5 July 2023
EMA/COMP/285310/2023
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 11-13 July 2023

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

11 July 2023, 09:00-19:30, room 2A

12 July 2023, 08:30-19:30, room 2A

13 July 2023, 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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Table of contents

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 11-13 July 2023. See July 2023 COMP minutes (to be published post September 2023 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 11-13 July 2023.

1.3. Adoption of the minutes

COMP minutes for 13-15 June 2023.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000134159

Treatment of tuberculosis

Action: For information

Note: Withdrawal request received on 20 June 2023.

2.1.2. - EMA/OD/0000077804

Treatment of non-traumatic spontaneous intracerebral haemorrhage

Action: For adoption, Oral explanation to be held on 11 July 2023 at 11:30

2.1.3. - EMA/OD/0000135389

Treatment of amyotrophic lateral sclerosis

Action: For information

Note: Withdrawal request received on 22 June 2023.

2.1.4. - EMA/OD/0000126381

Treatment in allogeneic haematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 12 July 2023 at 10:45

2.1.5. - EMA/OD/0000131231

Treatment of malaria

Action: For adoption, Oral explanation to be held on 12 July 2023 at 09:00

2.1.6. - EMA/OD/0000134554

Treatment of neurofibromatosis type 1 (NF1)

Action: For information

Note: Withdrawal request received on 23 June 2023.

2.1.7. - EMA/OD/0000135309

Treatment of glioma

Action: For adoption, Oral explanation to be held on 12 July 2023 at 15:30

2.1.8. - EMA/OD/0000131435

Treatment of gastro-entero-pancreatic neuroendocrine tumours

Action: For adoption, Oral explanation to be held on 13 July 2023 at 09:00

2.1.9. - EMA/OD/0000134260

Treatment of amyotrophic lateral sclerosis (ALS)

Action: For information

Note: Withdrawal request received on 22 June 2023.

2.1.10. - EMA/OD/0000101423

Treatment in solid organ transplant

Action: For adoption, Oral explanation to be held on 13 July 2023 at 12:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000122901

Treatment of thalassaemia intermedia and major

Action: For discussion/adoption

2.2.2. - EMA/OD/0000124476

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.3. - EMA/OD/0000128591

Treatment of Huntington’s disease

Action: For discussion/adoption

2.2.4. - EMA/OD/0000128649

Treatment of transthyretin-mediated amyloidosis

Action: For discussion/adoption

2.2.5. - EMA/OD/0000131298

Treatment of glioma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000133480

Treatment of Stargardt's disease

Action: For discussion/adoption

2.2.7. - EMA/OD/0000133609

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.8. - EMA/OD/0000134075

Treatment of Kleine-Levin syndrome

Action: For discussion/adoption

2.2.9. - EMA/OD/0000135016

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.10. - EMA/OD/0000135549

Treatment of Pelizaeus-Merzbacher disease

Action: For discussion/adoption

2.2.11. - EMA/OD/0000136490

Treatment of arrhythmogenic right ventricular cardiomyopathy (ARVC) due to plakophilin-2 gene (*PKP2*) mutations

Action: For discussion/adoption

2.2.12. - EMA/OD/0000137651

Treatment of Smith-Magenis syndrome

Action: For discussion/adoption

2.2.13. - EMA/OD/0000138272

Treatment of hypothalamic obesity

Action: For discussion/adoption

2.2.14. - EMA/OD/0000138974

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.15. - EMA/OD/0000139967

Treatment of Guillain-Barre syndrome

Action: For discussion/adoption

2.2.16. - EMA/OD/0000140431

Treatment of patients with myelodysplastic syndromes and chronic myelomonocytic leukaemia

Action: For discussion/adoption

2.2.17. - EMA/OD/0000140620

Treatment of glioma

Action: For discussion/adoption

2.2.18. - EMA/OD/0000140879

Treatment of limb-girdle muscular dystrophy (LGMD)

Action: For discussion/adoption

2.2.19. - EMA/OD/0000140986

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.20. - EMA/OD/0000141035

Treatment of amyotrophic lateral sclerosis (ALS)

Action: For discussion/adoption

2.2.21. - EMA/OD/0000141142

Treatment of Danon disease

Action: For discussion/adoption

2.2.22. - EMA/OD/0000142116

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

Document(s) tabled:
OMPD applications - appointment of rapporteurs at the 11-13 July 2023 COMP meeting

2.7. Evaluation on-going

2 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 achondroplasia

Action: For discussion/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. - tislelizumab - EMEA/H/C/005919/0000, EU/3/20/2357, EMA/OD/0000129253

Novartis Europharm Limited; Treatment of oesophageal cancer

Action: For discussion/adoption

4.2.2. - quizartinib - EMEA/H/C/005910/0000, EU/3/09/622, EMA/OD/0000134652

Daiichi Sankyo Europe GmbH; Treatment of acute myeloid leukaemia

Action: For discussion/adoption

4.2.3. - cedazuridine, decitabine - EMEA/H/C/005823/0000, EU/3/21/2548, EMA/OD/0000141337

Otsuka Pharmaceutical Netherlands B.V.; Treatment of acute myeloid leukaemia

Action: For information

Note: Orphan designation withdrawal request received on 28 June 2023.

4.2.4. Bylvay - odevixibat - EMEA/H/C/004691/II/0011, EU/3/12/1040, EMA/OD/0000123138

Albireo AB; Treatment of Alagille syndrome

CHMP Rapporteur: Johann Lodewijk Hillege

Action: For adoption, Oral explanation to be held on 11 July 2023 at 15:30

4.2.5. - epcoritamab - EMEA/H/C/005985/0000, EU/3/22/2581, EMA/OD/0000104478

Abbvie Deutschland GmbH & Co. KG; Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 12 July 2023 at 12:00

4.2.6. - talquetamab - EMEA/H/C/005864/0000, EU/3/21/2486, EMA/OD/0000126657

Accelerated assessment

Janssen - Cilag International; Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 12 July 2023 at 14:15

4.3. Appeal

None

4.4. On-going procedures

Action: For information

Document(s) tabled:
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. Carvykti - ciltacabtagene autoleucel - EMEA/H/C/005095/II/0021, EU/3/20/2252, EMA/OD/0000141581

Janssen - Cilag International; Treatment of multiple myeloma

CHMP Rapporteur: Jan Mueller-Berghaus

Action: For discussion/adoption

5.2.2. Adcetris - brentuximab vedotin - EMEA/H/C/002455/II/0107, EU/3/08/596, EMA/OD/0000136638

Takeda Pharma A/S; Treatment of Hodgkin lymphoma

CHMP Rapporteur: Johann Lodewijk Hillege; CHMP Co-Rapporteur: Jan Mueller-Berghaus

Action: For discussion/adoption

5.2.3. Onivyde pegylated liposomal - irinotecan- EMEA/H/C/004125/II/0034, EU/3/11/933

Les Laboratoires Servier; Treatment of pancreatic cancer

CHMP Rapporteur: Filip Josephson

Action: For discussion/adoption

5.3. Appeal

None

5.4. On-going procedures

Action: For information

Document(s) tabled:

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

SRLM meeting in Madrid under the Spanish Presidency of the Council of the EU

Action: For discussion

7.1.4. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 11 July 2023 at 17:00

Document tabled:

PAWG draft agenda for 11 July 2023

7.1.5. COMP Decisions Database

Action: For discussion

Document tabled:

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 June 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)

None

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 Presentation: Results on historical review of indirect comparisons

Action: For discussion

8.2. COMP members nominated on EMA's recommendation by the European Commission

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/