8 June 2022
EMA/COMP/577731/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 14-16 June 2022

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

14 June 2022, 09:00-19:00, room 1D / virtual meeting

15 June 2022, 08:30-19:30, room 1D / virtual meeting

16 June 2022, 08:30-17:30, room 1D / 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
An agency of the European Union

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 14-16 June 2022. See June 2022 COMP minutes (to be published post July 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 14-16 June 2022.

1.3. Adoption of the minutes

COMP minutes for 10-12 May 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000073118

Treatment of perinatal asphyxia

Action: For adoption, Oral explanation to be held on 14 June 2022 at 09:30

2.1.2. - EMA/OD/0000076399

Treatment of fracture nonunion

Action: For adoption, Oral explanation to be held on 14 June 2022 at 11:00

2.1.3. - EMA/OD/0000083166

Treatment of neurofibromatosis type 2

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

2.1.4. - EMA/OD/0000083331

Treatment of Lambert-Eaton myasthenia syndrome (LEMS)

Action: For information

Note: Withdrawal request received on 18 May 2022.

2.1.5. - EMA/OD/0000084283

Treatment of pulmonary arterial hypertension

Action: For adoption, Oral explanation to be held on 14 June 2022 at 15:45

2.1.6. - EMA/OD/0000077315

Treatment of type 1 diabetes in DQ8 positive patients with residual beta cell function

Action: For adoption, Oral explanation to be held on 14 June 2022 at 17:00

2.1.7. - EMA/OD/0000083873

Treatment of choroideremia

Action: For information

Note: Withdrawal request received on 26 May 2022.

2.1.8. - EMA/OD/0000083791

Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 15 June 2022 at 09:00

2.1.9. - EMA/OD/0000079201

Treatment of non-infectious intermediate, posterior and chronic anterior uveitis

Action: For adoption, Oral explanation to be held on 15 June 2022 at 10:30

2.1.10. - EMA/OD/0000079978

Treatment of malignant mesothelioma

Action: For adoption, Oral explanation to be held on 15 June 2022 at 12:00

2.1.11. - EMA/OD/0000080896

Treatment of Prader-Willi syndrome

Action: For information

Note: Withdrawal request received on 20 May 2022.

2.1.12. - EMA/OD/0000083574

Treatment of West syndrome

Action: For information

Note: Withdrawal request received on 24 May 2022.

2.1.13. - EMA/OD/0000083982

Treatment of cutaneous T-cell lymphoma

Action: For adoption, Oral explanation to be held on 14 June 2022 at 14:30

2.1.14. - EMA/OD/0000083254

Prevention of graft rejection following solid organ transplantation

Action: For adoption, Oral explanation to be held on 15 June 2022 at 14:30

2.1.15. - EMA/OD/0000075761

Treatment of mantle cell lymphoma

Action: For adoption, Oral explanation to be held on 15 June 2022 at 16:30

2.1.16. - EMA/OD/0000080688

Treatment of Brugada syndrome

Action: For adoption, Oral explanation to be held on 16 June 2022 at 09:00

2.1.17. - EMA/OD/0000083246

Treatment of pulmonary arterial hypertension

Action: For adoption, Oral explanation to be held on 16 June 2022 at 10:30

2.1.18. - EMA/OD/0000082229

Treatment of GM1 gangliosidosis

Action: For information

Note: Withdrawal request received on 17 May 2022.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000021158

