



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

17 February 2025
EMA/COMP/42497/2025
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 18-19 February 2025

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

18 February 2025, virtual meeting room

19 February 2025, 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

An agency of the European
Union



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
2.1.1.	- EMA/OD/0000230735	5
2.1.2.	- EMA/OD/0000232365	5
2.1.3.	- EMA/OD/0000233979	5
2.2.	For discussion / preparation for an opinion.....	5
2.2.1.	- EMA/OD/0000224127	5
2.2.2.	- EMA/OD/0000227245	5
2.2.3.	- EMA/OD/0000228898	6
2.2.4.	- EMA/OD/0000229932	6
2.2.5.	- EMA/OD/0000232820	6
2.2.6.	- EMA/OD/0000235476	6
2.2.7.	- EMA/OD/0000236424	6
2.2.8.	- EMA/OD/0000236836	6
2.2.9.	- EMA/OD/0000237565	6
2.2.10.	- EMA/OD/0000237791	6
2.3.	Revision of the COMP opinions	6
2.4.	Amendment of existing orphan designations.....	7
2.5.	Appeal	7
2.6.	Nominations	7
2.6.1.	New applications for orphan medicinal product designation - Appointment of COMP rapporteurs.....	7
2.7.	Evaluation on-going.....	7
3.	Requests for protocol assistance with significant benefit question	7
3.1.	Ongoing procedures	7
3.1.1.	-	7
3.1.2.	-	7
3.1.3.	-	7
3.1.4.	-	7
4.	Review of orphan designation for orphan medicinal products at time of initial marketing authorisation	8
4.1.	Orphan designated products for which CHMP opinions have been adopted	8
4.2.	Orphan designated products for discussion prior to adoption of CHMP opinion	8

4.2.1.	Kizfizo - temozolomide - EMEA/H/C/006169, EU/3/19/2188, EMA/OD/0000178760	8
4.2.2.	Fabhalta - iptacopan hydrochloride - EMEA/H/C/005764/II/0001, EU/3/18/2104, EMA/OD/0000224423	8
4.2.3.	- beremagene geperpavec - EMEA/H/C/006330, EU/3/18/2012, EMA/OD/0000233504	8
4.3.	Appeal	8
4.4.	On-going procedures	8
4.5.	Orphan Maintenance Reports.....	8
5.	Review of orphan designation for authorised orphan medicinal products at time marketing authorisation extension	9
5.1.	After adoption of CHMP opinion	9
5.2.	Prior to adoption of CHMP opinion	9
5.2.1.	Columvi - glofitamab - EMEA/H/C/005751/II/0005, EU/3/21/2497, EMA/OD/0000225358 ..	9
5.3.	Appeal	9
5.4.	On-going procedures	9
6.	Application of Article 8(2) of the Orphan Regulation	9
7.	Organisational, regulatory and methodological matters	9
7.1.	Mandate and organisation of the COMP	9
7.1.1.	COMP membership.....	9
7.1.2.	Vote by proxy	9
7.1.3.	Strategic Review & Learning meetings.....	9
7.1.4.	Protocol Assistance Working Group (PAWG)	10
7.1.5.	COMP Decisions Database.....	10
7.1.6.	Committee Meeting Dates for 2027-2028.....	10
7.1.7.	COMP Agenda in IRIS.....	10
7.2.	Coordination with EMA Scientific Committees or CMDh-v	10
7.2.1.	Recommendation on eligibility to PRIME – report	10
7.3.	Coordination with EMA Working Parties/Working Groups/Drafting Groups	10
7.3.1.	Working Party with Patients’ and Consumers’ Organisations (PCWP) and Working Party with Healthcare Professionals’ Organisations (HCPWP)	10
7.3.2.	Innovation Task Force (ITF) meetings	10
7.4.	Cooperation within the EU regulatory network.....	10
7.4.1.	European Commission	10
7.5.	Cooperation with International Regulators.....	11
7.5.1.	Food and Drug Administration (FDA)	11
7.5.2.	Japanese Pharmaceuticals and Medical Devices Agency (PMDA).....	11
7.5.3.	Therapeutic Goods Administration (TGA), Australia	11
7.5.4.	Health Canada.....	11
7.6.	Contacts of the COMP with external parties and interaction with the Interested Parties to the Committee.....	11

7.7.	COMP work plan	11
7.8.	Planning and reporting	11
7.8.1.	List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2025	11
7.8.2.	Overview of orphan marketing authorisations/applications.....	11
8.	Any other business	11
8.1.	EURORDIS activities and rare disease day campaign.....	11
9.	Explanatory notes	11

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 18-19 February 2025. See February 2025 COMP minutes (to be published post March 2025 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 18-19 February 2025.

