8 November 2022
EMA/COMP/808207/2022 Corr.1 [1]
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 08-10 November 2022

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

08 November 2022, 09:00-19:30, room 1A / virtual meeting

09 November 2022, 08:30-19:30, room 1A / virtual meeting

10 November 2022, 08:30-17:00, room 1A / virtual meeting

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] Correction in section 4.3.1.

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 An agency of the European Union

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 08-10 November 2022. See November 2022 COMP minutes (to be published post December 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 8-10 November 2022.

1.3. Adoption of the minutes

COMP minutes for 4-6 October 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000099427

Treatment of small cell lung cancer

Action: For adoption

2.1.2. - EMA/OD/0000091248

Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 8 November 2022 at 09:30

2.1.3. - EMA/OD/0000082375

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 8 November 2022 at 14:15

2.1.4. - EMA/OD/0000096261

Treatment of progressive supranuclear palsy

Action: For adoption, Oral explanation to be held on 8 November 2022 at 12:15

2.1.5. - EMA/OD/0000098623

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For adoption, Oral explanation to be held on 9 November 2022 at 09:00

2.1.6. - EMA/OD/0000096686

Diagnosis of ATTR amyloidosis

Action: For adoption, Oral explanation to be held on 9 November 2022 at 10:30

2.1.7. - EMA/OD/0000099136

Treatment of perinatal asphyxia

Action: For information

Note: Withdrawal request received on 20 October 2022.

2.1.8. - EMA/OD/0000096688

Treatment of autosomal dominant polycystic kidney disease (ADPKD)

Action: For adoption, Oral explanation to be held on 9 November 2022 at 14:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000089519

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000093338

Treatment of myotonic disorders

Action: For discussion/adoption

2.2.3. - EMA/OD/0000095228

Treatment of carcinoid syndrome

Action: For discussion/adoption

2.2.4. - EMA/OD/0000096050

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.5. - EMA/OD/0000097397

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.6. - EMA/OD/0000097578

Diagnosis of neuroendocrine neoplasms (NEN)

Action: For discussion/adoption

2.2.7. - EMA/OD/0000098389

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000099257

Treatment of Canavan disease

Action: For discussion/adoption

2.2.9. - EMA/OD/0000099349

Treatment of peripheral T-cell lymphoma

Action: For discussion/adoption

2.2.10. - EMA/OD/0000099774

Prevention of tuberculosis

Action: For discussion/adoption

2.2.11. - EMA/OD/0000101416

Treatment of acute lymphoblastic leukaemia

Action: For discussion/adoption

2.2.12. - EMA/OD/0000102490

Treatment in haematopoietic stem cell transplantation

Action: For discussion/adoption

2.2.13. - EMA/OD/0000103269

Treatment of peripheral T-cell lymphoma

Action: For discussion/adoption

2.2.14. - EMA/OD/0000103787

Treatment of primary sclerosing cholangitis

Action: For discussion/adoption

2.2.15. - EMA/OD/0000104107

Treatment of glioma

Action: For discussion/adoption

2.2.16. - EMA/OD/0000104148

Treatment of adrenoleukodystrophy

Action: For discussion/adoption

2.2.17. - EMA/OD/0000105068

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.18. - EMA/OD/0000105169

Treatment of ornithine transcarbamylase (OTC) deficiency

Action: For discussion/adoption

2.2.19. - EMA/OD/0000105219

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.20. - EMA/OD/0000106875

Treatment of narcolepsy

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 8-10 November 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. -

Treatment of primary IgA nephropathy

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

CLS Behring GmbH; Treatment of haemophilia B

Action: For discussion

4.2.2. – omburtamab I-131 - EMEA/H/C/005499, EU/3/17/1839, EMA/OD/0000063579

Y-Mabs Therapeutics A/S; Treatment of neuroblastoma

Action: For information

4.2.3. 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

4.3. Appeal

4.3.1. Zynlonta – loncastuximab tesirine - EMEA/H/C/005685, EU/3/21/2481, EMA/OD/0000115078

ADC Therapeutics (NL) B.V.; Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 08 October 2022 at 15:45

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 8 November 2022

Document tabled:

PAWG draft agenda

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 October 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 information

Document(s) tabled:

Draft Agenda - Annual PCWP-HCPWP meeting with all eligible organisation - 15 November 2022

Meeting Summary PCWP meeting 22 September 2022

Meeting Summary HCPWP meeting 22 September 2022

Meeting Summary PCWP HCPWP meeting 22 September 2022

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

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 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. Public Consultation on Good Practice Guide and Data Quality Framework

Action: For discussion

8.3. Upcoming ITF meetings

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.

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