12 June 2020
EMA/COMP/285717/2020
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 16-18 June 2020

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

16 June 2020, 09:00-19:30, remote virtual meeting

17 June 2020, 08:30-19:30, remote virtual meeting

18 June 2020, 08:30-16:00, 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 16-18 June 2020. See June 2020 COMP minutes (to be published post July 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 16-18 June 2020.

1.3. Adoption of the minutes

COMP minutes for 18-20 May 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000030023

Treatment of peripheral T-cell lymphoma (PTCL)

Action: For information

Note: Withdrawal request received on 29 May 2020.

2.1.2. - EMA/OD/0000029989

Treatment of acute respiratory distress syndrome (ARDS)

Action: For adoption, Oral explanation to be held on 16 June 2020 at 11:15

2.1.3. - EMA/OD/0000030112

Treatment of glycogen storage disease type II (Pompe's disease)

Action: For adoption

2.1.4. - EMA/OD/0000030089

Treatment of GM1-gangliosidosis

Action: For adoption, Oral explanation to be held on 18 June 2020 at 09:00

2.1.5. - EMA/OD/0000030352

Treatment of hypertrophic cardiomyopathy

Action: For adoption, Oral explanation to be held on 16 June 2020 at 15:00

2.1.6. - EMA/OD/0000029210

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 17 June 2020 at 10:30

2.1.7. - EMA/OD/0000030109

Treatment of long-chain fatty acid oxidation disorders (LC-FAOD)

Action: For adoption, Oral explanation to be held on 17 June 2020 at 14:00

2.1.8. - EMA/OD/0000023895

Treatment of pulmonary arterial hypertension (PAH)

Action: For adoption, Oral explanation to be held on 17 June 2020 at 15:30

2.1.9. - EMA/OD/0000030276

Treatment of gestational hypermethioninemia

Action: For information

Note: Withdrawal request received on 3 June 2020.

2.1.10. - EMA/OD/0000026421

Treatment of hematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 16 June 2020 at 16:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000016117

Prevention of bronchopulmonary dysplasia

Action: For discussion/adoption

2.2.2. - EMA/OD/0000019700

Treatment of metastatic pancreatic ductal adenocarcinoma (PDAC)

Action: For discussion/adoption

2.2.3. - EMA/OD/0000023667

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.4. - EMA/OD/0000024640

Treatment of retinitis pigmentosa

Action: For discussion/adoption

2.2.5. - EMA/OD/0000028003

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

Action: For discussion/adoption

2.2.6. - EMA/OD/0000028006

Treatment of myoclonic epilepsy with Ragged-Red Fibres

Action: For discussion/adoption

2.2.7. - EMA/OD/0000028172

Treatment of Sickle cell disease

Action: For discussion/adoption

2.2.8. - EMA/OD/0000029026

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.9. - EMA/OD/0000029070

Treatment of achondroplasia

Action: For discussion/adoption

2.2.10. - EMA/OD/0000029150

Diagnosis of progressive supranuclear palsy (PSP)

Action: For discussion/adoption

2.2.11. - EMA/OD/0000029282

Treatment in solid organ transplant

Action: For discussion/adoption

2.2.12. - EMA/OD/0000029906

Treatment of non-traumatic subarachnoid haemorrhage

Action: For discussion/adoption

2.2.13. - EMA/OD/0000030237

Treatment of biliary atresia

Action: For discussion/adoption

2.2.14. - EMA/OD/0000030305

Treatment of primary sclerosing cholangitis

Action: For discussion/adoption

2.2.15. - EMA/OD/0000031667

Prevention of haemolytic uraemic syndrome

Action: For discussion/adoption

2.2.16. - EMA/OD/0000031867

Treatment of haematopoietic stem cell transplantation

Action: For discussion/adoption

2.2.17. - EMA/OD/0000031911

Treatment of inherited disorders of oxidative phosphorylation

Action: For discussion/adoption

2.2.18. - EMA/OD/0000031951

Treatment of myelodysplastic syndromes

Action: For discussion/adoption

2.2.19. - EMA/OD/0000031991

Prevention of retinopathy of prematurity

Action: For discussion/adoption

2.2.20. - EMA/OD/0000032007

Prevention of bronchopulmonary dysplasia

Action: For discussion/adoption

2.2.21. - EMA/OD/0000032028

Treatment of T-cell prolymphocytic leukaemia

Action: For discussion/adoption

2.2.22. - EMA/OD/0000032154

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

2.5.1. benzyl benzoate, beta-caryophyllene, cineole, cinnamaldehyde, cinnamyl acetate, linalool, trans-2-methoxycinnamaldehyde - EMA/OD/0000036241

