10 May 2022
EMA/COMP/262112/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 10-12 May 2022

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

10 May 2022, 08:30-19:30, remote virtual meeting

11 May 2022, 08:30-19:30, remote virtual meeting

12 May 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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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 10-12 May 2022. See May 2022 COMP minutes (to be published post June 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 10-12 May 2022.

1.3. Adoption of the minutes

COMP minutes for 11-13 April 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000077417

Treatment of tubular aggregate myopathies (including York platelet syndrome and Stormorken syndrome)

Action: For adoption, Oral explanation to be held on 10 May 2022 at 09:15

2.1.2. - EMA/OD/0000080709

Prevention of retinopathy of prematurity

Action: For adoption, Oral explanation to be held on 11 May 2022 at 14:30

2.1.3. - EMA/OD/0000077171

Treatment of chronic myeloid leukemia

Action: For adoption, Oral explanation to be held on 10 May 2022 at 12:00

2.1.4. - EMA/OD/0000076247

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 11 May 2022 at 10:30

2.1.5. - EMA/OD/0000080466

Prevention of risk of graft failure following allogenic hematopoietic stem cell transplantation

Action: For information

Note: Withdrawal request received on 27 April 2022.

2.1.6. - EMA/OD/0000080468

Treatment of chromosome 15q11.2-13.1 duplication syndrome (dup15q)

Action: For adoption, Oral explanation to be held on 11 May 2022 at 16:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000068847

Treatment of phenylketonuria

Action: For discussion/adoption

2.2.2. - EMA/OD/0000073118

Treatment of perinatal asphyxia

Action: For discussion/adoption

2.2.3. - EMA/OD/0000075685

Treatment of osteogenesis imperfecta

Action: For discussion/adoption

2.2.4. - EMA/OD/0000075740

Treatment of primary IgA nephropathy

Action: For discussion/adoption

2.2.5. - EMA/OD/0000075761

Treatment of mantle cell lymphoma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000076332

Treatment of hereditary angioedema

Action: For discussion/adoption

2.2.7. - EMA/OD/0000076399

Treatment of fracture nonunion

Action: For discussion/adoption

2.2.8. - EMA/OD/0000076571

Treatment of erythromelalgia

Action: For discussion/adoption

2.2.9. - EMA/OD/0000077315

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

Action: For discussion/adoption

2.2.10. - EMA/OD/0000079201

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

Action: For discussion/adoption

2.2.11. - EMA/OD/0000079978

Treatment of malignant mesothelioma

Action: For discussion/adoption

2.2.12. - EMA/OD/0000080409

Treatment of SCN8A developmental and epileptic encephalopathy (SCN8A-DEE)

Action: For discussion/adoption

2.2.13. - EMA/OD/0000080460

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.14. - EMA/OD/0000080688

Treatment of Brugada syndrome

Action: For discussion/adoption

2.2.15. - EMA/OD/0000080823

Treatment of TBR1-related disorder

Action: For discussion/adoption

2.2.16. - EMA/OD/0000080896

Treatment of Prader-Willi Syndrome

Action: For discussion/adoption

2.2.17. - EMA/OD/0000081767

Treatment of lymphatic malformations

Action: For discussion/adoption

2.2.18. - EMA/OD/0000082229

Treatment of GM1 gangliosidosis

Action: For discussion/adoption

2.2.19. - EMA/OD/0000082687

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.20. - EMA/OD/0000082957

Treatment of neuronal ceroid lipofuscinosis (NCLs)

Action: For discussion/adoption

2.2.21. - EMA/OD/0000083166

Treatment of neurofibromatosis type 2

Action: For discussion/adoption

2.2.22. - EMA/OD/0000083246

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

2.2.23. - EMA/OD/0000083254

Prevention of graft rejection following solid organ transplantation

Action: For discussion/adoption

2.2.24. - EMA/OD/0000083331

Treatment of Lambert-Eaton myasthenia syndrome (LEMS)

