09 September 2024
EMA/COMP/347157/2024
Human Medicines Division

# Committee for Orphan Medicinal Products (COMP)

## Draft agenda for the meeting on 10-12 September 2024

Chair: Violeta Stoyanova-Beninska

10 September 2024, 09:00-19:30, room 2A

11 September 2024, 08:30-19:30, room 2A

12 September 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 10-12 September 2024. See September 2024 COMP minutes (to be published post October 2024 COMP meeting).

## 1.2. Adoption of agenda

COMP agenda for 10-12 September 2024.

## 1.3. Adoption of the minutes

COMP minutes for 16-18 July 2024.

# 2. Applications for orphan medicinal product designation

## 2.1. For opinion

### 2.1.1. - EMA/OD/0000174573

Treatment of chondrosarcoma

**Action:** For adoption

### 2.1.2. - EMA/OD/0000154786

Prevention of arteriovenous access dysfunction in patients undergoing surgical creation of an arteriovenous fistula for hemodialysis

**Action:** For adoption, Oral explanation to be held on 10 September 2024 at 15:00

### 2.1.3. - EMA/OD/0000178304

Treatment of pouchitis

**Action:** For adoption, Oral explanation to be held on 11 September 2024 at 09:15

### 2.1.4. - EMA/OD/0000174349

Treatment of complex regional pain syndrome

**Action:** For information

Note: Withdrawal request received on 27 August 2024.

### 2.1.5. - EMA/OD/0000171663

Treatment of cystic fibrosis

**Action:** For adoption, Oral explanation to be held on 11 September 2024 at 14:15

### 2.1.6. - EMA/OD/0000173551

Treatment of Wilson's disease

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

### 2.1.7. - EMA/OD/0000179561

Treatment of cutaneous T-cell lymphoma (CTCL)

**Action:** For adoption, Oral explanation to be held on 10 September 2024 at 11:30

### 2.1.8. - EMA/OD/0000160667

Treatment of anti-neutrophil cytoplasmic antibody (ANCA) – associated vasculitis (AAV)

**Action:** For adoption, Oral explanation to be held on 10 September 2024 at 16:30

## 2.2. For discussion / preparation for an opinion

### 2.2.1. - EMA/OD/0000172976

Treatment of pancreatic cancer

**Action:** For discussion/adoption

### 2.2.2. - EMA/OD/0000175107

Treatment of glioma

**Action:** For discussion/adoption

### 2.2.3. - EMA/OD/0000175157

Treatment of idiopathic pulmonary fibrosis

**Action:** For discussion/adoption

### 2.2.4. - EMA/OD/0000178549

Treatment of hereditary angioedema

**Action:** For discussion/adoption

### 2.2.5. - EMA/OD/0000178877

Treatment of myotonic dystrophies

**Action:** For discussion/adoption

### 2.2.6. - EMA/OD/0000179156

Treatment of frontotemporal dementia

**Action:** For discussion/adoption

### 2.2.7. - EMA/OD/0000179541

Treatment of retinoblastoma

**Action:** For discussion/adoption

### 2.2.8. - EMA/OD/0000179931

Treatment of autosomal dominant polycystic liver disease

**Action:** For discussion/adoption

### 2.2.9. - EMA/OD/0000179978

Treatment of ATTR amyloidosis

**Action:** For discussion/adoption

### 2.2.10. - EMA/OD/0000180597

Treatment of immunoglobulin A nephropathy (IgAN)

**Action:** For discussion/adoption

### 2.2.11. - EMA/OD/0000182415

Treatment of amyotrophic lateral sclerosis

**Action:** For discussion/adoption

### 2.2.12. - EMA/OD/0000182648

Treatment of spinal and bulbar muscular atrophy

**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 10-12 September 2024 COMP meeting

## 2.7. Evaluation on-going

None

# 3. Requests for protocol assistance with significant benefit question

## 3.1. Ongoing procedures

### 3.1.1. -

Treatment of hereditary angioedema

**Action:** For adoption

### 3.1.2. -

Treatment of ovarian cancer

**Action:** For adoption

### 3.1.3. -

Treatment of Dravet syndrome

**Action:** For adoption

### 3.1.4. -

Treatment of STXBP1 developmental and epileptic encephalopathy

**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. - serplulimab - EMEA/H/C/006170, EU/3/22/2731, EMA/OD/0000155775

Henlius Europe GmbH; Treatment of small cell lung cancer

**Action:** For discussion/adoption

### 4.2.2. - vorasidenib - EMEA/H/C/006284, EU/3/22/2737, EMA/OD/0000159477

Les Laboratoires Servier; Treatment of glioma

**Action:** For discussion/adoption

### 4.2.3. - mirvetuximab soravtansine - EMEA/H/C/005036, EU/3/15/1458, EMA/OD/0000178274

Abbvie Deutschland GmbH & Co. KG; Treatment of ovarian cancer

**Action:** For discussion/adoption

### 4.2.4. - marstacimab - EMEA/H/C/006240

Pfizer Europe MA EEIG

a) Treatment of haemophilia B, EU/3/23/2866, EMA/OD/0000179102

**Action:** For discussion/adoption

b) Treatment of haemophilia A, EU/3/16/1752, EMA/OD/0000179103

**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. Columvi - glofitamab - EMEA/H/C/005751/II/0005, EU/3/21/2497, EMA/OD/0000225358

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

**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 – Hungary

Update on the SRLM meeting to be held in Budapest on 29-30 October 2024

**Action**: For information

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

Proposed meeting time on 6 September 2024 at 09:00

PAWG draft agenda for 6 September 2024 meeting

### 7.1.5. COMP Decisions Database

**Action:** For discussion

### 7.1.6. Election of COMP Vice-Chairperson

**Action:** For adoption

## 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 July 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)

**Action:** For information

### 7.3.2. 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

## 7.5. 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. Update on 2024 work plan

Scope: Mid-year report

**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. Large B-cell lymphoma - condition and prevalence

**Action**: For information

## 8.2. The European Cancer Information System (ECIS) - Incidence, Mortality, Survival and Prevalence indicators

**Action**: For information

## 8.3. Committee meetings in Microsoft Teams and new tool for voting

**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/