6 November 2023
EMA/COMP/458095/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 07-09 November 2023

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

07 November 2023, 08:30-19:30, virtual meeting room

08 November 2023, 08:30-19:30, virtual meeting room

09 November 2023, 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).

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 07-09 November 2023. See (current) November 2023 COMP minutes (to be published post December 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 07-09 November 2023.

1.3. Adoption of the minutes

COMP minutes for 03-05 October 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000142974

Treatment of Duchenne muscular dystrophy

Action: For adoption, Oral explanation to be held on 07 November 2023 at 14:00

2.1.2. - EMA/OD/0000146696

Treatment of pancreatic cancer

Action: For information

Note: Withdrawal request received on 19 October 2023.

2.1.3. - EMA/OD/0000143407

Treatment of follicular lymphoma

Action: For adoption, Oral explanation to be held on 07 November 2023 at 16:00

2.1.4. - EMA/OD/0000145792

Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 08 November 2023 at 16:00

2.1.5. - EMA/OD/0000142982

Treatment of moderate to severe traumatic brain injury

Action: For information

Note: Withdrawal request received on 12 October 2023.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000137167

Prevention of primary graft dysfunction following lung transplantation

Action: For discussion/adoption

2.2.2. - EMA/OD/0000140599

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.3. - EMA/OD/0000142148

Treatment of perinatal asphyxia

Action: For discussion/adoption

2.2.4. - EMA/OD/0000143601

Treatment of glioma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000147097

Treatment of Leigh syndrome

Action: For discussion/adoption

2.2.6. - EMA/OD/0000147441

Treatment of peripheral T cell lymphoma

Action: For discussion/adoption

2.2.7. - EMA/OD/0000148726

Treatment of Angelman syndrome

Action: For discussion/adoption

2.2.8. - EMA/OD/0000148755

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.9. - EMA/OD/0000149115

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000149222

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000149464

Treatment of hyperinsulinism

Action: For discussion/adoption

2.2.12. - EMA/OD/0000149586

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.13. - EMA/OD/0000149631

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.14. - EMA/OD/0000150150

Treatment of ricin poisoning

Action: For discussion/adoption

2.2.15. - EMA/OD/0000150249

Treatment of autosomal dominant polycystic kidney disease

Action: For discussion/adoption

2.2.16. - EMA/OD/0000150398

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Action: For discussion/adoption

2.2.17. - EMA/OD/0000150558

Treatment of eosinophilic esophagitis

Action: For discussion/adoption

2.2.18. - EMA/OD/0000150801

Treatment of tenosynovial giant-cell tumour (TGCT)

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 07-09 November 2023 COMP meeting

2.7. Evaluation on-going

17 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 ovarian 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. Rezzayo – rezafungin - EMEA/H/C/005900, EU/3/20/2385, EMA/OD/0000140230

Mundipharma GmbH; Treatment of invasive candidiasis

Action: For adoption, Oral explanation to be held on 08 November 2023 at 14:15

4.1.2. Elrexfio - humanised IgG2k Fc-modified bispecific monoclonal antibody against CD3 and BCMA - EMEA/H/C/005908, EU/3/21/2471, EMA/OD/0000147440

Pfizer Europe MA EEIG; Treatment of multiple myeloma

Action: For information

Note: Withdrawal request received on 25 October 2023.

4.1.3. Albrioza – sodium phenylbutyrate / ursodoxicoltaurine - EMEA/H/C/005901, EU/3/20/2284, EMA/OD/0000096503

Amylyx Pharmaceuticals EMEA; Treatment of amyotrophic lateral sclerosis

Action: For information

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

4.2.1. - sparsentan - EMEA/H/C/005783/0000, EU/3/20/2345, EMA/OD/0000110380

Vifor France; Treatment of primary IgA nephropathy

Action: For discussion/adoption

4.2.2. - leniolisib - EMEA/H/C/005927/0000, EU/3/20/2339, EMA/OD/0000117371

Pharming Technologies B.V.; Treatment of activated phosphoinositide 3-kinase delta syndrome

Action: For discussion/adoption

4.2.3. - rozanolixizumab - EMEA/H/C/005824/0000, EU/3/20/2272, EMA/OD/0000129455

UCB Pharma; Treatment of myasthenia gravis

Action: For discussion/adoption

4.2.4. - trametinib dimethyl sulfoxide - EMEA/H/C/005886/0000, EU/3/20/2374, EMA/OD/0000134200

Novartis Europharm Limited; Treatment of glioma

Action: For discussion/adoption

4.2.5. - momelotinib dihydrochloride - EMEA/H/C/005768/0000, EU/3/11/886, EMA/OD/0000129901

GlaxoSmithKline Trading Services Limited; Treatment of post-polycythaemia vera myelofibrosis

Action: For discussion/adoption

4.2.6. - momelotinib dihydrochloride - EMEA/H/C/005768/0000, EU/3/11/887, EMA/OD/0000130955

GlaxoSmithKline Trading Services Limited; Treatment of post-essential thrombocythaemia myelofibrosis

Action: For discussion/adoption

4.2.7. - momelotinib dihydrochloride - EMEA/H/C/005768/0000, EU/3/11/888, EMA/OD/0000130957

GlaxoSmithKline Trading Services Limited; Treatment of primary myelofibrosis

Action: For discussion/adoption

4.2.8. Ayvakyt - avapritinib - EMEA/H/C/005208/II/0023, EU/3/18/2074, EMA/OD/0000127063

Blueprint Medicines; Treatment of mastocytosis

Action: For discussion/adoption

4.2.9. Livmarli - maralixibat - EMEA/H/C/005857/II/0003/G, EU/3/13/1216, EMA/OD/0000136132

Mirum Pharmaceuticals International B.V.; Treatment of progressive familial intrahepatic cholestasis

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

None

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

SRLM meeting in Madrid under the Spanish Presidency of the Council of the EU

Action: For discussion

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 06 November 2023 at 10:00

PAWG draft agenda for 06 November 2023

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 October 2023

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

Draft Agenda - Annual PCWP-HCPWP meeting with all eligible organisation – 14 - 15 November 2023

7.3.2. Upcoming 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. C4C multistakeholder meeting on perinatal asphyixia

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 2023

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Presentation of Reflection Paper on the use of real-world data to generate real-world evidence in non-interventional studies

Action: For discussion

8.2. Nomination of COMP representative for ENCePP Steering Group 2024-2026

Action: For discussion

8.3. Pilot on new maintenance procedure

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/