18 March 2025
EMA/COMP/77031/2025
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 18-20 March 2025

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

18 March 2025, room 2A

19 March 2025, room 2A

20 March 2025, 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 18-20 March 2025. See March 2025 COMP minutes (to be published post April 2025 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 18-20 March 2025.

1.3. Adoption of the minutes

COMP minutes for 18-19 February 2025.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000236836

Treatment of small cell lung cancer

Action: For information

Notes: Withdrawal request received on 3 March 2025.

2.1.2. - EMA/OD/0000235476

Treatment of non-infectious uveitis

Action: For adoption, Oral explanation to be held on 19 March 2025 at 09:30

2.1.3. - EMA/OD/0000236424

Treatment of primary IgA nephropathy

Action: For adoption, Oral explanation to be held on 19 March 2025 at 14:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000166069

Prevention of foetal and neonatal alloimmune thrombocytopenia

Action: For discussion/adoption

2.2.2. - EMA/OD/0000236117

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.3. - EMA/OD/0000236884

Treatment of spinal muscular atrophy

Action: For discussion/adoption

2.2.4. - EMA/OD/0000239285

Diagnosis of ATTR amyloidosis

Action: For discussion/adoption

2.2.5. - EMA/OD/0000239625

Treatment of pre-eclampsia

Action: For discussion/adoption

2.2.6. - EMA/OD/0000240898

Treatment of familial adenomatous polyposis

Action: For discussion/adoption

2.2.7. - EMA/OD/0000241887

Treatment of Duchenne muscular dystrophy and Becker muscular dystrophy

Action: For discussion/adoption

2.2.8. - EMA/OD/0000242100

Treatment of primary ubiquinone deficiency (PUD)

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 18-20 March 2025 COMP meeting

2.7. Evaluation on-going

15 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 beta-thalassaemia intermedia and major

Action: For adoption

3.1.2. -

Treatment of sickle cell disease

Action: For adoption

3.1.3. -

Treatment of graft-versus-host disease

Action: For adoption

3.1.4. -

Treatment of pancreatic cancer

Action: For adoption

3.1.5. -

Diagnosis of marginal zone lymphoma

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. - zanidatamab - EMEA/H/C/006380, EU/3/21/2458, EMA/OD/0000241913

Jazz Pharmaceuticals Ireland Limited; Treatment of biliary tract cancer

Action: For discussion/adoption

4.2.2. - givinostat - EMEA/H/C/006079, EU/3/12/1009, EMA/OD/0000178186

Italfarmaco S.p.A.; Treatment of Duchenne muscular dystrophy

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

5.1.1. 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

CHMP Rapporteur: Boje Kvorning Pires Ehmsen

Action: For adoption, Oral explanation to be held on 18 March 2025 at 14:30

5.1.2. Kaftrio - ivacaftor / tezacaftor / elexacaftor - EMEA/H/C/005269/II/0042/G, EU/3/18/2116, EMA/OD/0000160021

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP Rapporteur: Peter Mol

Action: For discussion/adoption

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: Update on the SRLM meeting to be held face-to-face on 29-30 April 2025 in Warsaw, Poland

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 14 March 2025 at 10:00

PAWG draft agenda for 14 March 2025 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 February 2025

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

7.3.1. Patients and Consumers Working Party (PCWP) Healthcare Professionals Working Party (HCPWP)

7.3.1.1. Updates to Rules of Procedure and mandates for PCWP and HCPWP

Scope: Presentation on updates to Rules of Procedures and mandates for PCWP and HCPWP

Action: For adoption

7.3.1.2. Agenda and Minutes

Scope: Meeting summary of the PCWP/HCPWP and all eligible organisations meeting held on 20 November 2024 and draft agenda of the PCWP-HCPWP meeting to be held on 1-2 April 2025

Action: For information

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

7.4.2. 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

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 2025

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. EMA rules of reimbursement

Action: For information

8.2. Revision of EMA policy 0044 on handling of competing interests

Scope: The main changes in the revision of policy 0044, the updated declaration of interests form and the next steps for experts will be presented

Action: For information

8.3. Revisions made to the reflection paper on real-world evidence (RWE)

Scope: Present the main changes introduced to the reflection paper on use of real-world data (RWD) in non-interventional studies (NIS) to generate RWE for regulatory purposes after the public consultation

Action: For information

8.4. Draft reflection paper on Patient Experience Data (PED) for internal consultation

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/