1 September 2022
EMA/COMP/653513/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 06-08 September 2022

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

06 September 2022, 09:00-19:30, room 1A / virtual meeting

07 September 2022, 08:30-19:30, room 1A / virtual meeting

08 September 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).

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 06-08 September 2022. See September 2022 COMP minutes (to be published post October 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 06-08 September 2022.

1.3. Adoption of the minutes

COMP minutes for 12-14 July 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000093474

Treatment of Werner's syndrome

Action: For adoption, Oral explanation to be held on 06 September 2022 at 17:00

2.1.2. - EMA/OD/0000092639

Treatment of linear IgA bullous dermatosis

Action: For adoption, Oral explanation to be held on 06 September 2022 at 09:30

2.1.3. - EMA/OD/0000085970

Treatment of microvillous inclusion disease

Action: For adoption, Oral explanation to be held on 06 September 2022 at 14:15

2.1.4. - EMA/OD/0000084535

Treatment of Duchenne muscular dystrophy

Action: For information

Note: Withdrawal request received on 16 August 2022.

2.1.5. - EMA/OD/0000092197

Treatment of pneumonia due to *Pseudomonas aeruginosa*

Action: For information

Note: Withdrawal request received on 18 August 2022.

2.1.6. - EMA/OD/0000090156

Treatment of apolipoprotein L1-mediated kidney disease (AMKD)

Action: For adoption, Oral explanation to be held on 06 September 2022 at 15:30

2.1.7. - EMA/OD/0000090261

Treatment of Duchenne muscular dystrophy (DMD)

Action: For information

Note: Withdrawal request received on 01 August 2022.

2.1.8. - EMA/OD/0000083978

Treatment of Type 1 diabetes mellitus in individuals positive for GAD65 antibody and carrying the genetic human leukocyte antigen (HLA) DR3-DQ2 haplotype

Action: For adoption, Oral explanation to be held on 07 September 2022 at 10:30

2.1.9. - EMA/OD/0000083743

Treatment of focal cortical dysplasia

Action: For adoption, Oral explanation to be held on 07 September 2022 at 12:00

2.1.10. - EMA/OD/0000092484

Treatment of congenital ichthyosis

Action: For adoption, Oral explanation to be held on 07 September 2022 at 14:00

2.1.11. - EMA/OD/0000083190

Treatment of Type 1 diabetes with residual β-cell function defined by stimulated C-peptide levels ranging between 0.2 and 0.6 nmol/L

Action: For adoption, Oral explanation to be held on 07 September 2022 at 15:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000082252

Treatment of amyotrophic lateral sclerosis (ALS)

Action: For discussion/adoption

2.2.2. - EMA/OD/0000086488

Treatment of acute myeloid leukemia

Action: For discussion/adoption

2.2.3. - EMA/OD/0000088236

Treatment of ovarian cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000089323

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.5. - EMA/OD/0000089368

Treatment of chronic intestinal pseudo-obstruction

Action: For discussion/adoption

2.2.6. - EMA/OD/0000091780

Treatment of otoferlin gene-mediated hearing loss

Action: For discussion/adoption

2.2.7. - EMA/OD/0000091801

Treatment of tuberculosis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000092213

Treatment of glioma

Action: For discussion/adoption

2.2.9. - EMA/OD/0000092280

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000093040

Treatment of pseudoxanthoma elasticum

Action: For discussion/adoption

2.2.11. - EMA/OD/0000095828

Treatment of vanishing white matter disease

Action: For discussion/adoption

2.2.12. - EMA/OD/0000095946

Treatment of myelofibrosis

Action: For discussion/adoption

2.2.13. - EMA/OD/0000096114

Diagnosis of AL amyloidosis

Action: For discussion/adoption

2.2.14. - EMA/OD/0000096220

Treatment of Rett syndrome

Action: For discussion/adoption

2.2.15. - EMA/OD/0000096314

Treatment of Guillain-Barré syndrome

Action: For discussion/adoption

2.2.16. - EMA/OD/0000096322

Treatment of familial cerebral cavernous malformation

Action: For discussion/adoption

2.2.17. - EMA/OD/0000096338

Treatment of familial cerebral cavernous malformation

Action: For discussion/adoption

2.2.18. - EMA/OD/0000096494

Treatment of multiple system atrophy

Action: For discussion/adoption

2.2.19. - EMA/OD/0000096637

Treatment of GM1 gangliosidosis

Action: For discussion/adoption

2.2.20. - EMA/OD/0000096917

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

Action: For discussion/adoption

2.2.21. - EMA/OD/0000096942

Treatment of West syndrome

Action: For discussion/adoption

2.2.22. - EMA/OD/0000096995

Treatment of essential thrombocythaemia

Action: For discussion/adoption

2.2.23. - EMA/OD/0000097127

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

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/0000099365

Prevention of spaceflight-related radiation and microgravity

Action: For information

Note: Withdrawal request received on 30 August 2022.

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 06-08 September 2022 COMP meeting

2.7. Evaluation on-going

14 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 congenital alpha-1 antitrypsin deficiency

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. - maribavir - EMEA/H/C/005787/0000

Takeda Pharmaceuticals International AG Ireland Branch

a) Prevention of cytomegalovirus (CMV) disease in patients with impaired cell mediated immunity deemed at risk, EU/3/07/519, EMA/OD/0000091090

Action: For discussion/adoption

b) Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity, EU/3/13/1133, EMA/OD/0000091101

Action: For discussion/adoption

4.2.2. – mitapivat - EMEA/H/C/005540, EU/3/20/2270, EMA/OD/0000068458

Agios Netherlands B.V.; Treatment of pyruvate kinase deficiency

Action: For information

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

Amryt Pharmaceuticals Designated Activity Company; Treatment of acromegaly

Action: For discussion/adoption

4.2.4. – sutimlimab - EMEA/H/C/005776, EU/3/16/1609, EMA/OD/0000082097

Genzyme Europe BV; Treatment of autoimmune haemolytic anaemia

Action: For discussion/adoption

4.2.5. – 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 discussion/adoption

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

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

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. Yescarta - axicabtagene ciloleucel - EMEA/H/C/004480/II/0046, EU/3/14/1393, EMA/OD/0000076832

Kite Pharma EU B.V.; Treatment of diffuse large B cell lymphoma

Action: For discussion/adoption

5.2.2. Iclusig - ponatinib - EMEA/H/C/002695/II/0064, EU/3/09/715,

Incyte Biosciences Distribution B.V.; Treatment of acute lymphoblastic leukaemia

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

COMP SRLM under the Czech Presidency of the Council of the EU to be 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 6th September 2022 at 13:15

Document tabled:

PAWG draft agenda for 6th September 2022 meeting

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:

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)

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. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q3/2022 Update of the Business Pipeline report for the human scientific committees

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/