30 November 2020
EMA/COMP/598981/2020
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 01-03 December 2020

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

01 December 2020, 08:30-19:30, remote virtual meeting

02 December 2020, 08:30-19:30, remote virtual meeting

03 December 2020, 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 1-3 December 2020. See (current) December 2020 COMP minutes (to be published post January 2021 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 01-03 December 2020.

1.3. Adoption of the minutes

COMP minutes for 03-05 November 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000040564

Treatment of primary biliary cholangitis

Action: For adoption

2.1.2. - EMA/OD/0000041707

Treatment of glioma

Action: For adoption, Oral explanation to be held on 01 December 2020 at 09:00

2.1.3. - EMA/OD/0000037877

Treatment of focal segmental glomerulosclerosis

Action: For adoption, Oral explanation to be held on 01 December 2020 at 10:30

2.1.4. - EMA/OD/0000041257

Treatment of LAMA2 congenital muscular dystrophy

Action: For adoption, Oral explanation to be held on 01 December 2020 at 15:00

2.1.5. - EMA/OD/0000034920

Treatment of mitochondrial epilepsy

Action: For adoption, Oral explanation to be held on 01 December 2020 at 16:30

2.1.6. - EMA/OD/0000042085

Treatment of primary intracerebral hemorrhage

Action: For adoption, Oral explanation to be held on 01 December 2020 at 18:00

2.1.7. - EMA/OD/0000042012

Treatment of follicular lymphoma

Action: For adoption, Oral explanation to be held on 02 December 2020 at 15:30

2.1.8. - EMA/OD/0000020657

Treatment of renal transplant interstitial fibrosis and tubular atrophy

Action: For adoption, Oral explanation to be held on 02 December 2020 at 10:30

2.1.9. - EMA/OD/0000037806

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 02 December 2020 at 13:30

2.1.10. - EMA/OD/0000040482

Treatment of pancreatic cancer

Action: For information

Note: Withdrawal request received on 17 November 2020.

2.1.11. - EMA/OD/0000042079

Treatment of pericarditis

Action: For adoption, Oral explanation to be held on 02 December 2020 at 17:00

2.1.12. - EMA/OD/0000039384

Treatment of idiopathic hypersomnia

Action: For adoption, Oral explanation to be held on 01 December 2020 at 13:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000030100

Treatment of uterine serous carcinoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000033964

Treatment of invasive candidiasis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000038364

Treatment of spinocerebellar ataxia

Action: For discussion/adoption

2.2.4. - EMA/OD/0000041637

Treatment of Angelman syndrome

Action: For discussion/adoption

2.2.5. - EMA/OD/0000042048

Treatment of acute respiratory distress syndrome

Action: For discussion/adoption

2.2.6. - EMA/OD/0000042795

Treatment of mucopolysaccharidosis, type II (Hunter syndrome)

Action: For discussion/adoption

2.2.7. - EMA/OD/0000042924

Treatment of eosinophilic gastritis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000043059

Treatment of dermatomyositis

Action: For discussion/adoption

2.2.9. - EMA/OD/0000043071

Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion/adoption

2.2.10. - EMA/OD/0000043102

Treatment of haemophilia A

Action: For discussion/adoption

2.2.11. - EMA/OD/0000043114

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.12. - EMA/OD/0000043121

Treatment of haemophilia B

Action: For discussion/adoption

2.2.13. - EMA/OD/0000043209

Treatment of Pitt-Hopkins syndrome

Action: For discussion/adoption

2.2.14. - EMA/OD/0000043217

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.15. - EMA/OD/0000043391

Treatment of Phelan-McDermid syndrome

Action: For discussion/adoption

2.2.16. - EMA/OD/0000043404

Treatment of leishmaniasis

Action: For discussion/adoption

2.2.17. - EMA/OD/0000043454

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.18. - EMA/OD/0000043459

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

2.2.19. - EMA/OD/0000043498

Treatment of eosinophilic enteritis

Action: For discussion/adoption

2.2.20. - EMA/OD/0000043607

Prevention of retinopathy of prematurity

Action: For discussion/adoption

2.2.21. - EMA/OD/0000043612

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.22. - EMA/OD/0000043730

Treatment of primary aldosteronism

Action: For discussion/adoption

2.2.23. - EMA/OD/0000043828

Treatment of gastric cancer

Action: For discussion/adoption

2.2.24. - EMA/OD/0000043829

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.25. - EMA/OD/0000043857

Treatment of cystinosis

Action: For discussion/adoption

2.2.26. - EMA/OD/0000043899

Treatment of Fabry's disease

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 discussion/adoption

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 01-03 December 2020 COMP meeting

