20 May 2019
EMA/COMP/240339/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

# Committee for Orphan Medicinal Products (COMP)

## Draft agenda for the meeting on 21-23 May 2019

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

21 May 2019, 09:00-19:30, room 2-A

22 May 2019, 08:30-19:30, room 2-A

23 May 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 21-23 May 2019. See May 2019 COMP minutes (to be published post 18-20 June 2019 COMP meeting).

## 1.2. Adoption of agenda

COMP agenda for 21-23 May 2019.

## 1.3. Adoption of the minutes

COMP minutes for 15-17 April 2019.

# 2. Applications for orphan medicinal product designation

## 2.1. For opinion

### 2.1.1. - EMA/OD/0000004216

Treatment of mantle cell lymphoma

**Action:** For adoption, Oral explanation to be held on 21 May 2019 at 09:30

### 2.1.2. - EMA/OD/0000003698

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

**Action:** For adoption, Oral explanation to be held on 21 May 2019 at 10:30

### 2.1.3. - EMA/OD/0000004038

Treatment of haemophilia A

**Action:** For adoption, Oral explanation to be held on 21 May 2019 at 12:00

### 2.1.4. - EMA/OD/0000004041

Treatment of Cushing’s syndrome (hyperadrenocorticism)

**Action:** For adoption, Oral explanation to be held on 21 May 2019 at 14:30

### 2.1.5. - EMA/OD/0000003941

Treatment of cyclin-dependent kinase-like 5 deficiency disorder

**Action:** For adoption, Oral explanation to be held on 22 May 2019 at 09:30

### 2.1.6. - EMA/OD/0000004036

Treatment of nontuberculous mycobacterial lung disease

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

### 2.1.7. - EMA/OD/0000004076

Treatment of Angelman syndrome

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

### 2.1.8. - EMA/OD/0000003833

Treatment of heat stroke

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

### 2.1.9. - EMA/OD/0000003859

Treatment of eosinophilic esophagitis

**Action:** For information

Note: Withdrawal request received 30 April 2019.

### 2.1.10. - EMA/OD/0000004363

Treatment of maternally inherited diabetes and deafness

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

### 2.1.11. - EMA/OD/0000003105

Prevention of intradialytic hypotension

Action: For adoption, Oral explanation to be held on 23 May 2019 at 10:30

### 2.1.12. - EMA/OD/0000003694

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

**Action:** For information

Note: Withdrawal request received 30 April 2019.

## 2.2. For discussion / preparation for an opinion

### 2.2.1. - EMA/OD/0000002916

Treatment of centronuclear myopathies (CNM)

**Action:** For discussion/adoption

### 2.2.2. - EMA/OD/0000003591

Treatment of acute radiation syndrome

**Action:** For discussion/adoption

### 2.2.3. - EMA/OD/0000003689

Treatment of Gaucher disease

**Action:** For discussion/adoption

### 2.2.4. - EMA/OD/0000003838

Treatment of Duchenne muscular dystrophy

**Action:** For discussion/adoption

### 2.2.5. - EMA/OD/0000003987

Treatment of glioma

**Action:** For discussion/adoption

### 2.2.6. - EMA/OD/0000004238

Treatment of spinal cord injury

**Action:** For discussion/adoption

### 2.2.7. - EMA/OD/0000004268

Treatment of spinal muscular atrophy

**Action:** For discussion/adoption

### 2.2.8. - EMA/OD/0000004774

Treatment of mitochondrial encephalopathy, lactic acidosis, and stroke-like episodes

