12 January 2022
EMA/COMP/749019/2021
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 18-20 January 2022

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

18 January 2022, 08:30-19:30, remote virtual meeting

19 January 2022, 08:30-19:30, remote virtual meeting

20 January 2022, 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 18-20 January 2022. See January 2022 COMP minutes (to be published post February 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 18-20 January 2022.

1.3. Adoption of the minutes

COMP minutes for 7-9 December 2021.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000068060

Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 18 January 2022 at 11:00

2.1.2. - EMA/OD/0000070454

Treatment of amyotrophic lateral sclerosis (ALS)

Action: For adoption, Oral explanation to be held on 18 January 2022 at 14:00

2.1.3. - EMA/OD/0000069751

Treatment of pancreatic cancers

Action: For adoption, Oral explanation to be held on 19 January 2022 at 09:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000061188

Treatment of peripheral T-cell lymphoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000063681

Treatment of mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes

Action: For discussion/adoption

2.2.3. - EMA/OD/0000064376

Treatment of incomplete spinal cord injury

Action: For discussion/adoption

2.2.4. - EMA/OD/0000068622

Treatment of narcolepsy

Action: For discussion/adoption

2.2.5. - EMA/OD/0000068912

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For discussion/adoption

2.2.6. - EMA/OD/0000069374

Treatment of pyridoxamine 5'-phosphate oxidase deficiency

Action: For discussion/adoption

2.2.7. - EMA/OD/0000069674

Treatment of respiratory distress syndrome

Action: For discussion/adoption

2.2.8. - EMA/OD/0000069873

Treatment of hypoparathyroidism

Action: For discussion/adoption

2.2.9. - EMA/OD/0000072303

Treatment of Dravet syndrome

Action: For discussion/adoption

2.2.10. - EMA/OD/0000072308

Treatment of pyruvate kinase deficiency

Action: For discussion/adoption

2.2.11. - EMA/OD/0000072899

Treatment of diffuse large B-cell lymphoma (DLBCL)

Action: For discussion/adoption

2.2.12. - EMA/OD/0000073562

Treatment of Ehlers-Danlos syndrome

Action: For discussion/adoption

2.2.13. - EMA/OD/0000073624

Prevention of graft versus host disease

Action: For discussion/adoption

2.2.14. - EMA/OD/0000073716

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.15. - EMA/OD/0000073948

Treatment of fragile X syndrome (FXS)

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 18-20 January 2022 COMP meeting

2.7. Evaluation on-going

13 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 cystic fibrosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of myasthenia gravis

Action: For information

3.2.2. -

Treatment of tuberous sclerosis

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. – artesunate - EMEA/H/C/005718/0000, EMA/OD/043/15, EU/3/15/1521, EMA/OD/0000063220

B And O Pharm; Treatment of malaria

Action: For information

4.2.2. AYVAKYT – avapritinib - EMEA/H/C/005208/X/0004/G, EMA/OD/079/18, EU/3/18/2074, EMA/OD/0000071880

Blueprint Medicines (Netherlands) B.V. Treatment of mastocytosis

CHMP Rapporteur: Blanca Garcia-Ochoa; CHMP Co-Rapporteur: Ingrid Wang**Action:** For discussion/adoption

Action: For discussion/adoption

4.2.3. – lisocabtagene maraleucel - EMEA/H/C/004731/0000

Celgene Europe B.V.

a) Treatment of primary mediastinal large-B-cell lymphoma, EMA/OD/0000001127, EU/3/18/2099, EMA/OD/0000039978

Action: For discussion/adoption

b) Treatment of diffuse large B-cell lymphoma, EMA/OD/045/17, EU/3/17/1890, EMA/OD/0000039934

Action: For discussion/adoption

c) Treatment of follicular lymphoma, EMA/OD/260/17, EU/3/18/2018, EMA/OD/0000039979

Action: For discussion/adoption

4.2.4. – tebentafusp - EMEA/H/C/004929/0000, EMA/OD/0000030272, EU/3/21/2397, EMA/OD/0000068646

Accelerated assessment

Immunocore Ireland Limited; Treatment of uveal melanoma

Action: For discussion/adoption

4.3. Appeal

4.3.1. Nexviadyme - avalglucosidase alfa - EMEA/H/C/005501/0000, EU/3/14/1251

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

Action: For information, appointment of rapporteurs

4.3.2. Uplizna – inebilizumab - EMEA/H/C/005818/0000, EMA/OD/267/16, EU/3/17/1856, EMA/OD/0000079956

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

Action: For information, appointment of rapporteurs

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 17 January 2022 at 10:00

Document tabled:

PAWG draft agenda for 17 January 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

Action: For information

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes December 2021

7.2.2. PRIME – Analysis of the first 5 years' experience

Action: For discussion

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

Action: For adoption

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 and 2022

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

None

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/