12 July 2022
EMA/COMP/602828/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 12-14 July 2022

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

12 July 2022, 08:30-19:30, remote virtual meeting

13 July 2022, 08:30-19:30, remote virtual meeting

14 July 2022, 08:30-17:00, remote 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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Table of contents

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 12-14 July 2022. See July 2022 COMP minutes (to be published post September 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 12-14 July 2022.

1.3. Adoption of the minutes

COMP minutes for 14-16 June 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000085890

Treatment of idiopathic hypersomnia

Action: For adoption

2.1.2. - EMA/OD/0000085805

Treatment of mastocytosis

Action: For information

Note: Withdrawal request received on 29 June 2022.

2.1.3. - EMA/OD/0000086580

Treatment of graft-versus-host-disease

Action: For adoption, Oral explanation to be held on 12 July 2022 at 11:00

2.1.4. - EMA/OD/0000077279

Treatment of retinopathy of prematurity (ROP)

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

2.1.5. - EMA/OD/0000085640

Treatment of Covid-19 and Dengue co-infection

Action: For information

Note: Withdrawal request received on 24 June 2022.

2.1.6. - EMA/OD/0000073417

Treatment of CTLA-4 haploinsufficiency with autoimmune infiltration disease

Action: For adoption, Oral explanation to be held on 13 July 2022 at 09:00

2.1.7. - EMA/OD/0000086052

Treatment of Alstrom syndrome

Action: For adoption, Oral explanation to be held on 13 July 2022 at 17:00

2.1.8. - EMA/OD/0000086055

Treatment of Bardet-Biedl syndrome

Action: For adoption, Oral explanation to be held on 13 July 2022 at 17:00

2.1.9. - EMA/OD/0000076480

Treatment of myasthenia gravis

Action: For information

Note: Withdrawal request received on 28 June 2022.

2.1.10. - EMA/OD/0000086562

Prevention of acute liver failure

Action: For adoption, Oral explanation to be held on 12 July 2022 at 14:15

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000072597

Treatment of mucopolysaccharidosis Type IV A, Morquio A syndrome

Action: For discussion/adoption

2.2.2. - EMA/OD/0000076094

Treatment of osteosarcoma

Action: For discussion/adoption

2.2.3. - EMA/OD/0000081808

Treatment of peripheral T-cell lymphoma

Action: For discussion/adoption

2.2.4. - 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 discussion/adoption

2.2.5. - EMA/OD/0000083743

Treatment of focal cortical dysplasia

Action: For discussion/adoption

2.2.6. - 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 discussion/adoption

2.2.7. - EMA/OD/0000084294

Treatment of familial adenomatous polyposis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000084535

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.9. - EMA/OD/0000085853

Treatment of primary sclerosing cholangitis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000085970

Treatment of microvillous inclusion disease

Action: For discussion/adoption

2.2.11. - EMA/OD/0000086532

Treatment of haemophilia A

Action: For discussion/adoption

2.2.12. - EMA/OD/0000086550

Treatment of neuroblastoma

Action: For information

Note: Withdrawal request received on 5 July 2022.

2.2.13. - EMA/OD/0000089012

Treatment of nontuberculous mycobacterial lung disease

Action: For discussion/adoption

2.2.14. - EMA/OD/0000090156

Treatment of apolipoprotein L1-mediated kidney disease (AMKD)

Action: For discussion/adoption

2.2.15. - EMA/OD/0000090261

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.16. - EMA/OD/0000090545

Treatment of frontotemporal dementia

Action: For discussion/adoption

2.2.17. - EMA/OD/0000091543

Treatment of Stargardt's disease

Action: For discussion/adoption

2.2.18. - EMA/OD/0000091771

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.19. - EMA/OD/0000091878

Treatment of myasthenia gravis

Action: For discussion/adoption

2.2.20. - EMA/OD/0000091899

Treatment of citrullinemia type 1

Action: For discussion/adoption

2.2.21. - EMA/OD/0000092111

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.22. - EMA/OD/0000092197

Treatment of pneumonia due to *Pseudomonas aeruginosa*

Action: For discussion/adoption

2.2.23. - EMA/OD/0000092484

Treatment of congenital ichthyosis

Action: For discussion/adoption

2.2.24. - EMA/OD/0000092639

Treatment of linear IgA bullous dermatosis

Action: For discussion/adoption

2.2.25. - EMA/OD/0000093202

Treatment of non-tuberculous mycobacterial lung disease

Action: For discussion/adoption

2.2.26. - EMA/OD/0000093305

Treatment of myelodysplastic syndrome (MDS)