Treatment of autoimmune haemolytic anaemia

Action: For discussion/adoption

2.2.2. - EMA/OD/0000073417

Treatment of CTLA-4 haploinsufficiency with autoimmune infiltration disease

Action: For discussion/adoption

2.2.3. - EMA/OD/0000076464

Treatment of invasive candidiasis

Action: For discussion/adoption

2.2.4. - EMA/OD/0000076480

Treatment of myasthenia gravis

Action: For discussion/adoption

2.2.5. - EMA/OD/0000076916

Treatment of invasive aspergillosis

Action: For discussion/adoption

2.2.6. - EMA/OD/0000077279

Treatment of retinopathy of prematurity

Action: For discussion/adoption

2.2.7. - EMA/OD/0000077782

Treatment of ornithine transcarbamylase deficiency

Action: For discussion/adoption

2.2.8. - EMA/OD/0000078610

Treatment in solid organ transplantation

Action: For discussion/adoption

2.2.9. - EMA/OD/0000080170

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.10. - EMA/OD/0000080529

Treatment of myasthenia gravis

Action: For discussion/adoption

2.2.11. - EMA/OD/0000081208

Treatment of multiple system atrophy

Action: For discussion/adoption

2.2.12. - EMA/OD/0000082060

Treatment of pemphigus

Action: For discussion/adoption

2.2.13. - EMA/OD/0000082579

Treatment of fibrodysplasia ossificans progressiva

Action: For discussion/adoption

2.2.14. - EMA/OD/0000082615

Treatment of small-cell lung cancer

Action: For discussion/adoption

2.2.15. - EMA/OD/0000083083

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.16. - EMA/OD/0000083733

Treatment of autoimmune haemolytic anaemia

Action: For discussion/adoption

2.2.17. - EMA/OD/0000085141

Treatment of glioma

Action: For discussion/adoption

2.2.18. - EMA/OD/0000085459

Treatment of glioma

Action: For discussion/adoption

2.2.19. - EMA/OD/0000085590

Treatment of pyoderma gangrenosum

Action: For discussion/adoption

2.2.20. - EMA/OD/0000085640

Treatment of Covid-19 and dengue co-infection

Action: For discussion/adoption

2.2.21. - EMA/OD/0000085676

Treatment of maple syrup urine disease

Action: For discussion/adoption

2.2.22. - EMA/OD/0000085783

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.23. - EMA/OD/0000085805

Treatment of mastocytosis

Action: For discussion/adoption

2.2.24. - EMA/OD/0000085890

Treatment of idiopathic hypersomnia

Action: For discussion/adoption

2.2.25. - EMA/OD/0000085913

Treatment of PLPHP deficiency

Action: For discussion/adoption

2.2.26. - EMA/OD/0000086046

Treatment of non-traumatic spontaneous acute intracerebral haemorrhage

Action: For discussion/adoption

2.2.27. - EMA/OD/0000086049

Treatment of Wolfram syndrome

Action: For discussion/adoption

2.2.28. - EMA/OD/0000086052

Treatment of Alstrom syndrome

Action: For discussion/adoption

2.2.29. - EMA/OD/0000086055

Treatment of Bardet-Biedl syndrome

Action: For discussion/adoption

2.2.30. - EMA/OD/0000086361

Treatment of pachyonychia congenita

Action: For discussion/adoption

2.2.31. - EMA/OD/0000086527

Treatment of nasopharyngeal cancer

Action: For discussion/adoption

2.2.32. - EMA/OD/0000086562

Prevention of acute liver failure

Action: For discussion/adoption

2.2.33. - EMA/OD/0000086580

Treatment of graft-versus-host-disease

Action: For discussion/adoption

2.2.34. - EMA/OD/0000086748

Treatment of familial adenomatous polyposis

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 June 2022 COMP meeting

2.7. Evaluation on-going

13 applications for orphan designation will not be discussed as evaluation is ongoing.

Action: For information

Notes:
See 7.8.1. Table

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of pancreatic cancer

Action: For adoption

3.1.2. -

Treatment of mucopolysaccharidosis II (Hunter's syndrome)

Action: For adoption

3.1.3. -

Treatment of acute myeloid leukaemia

Action: For adoption

3.1.4. -

Treatment of myelofibrosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of multiple myeloma

Action: For information

3.2.2. -

Treatment of primary biliary cholangitis

Action: For information

3.2.3. -

Treatment of myelodysplastic syndromes

Action: For information

3.3. New requests

None

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

Accelerated assessment

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

Action: For adoption

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

BioMarin International Limited; Treatment of haemophilia A

Action: For discussion/adoption

4.2.3. - efgartigimod alfa - EMEA/H/C/005849/0000, EU/3/18/1992, EMA/OD/0000083237

Argenx; Treatment of myasthenia gravis

Action: For discussion/adoption

4.2.4. Crysvita - burosumab - EMEA/H/C/004275/II/0023, EU/3/18/2011, EMA/OD/0000051257

Kyowa Kirin Holdings B.V.; Treatment of phosphaturic mesenchymal tumour

CHMP Rapporteur: Kristina Dunder; CHMP Co-Rapporteur: Jayne Crowe**Action:** For discussion/adoption

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

Novartis Europharm Limited; Treatment of chronic myeloid leukaemia

Action: For discussion/adoption

4.2.6. - maribavir - EMEA/H/C/005787/0000, EU/3/13/1133, EMA/OD/0000091101

Takeda Pharmaceuticals International AG Ireland Branch; Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity

Action: For discussion/adoption

4.2.7. - melphalan flufenamide - EMEA/H/C/005681/0000, EU/3/15/1463, EMA/OD/0000063986

Oncopeptides AB; Treatment of plasma cell myeloma

Action: For discussion/adoption

4.2.8. 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

CHMP Rapporteur: Jan Mueller-Berghaus**Action:** For discussion/adoption

4.2.9. - synthetic double-stranded siRNA oligonucleotide targeted against transthyretin mRNA, with six phosphorothioate linkages in the backbone, and nine 2'-fluoro and thirty-five 2'-O-methyl nucleoside residues in the sequence, which is covalently linked via a phosphodiester group to a ligand containing three N-acetylgalactosamine residues - EMEA/H/C/005852/0000, EU/3/18/2026, EMA/OD/0000085855

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

Action: For discussion/adoption

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

Accelerated assessment

Janssen-Cilag International; Treatment of multiple myeloma

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

None

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 10 June 2022 at 14:00

Document tabled:

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 May 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

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

Action: For information

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:
Q2/2022 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/