6 October 2020
EMA/COMP/491947/2020
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 06-08 October 2020

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

06 October 2020, 08:30-19:30, remote virtual meeting

07 October 2020, 08:30-19:30, remote virtual meeting

08 October 2020, 08:30-17:00, 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).

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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 06-08 October 2020. See October 2020 COMP minutes (to be published post November 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 06-08 October 2020.

1.3. Adoption of the minutes

COMP minutes for 08-10 September 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000037416

Treatment of non-small cell lung cancer with EGFR and MET alterations

Action: For information

Note: Withdrawal request received on 18 September 2020.

2.1.2. - EMA/OD/0000031771

Treatment of traumatic brain injury with development of oedema

Action: For adoption, Oral explanation to be held on 06 October 2020 at 13:30

2.1.3. - EMA/OD/0000032268

Treatment of gastric cancer

Action: For adoption, Oral explanation to be held on 06 October 2020 at 16:30

2.1.4. - EMA/OD/0000037822

Treatment of frontotemporal dementia (FTD)

Action: For adoption, Oral explanation to be held on 06 October 2020 at 15:00

2.1.5. - EMA/OD/0000036055

Treatment of oesophageal cancer

Action: For adoption, Oral explanation to be held on 07 October 2020 at 17:00

2.1.6. - EMA/OD/0000034870

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 07 October 2020 at 11:30

2.1.7. - EMA/OD/0000037899

Treatment of congenital pulmonary hypoplasia in infancy

Action: For adoption, Oral explanation to be held on 07 October 2020 at 15:30

2.1.8. - EMA/OD/0000034572

Treatment of hereditary angioedema

Action: For information

Note: Withdrawal request received on 22 September 2020.

2.1.9. - EMA/OD/0000030636

Treatment of unclassifiable interstitial lung disease

Action: For adoption, Oral explanation to be held on 06 October 2020 at 09:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000028397

Treatment of fibrodysplasia ossificans progressiva

Action: For discussion/adoption

2.2.2. - EMA/OD/0000033691

Treatment of microvillus inclusion disease

Action: For discussion/adoption

2.2.3. - EMA/OD/0000034375

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.4. - EMA/OD/0000035302

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.5. - EMA/OD/0000035896

Treatment of glioma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000036404

Treatment of relapsed refractory myelodysplastic syndrome

Action: For discussion/adoption

2.2.7. - EMA/OD/0000037176

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000037744

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

2.2.9. - EMA/OD/0000037871

Treatment of carnitine palmitoyltransferase I deficiency

Action: For discussion/adoption

2.2.10. - EMA/OD/0000038040

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000038423

Treatment of Huntington's disease

Action: For discussion/adoption

2.2.12. - EMA/OD/0000038481

Treatment of X-linked severe combined immunodeficiency (X-SCID)

Action: For discussion/adoption

2.2.13. - EMA/OD/0000038634

Treatment of progressive multifocal eukoencephalopathy

Action: For discussion/adoption

2.2.14. - EMA/OD/0000039164

Treatment of primary ciliary dyskinesia (PCD)

Action: For discussion/adoption

2.2.15. - EMA/OD/0000039198

Treatment of peripheral artery disease in patients with end-stage kidney disease receiving haemodialysis

Action: For discussion/adoption

2.2.16. - EMA/OD/0000039389

Treatment of retinitis pigmentosa

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 tabled:
OMPD applications - appointment of rapporteurs at the 06-08 October 2020 COMP meeting

2.7. Evaluation on-going

27 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 systemic mastocytosis

Action: For adoption

3.1.2. -

Treatment of neuroblastoma

Action: For adoption

3.1.3. -

Treatment of immune thrombocytopenia

Action: For adoption

3.1.4. -

Treatment of non-infectious uveitis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of bullous pemphigoid

Action: For information

3.2.2. -

Treatment of amyotrophic lateral sclerosis

Action: For information

3.3. New requests

3.3.1. -

Treatment of desmoid tumours

Action: For information

3.3.2. -

Treatment of marginal zone lymphoma

Action: For information

3.3.3. -

Treatment of glioblastoma

Action: For information

3.3.4. -

Treatment of ornithine transcarbamylase deficiency

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. - fenfluramine hydrochloride - EMEA/H/C/003933/0000, EU/3/13/1219, EMA/OD/0000024920

Zogenix ROI Limited; Treatment of Dravet syndrome

Action: For information

4.2.2. 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 information

4.2.3. - autologous CD34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector encoding the human arylsulfatase a gene - EMEA/H/C/005321/0000, EU/3/07/446, EMA/OD/0000023359

Accelerated assessment

Orchard Therapeutics (Netherlands) B.V.; Treatment of metachromatic leukodystrophy

Action: For information

4.2.4. - lumasiran - EMEA/H/C/005040/0000, EU/3/16/1637, EMA/OD/0000034914

Accelerated assessment

Alnylam Netherlands B.V.; Treatment of primary hyperoxaluria type 1

Action: For discussion

4.2.5. - Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured - EMEA/H/C/005102/0000, EU/3/19/2220, EMA/OD/0000026061

Accelerated assessment

Kite Pharma EU B.V.; Treatment of mantle cell lymphoma

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

5.1.1. Zejula - niraparib - EMEA/H/C/004249/II/0019, EU/3/10/760, EMA/OD/0000031233

GlaxoSmithKline (Ireland) Limited; Treatment of ovarian cancer

CHMP Rapporteur: Bjorg Bolstad; CHMP Co-Rapporteur: Alexandre Moreau

Action: For adoption, Oral explanation to be held on 07 October 2020 at 13:30

5.2. Prior to adoption of CHMP opinion

5.2.1. Kaftrio - ivacaftor/tezacaftor/elexacaftor - EMEA/H/C/005269/II/0001, EMA/OD/0000001208, EU/3/18/2116

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP Rapporteur: Johann Lodewijk Hillege

Action: For discussion

5.2.2. Kalydeco - ivacaftor - EMEA/H/C/002494/II/0089, EMA/OD/010/08, EU/3/08/556

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP Rapporteur: Maria Concepcion Prieto Yerro

Action: For discussion

5.2.3. 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 information

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 Meeting – COMP, 24-25 September 2020, Germany

Report from the meeting

Action: For information

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 2 October 2020 at 11:00

Document tabled:
PAWG draft agenda for 2 October 2020 meeting

7.1.3. COMP Workshop 2020 on support for orphan medicines development

Action: For information

Document(s) tabled: Background information

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 September 2020

7.2.1. COMP-CAT Working Group

Proposed meeting time on 5 October 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

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 2020

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. COMP meeting schedule (2022-2024)

Action: For adoption

Document(s) tabled: Documents: Committee meeting dates (2022-2024)

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/