13 April 2023
EMA/COMP/145882/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 18-20 April 2023

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

18 April 2023, 08:30-19:30, virtual meeting

19 April 2023, 08:30-19:30, virtual meeting

20 April 2023, 08:30-17:00, 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 18-20 April 2023. See April 2023 COMP minutes (to be published post May 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 18-20 April 2023.

1.3. Adoption of the minutes

COMP minutes for 21-23 March 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000118693

Treatment of myotonic disorders

Action: For adoption

2.1.2. - EMA/OD/0000114003

Treatment of sarcoidosis

Action: For information

Note: Withdrawal request received on 5 April 2023

2.1.3. - EMA/OD/0000119243

Treatment of paediatric osteosarcoma (melatonin monotherapy and/or in combination with cisplatin and/or doxorubicin)

Action: For information

Note: Withdrawal request received on 4 April 2023

2.1.4. - EMA/OD/0000098523

Treatment of adrenal insufficiency

Action: For information

Note: Withdrawal request received on 29 March 2023

2.1.5. - EMA/OD/0000120667

Treatment of Charcot-Marie-Tooth disease

Action: For adoption, Oral explanation to be held on 19 April 2023 at 11:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000061333

Treatment of hypomyelinating leukodystrophy-18

Action: For discussion/adoption

2.2.2. - EMA/OD/0000093914

Treatment of spinal cord injury

Action: For discussion/adoption

2.2.3. - EMA/OD/0000106321

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.4. - EMA/OD/0000112308

Treatment of moderate and severe closed traumatic brain injury

Action: For discussion/adoption

2.2.5. - EMA/OD/0000114445

Treatment of neurofibromatosis type 1

Action: For discussion/adoption

2.2.6. - EMA/OD/0000116156

Treatment of prosthetic joint infection

Action: For discussion/adoption

2.2.7. - EMA/OD/0000117653

Treatment of pouchitis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000117747

Treatment of pouchitis

Action: For discussion/adoption

2.2.9. - EMA/OD/0000117752

Treatment of pouchitis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000118613

Treatment of Dravet syndrome

Action: For discussion/adoption

2.2.11. - EMA/OD/0000119638

Treatment of myelodysplastic syndromes (MDS)

Action: For discussion/adoption

2.2.12. - EMA/OD/0000122073

Treatment of glioma

Action: For discussion/adoption

2.2.13. - EMA/OD/0000126177

Treatment of chronic inflammatory demyelinating polyneuropathy (CIDP)

Action: For discussion/adoption

2.2.14. - EMA/OD/0000126335

Treatment of systemic sclerosis

Action: For discussion/adoption

2.2.15. - EMA/OD/0000126745

Treatment of myelodysplastic syndrome

Action: For discussion/adoption

2.2.16. - EMA/OD/0000127495

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.17. - EMA/OD/0000127702

Treatment of recurrent respiratory papillomatosis

Action: For discussion/adoption

2.2.18. - EMA/OD/0000128546

Treatment of pemphigus

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 April 2023 COMP meeting

2.7. Evaluation on-going

15 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 homocystinuria

Action: For adoption

3.1.2. -

Treatment of creatine deficiency syndromes

Action: For adoption

3.1.3. -

Treatment of multiple myeloma

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

None

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

4.2.1. - futibatinib - EMEA/H/C/005627/0000, EU/3/19/2146, EMA/OD/0000122904

Taiho Pharma Netherlands B.V.; Treatment of biliary tract cancer

Action: For discussion/adoption

4.2.2. - pirtobrutinib - EMEA/H/C/005863, EU/3/21/2450, EMA/OD/0000124200

Eli Lilly Nederland B.V.; Treatment of mantle cell lymphoma

Action: For discussion/adoption

4.2.3. - ganaxolone - EMEA/H/C/005825, EU/3/19/2224, EMA/OD/0000071368

Marinus Pharmaceuticals Emerald Limited; Treatment of CDKL5 deficiency disorder

Action: For discussion/adoption

4.2.4. - lenadogene nolparvovec - EMEA/H/C/005047, EU/3/11/860, EMA/OD/0000053128

GenSight Biologics S.A.; Treatment of Leber's hereditary optic neuropathy

Action: For information

4.2.5. – glofitamab - EMEA/H/C/005751, EU/3/21/2497, EMA/OD/0000091986

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

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. AYVAKYT - avapritinib - EMEA/H/C/005208/II/0023, EU/3/18/2074, EMA/OD/0000127063

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

CHMP Rapporteur: Blanca Garcia-Ochoa

Action: For discussion/adoption

5.2.2. Abecma - idecabtagene vicleucel - EMEA/H/C/004662/II/0031, EU/3/17/1863, EMA/OD/0000132929

Bristol-Myers Squibb; Treatment of multiple myeloma

CAT Rapporteur: Rune Kjeken; CAT Co-Rapporteur: Heli Suila

Action: For discussion/adoption

5.2.3. Adcetris - brentuximab vedotin- EMEA/H/C/002455/II/0107, EU/3/08/596, EMA/OD/0000136638

Takeda Pharma A/S; Treatment of Hodgkin lymphoma

CHMP Rapporteur: Johann Lodewijk Hillege; CHMP Co-Rapporteur: Jan Mueller-Berghaus

Action: For discussion/adoption

5.2.4. Prevymis – letermovir - EMEA/H/C/004536/II/0033/G, EU/3/11/849, EMA/OD/0000133054

Merck Sharp & Dohme B.V.; Prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk

CHMP Rapporteur: Filip Josephson; CHMP Co-Rapporteur: Aaron Sosa Mejia

Action: For discussion/adoption

5.2.5. Reblozyl – luspatercept - EMEA/H/C/004444/II/0021, EU/3/14/1300, EMA/OD/0000134295

Bristol-Myers Squibb Pharma EEIG; Treatment of myelodysplastic syndromes

CHMP Rapporteur: Daniela Philadelphy; CHMP Co-Rapporteur: Ewa Balkowiec Iskra

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

None

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 17 April 2023 at 17:00

Document tabled: PAWG draft agenda for 17 April 2023 meeting

7.1.5. COMP Decisions Database

Action: For discussion

Document tabled:

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

PRIME eligibility requests - list of adopted outcomes March 2023

7.2.2. COMP members nominated on EMA's recommendation

Action: For information

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

7.3.2. Upcoming ITF meetings

Action: For discussion

Upcoming ITF meetings

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 2023

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Revision of EMA policy 0044 on handling of competing interests of scientific committees' members and experts

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/