1.3. Adoption of the minutes

COMP minutes for 21-23 January 2025.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000230735

Treatment of Duchenne muscular dystrophy

Action: For information

Note: Withdrawal received on 04 February 2025.

2.1.2. - EMA/OD/0000232365

Treatment of bradykinin-mediated angioedema

Action: For adoption, Oral explanation to be held on 18 February 2025 at 14:00

2.1.3. - EMA/OD/0000233979

Treatment of Rett syndrome

Action: For adoption, Oral explanation to be held on 19 February 2025 at 10:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000224127

Treatment of immune thrombocytopenia

Action: For discussion/adoption

2.2.2. - EMA/OD/0000227245

Treatment of non-traumatic spontaneous intracerebral haemorrhage

Action: For discussion/adoption

2.2.3. - [EMA/OD/0000228898](#)

Treatment of haemophilia B

Action: For discussion/adoption

2.2.4. - [EMA/OD/0000229932](#)

Treatment of haemophilia B

Action: For discussion/adoption

2.2.5. - [EMA/OD/0000232820](#)

Treatment of Marfan syndrome

Action: For discussion/adoption

2.2.6. - [EMA/OD/0000235476](#)

Treatment of non-infectious uveitis

Action: For discussion/adoption

2.2.7. - [EMA/OD/0000236424](#)

Treatment of primary IgA nephropathy

Action: For discussion/adoption

2.2.8. - [EMA/OD/0000236836](#)

Treatment of small cell lung cancer

Action: For discussion/adoption

2.2.9. - [EMA/OD/0000237565](#)

Treatment of primary IgA nephropathy

Action: For discussion/adoption

2.2.10. - [EMA/OD/0000237791](#)

Treatment of myelodysplastic syndromes (MDS)

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

OMP applications - appointment of rapporteurs at the 18-19 February 2025 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 erythropoietic protoporphyria

Action: For adoption

3.1.2. -

Diagnosis of marginal zone lymphoma

Action: For adoption

3.1.3. -

Treatment of tuberculosis

Action: For adoption

3.1.4. -

Treatment of Berardinelli-Seip syndrome (congenital generalised lipodystrophy) / Treatment of Lawrence syndrome (acquired generalised lipodystrophy)

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. Kizfizo - temozolomide - EMEA/H/C/006169, EU/3/19/2188, EMA/OD/0000178760

Orphelia Pharma S.A.S.; Treatment of neuroblastoma

Action: For discussion/adoption

4.2.2. Fabhalta - iptacopan hydrochloride - EMEA/H/C/005764/II/0001, EU/3/18/2104, EMA/OD/0000224423

Novartis Europharm Limited; Treatment of C3 glomerulopathy

Action: For adoption

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

Krystal Biotech Netherlands B.V.; Treatment of epidermolysis bullosa

Action: For 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

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

Action: For information

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 14 February 2025 at 10:00

PAWG draft agenda for 14 February 2025 meeting

7.1.5. COMP Decisions Database

Action: For discussion

7.1.6. Committee Meeting Dates for 2027-2028

Scope: Proposed committee meeting dates for the period of 2027-2028

Action: For information

7.1.7. COMP Agenda in IRIS

Scope: The Committee will be informed on the upcoming change and impact of the agenda in IRIS, and receives a demo of IRIS Network Portal features

Action: For discussion

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

None

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. Innovation Task Force (ITF) meetings

Scope: Upcoming ITF meetings and overview of the ITF activities in 2024

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

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. EURORDIS activities and rare disease day campaign

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/