20 April 2020
EMA/COMP/159945/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 21-23 April 2020

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

21 April 2020, 09:00-19:30, Remote virtual meeting

22 April 2020, 08:30-19:30, Remote virtual meeting

23 April 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 21-23 April 2020. See April 2020 COMP minutes (to be published post May 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 21-23 April 2020.

1.3. Adoption of the minutes

COMP minutes for 17-19 March 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000020940

Treatment of multiple myeloma

Action: For adoption

2.1.2. - EMA/OD/0000023090

Treatment of marginal zone lymphoma

Action: For adoption

2.1.3. - EMA/OD/0000023993

Treatment of insulinoma

Action: For adoption, Oral explanation to be held on 21 April 2020 at 09:30

2.1.4. - EMA/OD/0000009482

Treatment of retinal detachment

Action: For adoption, Oral explanation to be held on 21 April 2020 at 14:30

2.1.5. - EMA/OD/0000023410

Treatment of multiple myeloma

Action: For information

Note: Withdrawal request received on 2 April 2020.

2.1.6. - EMA/OD/0000019446

Treatment of Cushing's Disease

Action: For adoption, Oral explanation to be held on 21 April 2020 at 17:00

2.1.7. - EMA/OD/0000023461

Treatment of primary sclerosing cholangitis

Action: For information

Note: Withdrawal request received on 3 April 2020.

2.1.8. - EMA/OD/0000022335

Treatment of pericarditis

Action: For adoption, Oral explanation to be held on 22 April 2020 at 13:30

2.1.9. - EMA/OD/0000023994

Treatment of esophageal squamous cell carcinoma

Action: For adoption, Oral explanation to be held on 22 April 2020 at 09:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000009060

Treatment of myelodysplastic syndrome (MDS)

Action: For discussion/adoption

2.2.2. - EMA/OD/0000021615

Treatment of immune thrombocytopenia

Action: For discussion/adoption

2.2.3. - EMA/OD/0000021857

Treatment of aneurysmal subarachnoid haemorrhage

Action: For discussion/adoption

2.2.4. - EMA/OD/0000022027

Treatment of chromosome 15q duplication syndrome

Action: For information

Note: Withdrawal request received on 8 April 2020.

2.2.5. - EMA/OD/0000023613

Treatment of paroxysmal nocturnal haemoglobinuria (PNH)

Action: For discussion/adoption

2.2.6. - EMA/OD/0000025548

Treatment of blastic plasmacytoid dendritic cell neoplasm

Action: For discussion/adoption

2.2.7. - EMA/OD/0000025790

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.8. - EMA/OD/0000025945

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.9. - EMA/OD/0000026429

Treatment of neonatal encephalopathy

Action: For discussion/adoption

2.2.10. - EMA/OD/0000026509

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.11. - EMA/OD/0000027842

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

2.2.12. - EMA/OD/0000027959

Treatment of Becker muscular dystrophy

Action: For discussion/adoption

2.2.13. - EMA/OD/0000028068

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

Action: For discussion/adoption

2.2.14. - EMA/OD/0000028117

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.15. - EMA/OD/0000028197

Treatment of Canavan disease

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 21-23 April 2020 COMP meeting

2.7. Evaluation on-going

13 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 Niemann-Pick disease type C

Action: For adoption

3.1.2. -

Treatment of mucopolysaccharidosis type II (Hunter syndrome)

Action: For adoption

3.1.3. -

Treatment of hepatocellular carcinoma

Action: For adoption

3.1.4. -

Treatment of diffuse large B-cell lymphoma

Action: For adoption

3.2. Finalised letters

3.2.1. -

Prevention of ischaemia reperfusion injury associated with solid organ transplantation

Action: For information

3.2.2. -

Treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome)

Action: For information

3.3. New requests

3.3.1. -

Treatment of Duchenne muscular dystrophy

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. - isatuximab – EMEA/H/C/004977, EMA/OD/198/13, EU/3/14/1268, EMA/OD/0000019553

Sanofi-Aventis Groupe; Treatment of plasma cell myeloma

Action: For adoption, Oral explanation to be held on 22 April 2020 at 15:00

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

4.2.1. - 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 discussion

4.2.2. – 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 discussion

4.2.3. – 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/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

5. Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension

5.1. After adoption of CHMP opinion

5.1.1. Adcetris - brentuximab vedotin - Type II variation - EMEA/H/C/002455/II/0070, EMA/OD/072/08, EU/3/08/595, EMA/OD/0000007448

Takeda Pharma A/S; Treatment of peripheral T-cell lymphoma

CHMP Rapporteur: Paula Boudewina van Hennik; CHMP Co-Rapporteur: Jan Mueller-Berghaus

Action: adoption, Oral explanation to be held on 22 April 2020 at 11:00

5.2. Prior to adoption of CHMP opinion

5.2.1. Imbruvica – ibrutinib - Type II variation - EMEA/H/C/003791/II/0059, EMA/OD/156/11, EU/3/12/984, EMA/OD/0000026247

Janssen-Cilag International NV; Treatment of chronic lymphocytic leukaemia

CHMP Rapporteur: Filip Josephson; CHMP Co-Rapporteur: Sinan B. Sarac

Action: For discussion

5.2.2. 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 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 meeting – COMP, 12-14 February 2020, Zagreb, Croatia

Report from the meeting

Action: For information

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

Action: For information

7.1.3. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 21 April 2020 at 18:30, remote virtual meeting
Document tabled:
PAWG draft agenda for 21 April 2020 meeting

7.1.4. Committee for Orphan Medicinal Products Rules of Procedure

Action: For adoption

Document(s) tabled:
COMP Rules of Procedure Rev. 5 and related documents

7.1.5. Reassessment of the Orphan criteria

Action: For discussion

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 March 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

Document(s) tabled:

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

Action: For information

Document(s) tabled:

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

8.1.1. Working Party with Patients' and Consumers' Organisations (PCWP) and Working Party with Healthcare Professionals' Organisations (HCPWP)

Meeting report

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/