15 March 2019
EMA/COMP/141502/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 19-21 March 2019

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

19 March 2019, 09:00-19:30, room 0-H

20 March 2019, 08:30-18:00, room 0-H

21 March 2019, 08:30-15:00, room 0-H

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 March 2019. See March 2019 COMP minutes (to be published post April 2019 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 19-21 March 2019.

1.3. Adoption of the minutes

COMP minutes for 19-21 February 2019.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000002559

Treatment of Spinal cord injury

Action: For adoption

2.1.2. - EMA/OD/0000002563

Treatment of Fragile X Syndrome

Action: For adoption

2.1.3. - EMA/OD/0000002526

Treatment of Stargardt's Disease

Action: For adoption, Oral explanation to be held on 19 March 2019 at 10:00

2.1.4. - EMA/OD/0000002530

Treatment of Invasive Aspergillosis

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

2.1.5. - EMA/OD/0000002735

Treatment of Hepatocellular carcinoma

Action: For information

Note: Withdrawal request received 08 March 2019.

2.1.6. - EMA/OD/0000002861

Treatment of follicular lymphoma

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

2.1.7. - EMA/OD/0000002754

Treatment of Mucopolysaccharidosis type III (Sanfilippo syndrome)

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

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000002397

Treatment of Cushing syndrome

Action: For discussion/adoption

2.2.2. - EMA/OD/0000002775

Treatment of Amyotrophic Lateral Sclerosis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000003085

Treatment of non-small cell lung cancer with MET alterations

Action: For discussion/adoption

2.2.4. - EMA/OD/0000003147

Treatment of Lichen Planopilaris

Action: For discussion/adoption

2.2.5. - EMA/OD/0000003185

Treatment of Angioimmunoblastic T-cell lymphomas (AITL)

Action: For discussion/adoption

2.2.6. - EMA/OD/0000003203

Treatment of Enteropathy-associated T-cell lymphoma (EATL)

Action: For discussion/adoption

2.2.7. - EMA/OD/0000003207

Treatment of Propionic acidaemia

Action: For discussion/adoption

2.2.8. - EMA/OD/0000003216

Treatment of Ornithine transcarbamylase deficiency

Action: For discussion/adoption

2.2.9. - EMA/OD/0000003229

Treatment in solid organ transplantation

Action: For discussion/adoption

2.2.10. - EMA/OD/0000003554

Treatment of Loculated pleural effusion

Action: For discussion/adoption

2.2.11. - EMA/OD/0000004269

Treatment of lymphoplasmacytic lymphoma

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

2.4.1. Eculizumab – EMA/OD/0000003544

Alexion Europe S.A.S.; Treatment of neuromyelitis optica; Proposed new indication: Treatment of neuromyelitis optica spectrum disorder

Action: For adoption

Document(s) tabled:
Amended draft Summary report

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 March 2019 COMP meeting

2.7. Evaluation on-going

Twenty applications for orphan designation will not be discussed as evaluation is on-going.

Action: For information

Notes: See 7.8.1.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of ATTR amyloidosis

Action: For adoption

3.1.2. -

Treatment of congenital adrenal hyperplasia

Action: For adoption

3.1.3. -

Treatment of beta-thalassaemia intermedia and major

Action: For adoption

3.1.4. -

Treatment of hyperargininaemia

Action: For adoption

3.1.5. -

Treatment of adenosine deaminase-deficient-severe combined immunodeficiency

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of diffuse large B-cell lymphoma

Action: For information

3.2.2. -

Treatment of gastric carcinoid

Action: For information

3.3. New requests

3.3.1. -

Treatment of amyotrophic lateral sclerosis

Action: For information

3.3.2. -

Treatment of cystinuria

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

BioMarin International Limited; Treatment of hyperphenylalaninaemia

Action: For adoption

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

4.1.2. Waylivra - volanesorsen – EMEA/H/C/004538, EMA/OD/180/13, EU/3/14/1249

Akcea Therapeutics UK Ltd; Treatment of familial chylomicronemia syndrome

Action: For information

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

4.2.1. - 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/adoption

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

4.2.2. - Pegylated recombinant factor VIII – EMEA/H/C/004883, EMA/OD/144/11, EU/3/12/995

Novo Nordisk A/S; Treatment of haemophilia A

Action: For discussion/adoption

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

4.2.3. - Trientine dihydrochloride – EMEA/H/C/004111, EMEA/OD/043/03, EU/3/03/172

Univar BV; Treatment of Wilson's Disease

Action: For information

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

4.2.4. Soliris - ECULIZUMAB – Type II variation – EMEA/H/C/000791/II/0105, EMA/OD/087/13, EU/3/13/1185

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorder

Action: For discussion/adoption

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

4.2.5. - Larotrectinib - EMEA/H/C/004919

Bayer AG;

a) Treatment of salivary gland cancer EMA/OD/213/17, EU/3/18/1995

b) Treatment of soft tissue sarcoma EMA/OD/184/15, EU/3/15/1606

Action: For information

4.2.6. – Glutamine – EMEA/H/C/004734, EMA/OD/016/12, EU/3/12/1011

Emmaus Medical Europe Limited; Treatment of sickle cell disease

Action: For discussion/adoption

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

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 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 March 2019 at 18:00

Document tabled:
PAWG draft agenda for 19 March 2019 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 20 March 2019 at 18:00

7.1.4. Prevalence Working Group

Document tabled:

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 February 2019

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

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

Action: For information

7.3.3. SAWP/COMP joint membership

Call for interest to replace COMP alternate representative at SAWP - Deadline 8 March 2019

Action: For adoption

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.4.2. Handling of confidential information within the EU network

Action: For discussion

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

8.1. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q1/2019 Update of the Business Pipeline report for the human scientific committees

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/