Patients' and Consumers' (PCWP) and Healthcare Professionals' (HCPWP) Working Parties meeting with all eligible organisations held face-to-face on 14-15 November 2023

Draft agenda of the Patients' and Consumers' (PCWP) and Healthcare Professionals' (HCPWP) Working Parties joint meeting to be held face-to-face on 27-28 February 2024

7.3.2. Innovation Task Force (ITF) meetings

Action: For discussion

Upcoming ITF meetings

Overview of ITF activities for the year 2023

7.3.3. Revision of EMA guideline on epileptic disorders

Action: For discussion

Clinical investigation of medicinal products in the treatment of epileptic disorders - Scientific guideline

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.4.2. Feedback from the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP) Plenary

Action: For discussion

7.4.3. EURORDIS update on Rare Diseases Day events

Action: For discussion

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. New tool for searching scientific advice - Scientific Explorer

Action: For discussion

8.2. Overview of the relevant case-law of the Court of Justice of the European Union

Action: For discussion

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/