30 September 2022
EMA/COMP/762286/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 04-06 October 2022

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

04 October 2022, 08:30-19:30, virtual meeting

05 October 2022, 08:30-19:30, virtual meeting

06 October 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 04-06 October 2022. See (current) October 2022 COMP minutes (to be published post November 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 04-06 October 2022.

1.3. Adoption of the minutes

COMP minutes for 06-08 September 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000091801

Treatment of tuberculosis

Action: For adoption

2.1.2. - EMA/OD/0000096114

Diagnosis of AL amyloidosis

Action: For adoption, Oral explanation to be held on 04 October 2022 at 09:30

2.1.3. - EMA/OD/0000096494

Treatment of multiple system atrophy

Action: For information

Note: Withdrawal request received on 12 September 2022

2.1.4. - EMA/OD/0000097127

Treatment of 22q11.2 deletion syndrome (22qDS)

Action: For adoption, Oral explanation to be held on 05 October 2022 at 11:30

2.1.5. - EMA/OD/0000096942

Treatment of West syndrome

Action: For adoption, Oral explanation to be held on 05 October 2022 at 09:00

2.1.6. - EMA/OD/0000096322

Treatment of familial Cerebral Cavernous Malformation (fCCM)

Action: For adoption, Oral explanation to be held on 04 October 2022 at 15:30

2.1.7. - EMA/OD/0000096338

Treatment of familial Cerebral Cavernous Malformation (fCCM)

Action: For adoption, Oral explanation to be held on 04 October 2022 at 15:30

2.1.8. - EMA/OD/0000096917

Treatment of primary immune complex membranoproliferative glomerulonephritis (IC-MPGN)

Action: For adoption, Oral explanation to be held on 05 October 2022 at 14:00

2.1.9. - EMA/OD/0000088236

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 05 October 2022 at 15:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000082375

Treatment of ovarian cancer

Action: For discussion/adoption

2.2.2. - EMA/OD/0000091248

Treatment of haemophilia B

Action: For discussion/adoption

2.2.3. - EMA/OD/0000095176

Treatment of Menkes disease

Action: For discussion/adoption

2.2.4. - EMA/OD/0000096261

Treatment of progressive supranuclear palsy

Action: For discussion/adoption

2.2.5. - EMA/OD/0000096686

Diagnosis of ATTR amyloidosis

Action: For discussion/adoption

2.2.6. - EMA/OD/0000096688

Treatment of autosomal dominant polycystic kidney disease (ADPKD)

Action: For discussion/adoption

2.2.7. - EMA/OD/0000096385

Treatment of spinocerebellar ataxia

Action: For discussion/adoption

2.2.8. - EMA/OD/0000098317

Treatment of adrenoleukodystrophy

Action: For discussion/adoption

2.2.9. - EMA/OD/0000098623

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For discussion/adoption

2.2.10. - EMA/OD/0000098673

Treatment of adrenoleukodystrophy

Action: For discussion/adoption

2.2.11. - EMA/OD/0000099049

Treatment of cholangiocarcinoma

Action: For discussion/adoption

2.2.12. - EMA/OD/0000099136

Treatment of perinatal asphyxia

Action: For discussion/adoption

2.2.13. - EMA/OD/0000099342

Treatment of hereditary angioedema

Action: For discussion/adoption

2.2.14. - EMA/OD/0000099427

Treatment of small cell lung cancer

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

2.5.1. - EMA/OD/0000103671

Treatment of retinopathy of prematurity

Action: For adoption, Oral explanation to be held on 04 October 2022 at 10:45

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 04-06 October 2022 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 in solid organ transplantation

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. – loncastuximab tesirine - EMEA/H/C/005685, EU/3/21/2481, EMA/OD/0000094879

FGK Representative Service GmbH; Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 04 October 2022 at 12:15

4.1.2. - octreotide acetate - EMEA/H/C/005826/0000, EU/3/13/1170, EMA/OD/0000086000

Amryt Pharmaceuticals Designated Activity Company; Treatment of acromegaly

Action: For adoption, Oral explanation to be held on 04 October 2022 at 14:15

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

4.2.1. – maralixibat - EMEA/H/C/005857, EU/3/13/1214, EMA/OD/0000078931

Mirum Pharmaceuticals International B.V.; Treatment of Alagille syndrome

Action: For information

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 information

4.2.3. – tabelecleucel - EMEA/H/C/004577, EU/3/16/1627, EMA/OD/0000076907

Atara Biotherapeutics Ireland Limited; Treatment of post-transplant lymphoproliferative disorder

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

Feedback from the COMP SRLM under the Czech Presidency of the Council of the EU held F-2-F on 21-23 September 2022 in Bonn, Germany.

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 3rd October 2022 at 13:30

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 September 2022

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP)

Action: For information

Document(s) tabled:

7.3.2. Working Party with Healthcare Professionals' Organisations (HCPWP)

Action: For information

Document(s) tabled:

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)

FDA and its implementation of the US Federal Act for Amyotrophic Lateral Sclerosis.

FDA and recent developments in the US regarding Diffuse large B-cell lymphoma (DLBCL) prevalence.

Action: For discussion

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. Real World Evidence update

Action: For discussion

8.2. Follow up on expert consultation group on IRD

Action: For discussion

8.3. Innovative therapies project

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/