Action: For discussion/adoption

2.2.27. - EMA/OD/0000093471

Treatment of Hutchinson-Gilford Progeria Syndrome

Action: For discussion/adoption

2.2.28. - EMA/OD/0000093474

Treatment of Werner's syndrome

Action: For discussion/adoption

2.2.29. - EMA/OD/0000093683

Treatment of chondrosarcoma

Action: For discussion/adoption

2.2.30. - EMA/OD/0000093873

Treatment of cryptococcosis

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 12-14 July 2022 COMP meeting

2.7. Evaluation on-going

0 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

None

3.2. Finalised letters

3.2.1. -

Treatment of pancreatic cancer

Action: For information

3.2.2. -

Treatment of mucopolysaccharidosis II (Hunter's syndrome)

Action: For information

3.2.3. -

Treatment of acute myeloid leukaemia

Action: For information

3.2.4. -

Treatment of myelofibrosis

Action: For information

3.3. New requests

3.3.1. - EMA/SA/0000086685

Treatment of congenital alpha-1 antitrypsin deficiency

Action: For information

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. Roctavian - valoctocogene roxaparvovec - EMEA/H/C/005830/0000, EU/3/16/1622, EMA/OD/0000067127

Biomarin International Limited; Treatment of haemophilia A

Action: For adoption, Oral explanation to be held on 13 July 2022 at 14:00

4.1.2. Scemblix - asciminib - EMEA/H/C/005605/0000, EU/3/20/2261, EMA/OD/0000068920

Novartis Europharm Limited; Treatment of chronic myeloid leukaemia

Action: For adoption, Oral explanation to be held on 13 July 2022 at 10:30

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

4.2.1. - vutrisiran - EMEA/H/C/005852/0000, EU/3/18/2026, EMA/OD/0000085855

Alnylam Netherlands B.V.; Treatment of transthyretin-mediated amyloidosis

Action: For adoption, Oral explanation to be held on 12 July 2022 at 15:30

4.2.2. - teclistamab - EMEA/H/C/005865/0000, EU/3/20/2331, EMA/OD/0000083072

Accelerated assessment

Janssen-Cilag International; Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 12 July 2022 at 17:00

4.2.3. – fosdenopterin - EMEA/H/C/005378/0000, EU/3/10/777, EMA/OD/0000074822

Comharsa Life Sciences Ltd; Treatment of molybdenum cofactor deficiency type A

Action: For adoption

4.2.4. Imcivree - setmelanotide - EMEA/H/C/005089/II/0002/G, EU/3/19/2192, EMA/OD/0000074865

Rhythm Pharmaceuticals Netherlands B.V.; Treatment of Bardet Biedl syndrome

Action: For adoption

4.2.5. - mitapivat sulfate - EMEA/H/C/005540/0000, EU/3/20/2270, EMA/OD/0000068458

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

Action: For information

4.2.6. Tecartus - brexucabtagene autoleucel - EMEA/H/C/005102/II/0008/G, EU/3/20/2344, EMA/OD/0000063560

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia

Action: For 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. Epidyolex – cannabidiol - EMEA/H/C/004675/II/0020

GW Pharma (International) B.V.

a) Treatment of Dravet syndrome, EMA/OD/0000097923, EU/3/14/1339

Action: For discussion

b) Treatment of Lennox-Gastaut syndrome, EMA/OD/0000098337, EU/3/17/1855
Action: For discussion

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

Draft agenda of the 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 discussion

7.1.4. Protocol Assistance Working Group (PAWG)

None

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

None

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

Documents tabled**:** Draft Agenda of September 2022 meeting and Minutes of June 2022 meeting

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. Real World Evidence update, including DARWIN EU®

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/