Committee for Orphan Medicinal Products (COMP)
Draft agenda for the meeting on 05-07 December 2023

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

05 December 2023, 09:00-19:30, room 2A
06 December 2023, 08:30-19:30, room 2A
07 December 2023, 09:00-17:00, 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 ongoing 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).
# Table of contents

1. Introduction .................................................. 5
   1.1. Welcome and declarations of interest of members and experts .................. 5
   1.2. Adoption of agenda ........................................ 5
   1.3. Adoption of the minutes .................................... 5

2. Applications for orphan medicinal product designation .................................. 5
   2.1. For opinion .................................................. 5
       - EMA/OD/0000150558 ........................................ 5
       - EMA/OD/0000149464 ........................................ 5
       - EMA/OD/0000149222 ........................................ 5
       - EMA/OD/0000148755 ........................................ 5
       - EMA/OD/0000149631 ........................................ 5
       - EMA/OD/0000150398 ........................................ 5
       - EMA/OD/0000150249 ........................................ 5
   2.2. For discussion / preparation for an opinion .............................................. 6
       - EMA/OD/0000139765 ........................................ 6
       - EMA/OD/0000144447 ........................................ 6
       - EMA/OD/0000144999 ........................................ 6
       - EMA/OD/0000145710 ........................................ 6
       - EMA/OD/0000146101 ........................................ 6
       - EMA/OD/0000149117 ........................................ 6
       - EMA/OD/0000149156 ........................................ 6
       - EMA/OD/0000149689 ........................................ 7
       - EMA/OD/0000150090 ........................................ 7
       - EMA/OD/0000152508 ........................................ 7
       - EMA/OD/0000152994 ........................................ 7
       - EMA/OD/0000153654 ........................................ 7
       - EMA/OD/0000153667 ........................................ 7
       - EMA/OD/0000154030 ........................................ 7
       - EMA/OD/0000154059 ........................................ 7
       - EMA/OD/0000154242 ........................................ 7
       - EMA/OD/0000154838 ........................................ 7
   2.3. Revision of the COMP opinions ....................................................... 8
   2.4. Amendment of existing orphan designations ............................................ 8
   2.5. Appeal ..................................................................................... 8
   2.6. Nominations ............................................................................. 8
       - New applications for orphan medicinal product designation - Appointment of COMP rapporteurs ........................................ 8
2.7. Evaluation on-going........................................................................................................ 8

3. Requests for protocol assistance with significant benefit question 8

3.1. Ongoing procedures ........................................................................................................ 8
3.1.1. - .......................................................................................................................... 8
3.1.2. - .......................................................................................................................... 8

4. Review of orphan designation for orphan medicinal products at time of initial marketing authorisation 9

4.1. Orphan designated products for which CHMP opinions have been adopted .......... 9
4.1.1. Omjjara - momelotinib dihydrochloride - EMEA/H/C/005768/0000 ......................... 9

4.2. Orphan designated products for discussion prior to adoption of CHMP opinion ...... 9
4.2.1. Livmarli - maralixibat - EMEA/H/C/005857/II/0003/G, EU/3/13/1216, EMA/OD/0000136132 ................................................................. 9
4.2.2. – exagamglogene autotemcel - EMEA/H/C/005763 ........................................................................ 9
4.2.3. - sparsentan - EMEA/H/C/005783, EU/3/20/2345, EMA/OD/0000110380 .................. 9
4.2.4. – omaveloxolone - EMEA/H/C/006084, EU/3/18/2037, EMA/OD/0000156841 .......... 10

4.3. Appeal .................................................................................................................. 10

4.4. On-going procedures .............................................................................................. 10

4.5. Orphan Maintenance Reports ................................................................................ 10

5. Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension 10

5.1. After adoption of CHMP opinion ............................................................................. 10
5.2. Prior to adoption of CHMP opinion ........................................................................ 10
5.3. Appeal .................................................................................................................. 10
5.4. On-going procedures ............................................................................................. 10

