17 May 2024
EMA/COMP/176815/2024
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 21-23 May 2024

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

21 May 2024, 08:30-19:30, virtual meeting room

22 May 2024, 08:30-19:30, virtual meeting room

23 May 2024, 08:30-17:00, 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).

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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 21-23 May 2024. See May 2024 COMP minutes (to be published post June 2024 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 21-23 May 2024.

1.3. Adoption of the minutes

COMP minutes for 16-18 April 2024.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000155489

Treatment of cutaneous T-cell lymphoma

Action: For information

Note: Withdrawal request received on 6 May 2024.

2.1.2. - EMA/OD/0000165835

Treatment of signet ring cell carcinoma

Action: For adoption, Oral explanation to be held on 21 May 2024 at 11:30

2.1.3. - EMA/OD/0000165562

Treatment of neonatal seizures

Action: For adoption, Oral explanation to be held on 21 May 2024 at 14:00

2.1.4. - EMA/OD/0000159474

Treatment of Duchenne muscular dystrophy

Action: For adoption, Oral explanation to be held on 22 May 2024 at 14:00

2.1.5. - EMA/OD/0000162667

Treatment of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 22 May 2024 at 11:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000139776

Prevention of T-cell engaging immunotherapy induced cytokine release syndrome (CRS)

Action: For discussion/adoption

2.2.2. - EMA/OD/0000160866

Treatment of pulmonary alveolar proteinosis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000162432

Treatment of complex regional pain syndrome

Action: For discussion/adoption

2.2.4. - EMA/OD/0000162927

Treatment of small cell lung cancer

Action: For discussion/adoption

2.2.5. - EMA/OD/0000163852

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000164215

Treatment of myasthenia gravis

Action: For discussion/adoption

2.2.7. - EMA/OD/0000164848

Treatment of hyperphenylalaninemia

Action: For discussion/adoption

2.2.8. - EMA/OD/0000164923

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.9. - EMA/OD/0000164949

Treatment of retinitis pigmentosa

Action: For discussion/adoption

2.2.10. - EMA/OD/0000165100

Treatment of idiopathic pulmonary fibrosis (IPF)

Action: For discussion/adoption

2.2.11. - EMA/OD/0000167882

Treatment of variegate porphyria

Action: For discussion/adoption

2.2.12. - EMA/OD/0000167926

Treatment of hepatitis D virus infection

Action: For discussion/adoption

2.2.13. - EMA/OD/0000168828

Treatment of pulmonary alveolar proteinosis (PAP)

Action: For discussion/adoption

2.2.14. - EMA/OD/0000168887

Treatment of pachyonychia congenita

Action: For discussion/adoption

2.2.15. - EMA/OD/0000168931

Treatment of C3 glomerulopathy

Action: For discussion/adoption

2.2.16. - EMA/OD/0000168982

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.17. - EMA/OD/0000169022

Treatment of symptomatic obstructive hypertrophic cardiomyopathy

Action: For discussion/adoption

2.2.18. - EMA/OD/0000169049

Treatment of hereditary orotic aciduria

Action: For discussion/adoption

2.2.19. - EMA/OD/0000169065

Treatment of eosinophilic esophagitis

Action: For discussion/adoption

2.2.20. - EMA/OD/0000169553

Treatment of peripheral T-cell lymphoma

Action: For discussion/adoption

2.2.21. - EMA/OD/0000169732

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

2.2.22. - EMA/OD/0000169763

Treatment of systemic sclerosis

Action: For discussion/adoption

2.2.23. - EMA/OD/0000169912

Treatment of GM1 gangliosidosis

Action: For discussion/adoption

2.2.24. - EMA/OD/0000169976

Treatment of poliovirus infection

Action: For discussion/adoption

2.2.25. - EMA/OD/0000170993

Treatment of amyotrophic lateral sclerosis

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 21-23 May 2024 COMP meeting

2.7. Evaluation on-going

19 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 pyruvate kinase deficiency

Action: For adoption

3.1.2. -

Treatment of Prader-Willi syndrome

Action: For adoption

3.1.3. -

Treatment of acute myeloid leukaemia

Action: For information

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. Nezglyal – leriglitazone - EMEA/H/C/005757, EU/3/16/1770, EMA/OD/0000144315

Minoryx Therapeutics S.L.; Treatment of adrenoleukodystrophy

Action: For information

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

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

CHMP Rapporteur: Martina Weise

Action: For discussion/adoption

4.2.3. - apadamtase alfa - EMEA/H/C/006198, EU/3/08/588, EMA/OD/0000150694

Takeda Manufacturing Austria AG; Treatment of thrombotic thrombocytopenic purpura

Action: For discussion/adoption

4.2.4. - polihexanide - EMEA/H/C/005858, EU/3/07/498, EMA/OD/0000152081

SIFI S.p.A.; Treatment of acanthamoeba keratitis

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. Kinpeygo - budesonide - EMEA/H/C/005653/II/0008, EU/3/16/1778, EMA/OD/0000157484

STADA Arzneimittel AG; Treatment of primary IgA nephropathy

CHMP Rapporteur: Christian Gartner

Action: For discussion/adoption

5.2.2. Blincyto – blinatumomab - EMEA/H/C/003731/II/0056, EU/3/09/650, EMA/OD/0000162410

Amgen Europe B.V.; Treatment of acute lymphoblastic leukaemia

CHMP Rapporteur: Alexandre Moreau

Action: For discussion/adoption

5.2.3. Adcetris – brentuximab vedotin - EMEA/H/C/002455/II/0111, EU/3/08/596

Takeda Pharma A/S; Treatment of Hodgkin lymphoma

Action: For information

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 21 May 2024 at 13:00

PAWG draft agenda for 21 May 2024 meeting

7.1.5. COMP Decisions Database

Action: For discussion

7.1.6. Scientific Committee Meetings – alternating face-to-face and virtual meetings schedule for 2025

Action: For discussion/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 April 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)

None

7.3.2. Innovation Task Force (ITF) meetings

Action: For discussion

Upcoming ITF meetings

7.3.3. Scientific Advice Working Party (SAWP): nomination of COMP member to SAWP

Action: For information

Election of Joint COMP-SAWP alternate member - call for nomination - Deadline 8 May 2024

7.3.4. Oncology Working Party (ONCWP)

Outcome of the ONCWP consultation

Action: For information

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 2024

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

7.8.3. Marketing Authorisation Applications (MAAs) 3-year forecast report (March 2024 to December 2026)

Action: For discussion

8. Any other business

8.1. Spinal muscular atrophy (SMA) registry report

Action: For discussion

8.2. Update on Real-World Evidence, including DARWIN EU®

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/