11 March 2024
EMA/COMP/79894/2024
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 12-14 March 2024

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

12 March 2024, 08:30-19:30, virtual meeting room

13 March 2024, 08:30-19:30, virtual meeting room

14 March 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 12-14 March 2024. See March 2024 COMP minutes (to be published post April 2024 meeting).

1.2. Adoption of agenda

COMP agenda for 12-14 March 2024.

1.3. Adoption of the minutes

COMP minutes for 13-15 February 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. – EMA/OD/0000158981

Treatment of congenital pseudarthrosis of long bones

Action: For adoption

2.1.2. – EMA/OD/0000155985

Treatment of traumatic spinal cord injury

Action: For adoption, Oral explanation to be held on 12 March 2024 at 09:45

2.1.3. – EMA/OD/0000159738

Treatment of ataxia-oculomotor apraxia-4

Action: For adoption, Oral explanation to be held on 12 March 2024 at 11:30

2.1.4. – EMA/OD/0000158137

Treatment of Duchenne muscular dystrophy (DMD)

Action: For adoption, Oral explanation to be held on 13 March 2024 at 14:00

2.2. For discussion / preparation for an opinion

2.2.1. – EMA/OD/0000133472

Treatment of tuberculosis

Action: For discussion/adoption

2.2.2. – EMA/OD/0000156967

Treatment of familial chylomicronemia syndrome (FCS)

Action: For discussion/adoption

2.2.3. – EMA/OD/0000157122

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.4. – EMA/OD/0000158039

Treatment of heparin-induced thrombocytopenia

Action: For discussion/adoption

2.2.5. – EMA/OD/0000160213

Treatment of neuroblastoma

Action: For discussion/adoption

2.2.6. – EMA/OD/0000161033

Treatment of hypophosphatasia

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 12-14 March 2024 COMP meeting

2.7. Evaluation on-going

16 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 Niemann-Pick disease, type C

Action: For adoption

3.1.2. –

Treatment of neurofibromatosis type 1

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

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

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

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

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

Novartis Europharm Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion/adoption

4.2.2. – efanesoctocog alfa - EMEA/H/C/005968, EU/3/19/2176, EMA/OD/0000160184

Swedish Orphan Biovitrum AB (publ); Treatment of haemophilia A

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. Reblozyl – luspatercept - EMEA/H/C/004444/II/0021, EU/3/14/1331, EMA/OD/0000134295

Bristol-Myers Squibb Pharma EEIG; Treatment of myelodysplastic syndromes

CHMP Rapporteur: Daniela Philadelphy; CHMP Co-Rapporteur: Ewa Balkowiec Iskra

Action: For adoption

5.2. Prior to adoption of CHMP opinion

5.2.1. Onivyde pegylated liposomal – irinotecan - EMEA/H/C/004125/II/0034, EU/3/11/933, EMA/OD/0000144740

Les Laboratoires Servier; Treatment of pancreatic cancer

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 11 March 2024 at 10:30

PAWG draft agenda for 11 March 2024

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 February 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 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

7.4.2. EU Network Training Centre (NTC): training webinar on the regulatory/HTA interface under the HTA Regulation

Action: For information

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. Update on Real World Evidence, including DARWIN EU®

Action: For discussion

8.2. Collaborare project: call for suggestions of conditions

Action: For discussion

8.3. EMA business Pipeline activity

Action: For information

Q1-2024 Update of the Business Pipeline report for the human scientific committees

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/