



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 February 2019
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Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 19-21 February 2019

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

19 February 2019, 08:30-19:30, room 03-E

20 February 2019, 08:30-18:00, room 03-E

21 February 2019, 08:30-15:00, room 03-E

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 19-21 February 2019. See February 2019 COMP minutes (to be published post March 2019 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 19-21 February 2019.

1.3. Adoption of the minutes

COMP minutes for 4-6 December 2018.

COMP minutes for 22-24 January 2019.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - [EMA/OD/0000001908](#)

Treatment of Polycythemia Vera

Action: For adoption, Oral explanation to be held on 19 February 2019 at 14:00

2.1.2. - [EMA/OD/0000001901](#)

Treatment of Duchenne muscular dystrophy

Action: For information

Note: Withdrawal request received 14 February 2019

2.1.3. - [EMA/OD/0000002181](#)

Treatment of endophthalmitis

Action: For adoption, Oral explanation to be held on 19 February 2019 at 15:30

2.1.4. - [EMA/OD/0000002333](#)

Treatment of Cystic fibrosis

Action: For adoption, Oral explanation to be held on 19 February 2019 at 17:00

2.1.5. - [EMA/OD/0000002264](#)

Treatment of Huntington's disease

Action: For adoption, Oral explanation to be held on 20 February 2019 at 10:00

2.1.6. - [EMA/OD/0000002429](#)

Treatment of Essential Thrombocythemia

Action: For adoption, Oral explanation to be held on 20 February 2019 at 11:30

2.2. For discussion / preparation for an opinion

2.2.1. - [EMA/OD/0000002304](#)

Treatment of Haemophilia B

Action: For discussion/adoption

2.2.2. - [EMA/OD/0000002457](#)

Treatment of Biliary Tract Cancer

Action: For discussion/adoption

2.2.3. - [EMA/OD/0000002498](#)

Treatment of Primary Sclerosing Cholangitis

Action: For discussion/adoption

2.2.4. - [EMA/OD/0000002526](#)

Treatment of Stargardt's Disease

Action: For discussion/ adoption

2.2.5. - [EMA/OD/0000002530](#)

Treatment of Invasive Aspergillosis

Action: For discussion/adoption

2.2.6. - [EMA/OD/0000002559](#)

Treatment of Spinal cord injury

Action: For discussion/adoption

2.2.7. - [EMA/OD/0000002563](#)

Treatment of Fragile X Syndrome

Action: For discussion/adoption

2.2.8. - EMA/OD/0000002735

Treatment of Hepatocellular carcinoma

Action: For discussion/adoption

2.2.9. - EMA/OD/0000002754

Treatment of Mucopolysaccharidosis type III (Sanfilippo syndrome)

Action: For discussion/adoption

2.2.10. - EMA/OD/0000002821

Treatment of Friedreich ataxia

Action: For discussion/adoption

2.2.11. - EMA/OD/0000002861

Treatment of follicular lymphoma

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 19-21 February 2019 COMP meeting

2.7. Evaluation on-going

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 diffuse large B-cell lymphoma

Action: For adoption

3.1.2. -

Treatment of ATTR amyloidosis

Action: For adoption

3.1.3. -

Treatment of congenital adrenal hyperplasia

Action: For adoption

3.1.4. -

Treatment of gastric carcinoid

Action: For adoption

3.1.5. - -

Treatment of beta-thalassaemia intermedia and major

Action: For adoption

3.1.6. -

Treatment of hyperargininaemia

Action: For adoption

3.2. Finalised letters

3.2.1. - -

Treatment of multiple myeloma

Action: For adoption

3.2.2. -

Treatment of diffuse large B-cell lymphoma

Action: For adoption

3.3. New requests

3.3.1. -

Treatment of adenosine deaminase-deficient-severe combined immunodeficiency

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. - Pegvaliase – EMEA/H/C/004744, EMEA/OD/112/09, EU/3/09/708

Treatment of hyperphenylalaninaemia

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.2. - Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human betaA-T87Q-globin gene – EMEA/H/C/003691, EMA/OD/146/12, EU/3/12/1091

Treatment of beta-thalassemia intermedia and major

Action: For discussion

Document(s) tabled:

Draft report on review of OMPD

4.2.3. - Pacritinib - EMEA/H/C/004793

a) Treatment of post-essential thrombocythaemia myelofibrosis EMA/OD/058/10, EU/3/10/767

b) Treatment of primary myelofibrosis EMA/OD/019/10, EU/3/10/768

c) Treatment of post-polycythemia vera myelofibrosis EMA/OD/057/10, EU/3/10/769

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

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

5.2.1. Imnovid – pomalidomide – Type II variation – EMEA/OD/053/09, EU/3/09/672, EMEA/H/C/002682/II/0031/G

Celgene Europe Limited; Treatment of multiple myeloma

CHMP rapporteur: Greg Markey;

Action: For discussion/adoption

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

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 19 February 2019 at 13:00

Document tabled:

PAWG draft agenda for 19 February 2019 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 20 February 2019 at 13:00

7.1.4. Prevalence Working Group

Proposed meeting time on 20 February 2019 at 08:30

7.2. Coordination with EMA Scientific Committees or CMDh-v

None

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. The 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 rapporteurships 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. Concepts of significant benefit (follow-up to COMP Work Plan 2017)

Action: For discussion

8.2. Presentation on Study to support the evaluation of the EU Orphan Regulation

Action: For discussion

Document(s) tabled:

EMA COMP presentation 21 Feb 2019_final

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/