12 March 2021
EMA/COMP/108776/2021
Human Medicines Division

# Committee for Orphan Medicinal Products (COMP)

## Draft agenda for the meeting on 16-18 March 2021

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

16 March 2021, 08:30-19:30, remote virtual meeting

17 March 2021, 08:30-19:30, remote virtual meeting

18 March 2021, 08:30-17:30, remote virtual meeting

**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 16-18 March 2021. See March 2021 COMP minutes (to be published post April 2021 COMP meeting).

## 1.2. Adoption of agenda

COMP agenda for 16-18 March 2021.

## 1.3. Adoption of the minutes

COMP minutes for 16-18 February 2021.

# 2. Applications for orphan medicinal product designation

## 2.1. For opinion

### 2.1.1. - EMA/OD/0000038966

Treatment of pulmonary hypertension associated with interstitial lung disease

**Action:** For adoption, Oral explanation to be held on 16 March 2021 at 10:30

### 2.1.2. - EMA/OD/0000048469

Treatment of non-functioning pituitary adenomas

**Action:** For information

Notes: Withdrawal request received on 1 March 2021.

### 2.1.3. - EMA/OD/0000047634

Treatment of ovarian cancer

**Action:** For adoption, Oral explanation to be held on 17 March 2021 at 11:00

### 2.1.4. - EMA/OD/0000048121

Treatment of cutaneous T-cell lymphoma

**Action:** For adoption, Oral explanation to be held on 17 March 2021 at 09:00

### 2.1.5. - EMA/OD/0000052275

Treatment of eosinophilic oesophagitis

**Action:** For adoption, Oral explanation to be held on 17 March 2021 at 17:30

### 2.1.6. - EMA/OD/0000048721

Treatment of non-small cell lung cancer with EGFR alterations

**Action:** For adoption, Oral explanation to be held on 16 March 2021 at 15:00

### 2.1.7. - EMA/OD/0000042673

Treatment of multiple myeloma

**Action:** For adoption, Oral explanation to be held on 16 March 2021 at 17:00

### 2.1.8. - EMA/OD/0000044231

Treatment of pancreatic cancer

**Action:** For adoption, Oral explanation to be held on 18 March 2021 at 11:30

## 2.2. For discussion / preparation for an opinion

### 2.2.1. - EMA/OD/0000037733

Treatment of systemic sclerosis

**Action:** For discussion/adoption

### 2.2.2. - EMA/OD/0000047579

Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma

**Action:** For discussion/adoption

### 2.2.3. - EMA/OD/0000047784

Treatment of multiple system atrophy

**Action:** For discussion/adoption

### 2.2.4. - EMA/OD/0000049059

Treatment of generalised pustular psoriasis (GPP)

**Action:** For discussion/adoption

### 2.2.5. - EMA/OD/0000049823

Treatment of cystic fibrosis

**Action:** For discussion/adoption

### 2.2.6. - EMA/OD/0000049973

Treatment of amyotrophic lateral sclerosis

**Action:** For discussion/adoption

### 2.2.7. - EMA/OD/0000050198

Treatment of glioma

**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 tabled:
OMPD applications - appointment of rapporteurs at the 16-18 March 2021 COMP meeting

## 2.7. Evaluation on-going

19 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 pancreatic cancer

**Action**: For adoption

### 3.1.2. -

Treatment of Gaucher disease

**Action**: For adoption

### 3.1.3. -

Treatment of growth hormone deficiency

**Action**: For adoption

## 3.2. Finalised letters

### 3.2.1. -

Treatment of Fabry disease

**Action**: For information

### 3.2.2. –

Treatment of relapsed or refractory multiple myeloma

**Action**: For information

### 3.2.3. -

Treatment of sickle cell disease

**Action**: For information

## 3.3. New requests

### 3.3.1. -

Treatment of glioma

**Action**: For information

### 3.3.2. -

Treatment of mucopolysaccharidosis type I

**Action**: For information

### 3.3.3. -

Diagnosis of AL amyloidosis

**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. Epidyolex - cannabidiol - EMEA/H/C/004675/II/0005, EMA/OD/165/17, EU/3/17/1959, EMA/OD/0000033940

GW Pharma (International) B.V.; Treatment of tuberous sclerosis

CHMP Rapporteur: Kirstine Moll Harboe; CHMP Co-Rapporteur: Ondřej Slanař

**Action:** For adoption, Oral explanation to be held on 16 March 2021 at 13:30

### 4.1.2. Orladeyo – berotralstat - EMEA/H/C/005138/0000, EMA/OD/003/18, EU/3/18/2028, EMA/OD/0000045564

BioCryst Ireland Limited; Treatment of hereditary angioedema

CHMP Rapporteur: Peter Kiely; CHMP Co-Rapporteur: Margareta Bego

**Action:** For adoption, Oral explanation to be held on 17 March 2021 at 14:00

### 4.1.3. Sibnayal – potassium - EMEA/H/C/005407, EMA/OD/016/17, EU/3/17/1888, EMA/OD/0000032257

Advicenne Pharma S.A.; Treatment of distal renal tubular acidosis

CHMP Rapporteur: Johann Lodewijk Hillege; CHMP Co-Rapporteur: Tomas Radimersky

**Action:** For adoption, Oral explanation to be held on 17 March 2021 at 15:30

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

### 4.2.1. – satralizumab - EMEA/H/C/004788, EMA/OD/014/16, EU/3/16/1680, EMA/OD/0000016001

Roche Registration GmbH; Treatment of neuromyelitis optica spectrum disorders

**Action:** For information

### 4.2.2. – elivaldogene autotemcel - EMEA/H/C/003690/0000, EMA/OD/009/12, EU/3/12/1003, EMA/OD/0000044429

**Accelerated Assessment**

bluebird bio (Netherlands) B.V; Treatment of adrenoleukodystrophy

**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 of marketing authorisation extension

## 5.1. After adoption of CHMP opinion

None

## 5.2. Prior to adoption of CHMP opinion

### 5.2.1. Kaftrio - ivacaftor/tezacaftor/elexacaftor - EMEA/H/C/005269/II/0001, EMA/OD/0000001208, EU/3/18/2116, EMA/OD/0000042077

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP Rapporteur: Johann Lodewijk Hillege

**Action:** For adoption

### 5.2.2. Kalydeco - ivacaftor - EMEA/H/C/002494/II/0089, EMA/OD/010/08, EU/3/08/556, EMA/OD/0000042076

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP Rapporteur: Maria Concepcion Prieto Yerro

**Action:** For adoption

## 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 12 March 2021 at 12:00

Document tabled:
PAWG draft agenda for 12 March 2021

## 7.2. Coordination with EMA Scientific Committees or CMDh-v

### 7.2.1. Recommendation on eligibility to PRIME – report from CHMP

**Action:** For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes February 2021

### 7.2.2. COMP-CAT Working Group

Proposed meeting time on 15 March 2021 at 17:30

**Action:** For discussion

Document(s) tabled: Agenda and related documents

## 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. 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

Translating Real-World Data into Real-World Evidence and its Applications to the Liver, PSC, and the Rare Diseases Forums

**Action:** For information

## 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 2021

**Action**: For information

### 7.8.2. Overview of orphan marketing authorisations/applications

**Action**: For information

# 8. Any other business

## 8.1. Inter-Committee SAG Oncology

**Action:** For discussion

## 8.2. EMA Business Pipeline activity and Horizon scanning

**Action:** For information

Document tabled:
Q1/2021 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/