01 November 2024
EMA/COMP/487032/2024
Human Medicines Division

# Committee for Orphan Medicinal Products (COMP)

## Draft agenda for the meeting on 05-07 November 2024

Chair: Tim Leest – Vice-Chair: Frauke Naumann-Winter

05 November 2024, 09:30-19:30, room 2A

06 November 2024, 08:30-19:30, room 2A

07 November 2024, 08:30-17:00, room 2A

**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 05-07 November 2024. See November 2024 COMP minutes (to be published post December 2024 COMP meeting).

## 1.2. Adoption of agenda

COMP agenda for 05-07 November 2024.

## 1.3. Adoption of the minutes

COMP minutes for 08-10 October 2024.

# 2. Applications for orphan medicinal product designation

## 2.1. For opinion

### 2.1.1. - EMA/OD/0000182883

Treatment of AL amyloidosis

**Action:** For information

Note: Withdrawal request received on 18 October 2024.

### 2.1.2. - EMA/OD/0000175842

Treatment of Duchenne muscular dystrophy

**Action:** For adoption, Oral explanation to be held on 05 November 2024 at 14:00

### 2.1.3. - EMA/OD/0000183952

Treatment of Duchenne muscular dystrophy

**Action:** For adoption, Oral explanation to be held on 05 November 2024 at 10:15

### 2.1.4. - EMA/OD/0000222362

Treatment of pulmonary arterial hypertension

**Action:** For information

Note: Withdrawal request received on 18 October 2024.

### 2.1.5. - EMA/OD/0000182940

Treatment of sickle cell disease

**Action:** For adoption, Oral explanation to be held on 06 November 2024 at 16:15

### 2.1.6. - EMA/OD/0000177828

Treatment of cystic fibrosis

**Action:** For adoption, Oral explanation to be held on 06 November 2024 at 09:00

### 2.1.7. - EMA/OD/0000175548

Treatment of focal segmental glomerulosclerosis

**Action:** For adoption, Oral explanation to be held on 06 November 2024 at 14:00

### 2.1.8. - EMA/OD/0000222517

Treatment of autoimmune haemolytic anaemia

**Action:** For information

Note: Withdrawal request received on 17 October 2024.

### 2.1.9. - EMA/OD/0000179368

Treatment of soft tissue sarcoma

**Action:** For information

Note: Withdrawal request received on 21 October 2024.

### 2.1.10. - EMA/OD/0000222144

Treatment in solid organ transplantation

**Action:** For adoption

## 2.2. For discussion / preparation for an opinion

### 2.2.1. - EMA/OD/0000166372

Treatment of polycythaemia vera

**Action:** For discussion/adoption

### 2.2.2. - EMA/OD/0000182637

Treatment of hypochondroplasia

**Action:** For discussion/adoption

### 2.2.3. - EMA/OD/0000183015

Treatment of uveal melanoma

**Action:** For discussion/adoption

### 2.2.4. - EMA/OD/0000184238

Treatment of Huntington's disease

**Action:** For discussion/adoption

### 2.2.5. - EMA/OD/0000222656

Treatment of Duchenne muscular dystrophy

**Action:** For discussion/adoption

### 2.2.6. - EMA/OD/0000223155

Treatment of pancreatic cancer

**Action:** For discussion/adoption

### 2.2.7. - EMA/OD/0000223853

Treatment of primary biliary cholangitis (PBC)

**Action:** For discussion/adoption

### 2.2.8. - EMA/OD/0000224328

Treatment of amyotrophic lateral sclerosis

**Action:** For discussion/adoption

### 2.2.9. - EMA/OD/0000224413

Treatment of neurofibromatosis type 2

**Action:** For discussion/adoption

### 2.2.10. - EMA/OD/0000224798

Treatment of congenital alpha-1 antitrypsin deficiency

**Action:** For discussion/adoption

### 2.2.11. - EMA/OD/0000225406

Treatment of inherited retinal dystrophies due to dysfunction in the *ABCA4* gene

