18 January 2019
EMA/COMP/862725/2018
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 22-24 January 2019

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

22 January 2019, 08:30-19:30, room 02-A

23 January 2019, 08:30-19:30, room 02-A

24 January 2019, 08:30-15:00, room 02-A

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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Table of contents

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 22-24 January 2019. See January 2019 COMP minutes (to be published post February 2019 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 22-24 January 2019.

1.3. Adoption of the minutes

COMP minutes for 4-6 December 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000001655

Treatment of Non-traumatic osteonecrosis

Action: For adoption

2.1.2. - EMA/OD/0000001582

Treatment of acute myeloid leukaemia (AML)

Action: For adoption, Oral explanation to be held on 22 January 2019 at 09:00

2.1.3. - EMA/OD/0000001854

Treatment of Myasthenia gravis

Action: For adoption, Oral explanation to be held on 22 January 2019 at 10:00

2.1.4. - EMA/OD/0000001317

Treatment of Small cell lung cancer

Action: For adoption, Oral explanation to be held on 22 January 2019 at 15:30

2.1.5. - EMA/OD/0000001606

Treatment of Pancreatic Carcinoma

Action: For adoption, Oral explanation to be held on 22 January 2019 at 17:00

2.1.6. - EMA/OD/0000001829

Treatment of Ulcerative Proctitis

Action: For adoption, Oral explanation to be held on 23 January 2019 at 09:00

2.1.7. - EMA/OD/0000001604

Treatment of Tuberous Sclerosis Complex

Action: For information

Note: Withdrawal request received 04 January 2019

2.1.8. - EMA/OD/0000001791

Treatment of follicular lymphoma

Action: For information

Note: Withdrawal request received 14 December 2018

2.1.9. - EMA/OD/0000001881

Treatment of Pancreatic Cancer

Action: For adoption, Oral explanation to be held on 23 January 2019 at 15:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000001793

Treatment of Mucopolysaccharidosis II (Hunter syndrome)

Action: For discussion/adoption

2.2.2. - EMA/OD/0000001899

Treatment of Spinal Muscular Atrophy

Action: For discussion/adoption

2.2.3. - EMA/OD/0000001901

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.4. - EMA/OD/0000001908

Treatment of Polycythemia Vera

Action: For discussion/adoption

2.2.5. - EMA/OD/0000002181

Treatment of endophthalmitis

Action: For discussion/adoption

2.2.6. - EMA/OD/0000002264

Treatment of Huntington's disease

Action: For discussion/adoption

2.2.7. - EMA/OD/0000002279

Treatment of Infantile neuroaxonal dystrophy

Action: For discussion/adoption

2.2.8. - EMA/OD/0000002293

Treatment of Haemophilia B

Action: For discussion/adoption

2.2.9. - EMA/OD/0000002333

Treatment of Cystic fibrosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000002383

Treatment of Epidermolysis bullosa

Action: For discussion/adoption

2.2.11. - EMA/OD/0000002426

Treatment of Post-transplant lymphoproliferative disorder

Action: For discussion/adoption

2.2.12. - EMA/OD/0000002552

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.13. - EMA/OD/0000002975

Treatment of DiGeorge Syndrome

Action: For discussion/adoption

2.2.14. - EMA/OD/0000002977

Treatment of CHARGE syndrome

Action: For discussion/adoption

2.2.15. - EMA/OD/0000002979

Treatment of FOXN1 deficiency

Action: For discussion/adoption

2.2.16. -EMA/OD/0000002429

Treatment of Essential Thrombocythemia

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 22-24 January 2019 COMP meeting

2.7. Evaluation on-going

Eleven applications for orphan designation will not be discussed as evaluation is 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 multiple myeloma

Action: For adoption

3.1.2. -

Treatment of diffuse large B-cell lymphoma

Action: For adoption

3.1.3. -

Treatment of diffuse large B-cell lymphoma

Action: For adoption

3.2. Finalised letters

3.2.1. -

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

Action: For information

3.2.2. -

Treatment of neurofibromatosis type 1

Action: For information

3.3. New requests

3.3.1. -

Treatment of ATTR amyloidosis

Action: For information

3.3.2. -

Treatment of congenital adrenal hyperplasia

Action: For information

3.3.3. -

Treatment of gastric carcinoid

Action: For information

3.3.4. -

Treatment of beta-thalassaemia intermedia and major

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. – CANNABIDIOL - EMEA/H/C/004675

GW Research Ltd;

a) Treatment of Dravet syndrome EMA/OD/083/14, EU/3/14/1339

b) Treatment of Lennox-Gastaut syndrome EMA/OD/275/16, EU/3/17/1855

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD

4.2.2. - pacritinib - EMEA/H/C/004793

CTI Life Sciences Ltd - United Kingdom;

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

Document(s) tabled:
Draft report on review of OMPD

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

5.1.1. Adcetris - Brentuximab vedotin – Type II variation – EMEA/H/C/002455/II/0055, EMEA/OD/073/08, EU/3/08/596

Takeda Pharma A/S; Treatment of Hodgkin lymphoma

CHMP rapporteur: Paula Boudewina van Hennik; CHMP co-rapporteur: Jan Mueller-Berghaus;

Action: For adoption, Oral explanation to be held on 22 January 2019 at time 14:00

Document(s) tabled:
Draft report on review of OMPD
Sponsor's report

5.2. Prior to adoption of CHMP opinion

5.2.1. Imbruvica – ibrutinib - Type II variation – EMEA/H/C/003791/II/0046, EMA/OD/0000002783

Janssen-Cilag International NV;

a) Treatment of chronic lymphocytic leukaemia EMA/OD/156/11, EU/3/12/984

b) Treatment of mantle cell lymphoma EMA/OD/171/12, EU/3/13/1115

c) Treatment of lymphoplasmacytic lymphoma EMA/OD/185/13, EU/3/14/1264

CHMP rapporteur: Filip Josephson

Action: For discussion

5.2.2. Imbruvica – ibrutinib - Type II variation – EMEA/H/C/003791/II/0047, EMA/OD/0000002367

Janssen-Cilag International NV;

a) Treatment of chronic lymphocytic leukaemia EMA/OD/156/11, EU/3/12/984

b) Treatment of mantle cell lymphoma EMA/OD/171/12, EU/3/13/1115

c) Treatment of lymphoplasmacytic lymphoma EMA/OD/185/13, EU/3/14/1264

CHMP rapporteur: Filip Josephson

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 22 January 2019 at 13:00

Document tabled:
PAWG draft agenda for 22 January 2019 meeting

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes December 2018

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 adoption

Documents tabled:

Draft EMA PCWP Mandate and composition

Draft EMA HCPWP Mandate and composition
Draft EMA PCWP and HCPWP Rules of procedure

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Action: For information

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 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

None

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/