6. Application of Article 8(2) of the Orphan Regulation 10

7. Organisational, regulatory and methodological matters 11

7.1. Mandate and organisation of the COMP .................................................................. 11
7.1.1. COMP membership ............................................................................................. 11
7.1.2. Vote by proxy ........................................................................................................ 11
7.1.3. Strategic Review & Learning meetings................................................................. 11
7.1.4. Protocol Assistance Working Group (PAWG) ..................................................... 11
7.1.5. COMP Decisions Database ................................................................................ 11

7.2. Coordination with EMA Scientific Committees or CMDh-v ................................. 11
7.2.1. Recommendation on eligibility to PRIME – report ........................................... 11

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups .......... 11
7.3.1. Working Party with Patients’ and Consumers’ Organisations (PCWP) and Working Party with Healthcare Professionals’ Organisations (HCPWP) .................................................. 11
7.3.2. Upcoming ITF meetings ...................................................................................... 11
7.4. Cooperation within the EU regulatory network ..................................................... 11
  7.4.1. European Commission .......................................................................................... 11

7.5. Cooperation with International Regulators ............................................................ 12
  7.5.1. Food and Drug Administration (FDA) ....................................................................... 12
  7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA) ......................... 12
  7.5.3. Therapeutic Goods Administration (TGA), Australia ............................................ 12
  7.5.4. Health Canada ........................................................................................................ 12

7.6. Contacts of the COMP with external parties and interaction with the Interested
     Parties to the Committee .............................................................................................. 12

7.7. COMP work plan ....................................................................................................... 12

7.8. Planning and reporting ............................................................................................. 12
  7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid
         applications submitted in 2023 .................................................................................. 12
  7.8.2. Overview of orphan marketing authorisations/applications ..................................... 12

8. Any other business ........................................................................................................ 12
  8.1. Update on the patient experience data - major contribution to patient care project
       ..................................................................................................................................... 12
  8.2. EMA business pipeline activity ................................................................................ 12

9. Explanatory notes ......................................................................................................... 13
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 December 2023. See December 2023 COMP minutes (to be published post January 2024 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 05-07 December 2023.

1.3. Adoption of the minutes

COMP minutes for 07-09 November 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000150558

Treatment of eosinophilic esophagitis

**Action:** For adoption, Oral explanation to be held on 06 December 2023 at 12:00

2.1.2. - EMA/OD/0000149464

Treatment of hyperinsulinism

**Action:** For adoption, Oral explanation to be held on 05 December 2023 at 14:00

2.1.3. - EMA/OD/0000149222

Treatment of diffuse large B-cell lymphoma

**Action:** For adoption, Oral explanation to be held on 05 December 2023 at 15:30

2.1.4. - EMA/OD/0000148755

Treatment of amyotrophic lateral sclerosis

**Action:** For adoption, Oral explanation to be held on 06 December 2023 at 09:00

2.1.5. - EMA/OD/0000149631

Treatment of amyotrophic lateral sclerosis

**Action:** For adoption, Oral explanation to be held on 06 December 2023 at 10:30
2.1.6. - EMA/OD/0000150398

Treatment of eosinophilic granulomatosis with polyangiitis (EGPA)

