15 May 2020
EMA/COMP/235630/2020
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 18-20 May 2020

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

18 May 2020, 09:00-19:30, remote virtual meeting

19 May 2020, 08:30-19:30, remote virtual meeting

20 May 2020, 08:30-13:00, 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 18-20 May 2020. See May 2020 COMP minutes (to be published post June 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 18-20 May 2020.

1.3. Adoption of the minutes

COMP minutes for 21-23 April 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000009060

Treatment of myelodysplastic syndrome (MDS)

Action: For adoption, Oral explanation to be held on 18 May 2020 at 16:30.

2.1.2. - EMA/OD/0000025548

Treatment of blastic plasmacytoid dendritic cell Neoplasm

Action: For adoption

2.1.3. - EMA/OD/0000025945

Treatment of idiopathic pulmonary fibrosis

Action: For adoption, Oral explanation to be held on 18 May 2020 at 14:30.

2.1.4. - EMA/OD/0000027959

Treatment of ecker muscular dystrophy

Action: For adoption, Oral explanation to be held on 19 May 2020 at 15:30.

2.1.5. - EMA/OD/0000028068

Diagnosis of Diagnosis of gastro-entero-pancreatic neuroendocrine tumours (GEP-NETs)

Action: For adoption

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000020970

Treatment of bullous pemphigoid

Action: For discussion/adoption

2.2.2. - EMA/OD/0000021553

Treatment of primary hyperoxaluria

Action: For discussion/adoption

2.2.3. - EMA/OD/0000022629

Treatment of glioma

Action: For discussion/adoption

2.2.4. - EMA/OD/0000023895

Treatment of pulmonary arterial hypertension (PAH)

Action: For discussion/adoption

2.2.5. - EMA/OD/0000026421

Treatment of hematopoietic stem cell transplantation

Action: For discussion/adoption

2.2.6. - EMA/OD/0000029210

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.7. - EMA/OD/0000029989

Treatment of acute respiratory distress syndrome (ARDS)

Action: For discussion/adoption

2.2.8. - EMA/OD/0000030023

Treatment of peripheral T-cell lymphoma (PTCL)

Action: For discussion/adoption

2.2.9. - EMA/OD/0000030089

Treatment of GM1-gangliosidosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000030104

Treatment of marginal zone lymphoma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000030109

Treatment of Long-chain fatty acid oxidation Disorders (LC-FAOD)

Action: For discussion/adoption

2.2.12. - EMA/OD/0000030112

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

Action: For discussion/adoption

2.2.13. - EMA/OD/0000030202

Treatment of metachromatic leukodystrophy (MLD)

Action: For discussion/adoption

2.2.14. - EMA/OD/0000030276

Treatment of gestational hypermethioninemia

Action: For discussion/adoption

2.2.15. - EMA/OD/0000030352

Treatment of hypertrohpic cardiomyopathy

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 tabled:
OMPD applications - appointment of rapporteurs at the 18-20 May 2020 COMP meeting

2.7. Evaluation on-going

22 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 II (Hunter syndrome)

Action: For adoption

3.1.2. -

Treatment of hepatocellular carcinoma

Action: For adoption

3.1.3. -

Treatment of Duchenne muscular dystrophy

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of Niemann-Pick disease type C

Action: For information

3.2.2. -

Treatment of diffuse large B-cell lymphoma

Action: For information

3.3. New requests

3.3.1. -

Treatment of ß-thalassaemia intermedia and major

Action: For information

3.3.2. -

Treatment of amyotrophic lateral sclerosis

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. Daurismo – glasdegib - EMEA/H/C/004878, EMA/OD/106/17, EU/3/17/1923, EMA/OD/0000020246

Pfizer Europe MA EEIG; Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 18 May 2020 at 11:30.

4.1.2. Reblozyl - luspatercept

Celgene Europe BV;

a) Treatment of beta-thalassaemia intermedia and major, EMEA/H/C/004444, EMA/OD/047/14, EU/3/14/1300, EMA/OD/0000008931
b) Treatment of myelodysplastic syndromes, EMEA/H/C/004444, EMA/OD/048/14, EU/3/14/1331, EMA/OD/0000009353

Action: For adoption

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

4.2.1. - 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 discussion

4.2.2. - 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

4.2.3. - moxetumomab pasudotox– EMEA/H/C/005322, EMA/OD/066/08, EU/3/08/592, EMA/OD/0000013333

AstraZeneca AB; Treatment of hairy cell leukaemia

Action: For information

4.2.4. - bulevirtide – EMEA/H/C/004854, EMA/OD/329/14, EU/3/15/1500, EMA/OD/0000018086

Accelerated assessment

MYR GmbH; Treatment of hepatitis delta virus infection

Action: For 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

None

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. Kyprolis – carfilzomib - Type II variation - EMEA/H/C/003790/II/0045, EMA/OD/120/07, EU/3/08/548, EMA/OD/0000030043

Amgen Europe B.V.; Treatment of multiple myeloma

CHMP Rapporteur: Jorge Camarero Jiménez; CHMP Co-Rapporteur: Alexandre Moreau

Action: For 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. Strategic Review & Learning meeting – joint COMP/CAT/PDCO, 21-22 November 2019, Helsinki, Finland

Action: For information

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 18 May 2020 at 18:30

Document tabled:
PAWP draft agenda for 18 May 2020 meeting

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 April 2020

7.2.2. COMP-CAT Working Group

Proposed meeting time on 19 May 2020 at 18:30

Action: For discussion

Document(s) tabled: Agenda and related documents

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

Document(s) tabled: Meeting Summary form the PCWP/HCPWP Joint meeting on 3-4 March 2020

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. Human Medicines Division

Presentation on the new structure

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/