**Action:** For discussion/adoption

### 2.2.12. - EMA/OD/0000226175

Treatment of activity-dependent neuroprotective protein (ADNP) syndrome

**Action:** For discussion/adoption

## 2.2.13. - EMA/OD/0000226273

Treatment of glycogen storage disease type I

**Action:** For discussion/adoption

## 2.2.14. - EMA/OD/0000226506

Treatment of inherited retinal dystrophy due to defects in the *RPE65* gene

**Action:** For discussion/adoption

## 2.2.15. - EMA/OD/0000226832

Treatment of chronic pancreatitis

**Action:** For discussion/adoption

## 2.2.16. - EMA/OD/0000227422

Treatment of non-traumatic osteonecrosis

**Action:** For discussion/adoption

## 2.2.17. - EMA/OD/0000227727

Treatment of autosomal dominant polycystic kidney disease

**Action:** For discussion/adoption

## 2.2.18. - EMA/OD/0000227741

Treatment of hepatitis delta virus infection

**Action:** For discussion/adoption

## 2.2.19. - EMA/OD/0000227841

Treatment of myasthenia gravis

**Action:** For discussion/adoption

## 2.2.20. - EMA/OD/0000227852

Treatment of hepatitis delta virus infection

**Action:** For discussion/adoption

## 2.2.21. - EMA/OD/0000227928

Treatment of glioma

**Action:** For discussion/adoption

### 2.2.22. - EMA/OD/0000228058

Treatment of autosomal recessive congenital ichthyosis (ARCI)

**Action:** For discussion/adoption

### 2.2.23. - EMA/OD/0000228105

Treatment of acute promyelocytic leukaemia

**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 05-07 November 2024 COMP meeting

## 2.7. Evaluation on-going

2 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

None

# 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. Wainzua - eplontersen - EMEA/H/C/006295, EU/3/23/2828, EMA/OD/0000177780

AstraZeneca AB; Treatment of transthyretin-mediated amyloidosis

**Action:** For adoption, Oral explanation to be held on 05 November 2024 at 11:45

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

### 4.2.1. - imetelstat - EMEA/H/C/006105, EU/3/20/2305, EMA/OD/0000225798

Geron Netherlands B.V.; Treatment of myelodysplastic syndromes

**Action:** For discussion/adoption

### 4.2.2. Pemazyre - pemigatinib - EMEA/H/C/005266/II/0015, EU/3/19/2216, EMA/OD/0000167021

Incyte Biosciences; Treatment of myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2

CHMP Rapporteur: Alexandre Moreau; CHMP Co-Rapporteur: Janet Koenig

**Action:** For discussion/adoption

### 4.2.3. - garadacimab - EMEA/H/C/006116, EU/3/21/2532, EMA/OD/0000133460

CSL Behring GmbH; Treatment of hereditary angioedema

**Action:** For discussion/adoption

### 4.2.4. - beremagene geperpavec - EMEA/H/C/006330, EU/3/18/2012, EMA/OD/0000233504

Krystal Biotech Netherlands; Treatment of epidermolysis bullosa

**Action:** For discussion/adoption

### 4.2.5. - seladelpar lysine dihydrate - EMEA/H/C/004692, EU/3/17/1930, EMA/OD/0000170646

Cymabay Ireland Limited; Treatment of primary biliary cholangitis

**Action:** For discussion/adoption

### 4.2.6. - tiratricol - EMEA/H/C/005220, EU/3/17/1945, EMA/OD/0000168628

Rare Thyroid Therapeutics; Treatment of monocarboxylate transporter 8 (MCT8) deficiency

**Action:** For discussion/adoption

### 4.2.7. - acoramidis - EMEA/H/C/006333, EU/3/18/2081, EMA/OD/0000224696

BridgeBio Europe B.V.; Treatment of ATTR amyloidosis

**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

None

## 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

Scope: Preliminary feedback from the SRLM meeting in Budapest under the Hungarian Presidency of the Council of the EU

**Action:** For discussion

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

None

### 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 October 2024

## 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)

Scope: Call for COMP representatives to PCWP and HCPWP

**Action**: For adoption

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

Scope: Upcoming ITF meetings

**Action**: For discussion

## 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)

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. Draft COMP Work Plan for 2025

COMP Chair: Tim Leest

**Action**: For discussion

## 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. Patient engagement methodologies

**Action**: For 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.

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/