07 April 2022
EMA/COMP/209670/2022
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 11-13 April 2022

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

11 April 2022, 08:30-19:30, remote virtual meeting

12 April 2022, 08:30-19:30, remote virtual meeting

13 April 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 11-13 April 2022. See April 2022 COMP minutes (to be published post May 2022 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 11-13 April 2022.

1.3. Adoption of the minutes

COMP minutes for 15-17 March 2022.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000077548

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

Action: For adoption, Oral explanation to be held on 12 April 2022 at 18:15

2.1.2. - EMA/OD/0000076540

Treatment of essential thrombocythemia

Action: For adoption, Oral explanation to be held on 13 April 2022 at 09:00

2.1.3. - EMA/OD/0000075402

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 11 April 2022 at 15:00

2.1.4. - EMA/OD/0000077023

Treatment of Angelman syndrome

Action: For adoption, Oral explanation to be held on 11 April 2022 at 16:15

2.1.5. - EMA/OD/0000077207

Treatment of epidermolysis bullosa

Action: For information

Note: Withdrawal request received on 1 April 2022.

2.1.6. - EMA/OD/0000077756

Treatment of epidermolysis bullosa

Action: For adoption, Oral explanation to be held on 11 April 2022 at 18:45

2.1.7. - EMA/OD/0000076679

Treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN)

Action: For adoption, Oral explanation to be held on 12 April 2022 at 11:45

2.1.8. - EMA/OD/0000076545

Prevention of spaceflight-related radiation and microgravity

Action: For adoption, Oral explanation to be held on 12 April 2022 at 17:00

2.1.9. - EMA/OD/0000077200

Treatment of COVID-19 related ARDS and survival

Action: For information

Note: Withdrawal request received on 28 March 2022.

2.1.10. - EMA/OD/0000072068

Treatment of focal segmental glomerulosclerosis (FSGS)

Action: For adoption, Oral explanation to be held on 11 April 2022 at 17:30

2.1.11. - EMA/OD/0000073629

Treatment of glioma

Action: For adoption, Oral explanation to be held on 11 April 2022 at 13.30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000076247

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000077171

Treatment of chronic myeloid leukemia

Action: For discussion/adoption

2.2.3. - EMA/OD/0000077407

Treatment of spinal cord injury

Action: For discussion/adoption

2.2.4. - EMA/OD/0000077417

Treatment of tubular aggregate myopathies (including York platelet syndrome and Stormorken syndrome)

Action: For discussion/adoption

2.2.5. - EMA/OD/0000077676

Treatment of primary lymphatic malformations

Action: For discussion/adoption

2.2.6. - EMA/OD/0000077720

Treatment of myopathic mitochondrial DNA depletion syndrome

Action: For discussion/adoption

2.2.7. - EMA/OD/0000078233

Treatment of fragile X syndrome

Action: For discussion/adoption

2.2.8. - EMA/OD/0000078678

Treatment of limb-girdle muscular dystrophy (LGMD)

Action: For discussion/adoption

2.2.9. - EMA/OD/0000079230

Treatment of hyper IgM1 syndrome (HIGM1)

Action: For discussion/adoption

2.2.10. - EMA/OD/0000079683

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.11. - EMA/OD/0000080466

Prevention of risk of graft failure following allogenic hematopoietic stem cell transplantation

Action: For discussion/adoption

2.2.12. - EMA/OD/0000080468

Treatment of chromosome 15q11.2-13.1 duplication syndrome (dup15q)

Action: For discussion/adoption

2.2.13. - EMA/OD/0000080709

Prevention of retinopathy of prematurity

Action: For discussion/adoption

2.2.14. - EMA/OD/0000080809

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.15. - EMA/OD/0000081138

Treatment of noninsulinoma pancreatogenous hypoglycemia syndrome (NIHPS)

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 11-13 April 2022 COMP meeting

2.7. Evaluation on-going

36 applications for orphan designation will not be discussed as evaluation is ongoing.

Action: For information

Notes:
See 7.8.1. Table

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 multiple myeloma

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of acute myeloid leukaemia

Action: For information

3.3. New requests

3.3.1. -

Treatment of multiple myeloma

Action: For information

3.3.2. -

Treatment of primary biliary cholangitis

Action: For information

3.3.3. -

Treatment of myelodysplastic syndromes

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. Carvykti - 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 adoption, Oral explanation to be held on 12 April 2022 at 15:45

4.1.2. Kymriah – tisangenlecleucel - EMEA/H/C/004090/II/0044, EU/3/21/2464, EMA/OD/0000054173

Novartis Europharm Limited; Treatment of follicular lymphoma

Action: For adoption, Oral explanation to be held on 13 April 2022 at 10:30

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

4.2.1. Yescarta - axicabtagene ciloleucel - EMEA/H/C/004480/II/0042, EU/3/15/1579, EMA/OD/0000068456

Kite Pharma EU B.V.; Treatment of follicular lymphoma

Action: For adoption, Oral explanation to be held on 11 April 2022 10:45

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. - 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

4.2.4. – olipudase alfa - EMEA/H/C/004850, EU/3/01/056, EMA/OD/0000072975

Accelerated assessment

Genzyme Europe BV; Treatment of Niemann-Pick disease

Action: For discussion/adoption

4.2.5. – fosdenopterin - EMEA/H/C/005378/0000, EU/3/10/777, EMA/OD/0000074822

Accelerated assessment

Comharsa Life Sciences Ltd; Treatment of molybdenum cofactor deficiency type A

Action: For discussion/adoption

4.2.6. Imcivree - setmelanotide - EMEA/H/C/005089/II/0002/G, EU/3/19/2192, EMA/OD/0000074865

Rhythm Pharmaceuticals Netherlands B.V.; Treatment of Bardet Biedl syndrome (BBS)

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 adoption, Oral explanation to be held on 12 April 2022 at 14:00

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

Feedback from the joint COMP/PDCO meeting under the French Presidency of the Council of the EU, to held virtually on 31 March 2022

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 8 April 2022 at 10:00

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 March 2022

7.2.2. COMP-CAT Working Group

Proposed meeting time on 7 April 2022 at 16:00

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) 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. 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. Complex Clinical Trials Question and Answers document

Action: For discussion

Document(s) tabled:

8.2. EMA survey on Orphan Maintenance Assessment Report (OMAR)

Action: For information

Document(s) tabled:

8.3. Marketing Authorisation Applications 3-year forecast report

Action: For information

Document(s) tabled:

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/