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SCIENCE MEDICINES HEALTH

11 May 2023
EMA/COMP/192353/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 15-17 May 2023

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

15 May 2023, 09:00-19:30, room 2A/ virtual meeting

16 May 2023, 08:30-19:30, room 2A/ virtual meeting

17 May 2023, 08:30-17:00, room 2A/ 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

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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 15-17 May 2023. See May 2023 COMP minutes (to be published post June 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 15-17 May 2023.

1.3. Adoption of the minutes

COMP minutes for 18-20 April 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - [EMA/OD/0000112308](#)

Treatment of moderate and severe closed traumatic brain injury

Action: For adoption, Oral explanation to be held on 15 May 2023 at 09:45

2.1.2. - [EMA/OD/0000127495](#)

Treatment of idiopathic pulmonary fibrosis

Action: For information

Note: Withdrawal request received on 27 April 2023.

2.1.3. - [EMA/OD/0000117653](#)

Treatment of pouchitis

Action: For adoption, Oral explanation to be held on 15 May 2023 at 13:30

2.1.4. - [EMA/OD/0000117747](#)

Treatment of pouchitis

Action: For adoption, Oral explanation to be held on 15 May 2023 at 13:30

2.1.5. - [EMA/OD/0000117752](#)

Treatment of pouchitis

Action: For adoption, Oral explanation to be held on 15 May 2023 at 13:30

2.1.6. - EMA/OD/0000116156

Treatment of prosthetic joint infection

Action: For information

Note: Withdrawal request received on 27 April 2023.

2.1.7. - EMA/OD/0000128546

Treatment of pemphigus

Action: For adoption, Oral explanation to be held on 16 May 2023 at 10:45

2.1.8. - EMA/OD/0000122073

Treatment of glioma

Action: For adoption, Oral explanation to be held on 16 May 2023 at 15:30

2.1.9. - EMA/OD/0000126745

Treatment of myelodysplastic syndrome

Action: For adoption, Oral explanation to be held on 16 May 2023 at 17:00

2.1.10. - EMA/OD/0000126335

Treatment of systemic sclerosis

Action: For adoption, Oral explanation to be held on 17 May 2023 at 09:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000104786

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.2. - EMA/OD/0000116655

Treatment of patients with light chain (AL) amyloidosis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000119470

Treatment of osteosarcoma

Action: For discussion/adoption

2.2.4. - [EMA/OD/0000124587](#)

Treatment of gastric cancer

Action: For discussion/adoption

2.2.5. - [EMA/OD/0000124780](#)

Treatment of pre-eclampsia

Action: For discussion/adoption

2.2.6. - [EMA/OD/0000124931](#)

Treatment of primary biliary cholangitis

Action: For discussion/adoption

2.2.7. - [EMA/OD/0000125116](#)

Treatment of inherited retinal dystrophy due to dysfunction in the *ABCA4* gene

Action: For discussion/adoption

2.2.8. - [EMA/OD/0000125383](#)

Treatment of cryopyrin associated periodic syndromes

Action: For discussion/adoption

2.2.9. - [EMA/OD/0000127302](#)

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.10. - [EMA/OD/0000130058](#)

Treatment of congenital diaphragmatic hernia

Action: For discussion/adoption

2.2.11. - [EMA/OD/0000130111](#)

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.12. - [EMA/OD/0000130960](#)

Treatment of acute lymphoblastic leukaemia

Action: For discussion/adoption

2.2.13. - EMA/OD/0000131549

Treatment of Alport syndrome

Action: For discussion/adoption

2.2.14. - EMA/OD/0000131821

Treatment of nontuberculous mycobacterial pulmonary disease (NTM-PD)

Action: For discussion/adoption

2.2.15. - EMA/OD/0000131872

Treatment of soft tissue sarcoma

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 15-17 May 2023 COMP meeting

2.7. Evaluation on-going

25 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 Fabry disease

Action: For discussion/adoption

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. Lytgobi - 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 information

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

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

Action: For adoption, Oral explanation to be held on 16 May 2023 at 12:00

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

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

Action: For adoption, Oral explanation to be held on 16 May 2023 at 14:00

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

4.2.1. - 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

4.2.2. – sodium phenylbutyrate/ ursodoxicoltaurine - EMEA/H/C/005901, EU/3/20/2284, EMA/OD/0000096503

Amylyx Pharmaceuticals EMEA; Treatment of amyotrophic lateral sclerosis

Action: For information

4.2.3. **Sohonos - palovarotene - EMEA/H/C/004867, EU/3/14/1368, EMA/OD/0000101938**

Ipsen Pharma; treatment of fibrodysplasia ossificans progressiva

Action: For information

4.2.4. **- talquetamab - EMEA/H/C/005864, EU/3/21/2486, EMA/OD/0000126657**

Accelerated assessment

Janssen-Cilag International N.V.; 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

5.2.1. **Aspaveli - pegcetacoplan- EMEA/H/C/005553/II/0011, EU/3/17/1873,**

Swedish Orphan Biovitrum AB (publ); Treatment of paroxysmal nocturnal haemoglobinuria

CHMP Rapporteur: Alexandre Moreau; CHMP Co-Rapporteur: Selma Arapovic-Dzakula

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 Swedish Presidency of the Council of the European Union:
The COMP SRLM meeting in Uppsala 3-4 May 2023

Action: For discussion

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 15 May 2023
PAWG draft agenda for 15 May 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 April 2023

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

Agenda - HCPWP plenary meeting - 27 June 2023

Agenda - PCWP and HCPWP joint meeting - 28 June 2023

Agenda - PCWP plenary meeting - 27 June 2023

Meeting Summary - PCWP HCPWP meeting - 3 March 2023

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. Discussion on OMARs

Action: For discussion

8.2. Business Pipeline Report - 3 year Forecast report

Action: For discussion

8.3. ACT EU PA04 - Multi-stakeholder Workshop on ICH E6 R3 - Public Consultation

Action: For information/discussion

8.4. Report on experience with RWE studies to support EMA scientific committees

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/