18 January 2021
EMA/COMP/665917/2020
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 19-21 January 2021

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

19 January 2021, 08:30-19:30, remote virtual meeting

20 January 2021, 08:30-19:30, remote virtual meeting

21 January 2021, 08:30-17:30, remote virtual meeting

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).

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

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 19-21 January 2021. See January 2021 COMP minutes (to be published post February 2021 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 19-21 January 2021.

1.3. Adoption of the minutes

COMP minutes for 1-3 December 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000043071

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For adoption

2.1.2. - EMA/OD/0000043114

Treatment of sickle cell disease

Action: For adoption

2.1.3. - EMA/OD/0000043857

Treatment of cystinosis

Action: For adoption

2.1.4. - EMA/OD/0000030100

Treatment of uterine serous carcinoma

Action: For adoption, Oral explanation to be held on 19 January 2021 at 09:00

2.1.5. - EMA/OD/0000043454

Treatment of acute myeloid leukaemia

Action: For information

Notes: Withdrawal request received on 17 December 2020.

2.1.6. - EMA/OD/0000043102

Treatment of Haemophilia A

Action: For adoption, Oral explanation to be held on 19 January 2021 at 13:30

2.1.7. - EMA/OD/0000043121

Treatment of Haemophilia B

Action: For adoption, Oral explanation to be held on 19 January 2021 at 13:30

2.1.8. - EMA/OD/0000043404

Treatment of leishmaniasis

Action: For adoption, Oral explanation to be held on 19 January 2021 at 15:00

2.1.9. - EMA/OD/0000043828

Treatment of gastric cancer

Action: For adoption, Oral explanation to be held on 19 January 2021 at 16:30

2.1.10. - EMA/OD/0000043607

Prevention of retinopathy of prematurity

Action: For adoption, Oral explanation to be held on 19 January 2021 at 18:00

2.1.11. - EMA/OD/0000043730

Treatment of primary aldosteronism

Action: For adoption, Oral explanation to be held on 20 January 2021 at 09:00

2.1.12. - EMA/OD/0000043217

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 20 January 2021 at 10:30

2.1.13. - EMA/OD/0000043459

Treatment of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 20 January 2021 at 11:30

2.1.14. - EMA/OD/0000042924

Treatment of eosinophilic gastritis

Action: For adoption, Oral explanation to be held on 20 January 2021 at 15:30

2.1.15. - EMA/OD/0000043498

Treatment of eosinophilic enteritis

Action: For adoption, Oral explanation to be held on 20 January 2021 at 15:30

2.1.16. - EMA/OD/0000038364

Treatment of spinocerebellar ataxia

Action: For adoption, Oral explanation to be held on 20 January 2021 at 18:00

2.1.17. - EMA/OD/0000043829

Treatment of sickle cell disease

Action: For adoption, Oral explanation to be held on 21 January 2021 at 09:00

2.1.18. - EMA/OD/0000042048

Treatment of acute respiratory distress syndrome

Action: For information

Notes: Withdrawal request received on 5 January 2021.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000041437

Treatment of lymphoplasmacytic lymphoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000041484

Treatment of fulminant hypermetabolic crisis secondary to calcium dysregulation in skeletal muscle

Action: For discussion/adoption

2.2.3. - EMA/OD/0000043808

Treatment of bronchopulmonary dysplasia

Action: For discussion/adoption

2.2.4. - EMA/OD/0000043946

Treatment of retinitis pigmentosa

Action: For discussion/adoption

2.2.5. - EMA/OD/0000044835

Treatment of creatine transporter deficiency syndrome

Action: For discussion/adoption

2.2.6. - EMA/OD/0000045680

Treatment of medullary thyroid carcinoma

Action: For discussion/adoption

2.2.7. - EMA/OD/0000045713

Treatment of spinal cord injury

Action: For discussion/adoption

2.2.8. - EMA/OD/0000046077

Treatment of Krabbe disease

Action: For discussion/adoption

2.2.9. - EMA/OD/0000046254

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.10. - EMA/OD/0000046325

Prevention of bronchopulmonary dysplasia

Action: For discussion/adoption

2.2.11. - EMA/OD/0000046383

Treatment of Leber congenital amaurosis

Action: For discussion/adoption

2.2.12. - EMA/OD/0000046433

Treatment of Gaucher disease

Action: For discussion/adoption

2.2.13. - EMA/OD/0000046448

Treatment of ATTR amyloidosis

Action: For discussion/adoption

2.2.14. - EMA/OD/0000048780

Treatment of Dravet syndrome

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

None

2.5. Appeal

2.5.1. tebentafusp - EMA/OD/0000047566

Pharma Gateway AB; Treatment of uveal melanoma

Action: For adoption, Oral explanation to be held on 19 January 2021 at 11:00

2.6. Nominations

2.6.1. New applications for orphan medicinal product designation - Appointment of COMP rapporteurs

Action: For adoption

Document tabled:
OMPD applications - appointment of rapporteurs at the 19-21 January 2021 COMP meeting

2.7. Evaluation on-going

14 applications for orphan designation will not be discussed as evaluation is ongoing.

