20 January 2020
EMA/COMP/615654/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Agenda for the meeting on 03-05 December 2019

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

03 December 2019, 09:00-19:30, room 2A

04 December 2019, 08:30-19:30, room 2A

05 December 2019, 08:30-14:30, room 2A

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 03-05 December 2019. See December 2019 COMP minutes (to be published post January 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 03-05 December 2019.

1.3. Adoption of the minutes

COMP minutes for 05-07 November 2019.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000015678

Treatment of Fabry disease

Action: For adoption

2.1.2. - EMA/OD/0000010610

Treatment of autoimmune haemolytic anaemia

Action: For information

Note: Withdrawal request received on 13 November 2019.

2.1.3. - EMA/OD/0000016622

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 03 December 2019 at 15:30

2.1.4. - EMA/OD/0000016648

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 03 December 2019 at 12:00

2.1.5. - EMA/OD/0000016302

Treatment of sickle cell disease

Action: For adoption, Oral explanation to be held on 03 December 2019 at 14:00

2.1.6. - EMA/OD/0000014361

Treatment of cystinuria

Action: For adoption, Oral explanation to be held on 03 December 2019 at 17:00

2.1.7. - EMA/OD/0000005940

Treatment of myasthenia gravis

Action: For information

Note: Withdrawal request received on 18 November 2019.

2.1.8. - EMA/OD/0000007445

Treatment of acute myeloid leukaemia

Action: For information

Note: Withdrawal request received on 15 November 2019.

2.1.9. - EMA/OD/0000016718

Treatment of primary hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 04 December 2019 at 16:00

2.1.10. - EMA/OD/0000014371

Treatment of pancreatic cancer

Action: For information

Note: Withdrawal request received on 21 November 2019.

2.1.11. - EMA/OD/0000016160

Treatment of Gaucher disease

Action: For information

Note: Withdrawal request received on 14 November 2019.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000010028

Treatment of Adrenoleukodystrophy (X-ALD)

Action: For discussion/adoption

2.2.2. - EMA/OD/0000010743

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000011265

Treatment of Huntington's Disease

Action: For discussion/adoption

2.2.4. - EMA/OD/0000013447

Treatment of mucopolysaccharidosis type IVA (Morquio A Syndrome)

Action: For discussion/adoption

2.2.5. - EMA/OD/0000013899

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.6. - EMA/OD/0000015279

Treatment of non-traumatic subarachnoid haemorrhage

Action: For discussion/adoption

2.2.7. - EMA/OD/0000017344

Treatment of systemic sclerosis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000018285

Treatment of Leber congenital amaurosis

Action: For discussion/adoption

2.2.9. - EMA/OD/0000018682

Treatment of cerebral hypoxia-ischaemia reperfusion injury after return of spontaneous circulation in cardiac arrest patients

Action: For discussion/adoption

2.2.10. - EMA/OD/0000018797

Treatment of Alström syndrome

Action: For discussion/adoption

2.2.11. - EMA/OD/0000018998

Treatment of systemic sclerosis

Action: For discussion/adoption

2.2.12. - EMA/OD/0000019103

Treatment of acute lymphoblastic leukaemia (ALL)

Action: For discussion/adoption

2.2.13. - EMA/OD/0000019108

Treatment of glioma

Action: For discussion/adoption

2.2.14. - EMA/OD/0000019154

Treatment of follicular lymphoma

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designation

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 coordinators at the 03-05 December 2019 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes:
See 7.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of glioma

Action: For adoption

3.1.2. -

Treatment of eosinophilic esophagitis

Action: For adoption

3.2. Finalised letters

None

3.3. New requests

3.3.1. -

Treatment of haemophilia A

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. Revlimid – lenalidomide - EMEA/H/C/000717/II/0107, EMA/OD/158/12, EU/3/12/1097, EMA/OD/0000005466

Celgene Europe BV; Treatment of follicular lymphoma

CHMP rapporteur: Alexandre Moreau; CHMP co-rapporteur: Filip Josephson

Action: For adoption, Oral explanation to be held on 04 December 2019 at 10:00

4.1.2. Polivy - polatuzumab vedotin – EMEA/H/C/004870, EMA/OD/231/17, EU/3/18/2013, EMA/OD/0000003161

Roche Registration GmbH; Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 04 December 2019 at 13:00

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

4.2.1. - Onasemnogene abeparvovec – EMEA/H/C/004750, EMA/OD/028/15, EU/3/15/1509, EMA/OD/0000003028

AveXis EU Limited; Treatment of spinal muscular atrophy

Action: For discussion

4.2.2. – givosiran – EMEA/H/C/004775, EMA/OD/125/16, EU/3/16/1731, EMA/OD/0000013235

Accelerated assessment

Alnylam Netherlands B.V.; Treatment of acute hepatic porphyria

Action: For discussion

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

Action: For information

Document(s) tabled:

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. Vyndaqel – tafamidis – EMEA/H/C/002294/X/0049/G

Pfizer Europe MA EEIG;

a) Treatment of familial amyloid polyneuropathy, EU/3/06/401, EMA/OD/0000024082

b) Treatment of senile systemic amyloidosis, EU/3/12/1066, EMA/OD/0000003853

CHMP rapporteur: Joseph Emmerich; CHMP co-rapporteur: Bruno Sepodes

Action: For discussion

5.2.2. Darzalex – daratumumab - EMEA/H/C/004077/II/0030, EMA/OD/038/13, EU/3/13/1153, EMA/OD/0000010020

Janssen-Cilag International NV; Treatment of plasma cell myeloma

CHMP rapporteur: Sinan B. Sarac Jiménez; CHMP co-rapporteur: Jorge Camarero

Action: For discussion

5.2.3. Crysvita – burosumab – EMEA/H/C/004275/II/0010/G, EMA/OD/133/14, EU/3/14/1351, EMA/OD/0000023281

Kyowa Kirin Holdings B.V.; Treatment of X-linked hypophosphataemia

CHMP rapporteur: Kristina Dunder; CHMP co-rapporteur: Jayne Crowe

Action: For information

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

Report from the meeting

Action: For information

Document(s) tabled:

Final Agenda and related documents

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 3 December 2019 at 18:30 in room 0H

Document tabled:

PAWG draft agenda for 3 December 2019 meeting

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 November 2019

7.2.2.

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 tabled:
Meeting Summary PCWP/HCPWP 25 September 2019

Draft agenda Annual PCWP/HCPWP meeting 20 November 2019

7.3.2. Working Party with Patients' and Consumers' Organisations (PCWP)

Action: For information

Document(s) tabled:
Meeting Summary PCWP meeting 24 September 2019

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

Action: For information

Document(s) tabled:
Meeting Summary HCPWP meeting 24 September 2019

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

Action: For adoption

Document tabled:

Draft work plan 2020

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2019

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:
Q4/2019 Update of the Business Pipeline report for the human scientific committees

8.2. EMA's move to the permanent building

Update

Action: For information

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/