



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

12 April 2019
EMA/COMP/193772/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 15-17 April 2019

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

15 April 2019, 09:00-19:30, room 2-A

16 April 2019, 08:30-19:30, room 2-A

17 April 2019, 08:30-17:00, room 2-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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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 15-17 April 2019. See April 2019 COMP minutes (to be published post May 2019 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 15-17 April 2019.

1.3. Adoption of the minutes

COMP minutes for 19-21 March 2019.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - [EMA/OD/0000002397](#)

Treatment of Cushing syndrome

Action: For adoption, Oral explanation to be held on 15 April 2019 at 09:30

2.1.2. - [EMA/OD/0000003147](#)

Treatment of lichen planopilaris

Action: For information

Note: Withdrawal request received 27 March 2019.

2.1.3. - [EMA/OD/0000003085](#)

Treatment of non-small cell lung cancer with MET alterations

Action: For adoption, Oral explanation to be held on 15 April 2019 at 17:00

2.1.4. - [EMA/OD/0000003229](#)

Treatment in solid organ transplantation

Action: For adoption, Oral explanation to be held on 16 April 2019 at 09:30

2.1.5. - [EMA/OD/0000003185](#)

Treatment of angioimmunoblastic T-cell lymphomas (AITL)

Action: For information

Note: Withdrawal request received 1 April 2019.

2.1.6. - [EMA/OD/0000003203](#)

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

Action: For information

Note: Withdrawal request received 1 April 2019.

2.1.7. - [EMA/OD/0000003554](#)

Treatment of loculated pleural effusion

Action: For adoption, Oral explanation to be held on 16 April 2019 at 17:00

2.1.8. - [EMA/OD/0000004269](#)

Treatment of lymphoplasmatic lymphoma

Action: For adoption, Oral explanation to be held on 17 April 2019 at 09:30

2.2. For discussion / preparation for an opinion

2.2.1. - [EMA/OD/0000001324](#)

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.2. - [EMA/OD/0000003013](#)

Treatment of epidermolysis bullosa

Action: For discussion/adoption

2.2.3. - [EMA/OD/0000003105](#)

Prevention of intradialytic hypotension

Action: For discussion/adoption

2.2.4. - [EMA/OD/0000003121](#)

Treatment of Stargardt disease

Action: For discussion/adoption

2.2.5. - [EMA/OD/0000003694](#)

Treatment of adult T-cell lymphomas/leukaemias (ATLL)

Action: For discussion/adoption

2.2.6. - [EMA/OD/0000003698](#)

Treatment of peripheral T-cell lymphoma - Not otherwise specified (PTCL-NOS)

Action: For discussion/adoption

2.2.7. - [EMA/OD/0000003833](#)

Treatment of heat stroke

Action: For discussion/adoption

2.2.8. - [EMA/OD/0000003859](#)

Treatment of eosinophilic esophagitis

Action: For discussion/adoption

2.2.9. - [EMA/OD/0000003878](#)

Treatment of acute myeloid leukemia (AML)

Action: For discussion/adoption

2.2.10. - [EMA/OD/0000003888](#)

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.11. - [EMA/OD/0000003941](#)

Treatment of cyclin-dependent kinase-like 5 deficiency disorder

Action: For discussion/adoption

2.2.12. - [EMA/OD/0000004036](#)

Treatment of nontuberculous mycobacterial lung disease

Action: For discussion/adoption

2.2.13. - [EMA/OD/0000004038](#)

Treatment of haemophilia A

Action: For discussion/adoption

2.2.14. - [EMA/OD/0000004041](#)

Treatment of Cushing's syndrome (hyperadrenocorticism)

Action: For discussion/adoption

2.2.15. - [EMA/OD/0000004076](#)

Treatment of Angelman syndrome

Action: For discussion/adoption

2.2.16. - [EMA/OD/0000004127](#)

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

2.2.17. - [EMA/OD/0000004137](#)

Treatment of citrullinemia Type 1 deficiency or Argininosuccinate synthetase deficiency

Action: For discussion/adoption

2.2.18. - [EMA/OD/0000004139](#)

Treatment of carbamoylphosphate synthase-1 deficiency

Action: For discussion/adoption

2.2.19. - [EMA/OD/0000004216](#)

Treatment of mantle cell lymphoma

Action: For discussion/adoption

2.2.20. - [EMA/OD/0000004363](#)

Treatment of maternally inherited diabetes and deafness

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 15-17 April 2019 COMP meeting

2.7. Evaluation on-going

Sixteen 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 amyotrophic lateral sclerosis

Action: For adoption

3.1.2. -

Treatment of cystinuria

Action: For adoption

3.1.3. - -

Treatment of beta-thalassaemia intermedia and major

Action: For information

3.2. Finalised letters

3.2.1. -

Treatment of ATTR amyloidosis

Action: For information

3.2.2. -

Treatment of congenital adrenal hyperplasia

Action: For information

3.2.3. -

Treatment of hyperargininaemia

Action: For information

3.2.4. -

Treatment of adenosine deaminase-deficient-severe combined immunodeficiency

Action: For information

3.3. New requests

3.3.1. -

Treatment of diffuse large B-cell lymphoma

Action: For information

3.3.2. -

Treatment of idiopathic pulmonary fibrosis

Action: For information

3.3.3. -

Treatment of tuberculosis

Action: For information

3.3.4. -

Treatment of Niemann-Pick disease, type C

Action: For information

3.3.5. -

Treatment of immune thrombocytopenia

Action: For information

3.3.6. -

Prevention of invasive aspergillosis

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. Soliris - – Type II variation – EMEA/H/C/000791/II/0105, EMA/OD/087/13, EU/3/13/1185, EMA/OD/0000004454

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorder

Action: For discussion/adoption

4.2.2. - 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

4.2.3. - ravulizumab – EMA/OD/246/15, EU/3/16/1661, EMEA/H/C/004954, EMA/OD/0000004229

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion

4.2.4. - 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

4.3. Appeal

4.3.1. Trecondi - treosulfan - EMEA/H/C/004751, EMEA/OD/075/03, EU/3/04/186, EMA/OD/0000002579

medac Gesellschaft für klinische Spezialpräparate mbH; Conditioning treatment prior to haematopoietic progenitor cell transplantation

Action: For adoption, Oral explanation to be held on 15 April 2019 at time 14:00

Notes: Status of the procedure at the CHMP: CHMP adopted positive opinion in December 2018.

4.4. On-going procedures

Action: For information

Document(s) tabled:

Review of orphan designation for OMP for MA - Ongoing procedures

4.5. Orphan Maintenance Reports

None

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. 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, Oral explanation to be held on 15 April 2019 at time 11:30

Document(s) tabled:

Draft report on review of OMPD

Sponsor's report

5.2. Prior to adoption of CHMP opinion

None

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:

Review of orphan designation for OMP for MA extension - Ongoing 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, 27-28 May 2019, Rome, Italy

Action: For information

Document tabled:

Draft agenda

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 15 April 2019 at 18:30

Document tabled:
PAWG draft agenda for 15 April 2019 meeting

7.1.3. Non-Clinical Working Group

Proposed meeting time on 16 April at 18:30

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 March 2019

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

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

COMP representative.

Action: For adoption

Document(s) tabled:

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

COMP representative.

Action: For adoption

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

Action: For information

8.2. **IRIS: a new way of supporting COMP procedures with a CRM (Customer Relationship Management software)**

Action: For discussion

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