2.7. Evaluation on-going

14 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 ATTR amyloidosis-cardiomyopathy

Action: For adoption

3.1.2. -

Treatment of cutaneous T-cell lymphoma

Action: For adoption

3.1.3. -

Treatment of thalassaemia

Action: For adoption

3.1.4. -

Treatment of glioblastoma

Action: For information

3.1.5. -

Treatment of ornithine transcarbamylase deficiency

Action: For information

3.2. Finalised letters

3.2.1. -

Treatment of desmoid tumours

Action: For information

3.2.2. -

Treatment of marginal zone lymphoma

Action: For information

3.2.3. -

Treatment of diffused large B-cell lymphoma

Action: For information

3.3. New requests

3.3.1. -

Treatment of sickle cell disease

Action: For information

3.3.2. -

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

Action: For information

3.3.3. -

Treatment of multiple myeloma

Action: For information

3.3.4. -

Treatment of ATTR amyloidosis-polyneuropathy

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. - selinexor - EMEA/H/C/005127, EMA/OD/087/14, EU/3/14/1355, EMA/OD/0000043722

Karyopharm Europe GmbH; Treatment of plasma cell myeloma

Action: For information

4.2.2. – potassium - EMEA/H/C/005407, EMA/OD/016/17, EU/3/17/1888, EMA/OD/0000032257

Advicenne Pharma S.A.; Treatment of distal renal tubular acidosis

Action: For discussion/adoption

4.2.3. – fedratinib

Celgene Europe BV

a) Treatment of primary myelofibrosis, EMEA/H/C/005026/0000, EMA/OD/069/10, EU/3/10/794, EMA/OD/0000029092

b) Treatment of post-essential thrombocythaemia myelofibrosis EMEA/H/C/005026/0000, EMA/OD/084/10, EU/3/10/810, EMA/OD/0000029093

c) Treatment of post-polycythaemia vera myelofibrosis EMEA/H/C/005026/0000, EMA/OD/092/10, EU/3/10/811, EMA/OD/0000029095

Action: For adoption, Oral explanation to be held on 02 December 2020 at 11:30

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 adoption

4.2.5. – moxetumomab pasudotox- EMEA/H/C/005322, EMA/OD/066/08, EU/3/08/592, EMA/OD/0000013333

AstraZeneca AB; Treatment of hairy cell leukaemia

Action: For information

4.2.6. – duvelisib

Verastem Europe GmbH

a) Treatment of Follicular lymphoma, EMEA/H/C/005381/0000, EMA/OD/047/13, EU/3/13/1157, EMA/OD/0000024085

b) Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma EMEA/H/C/005381/0000, EMA/OD/196/12, EU/3/13/1125, EMA/OD/0000026423

Action: For discussion

4.2.7. – idecabtagene vicleucel - EMEA/H/C/004662/0000, EU/3/17/1863, EMA/OD/0000035635

Accelerated assessment

Celgene Europe BV; Treatment of multiple myeloma

Action: For information

4.2.8. – 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 information

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/adoption

5. Review of orphan designation for authorised orphan medicinal products at time of marketing authorisation extension

5.1. After adoption of CHMP opinion

5.1.1. Blincyto – blinatumomab - EMEA/H/C/003731/II/0030, EMA/OD/029/09, EU/3/09/650, EMA/OD/00000016144

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

CHMP Rapporteur: Alexandre Moreau; CHMP Co-Rapporteur: Daniela Melchiorri

Action: For adoption

5.2. Prior to adoption of CHMP opinion

5.2.1. Blincyto – blinatumomab - EMEA/H/C/003731/II/0038, EMA/OD/029/09, EU/3/09/650, EMA/OD/0000048837

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

CHMP Rapporteur: Alexandre Moreau

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

None

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 27 November 2020 at 11:00

Document tabled:
PAWG draft agenda for 27 November 2020 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 November 2020

7.2.2. COMP-CAT Working Group

Proposed meeting time on 30 November 2020 at 17:30

Action: For discussion

Document(s) tabled: Agenda and related documents

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

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

None

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

None

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)

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

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

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. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q4/2020 Update of the Business Pipeline report for the human scientific committees

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

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.

More detailed information on the above terms can be found on the EMA website: www.ema.europa.eu/