Septeos S.A.S.; Treatment of eumycetoma

Action: For adoption, Oral explanation to be held on 17 June 2020 at time 09:00

2.5.2. melatonin - EMA/OD/0000036026

Worphmed S.r.l.; Treatment of intracerebral hemorrhage

Action: For adoption, Oral explanation to be held on 17 June 2020 at time 11:30

2.6. Nominations

2.6.1. New applications for orphan medicinal product designation - Appointment of COMP rapporteurs

Action: For adoption

Document tabled:
OMPD applications - appointment of rapporteurs at the 16-18 June 2020 COMP meeting

2.7. Evaluation on-going

5 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 ß-thalassaemia intermedia and major

Action: For adoption

3.1.2. -

Treatment of amyotrophic lateral sclerosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of mucopolysaccharidosis type II (Hunter syndrome)

Action: For information

3.2.2. -

Treatment of hepatocellular carcinoma

Action: For information

3.2.3. -

Treatment of Duchenne muscular dystrophy

Action: For information

3.3. New requests

3.3.1. -

Treatment of short bowel syndrome

Action: For information

3.3.2. -

Treatment of Philadelphia chromosome-positive chronic myelogenous leukaemia in chronic phase

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. – imlifidase - EMEA/H/C/004849, EMA/OD/237/16, EU/3/16/1826, EMA/OD/0000005755

Hansa Biopharma AB; Prevention of graft rejection following solid organ transplantation

Action: For discussion

4.2.2. - elexacaftor/tezacaftor/ivacaftor - EMEA/H/C/005269, EU/3/18/2116, EMA/OD/0000020155

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Action: For discussion

4.2.3. – tagraxofusp - EMEA/H/C/005031, EMA/OD/064/15, EU/3/15/1567, EMA/OD/0000004627

TMC Pharma (EU) Limited; Treatment of blastic plasmacytoid dendritic cell neoplasm

Action: For information

4.2.4. – crizanlizumab - EMEA/H/C/004874, EMA/OD/026/12, EU/3/12/1034, EMA/OD/0000009984

Novartis Europharm Limited; Treatment of sickle cell disease

Action: For discussion

4.2.5. – avapritinib - EMA/OD/037/17, EU/3/17/1889, EMA/OD/0000030630

Blueprint Medicines (Netherlands) B.V.; Treatment of gastrointestinal stromal tumours

Action: For discussion

4.2.6. – amikacin - EMEA/H/C/005264, EMA/OD/191/13, EU/3/14/1259, EMA/OD/0000030955

Insmed Netherlands B.V.; Treatment of nontuberculous mycobacterial lung

Action: For discussion

4.2.7. – deferiprone - EMEA/H/C/005004, EMA/OD/006/18, EU/3/18/2034, EMA/OD/0000011266

Apotex B.V.; Treatment of neurodegeneration with brain iron accumulation

Action: For discussion

4.2.8. – belantamab mafodotin - EMEA/H/C/004935/0000, EMA/OD/077/17, EU/3/17/1925, EMA/OD/0000028779

Accelerated assessment

GlaxoSmithKline (Ireland) Limited; Treatment of multiple myeloma

Action: For discussion

4.2.9. – pexidartinib - EMEA/H/C/004832, EMA/OD/279/14, EU/3/15/1457, EMA/OD/0000021360

Daiichi Sankyo Europe GmbH; Treatment of tenosynovial giant cell tumour, localised and diffuse type

Action: For information

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

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. Kalydeco– ivacaftor - Type II variation - EMEA/H/C/002494/II/0085, EMA/OD/010/08, EU/3/08/556, EMA/OD/0000036247

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP rapporteur: Maria Concepcion Prieto Yerro

Action: For discussion

5.2.2. Kalydeco – ivacaftor- Type II variation - EMEA/H/C/002494/II/0086, EMA/OD/010/08, EU/3/08/556, EMA/OD/0000036251

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP rapporteur: Maria Concepcion Prieto Yerro

Action: For discussion

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. Strategic Review & Learning meetings

None

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 16 June 2020 at 19:30

Document tabled:
PAWG draft agenda for 16 June 2020 meeting

7.1.3.

Action: For information

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes May 2020

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

7.3.2. Working Party with Healthcare Professionals' Organisations (HCPWP)

None

7.3.3.

7.3.4.

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)

Action: For information

Notes: Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes: Ad hoc basis meeting

7.5.3. Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes: Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes: Ad hoc basis meeting

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 2020

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q2/2020 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/