Action: For discussion/adoption

2.2.25. - EMA/OD/0000083574

Treatment of West syndrome

Action: For discussion/adoption

2.2.26. - EMA/OD/0000083607

Treatment of Angelman syndrome

Action: For discussion/adoption

2.2.27. - EMA/OD/0000083615

Treatment of partial deep dermal and full thickness burns

Action: For discussion/adoption

2.2.28. - EMA/OD/0000083787

Treatment of methylmalonic acidemia

Action: For discussion/adoption

2.2.29. - EMA/OD/0000083789

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.30. - EMA/OD/0000083791

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.31. - EMA/OD/0000083873

Treatment of choroideremia

Action: For discussion/adoption

2.2.32. - EMA/OD/0000083967

Treatment of ATTR amyloidosis

Action: For discussion/adoption

2.2.33. - EMA/OD/0000083982

Treatment of cutaneous T-cell lymphoma

Action: For discussion/adoption

2.2.34. - EMA/OD/0000084241

Treatment of myelodysplastic syndromes

Action: For discussion/adoption

2.2.35. - EMA/OD/0000084283

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

2.2.36. - EMA/OD/0000084390

Treatment of galactosaemia

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 10-12 May 2022 COMP meeting

2.7. Evaluation on-going

34 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 multiple myeloma

Action: For adoption

3.1.2. -

Treatment of primary biliary cholangitis

Action: For adoption

3.1.3. -

Treatment of myelodysplastic syndromes

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of mucopolysaccharidosis type I

Action: For information

3.2.2. -

Treatment of multiple myeloma

Action: For information

3.3. New requests

3.3.1. -

Treatment of mucopolysaccharidosis II (Hunter's syndrome)

Action: For information

3.3.2. -

Treatment of acute myeloid leukaemia

Action: For information

3.3.3. -

Treatment of pancreatic cancer

Action: For information

3.3.4. -

Treatment of myelofibrosis

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. Yescarta - axicabtagene ciloleucel - EMEA/H/C/004480/II/0042, EU/3/15/1579, EMA/OD/0000068456

Kite Pharma EU B.V.; Treatment of follicular lymphoma

CHMP Rapporteur: Jan Mueller-Berghaus; CHMP Co-Rapporteur: Claire Beuneu**Action:** For adoption, oral explanation to be held on 10 May 2022 16:00

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

4.2.1. - eladocagene exuparvovec - EMEA/H/C/005352/0000, EU/3/16/1786, EMA/OD/0000024196

PTC Therapeutics International Limited; Treatment of aromatic L-amino acid decarboxylase deficiency

Action: For discussion/adoption

4.2.2. - budesonide - EMEA/H/C/005653/0000, EU/3/16/1778, EMA/OD/0000066260

Calliditas Therapeutics AB; Treatment of primary IgA nephropathy

Action: For discussion/adoption

4.2.3. - lonafarnib - EMEA/H/C/005271/0000, EU/3/18/2118, EMA/OD/0000067500

Eigerbio Europe Limited; Treatment of Hutchinson-Gilford Progeria Syndrome

Action: For discussion/adoption

4.2.4. - 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 discussion/adoption

4.2.5. - olipudase alfa - EMEA/H/C/004850/0000, EU/3/01/056, EMA/OD/0000072975

Accelerated assessment

Genzyme Europe B.V.; Treatment of Niemann-Pick disease

Action: For information

4.2.6. - 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 information

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/3/15/1553, EMA/OD/0000076832

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

CHMP Rapporteur: Jan Mueller-Berghaus; CHMP Co-Rapporteur: Claire Beuneu**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

Feedback from the joint COMP/PDCO meeting under the French Presidency of the Council of the EU held virtually on 31 March 2022

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 06 May 2022 10:00

Document tabled:

PAWP draft agenda for 06 May 2022 meeting

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

None

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

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

None

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

None

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Meeting concerning Nexviadyme

Action: For information

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. Discussion on RWD (update on studies of multiple myeloma)

Action: For information

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/