**Action:** For adoption, Oral explanation to be held on 05 December 2023 at 12:00

2.1.7. - EMA/OD/0000150249

Treatment of autosomal dominant polycystic kidney disease

**Action:** For adoption, Oral explanation to be held on 07 December 2023 at 09:30

2.2. **For discussion / preparation for an opinion**

2.2.1. - EMA/OD/0000139765

Treatment of glioma

**Action:** For discussion/adoption

2.2.2. - EMA/OD/0000144447

Treatment of systemic sclerosis

**Action:** For discussion/adoption

2.2.3. - EMA/OD/0000144999

Treatment of glioma

**Action:** For discussion/adoption

2.2.4. - EMA/OD/0000145710

Diagnosis of glioma

**Action:** For discussion/adoption

2.2.5. - EMA/OD/0000146101

Treatment of small cell lung cancer

**Action:** For discussion/adoption

2.2.6. - EMA/OD/0000149117

Treatment of cystic fibrosis

**Action:** For discussion/adoption

2.2.7. - EMA/OD/0000149156

Treatment of cholangiocarcinoma
<table>
<thead>
<tr>
<th>Number</th>
<th>EMA/OD/xxxxxx</th>
<th>Condition</th>
<th>Action</th>
</tr>
</thead>
<tbody>
<tr>
<td>2.2.8</td>
<td>EMA/OD/0000149689</td>
<td>Treatment of epidermolysis bullosa</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.9</td>
<td>EMA/OD/0000150090</td>
<td>Treatment of acute myeloid leukaemia</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.10</td>
<td>EMA/OD/0000152508</td>
<td>Treatment of alpha-thalassaemia X-linked intellectual disability</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.11</td>
<td>EMA/OD/0000152994</td>
<td>Treatment of Rett syndrome</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.12</td>
<td>EMA/OD/0000153654</td>
<td>Treatment of mobilisation of progenitor cells prior to stem cell transplantation</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.13</td>
<td>EMA/OD/0000153667</td>
<td>Treatment of acquired factor X deficiency</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.14</td>
<td>EMA/OD/0000154030</td>
<td>Treatment of recurrent respiratory papillomatosis</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.15</td>
<td>EMA/OD/0000154059</td>
<td>Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)</td>
<td>For discussion/adoption</td>
</tr>
<tr>
<td>2.2.16</td>
<td>EMA/OD/0000154242</td>
<td>Treatment of ATTR amyloidosis</td>
<td>For discussion/adoption</td>
</tr>
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</table>
**Action:** For discussion/adoption

2.2.17. **EMA/OD/0000154838**

Treatment of thalassaemia alpha intermedia and major

**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 December 2023 COMP meeting

2.7. **Evaluation on-going**

12 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 gastrointestinal stromal tumours

**Action:** For adoption

3.1.2. -

Treatment of immune thrombocytopenia

**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**

4.1.1. **Omjjara - momelotinib dihydrochloride** - EMEA/H/C/005768/0000

GlaxoSmithKline Trading Services Limited
- a) Treatment of post-polycythaemia vera myelofibrosis, EU/3/11/886, EMA/OD/0000129901

**Action:** For adoption, Oral explanation to be held on 06 December 2023 at 14:00
- b) Treatment of post-essential thrombocythaemia myelofibrosis, EU/3/11/887, EMA/OD/0000130955

**Action:** For adoption, Oral explanation to be held on 06 December 2023 at 14:00
- c) Treatment of primary myelofibrosis, EU/3/11/888, EMA/OD/0000130957

**Action:** For adoption, Oral explanation to be held on 06 December 2023 at 14:00

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

4.2.1. **Livmarli - maralixibat** - EMEA/H/C/005857/II/0003/G, EU/3/13/1216, EMA/OD/0000136132

Miran Pharmaceuticals International B.V.; Treatment of progressive familial intrahepatic cholestasis

**Action:** For information

4.2.2. **- exagamglogene autotemcel** - EMEA/H/C/005763

Vertex Pharmaceuticals (Ireland) Limited
- a) Treatment of sickle cell disease, EU/3/19/2242, EMA/OD/0000146415

**Action:** For discussion/adoption
- b) Treatment of beta-thalassaemia intermedia and major, EU/3/19/2210, EMA/OD/0000146264

**Action:** For discussion/adoption

4.2.3. **- sparsentan** - EMEA/H/C/005783, EU/3/20/2345, EMA/OD/0000110380

Vifor France; Treatment of primary IgA nephropathy

**Action:** For discussion/adoption
4.2.4.  omaveloxolone - EMEA/H/C/006084, EU/3/18/2037, EMA/OD/0000156841

Reata Ireland Limited; Treatment of Friedreich’s ataxia

**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

None

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 01 December at 13:00
PAWG draft agenda for 01 December 2023 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 November 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)

Feedback from PCWP/HCPWP and all eligible meeting - 14 and 15 November 2023

7.3.2. Upcoming 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

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 2023

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Update on the patient experience data - major contribution to patient care project

Action: For discussion

8.2. EMA business pipeline activity

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.

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