4 December 2023
EMA/COMP/511996/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 05-07 December 2023

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

05 December 2023, 09:00-19:30, room 2A

06 December 2023, 08:30-19:30, room 2A

07 December 2023, 09:00-17:00, 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).

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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 05-07 December 2023. See December 2023 COMP minutes (to be published post January 2024 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 05-07 December 2023.

1.3. Adoption of the minutes

COMP minutes for 07-09 November 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000150558

Treatment of eosinophilic esophagitis

Action: For adoption, Oral explanation to be held on 06 December 2023 at 12:00

2.1.2. - EMA/OD/0000149464

Treatment of hyperinsulinism

Action: For adoption, Oral explanation to be held on 05 December 2023 at 14:00

2.1.3. - EMA/OD/0000149222

Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 05 December 2023 at 15:30

2.1.4. - EMA/OD/0000148755

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 06 December 2023 at 09:00

2.1.5. - EMA/OD/0000149631

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 06 December 2023 at 10:30

2.1.6. - EMA/OD/0000150398

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

Action: For adoption, Oral explanation to be held on 05 December 2023 at 12:00

2.1.7. - EMA/OD/0000150249

Treatment of autosomal dominant polycystic kidney disease

Action: For adoption, Oral explanation to be held on 07 December 2023 at 09:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000139765

Treatment of glioma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000144447

Treatment of systemic sclerosis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000144999

Treatment of glioma

Action: For discussion/adoption

2.2.4. - EMA/OD/0000145710

Diagnosis of glioma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000146101

Treatment of small cell lung cancer

Action: For discussion/adoption

2.2.6. - EMA/OD/0000149117

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.7. - EMA/OD/0000149156

Treatment of cholangiocarcinoma

Action: For discussion/adoption

2.2.8. - EMA/OD/0000149689

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.9. - EMA/OD/0000150090

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.10. - EMA/OD/0000152508

Treatment of alpha-thalassaemia X-linked intellectual disability

Action: For discussion/adoption

2.2.11. - EMA/OD/0000152994

Treatment of Rett syndrome

Action: For discussion/adoption

2.2.12. - EMA/OD/0000153654

Treatment of mobilisation of progenitor cells prior to stem cell transplantation

Action: For discussion/adoption

2.2.13. - EMA/OD/0000153667

Treatment of acquired factor X deficiency

Action: For discussion/adoption

2.2.14. - EMA/OD/0000154030

Treatment of recurrent respiratory papillomatosis

Action: For discussion/adoption

2.2.15. - EMA/OD/0000154059

Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)

Action: For discussion/adoption

2.2.16. - EMA/OD/0000154242

Treatment of ATTR amyloidosis

Action: For discussion/adoption

2.2.17. - EMA/OD/0000154838

Treatment of thalassaemia alpha intermedia and major

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 05-07 December 2023 COMP meeting

2.7. Evaluation on-going

12 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 gastrointestinal stromal tumours

Action: For adoption

3.1.2. -

Treatment of immune thrombocytopenia

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. Omjjara - momelotinib dihydrochloride - EMEA/H/C/005768/0000

GlaxoSmithKline Trading Services Limited

a) Treatment of post-polycythaemia vera myelofibrosis, EU/3/11/886, EMA/OD/0000129901

Action: For adoption, Oral explanation to be held on 06 December 2023 at 14:00

b) Treatment of post-essential thrombocythaemia myelofibrosis, EU/3/11/887, EMA/OD/0000130955

Action: For adoption, Oral explanation to be held on 06 December 2023 at 14:00

c) Treatment of primary myelofibrosis, EU/3/11/888, EMA/OD/0000130957

Action: For adoption, Oral explanation to be held on 06 December 2023 at 14:00

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

4.2.1. 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 information

4.2.2. – exagamglogene autotemcel - EMEA/H/C/005763

Vertex Pharmaceuticals (Ireland) Limited

a) Treatment of sickle cell disease, EU/3/19/2242, EMA/OD/0000146415

Action: For discussion/adoption

b) Treatment of beta-thalassaemia intermedia and major, EU/3/19/2210, EMA/OD/0000146264

Action: For discussion/adoption

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

Vifor France; Treatment of primary IgA nephropathy

Action: For discussion/adoption

4.2.4. – omaveloxolone - EMEA/H/C/006084, EU/3/18/2037, EMA/OD/0000156841

Reata Ireland Limited; Treatment of Friedreich's ataxia

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

None

5.2. Prior to adoption of CHMP opinion

None

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 01 December at 13:00

PAWG draft agenda for 01 December 2023 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 November 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)

Feedback from PCWP/HCPWP and all eligible meeting - 14 and 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

7.5. 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. Update on the patient experience data - major contribution to patient care project

Action: For discussion

8.2. EMA business pipeline activity

Action: For information

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/