15 January 2024
EMA/COMP/568809/2023
Human Medicines Division

# Committee for Orphan Medicinal Products (COMP)

## Draft agenda for the meeting on 16-18 January 2024

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

16 January 2024, 08:30-19:30, virtual meeting room

17 January 2024, 08:30-19:30, virtual meeting room

18 January 2024, 08:30-17:00, virtual meeting room

**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).

**Official address** Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
**Address for visits and deliveries** Refer to www.ema.europa.eu/how-to-find-us
**Send us a question** Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000
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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 16-18 January 2024. See January 2024 COMP minutes (to be published post February 2024 COMP meeting).

## 1.2. Adoption of agenda

COMP agenda for 16-18 January 2024.

## 1.3. Adoption of the minutes

COMP minutes for 05-07 December 2023.

# 2. Applications for orphan medicinal product designation

## 2.1. For opinion

### 2.1.1. - EMA/OD/0000149117

Treatment of cystic fibrosis

**Action:** For adoption, Oral explanation to be held on 16 January 2024 at 15:15

### 2.1.2. - EMA/OD/0000139765

Treatment of glioma

**Action:** For adoption, Oral explanation to be held on 16 January 2024 at 14:00

### 2.1.3. - EMA/OD/0000144999

Treatment of glioma

**Action:** For adoption, Oral explanation to be held on 17 January 2024 at 14:00

### 2.1.4. - EMA/OD/0000154242

Treatment of transthyretin-mediated amyloidosis

**Action:** For adoption, Oral explanation to be held on 17 January 2024 at 11:00

### 2.1.5. - EMA/OD/0000149156

Treatment of cholangiocarcinoma

**Action:** For adoption, Oral explanation to be held on 17 January 2024 at 09:00

## 2.2. For discussion / preparation for an opinion

### 2.2.1. - EMA/OD/0000140382

Treatment of ovarian cancer

**Action:** For discussion/adoption

### 2.2.2. - EMA/OD/0000142006

Treatment of mesothelioma

**Action:** For discussion/adoption

### 2.2.3. - EMA/OD/0000145757

Treatment of small cell lung cancer

**Action:** For discussion/adoption

### 2.2.4. - EMA/OD/0000146222

Treatment of Berardinelli-Seip syndrome (congenital generalised lipodystrophy)

**Action:** For discussion/adoption

### 2.2.5. - EMA/OD/0000147176

Treatment of gastric cancer

**Action:** For discussion/adoption

### 2.2.6. - EMA/OD/0000147895

Treatment of Lawrence syndrome (acquired generalised lipodystrophy)

**Action:** For discussion/adoption

### 2.2.7. - EMA/OD/0000150709

Treatment of pilonidal disease

**Action:** For discussion/adoption

### 2.2.8. - EMA/OD/0000155761

Treatment of focal segmental glomerulosclerosis

**Action:** For discussion/adoption

### 2.2.9. - EMA/OD/0000156328

Prevention of hereditary angioedema

**Action:** For discussion/adoption

### 2.2.10. - EMA/OD/0000156633

Treatment of soft tissue sarcoma

**Action:** For discussion/adoption

### 2.2.11. - EMA/OD/0000156752

Treatment of acute respiratory distress syndrome

**Action:** For discussion/adoption

### 2.2.12. - EMA/OD/0000157053

Treatment of AL amyloidosis

**Action:** For discussion/adoption

### 2.2.13. - EMA/OD/0000157307

Treatment of fibrodysplasia ossificans progressiva

**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

OMPD applications - appointment of rapporteurs at the 16-18 January 2024 COMP meeting

## 2.7. Evaluation on-going

8 applications for orphan designation will not be discussed as evaluation is ongoing.

**Action**: For information

# 3. Requests for protocol assistance with significant benefit question

## 3.1. Ongoing procedures

### 3.1.1. -

Treatment of malignant mesothelioma

**Action:** For adoption

# 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. – leriglitazone - EMEA/H/C/005757, EU/3/16/1770, EMA/OD/0000144315

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

**Action:** For discussion/adoption

### 4.2.2. – danicopan - EMEA/H/C/005517, EU/3/17/1946, EMA/OD/0000136076

Alexion Europe SAS; Treatment of paroxysmal nocturnal haemoglobinuria

**Action:** For discussion/adoption

## 4.3. Appeal

None

## 4.4. On-going procedures

**Action**: For information

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. Abecma – idecabtagene vicleucel - EMEA/H/C/004662/II/0031, EU/3/17/1863, EMA/OD/0000132929

Bristol-Myers Squibb; Treatment of multiple myeloma

CAT Rapporteur: Rune Kjeken; CAT Co-Rapporteur: Heli Suila

**Action:** For discussion/adoption

### 5.2.2. Aspaveli – pegcetacoplan - EMEA/H/C/005553/II/0011, EU/3/17/1873, EMA/OD/0000140083

Swedish Orphan Biovitrum AB (publ); Treatment of paroxysmal nocturnal haemoglobinuria

CHMP Rapporteur: Alexandre Moreau; CHMP Co-Rapporteur: Selma Arapovic

**Action:** For discussion/adoption

## 5.3. Appeal

None

## 5.4. On-going procedures

**Action**: For information

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. COMP membership

**Action:** For information

### 7.1.2. Vote by proxy

**Action:** For information

### 7.1.3. Strategic Review & Learning meetings

SRLM meeting in Leuven under the Belgian Presidency of the Council of the EU

**Action**: For discussion

### 7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 15 January 2024 at 10:00

PAWG draft agenda for 15 January 2024 meeting

### 7.1.5. COMP Decisions Database

**Action:** For discussion

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

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

PRIME eligibility requests - list of adopted outcomes December 2023

## 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)

None

### 7.3.2. Upcoming Innovation Task Force (ITF) meetings

**Action**: For discussion

Upcoming ITF meetings

## 7.4. Cooperation within the EU regulatory network

### 7.4.1. European Commission

None

### 7.4.2. Feedback from the European Network of Centres for Pharmacoepidemiology and Pharmacovigilance (ENCePP) Plenary

**Action**: For discussion

## 7.5. Cooperation with International Regulators

### 7.5.1. Food and Drug Administration (FDA)

None

### 7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

### 7.5.3. Therapeutic Goods Administration (TGA), Australia

None

### 7.5.4. Health Canada

None

## 7.6. Contacts of the COMP with external parties and interaction with the Interested Parties to the Committee

None

## 7.7. COMP work plan

### 7.7.1. Work plan for 2024

**Action**: For discussion/adoption

## 7.8. Planning and reporting

### 7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2024

**Action**: For information

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

**Action**: For information

# 8. Any other business

## 8.1. New tool for searching scientific advice - Scientific Explorer

**Action**: For discussion

## 8.2. Ultra-rare diseases project

**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.

For a list of acronyms and abbreviations, see:

Abbreviations used in EMA scientific committees & CMD documents and in relation to EMA's regulatory activities

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/