Action: For information

Notes: See 7.8.1. table

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of sickle cell disease

Action: For adoption

3.1.2. -

Treatment of paediatric patients with severe combined immunodeficiency (SCID) receiving allogeneic haematopoietic stem cell transplantation

Action: For adoption

3.1.3. -

Treatment of multiple myeloma

Action: For adoption

3.1.4. -

Treatment of ATTR amyloidosis-polyneuropathy

Action: For adoption

3.1.5. -

Treatment of ATTR amyloidosis-cardiomyopathy

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of cutaneous T-cell lymphoma

Action: For information

3.2.2. -

Treatment of thalassaemia

Action: For information

3.2.3. -

Treatment of glioblastoma

Action: For information

3.2.4. -

Treatment of ornithine transcarbamylase deficiency

Action: For information

3.3. New requests

3.3.1. -

Treatment of Fabry disease

Action: For information

3.3.2. –

Relapsed or refractory multiple myeloma

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. – risdiplam - EMEA/H/C/005145/0000, EMA/OD/0000001899, EU/3/19/2145, EMA/OD/0000039037

Accelerated assessment

Roche Registration GmbH; Treatment of spinal muscular atrophy

Action: For adoption, Oral explanation to be held on 20 January 2021 at 13:30

4.2.2. - selinexor - EMEA/H/C/005127/0000, EU/3/14/1355, EMA/OD/0000043722

Karyopharm Europe GmbH; Treatment of plasma cell myeloma

Action: For information

4.2.3. Epidyolex - cannabidiol - EMEA/H/C/004675/II/0005, EMA/OD/165/17, EU/3/17/1959, EMA/OD/0000033940

GW Pharma (International) B.V.; Treatment of tuberous sclerosis

CHMP Rapporteur: Mark Ainsworth; CHMP Co-Rapporteur: Ondřej Slanař

Action: For discussion

4.2.4. – pemigatinib - EMEA/H/C/005266, EMA/OD/038/18, EU/3/18/2066, EMA/OD/0000039241

Incyte Biosciences Distribution B.V.; Treatment of biliary tract cancer

Action: For information

4.2.5. – berotralstat - EMEA/H/C/005138/0000, EMA/OD/003/18, EU/3/18/2028, EMA/OD/0000045564

BioCryst Ireland Limited; Treatment of hereditary angioedema

Action: For discussion

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 of marketing authorisation extension

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Galafold - migalastat - EMEA/H/C/004059/II/0029, EMA/OD/105/05, EU/3/06/368

Amicus Therapeutics Europe Limited; Treatment of Fabry disease

CHMP Rapporteur: Johann Lodewijk Hillege; CHMP Co-Rapporteur: Ondřej Slanař

Action: For discussion

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. Strategic Review & Learning meetings

COMP-SRLM of the Portuguese Presidency to take place on 11th February 2021 - remote virtual meeting

(1-day meeting, Thursday before February COMP meeting)

Action: For information

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 15 January 2021 at 11:00

Document tabled:
PAWG draft agenda for 15 January 2021 meeting

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes December 2020

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

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP)

Action: For information

Document(s) tabled:

7.3.2. Working Party with Healthcare Professionals' Organisations (HCPWP)

Action: For information

Document(s) tabled:

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Revision of the EU legislation on medicines for children and rare diseases

Action: For discussion

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

Notes: Monthly teleconference

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

Action: For information

Notes: Ad hoc basis meeting

7.5.3. Therapeutic Goods Administration (TGA), Australia

Action: For information

Notes: Ad hoc basis meeting

7.5.4. Health Canada

Action: For information

Notes: Ad hoc basis meeting

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

None

7.7. COMP work plan

Draft COMP Work Plan 2021

Action: For discussion/adoption

7.8. Planning and reporting

7.8.1. List of all applications submitted/expected and the COMP rapporteurship distribution of valid applications submitted in 2020/2021

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Big Data Training Signpost

Action: For information

8.2. ENCePP in the time of COVID

Action: For discussion

8.3.

Action: For discussion

8.4. New Executive Director at the EMA meeting with COMP

Introduction of the new EMA Executive Director - Emer Cooke

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/