16 March 2022
EMA/COMP/149289/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 15-17 March 2022

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

15 March 2022, 08:30-19:30, remote virtual meeting

16 March 2022, 08:30-19:30, remote virtual meeting

17 March 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 15-17 March 2022. See March 2022 COMP minutes (to be published post April 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 15-17 March 2022.

1.3. Adoption of the minutes

COMP minutes for 15-17 February 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000072331

Treatment of X-linked protoporphyria (XLP)

Action: For information

Notes: Withdrawal request received on 09 March 2022.

2.1.2. - EMA/OD/0000075999

Treatment of epilepsy with myoclonic-atonic seizures

Action: For adoption, Oral explanation to be held on 15 March 2022 at 16:00

2.1.3. - EMA/OD/0000072395

Treatment of primary biliary cholangitis

Action: For adoption, Oral explanation to be held on 16 March 2022 at 09:00

2.1.4. - EMA/OD/0000076085

Prevention of ischaemia-reperfusion injury in solid organ transplantation

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

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000070101

Treatment of mantle cell lymphoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000071657

Treatment of hypertrophic cardiomyopathy due to mutations in the *MYBPC3* gene encoding cardiac myosin-binding protein C

Action: For discussion/adoption

2.2.3. - EMA/OD/0000072068

Treatment of focal segmental glomerulosclerosis (FSGS)

Action: For discussion/adoption

2.2.4. - EMA/OD/0000073629

Treatment of glioma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000074838

Treatment of giant axonal neuropathy

Action: For discussion/adoption

2.2.6. - EMA/OD/0000075402

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.7. - EMA/OD/0000075682

Treatment of uveal melanoma

Action: For discussion/adoption

2.2.8. - EMA/OD/0000076090

Treatment of biliary tract cancer

Action: For discussion/adoption

2.2.9. - EMA/OD/0000076343

Treatment of resistance to thyroid hormone Type Beta (RTH-β)

Action: For discussion/adoption

2.2.10. - EMA/OD/0000076540

Treatment of essential thrombocythemia

Action: For discussion/adoption

2.2.11. - EMA/OD/0000076545

Prevention of spaceflight-related radiation and microgravity

Action: For discussion/adoption

2.2.12. - EMA/OD/0000076679

Treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN)

Action: For discussion/adoption

2.2.13. - EMA/OD/0000077023

Treatment of Angelman syndrome

Action: For discussion/adoption

2.2.14. - EMA/OD/0000077175

Treatment of primary sclerosing cholangitis

Action: For discussion/adoption

2.2.15. - EMA/OD/0000077200

Treatment of COVID-19 related ARDS and survival

Action: For discussion/adoption

2.2.16. - EMA/OD/0000077207

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.17. - EMA/OD/0000077237

Treatment of acute lymphoblastic leukaemia

Action: For discussion/adoption

2.2.18. - EMA/OD/0000077493

Treatment of haemophilia A

Action: For discussion/adoption

2.2.19. - EMA/OD/0000077524

Treatment of Fabry disease

Action: For discussion/adoption

2.2.20. EMA/OD/0000077548

Treatment of inherited retinal dystrophies due to defects in the *RPGR* gene

Action: For discussion/adoption

2.2.21. - EMA/OD/0000077756

Treatment of epidermolysis bullosa

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 March 2022 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 mucopolysaccharidosis type I

Action: For adoption

3.1.2 -

Treatment of acute myeloid leukaemia

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of primary IgA nephropathy

Action: For information

3.3. New requests

3.3.1 -

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. – arimoclomol - EMEA/H/C/005203/0000, EU/3/14/1376, EMA/OD/0000064667

Orphazyme A/S; Treatment of Niemann-Pick disease, type C

Action: For information

4.2.2. - betulae cortex dry extract (DER 5-10 : 1), extraction solvent n-heptane 95% (w/w) - EMEA/H/C/005035/0000, EU/3/10/845, EMA/OD/0000070235

Amryt Pharmaceuticals Designated Activity Company; Treatment of epidermolysis bullosa

Action: For discussion/adoption

4.2.3. - ciltacabtagene autoleucel - EMEA/H/C/005095/0000, EU/3/20/2252, EMA/OD/0000060914

Janssen-Cilag International N.V.; Treatment of multiple myeloma

Action: For discussion/adoption

4.2.4. - 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

4.2.5. - mosunetuzumab - EMEA/H/C/005680/0000, EU/3/21/2517, EMA/OD/0000082933

Accelerated assessment

Roche Registration GmbH; Treatment of follicular lymphoma

Action: For discussion/adoption

4.3. Appeal

None

4.5. On-going procedures

Action: For information

Document(s) tabled:
Review of orphan designation for OMP for MA - On-going procedures

4.6. 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/adoption

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:

6. Review of orphan designation for OMP for MA extension - On-going proceduresApplication of Article 8(2) of the Orphan Regulation

7. NoneOrganisational, 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

Strategic Review & Learning meeting – joint COMP/PDCO meeting under the French Presidency of the Council of the EU, to be held virtually on 31 March 2022

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 14 March 2022 at 10.00

Action: For information

Document tabled:

PAWP draft agenda for 14 March 2022 meeting

7.1.5. Principal Decisions Database

Action: For discussion

7.1.6. Data protection notice - processing of scientific committees' members/alternates' contact details

Action: For information

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 February 2022

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)

Nomination of a representative (and alternate) for PCWP/HCPWP for a new three-year mandate (June 2022 to May 2025).

Action: For adoption

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. The 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. Introducing DARWIN EU® Coordination Centre and next steps for RWE

Action: For discussion

8.2. Study on multiple myeloma

Action: For discussion

8.3. Orphan Maintenance Assessment Report (OMAR) updates

Action: For discussion

8.4. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q1-2022 Update of the Business Pipeline report for the human scientific committees

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/