8 February 2023
EMA/COMP/41150/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 14-16 February 2023

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

14 February 2023, 08:30-19:30, virtual meeting

15 February 2023, 08:30-19:30, virtual meeting

16 February 2023, 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 14-16 February 2023. See February 2023 COMP minutes (to be published post March 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 14-16 February 2023.

1.3. Adoption of the minutes

COMP minutes for 17-19 January 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000083629

Treatment of scedosporiosis

Action: For adoption, Oral explanation to be held on 14 February 2023 at 17:00

2.1.2. - EMA/OD/0000116158

Treatment of Niemann-Pick disease type C (NP-C)

Action: For adoption, Oral explanation to be held on 14 February 2023 at 12:15

2.1.3. - EMA/OD/0000114452

Treatment of GM1 gangliosidosis

Action: For adoption, Oral explanation to be held on 14 February 2023 at 14:30

2.1.4. - EMA/OD/0000114581

Treatment of galactosialidosis

Action: For adoption, Oral explanation to be held on 14 February 2023 at 14:30

2.1.5. - EMA/OD/0000114584

Treatment of sialidosis

Action: For adoption, Oral explanation to be held on 14 February 2023 at 14:30

2.1.6. - EMA/OD/0000105112

Treatment of osteogenesis imperfecta

Action: For information

Note: Withdrawal request received on 31 January 2023

2.1.7. - EMA/OD/0000114282

Treatment of *RPE65* retinopathies

Action: For adoption, Oral explanation to be held on 15 February 2023 at 12:00

2.1.8. - EMA/OD/0000112018

Treatment of spinal muscular atrophy

Action: For information

Note: Withdrawal request received on 30 January 2023

2.1.9. - EMA/OD/0000114439

Treatment of primary sclerosing cholangitis

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

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000093062

Treatment of spinal cord injury

Action: For discussion/adoption

2.2.2. - EMA/OD/0000104687

Treatment of mucopolysaccharidosis type II (Hunter syndrome)

Action: For discussion/adoption

2.2.3. - EMA/OD/0000105611

Treatment of vanishing white matter

Action: For discussion/adoption

2.2.4. - EMA/OD/0000112208

Treatment of mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes

Action: For discussion/adoption

2.2.5. - EMA/OD/0000115658

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.6. - EMA/OD/0000115720

Treatment of hereditary haemorrhagic telangiectasia

Action: For discussion/adoption

2.2.7. - EMA/OD/0000116218

Treatment of gastrointestinal stromal tumours

Action: For discussion/adoption

2.2.8. - EMA/OD/0000117508

Treatment of berylliosis (chronic beryllium disease)

Action: For discussion/adoption

2.2.9. - EMA/OD/0000118779

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000119068

Treatment of CDKL5 deficiency disorder

Action: For discussion/adoption

2.2.11. - EMA/OD/0000120211

Treatment of primary CTLA-4 checkpoint related immunodeficiencies

Action: For discussion/adoption

2.2.12. - EMA/OD/0000120404

Treatment of Fragile X Syndrome

Action: For discussion/adoption

2.2.13. - EMA/OD/0000120634

Treatment of otoferlin gene (hOTOF)-mediated hearing loss

Action: For discussion/adoption

2.2.14. - EMA/OD/0000109485

Treatment of gastrointestinal stromal tumours

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 14-16 February 2023 COMP meeting

2.7. Evaluation on-going

9 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 multiple myeloma

Action: For discussion/adoption

3.1.2. -

Treatment of graft-versus-host disease

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. – pegunigalsidase alfa - EMEA/H/C/005618, EU/3/17/1953, EMA/OD/0000109504

Chiesi Farmaceutici S.p.A.; Treatment of Fabry disease

Action: For discussion/adoption

4.2.2. – ivosidenib - EMEA/H/C/005936

Les Laboratoires Servier

a) Treatment of biliary tract cancer, EU/3/18/1994, EMA/OD/0000115500

Action: For discussion/adoption

b) Treatment of acute myeloid leukaemia, EU/3/16/1802, EMA/OD/0000115491

Action: For discussion/adoption

4.2.3. – ivosidenib - EMEA/H/C/006174, EU/3/16/1802, EMA/OD/0000117514

Les Laboratoires Servier; Treatment of acute myeloid leukaemia

Action: For discussion/adoption

4.2.4. – sirolimus - EMEA/H/C/005896/0000, EU/3/17/1910, EMA/OD/0000108887

Plusultra pharma GmbH; Treatment of tuberous sclerosis

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

Blueprint Medicines; Treatment of mastocytosis

CHMP Rapporteur: Blanca Garcia-Ochoa

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 10 February 2023 at 15:15

7.1.5. COMP Decisions Database

Action: For discussion

Document tabled:

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes January 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

Document(s) tabled:

Draft Agenda PCWP-HCPWP meeting – 3 March 2023

Meeting Summary PCWP-HCPWP - 15 November 2022

7.3.2. Upcoming ITF meetings

Action: For discussion

Overview of ITF activities 2022

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 EMA sponsored RWE study for SMA

Action: For discussion

8.2. Revision of the PAWG COMP answer template

Action: For discussion

8.3. European Specialised Expert Community (ESEC)

Action: For discussion

8.4. Real World Evidence update, including DARWIN EU®

Action: For discussion

8.5. Committee representatives at SAWP: re-nomination

Action: For discussion

8.6. Outcome measures in Epidermolysis Bullosa

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/