2 December 2022
EMA/COMP/886990/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 06-08 December 2022

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

06 December 2022, 08:30-19:30, virtual meeting

07 December 2022, 08:30-19:30, virtual meeting

08 December 2022, 08:30-17:00, virtual 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).

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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 06-08 December 2022. See (current) December 2022 COMP minutes (to be published post January 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 06-08 December 2022.

1.3. Adoption of the minutes

COMP minutes for 08-10 November 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000105219

Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 07 December 2022 at 15:30

2.1.2. - EMA/OD/0000103787

Treatment of primary sclerosing cholangitis

Action: For adoption, Oral explanation to be held on 06 December 2022 at 14:30

2.1.3. - EMA/OD/0000103269

Treatment of peripheral T-cell lymphoma

Action: For adoption, Oral explanation to be held on 06 December 2022 at 12:15

2.1.4. - EMA/OD/0000089519

Treatment of soft tissue sarcoma (STS)

Action: For information

Note: Withdrawal request received on 21 November 2022

2.1.5. - EMA/OD/0000104107

Treatment of glioma

Action: For adoption, Oral explanation to be held on 06 December 2022 at 15:45

2.1.6. - EMA/OD/0000096050

Treatment of Duchenne muscular dystrophy

Action: For information

Note: Withdrawal request received

2.1.7. - EMA/OD/0000099774

Prevention of tuberculosis

Action: For adoption, Oral explanation to be held on 07 December 2022 at 10:30

2.1.8. - EMA/OD/0000106875

Treatment of narcolepsy

Action: For information

Note: Withdrawal request received on 18 November 2022

2.1.9. - EMA/OD/0000095228

Treatment of carcinoid syndrome

Action: For adoption, Oral explanation to be held on 07 December 2022 at 14:00

2.1.10. - EMA/OD/0000097397

Treatment of Duchenne muscular dystrophy

Action: For adoption, Oral explanation to be held on 07 December 2022 at 12:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000070986

Treatment of megacystis microcolon intestinal hypoperistalsis syndrome

Action: For discussion/adoption

2.2.2. - EMA/OD/0000086832

Treatment of glioma

Action: For discussion/adoption

2.2.3. - EMA/OD/0000100767

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000102985

Treatment of hereditary cerebral amyloid angiopathies

Action: For discussion/adoption

2.2.5. - EMA/OD/0000104665

Treatment of recombination activating gene 2 deficient – severe combined immunodeficiency (RAG2-SCID)

Action: For discussion/adoption

2.2.6. - EMA/OD/0000104730

Treatment of congenital alpha-1 antitrypsin deficiency

Action: For discussion/adoption

2.2.7. - EMA/OD/0000105270

Diagnosis of glioma

Action: For discussion/adoption

2.2.8. - EMA/OD/0000105836

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.9. - EMA/OD/0000108995

Treatment of autosomal dominant polycystic kidney disease

Action: For discussion/adoption

2.2.10. - EMA/OD/0000110129

Treatment of Merkel cell carcinoma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000110207

Treatment of sarcoidosis

Action: For discussion/adoption

2.2.12. - EMA/OD/0000111633

Treatment of invasive aspergillosis

Action: For discussion/adoption

2.2.13. - EMA/OD/0000111754

Treatment of dopamine transporter deficiency syndrome

Action: For discussion/adoption

2.2.14. - EMA/OD/0000111992

Treatment of Huntington's disease

Action: For discussion/adoption

2.2.15. - EMA/OD/0000112174

Treatment of chronic granulomatous disease type I

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

Document(s) tabled:
OMPD applications - appointment of rapporteurs at the 06-08 December 2022 COMP meeting

2.7. Evaluation on-going

15 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. - EMA/SA/0000099403

Treatment of respiratory distress syndrome

Action: For discussion/adoption

3.1.2. - EMA/SA/0000109761

Treatment of myelodysplastic syndrome (MDS)

Action: For discussion/adoption

3.1.3. - EMA/SA/0000105854

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion/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. Fintepla – fenfluramine hydrochloride - EMEA/H/C/003933/II/0012, EU/3/17/1836, EMA/OD/0000075867

Zogenix ROI Limited; Treatment of Lennox-Gastaut syndrome

CHMP Rapporteur: Thalia Marie Estrup Blicher; CHMP Co-Rapporteur: Johann Lodewijk Hillege

Action: For discussion/adoption

4.2.2. – etranacogene dezaparvovec - EMEA/H/C/004827, EU/3/18/1999, EMA/OD/0000087180

CLS Behring GmbH; Treatment of haemophilia B

Action: For discussion/adoption

4.2.3. – cipaglucosidase alfa - EMEA/H/C/005703, EU/3/18/2000, EMA/OD/0000098435

Amicus Therapeutics Europe Limited; Treatment of glycogen storage disease type II (Pompe's disease)

Action: For discussion/adoption

4.3. Appeal

None

4.4. On-going procedures

Action: For information

Document(s) tabled:
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. Reblozyl – luspatercept - EMEA/H/C/004444/II/0009, EU/3/14/1300, EMA/OD/0000072540

Bristol-Myers Squibb Pharma EEIG; Treatment of beta-thalassaemia intermedia and major

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

Action: For discussion/adoption

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:

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 1 December 2022 at 14.00

Document tabled:

PAWG draft agenda for 1 December 2022

7.1.5. Principal Decisions Database

Action: For discussion

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

Action: For information

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes November 2022

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 discussion

Feedback from PCWP/HCPWP annual meeting with all eligible organisations – 15 November 2022

7.3.2. 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

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 2022

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Preparation of EMA Regulatory & Scientific Conference on RNA-based medicines

Action: For discussion

8.2. Review of orphan designation criteria and OMAR preparation

Action: For discussion

8.3. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Forecast for 2023 and Q4/2022 Update of the Business Pipeline report for the human scientific committees

8.4. Feedback from the ENCePP Plenary

Action: For discussion

8.5. ICH M11 Public Consultation

Action: For discussion

8.6. Methodology Working Party

Action: For discussion

Introduction of MWP to the 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.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/