**Action:** For discussion/adoption

### 2.2.9. - EMA/OD/0000004784

Treatment of myasthenia gravis

**Action:** For discussion/adoption

### 2.2.10. - EMA/OD/0000004992

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

**Action:** For discussion/adoption

### 2.2.11. - EMA/OD/0000005124

Treatment of soft tissue sarcoma

**Action:** For discussion/adoption

### 2.2.12. - EMA/OD/0000005159

Treatment of non-infectious uveitis

**Action:** For discussion/adoption

### 2.2.13. - EMA/OD/0000005305

Treatment of beta-thalassaemia

**Action:** For discussion/adoption

### 2.2.14. - EMA/OD/0000005429

Treatment of argininosuccinic aciduria

**Action:** For discussion/adoption

### 2.2.15. - EMA/OD/0000005430

Treatment of hyperargininaemia

**Action:** For discussion/adoption

### 2.2.16. - EMA/OD/0000006865

Treatment of haematopoietic stem cell transplantation

**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 21-23 May 2019 COMP meeting

## 2.7. Evaluation on-going

Twenty-three 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 diffuse large B-cell lymphoma

**Action**: For adoption

### 3.1.2. -

Treatment of idiopathic pulmonary fibrosis

**Action**: For adoption

### 3.1.3. -

Treatment of tuberculosis

**Action**: For adoption

### 3.1.4. -

Treatment of Niemann-Pick disease, type C

**Action**: For adoption

### 3.1.5. -

Treatment of immune thrombocytopenia

**Action**: For adoption

### 3.1.6. - -

Treatment of beta-thalassaemia intermedia and major

**Action**: For information

## 3.2. Finalised letters

### 3.2.1. -

Treatment of amyotrophic lateral sclerosis

**Action**: For information

### 3.2.2. -

Treatment of cystinuria

**Action**: For information

## 3.3. New requests

### 3.3.1. -

Treatment of multiple myeloma

**Action**: For information

### 3.3.2. -

Treatment of transthyretin-mediated amyloidosis

**Action**: For information

### 3.3.3. -

Treatment of spinal muscular atrophy

**Action**: For information

### 3.3.4. -

Treatment of medullary thyroid carcinoma

**Action**: For information

### 3.3.5. -

Treatment of ovarian cancer

**Action**: For information

### 3.3.6. -

Treatment of acute myeloid leukaemia

**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. Ultomiris - 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 information

Notes: Status of the procedure at the CHMP: CHMP opinion adopted in April 2019. Withdrawal request received 08 May 2019.

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

### 4.2.1. - trientine dihydrochloride – EMEA/H/C/004111, EMEA/OD/043/03, EU/3/03/172

Univar BV; Treatment of Wilson's disease

**Action**: For discussion/adoption

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

### 4.2.2. - 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 discussion/adoption

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

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

**Accelerated assessment**

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

**Action**: For discussion/adoption

### 4.2.4. - tagraxofusp – EMEA/H/C/005031, EMA/OD/064/15, EU/3/15/1567, EMA/OD/0000004627

**Accelerated assessment**

TMC Pharma (EU) Limited; Treatment of blastic plasmacytoid dendritic cell neoplasm

**Action**: For discussion/adoption

#### 4.2.5. - edaravone – EMEA/H/C/004938, EMA/OD/032/15, EU/3/15/1510

Mitsubishi Tanabe Pharma Europe Ltd; Treatment of amyotrophic lateral sclerosis

**Action**: For information

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

AveXis Netherlands B.V.; Treatment of spinal muscular atrophy

**Action**: For discussion/adoption

### 4.3. Appeal

None

### 4.4. Ongoing procedures

**Action**: For information

Document(s) tabled:
Review of orphan designation for OMP for MA - ongoing 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. Darzalex - daratumumab

Janssen-Cilag International NV;

a) EMEA/H/C/004077/II/0029, EMA/OD/038/13, EU/3/13/1153 Treatment of plasma cell myeloma

b) EMEA/H/C/004077/II/0030, EMA/OD/038/13, EU/3/13/1153 Treatment of plasma cell myeloma

**Action**: For discussion

## 5.3. Appeal

None

## 5.4. Ongoing 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 21 May 2019 at 18:30

Document tabled:
PAWG draft agenda for 21 May 2019 meeting

### 7.1.3. Points to consider on the estimation and reporting on the prevalence of a condition for the purpose of orphan designation (COMP/436/01)

Update

**Action:** For adoption

## 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 April 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

## 7.4. 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. Preparedness of the system and capacity increase

**Action**: For discussion

## 8.2. Court case -

**Action:** For information

Document: Background 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/