6 December 2021
EMA/COMP/642669/2021
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 07-09 December 2021

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

07 December 2021, 08:30-19:30, remote virtual meeting

08 December 2021, 08:30-19:30, remote virtual meeting

09 December 2021, 08:30-17:00, remote 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).

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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 7-9 December 2021. See (current) December 2021 COMP minutes (to be published post January 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 7-9 December 2021.

1.3. Adoption of the minutes

COMP minutes for 3-5 November 2021.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000064907

Treatment of chronic inflammatory demyelinating polyneuropathy

Action: For adoption, Oral explanation to be held on 08 December 2021 at 09:00

2.1.2. - EMA/OD/0000054314

Treatment of high-grade B-cell lymphoma

Action: For information

Notes: Withdrawal request received on 22 November 2021.

2.1.3. - EMA/OD/0000068582

Treatment of multiple myeloma

Action: For information

Notes: Withdrawal request received on 30 November 2021.

2.1.4. - EMA/OD/0000069661

Treatment of sarcoidosis

Action: For information

Notes: Withdrawal request received on 11 November 2021.

2.1.5. - EMA/OD/0000056828

Treatment of upper tract urothelial carcinoma

Action: For adoption, Oral explanation to be held on 07 December 2021 at 09:30

2.1.6. - EMA/OD/0000069185

Treatment of primary biliary cholangitis

Action: For adoption, Oral explanation to be held on 07 December 2021 at 11:15

2.1.7. - EMA/OD/0000047544

Treatment of generalised pustular psoriasis

Action: For adoption, Oral explanation to be held on 08 December 2021 at 13:45

2.1.8. - EMA/OD/0000067714

Treatment of isolated optic neuritis

Action: For adoption, Oral explanation to be held on 08 December 2021 at 17:15

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000065122

Treatment of Merkel cell carcinoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000065934

Prevention of retinopathy of prematurity

Action: For discussion/adoption

2.2.3. - EMA/OD/0000067342

Treatment of glycogen storage disease type 1a (GSD1a)

Action: For discussion/adoption

2.2.4. - EMA/OD/0000068027

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.5. - EMA/OD/0000068060

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000068755

Treatment of mucopolysaccharidosis Type IIIA, Sanfilippo syndrome

Action: For discussion/adoption

2.2.7. - EMA/OD/0000069392

Treatment of glioma

Action: For discussion/adoption

2.2.8. - EMA/OD/0000069751

Treatment of pancreatic cancers

Action: For discussion/adoption

2.2.9. - EMA/OD/0000070454

Treatment of amyotrophic lateral sclerosis (ALS)

Action: For discussion/adoption

2.2.10. - EMA/OD/0000070461

Treatment of invasive scopulariopsis

Action: For discussion/adoption

2.2.11. - EMA/OD/0000071191

Treatment of primary hyperoxaluria

Action: For discussion/adoption

2.2.12. - EMA/OD/0000071211

Treatment of *SCN2A* developmental and epileptic encephalopathy (SCN2A-DEE)

Action: For discussion/adoption

2.2.13. - EMA/OD/0000071268

Treatment of limb girdle muscular dystrophy (LGMD)

Action: For discussion/adoption

2.2.14. - EMA/OD/0000071311

Treatment of pre-eclampsia

Action: For discussion/adoption

2.2.15. - EMA/OD/0000071547

Prevention of bronchopulmonary dysplasia

Action: For discussion/adoption

2.2.16. - EMA/OD/0000071656

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.17. - EMA/OD/0000071679

Treatment of bronchiolitis obliterans syndrome (BOS)

Action: For discussion/adoption

2.2.18. - EMA/OD/0000071835

Treatment of Leber's hereditary optic neuropathy

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 07-09 December 2021 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 myasthenia gravis

Action: For adoption

3.1.2. -

Treatment of tuberous sclerosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of multiple myeloma

Action: For information

3.2.2. -

Treatment of multiple myeloma

Action: For information

3.2.3. -

Treatment of primary hyperoxaluria

Action: For information

3.3. New requests

3.3.1. -

Treatment of primary IgA nephropathy

Action: For information

3.3.2. -

Treatment of cystic fibrosis

Action: For information

3.3.3. -

Treatment of neuronal ceroid lipofuscinosis

Action: For information

3.3.4. -

Treatment of unresectable recurrent glioblastoma/gliosarcoma

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. Uplizna – inebilizumab - EMEA/H/C/005818/0000, EMA/OD/267/16, EU/3/17/1856, EMA/OD/0000055830

Viela Bio B.V.; Treatment of neuromyelitis optica spectrum disorders

Action: For adoption, Oral explanation to be held on 08 December 2021 at 15:30

4.1.2. Nexviadyme - avalglucosidase alfa - EMEA/H/C/005501/0000, EU/3/14/1251, EMA/OD/0000048959

Genzyme Europe B.V.; Treatment of Pompe's disease

Action: For adoption, Oral explanation to be held on 07 December 2021 at 15:00

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

4.2.1. - somatrogon - EMEA/H/C/005633/0000, EU/3/12/1087, EMA/OD/0000063709

Pfizer Europe MA EEIG; Treatment of growth hormone deficiency

Action: For discussion/adoption

4.2.2. - 2-hydroxy-6-((2-(1-isopropyl-1h-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde - EMEA/H/C/004869/0000, EU/3/16/1769, EMA/OD/0000074918

Global Blood Therapeutics Netherlands B.V.; Treatment of sickle cell disease

Action: For discussion/adoption

4.2.3. - tebentafusp - EMEA/H/C/004929/0000, EU/3/21/2397, EMA/OD/0000068646

Accelerated assessment

Immunocore Ireland Limited; Treatment of uveal melanoma

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. Polivy – polatuzumab vedotin – EMEA/H/C/004870/II/0012, EU/3/18/2013, EMA/OD/0000074173

Roche Registration GmbH, Treatment of diffuse large B-cell lymphoma

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 – joint COMP/PDCO, 19 November 2021, Lisbon, Portugal

Feedback from the meeting

Action: For discussion

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 3 December 2021 at 10:00

Document tabled:

PAWG draft agenda for 3 December 2021 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) and Working Party with Healthcare Professionals' Organisations (HCPWP)

Action: For information

Document(s) tabled: Meeting Summary PCWP-HCPWP joint meeting on 21 and 22 September

Draft Agenda - Annual PCWP-HCPWP joint meeting with all Eligible Organisations on 24 November

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

Action: For discussion

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2021

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Lifecycle Regulatory Submissions Metadata Project

Action: For discussion

Document(s) tabled: presentation

8.2. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q4/2021 Update of the Business Pipeline report for the human scientific committees

8.3. Feedback from the ENCePP Plenary

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/