13 July 2020
EMA/COMP/343863/2020
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 14-16 July 2020

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

14 July 2020, 08:30-20:00, remote virtual meeting

15 July 2020, 08:30-20:00, remote virtual meeting

16 July 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).

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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 14-16 July 2020. See July 2020 COMP minutes (to be published post September 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 14-16 July 2020.

1.3. Adoption of the minutes

COMP minutes for 16-18 June 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000016117

Prevention of bronchopulmonary dysplasia

Action: For adoption

2.1.2. - EMA/OD/0000024640

Treatment of retinitis pigmentosa

Action: For adoption

2.1.3. - EMA/OD/0000031667

Prevention of haemolytic uraemic syndrome

Action: For adoption

2.1.4. - EMA/OD/0000031867

Treatment in haematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 14 July 2020 at 09:00

2.1.5. - EMA/OD/0000032154

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 14 July 2020 at 11:30

2.1.6. - EMA/OD/0000023667

Treatment of idiopathic pulmonary fibrosis

Action: For adoption, Oral explanation to be held on 14 July 2020 at 16:30

2.1.7. - EMA/OD/0000031911

Treatment of inherited disorders of oxidative phosphorylation

Action: For adoption, Oral explanation to be held on 14 July 2020 at 18:00

2.1.8. - EMA/OD/0000029150

Diagnosis of progressive supranuclear palsy (PSP)

Action: For adoption, Oral explanation to be held on 15 July 2020 at 09:00

2.1.9. - EMA/OD/0000029026

Treatment of follicular lymphoma

Action: For information

Note: Withdrawal request received on 26 June 2020.

2.1.10. - EMA/OD/0000030305

Treatment of primary sclerosing cholangitis

Action: For adoption, Oral explanation to be held on 15 July 2020 at 11:30

2.1.11. - EMA/OD/0000028003

Treatment of mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes

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

2.1.12. - EMA/OD/0000028006

Treatment of myoclonic epilepsy with Ragged-Red Fibres

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

2.1.13. - EMA/OD/0000031991

Prevention of retinopathy of prematurity

Action: For adoption, Oral explanation to be held on 15 July 2020 at 17:00

2.1.14. - EMA/OD/0000019700

Treatment of metastatic pancreatic ductal adenocarcinoma (PDAC)

Action: For information

Note: Withdrawal request received on 9 July 2020.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000009949

Treatment of polycythaemia vera

Action: For discussion/adoption

2.2.2. - EMA/OD/0000028614

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.3. - EMA/OD/0000028963

Treatment of acute liver failure

Action: For discussion/adoption

2.2.4. - EMA/OD/0000030250

Treatment of solid organ transplantation

Action: For discussion/adoption

2.2.5. - EMA/OD/0000030264

Treatment of KCNQ2 encephalopathy

Action: For discussion/adoption

2.2.6. - EMA/OD/0000030272

Treatment of uveal melanoma

Action: For discussion/adoption

2.2.7. - EMA/OD/0000031078

Treatment of soft tissue sarcomas

Action: For discussion/adoption

2.2.8. - EMA/OD/0000031602

Treatment of mucopolysaccharidosis type VI

Action: For discussion/adoption

2.2.9. - EMA/OD/0000032059

Treatment of Charcot-Marie-Tooth (CMT) disease

Action: For discussion/adoption

2.2.10. - EMA/OD/0000032752

Treatment of long-chain 3-hydroxyacyl-coenzyme A dehydrogenase deficiency

Action: For discussion/adoption

2.2.11. - EMA/OD/0000033107

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.12. - EMA/OD/0000033552

Treatment of IgA nephropathy

Action: For discussion/adoption

2.2.13. - EMA/OD/0000033614

Treatment of Menkes disease

Action: For discussion/adoption

2.2.14. - EMA/OD/0000034330

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.15. - EMA/OD/0000034496

Adjuvant treatment of acute respiratory failure in COVID-19 patients at ICU

Action: For discussion/adoption

2.2.16. - EMA/OD/0000034624

Treatment of Wilson's disease

Action: For discussion/adoption

2.2.17. - EMA/OD/0000034662

Treatment of sickle cell disease

Action: For discussion/adoption

2.2.18. - EMA/OD/0000034728

Treatment of mucopolysaccharidosis III

Action: For discussion/adoption

2.2.19. - EMA/OD/0000035036

Treatment of von-Hippel Lindau disease

Action: For discussion/adoption

2.2.20. - EMA/OD/0000035086

Treatment of GM2 gangliosidosis

Action: For discussion/adoption

2.2.21. - EMA/OD/0000035180

Diagnosis of corticobasal degeneration

Action: For discussion/adoption

2.2.22. - EMA/OD/0000035212

Treatment of Graft versus Host disease

Action: For discussion/adoption

2.2.23. - EMA/OD/0000035407

Treatment of Fabry disease

Action: For discussion/adoption

2.2.24. - EMA/OD/0000035408

Treatment of Gaucher disease

Action: For discussion/adoption

2.2.25. - EMA/OD/0000035563

Treatment of anal cancer

Action: For discussion/adoption

2.2.26. - EMA/OD/0000035570

Treatment of diffuse large B cell lymphoma

Action: For discussion/adoption

2.2.27. - EMA/OD/0000035595

Treatment of Friedreich's ataxia

Action: For discussion/adoption

2.2.28. - EMA/OD/0000035618

Treatment of acute lymphoblastic leukaemia

Action: For discussion/adoption

2.2.29. - EMA/OD/0000035662

Treatment of cholangiocarcinoma

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 14-16 July 2020 COMP meeting

2.7. Evaluation on-going

None

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1.

Treatment of short bowel syndrome

Action: For adoption

3.1.2.

Treatment of Philadelphia chromosome-positive chronic myelogenous leukaemia in chronic phase

Action: For adoption

3.2. Finalised letters

3.2.1.

Treatment of ß-thalassaemia intermedia and major

Action: For information

3.2.2.

Treatment of amyotrophic lateral sclerosis

Action: For information

3.3. New requests

3.3.1.

Treatment of bullous pemphigoid

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

4.1.1. Kaftrio - elexacaftor/tezacaftor/ivacaftor - EMEA/H/C/005269, EU/3/18/2116, EMA/OD/0000020155

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

Action: For discussion

4.2. Orphan designated products for discussion prior to adoption of CHMP opinion

4.2.1. – emapalumab - EMEA/H/C/004386, EU/3/10/749, EMEA/OD/153/09, EMA/OD/0000009274

Novimmune B.V.; Treatment of haemophagocytic lymphohistiocytosis

Action: For information

4.2.2. – fenfluramine - EMEA/H/C/003933, EMA/OD/140/13, EU/3/13/1219, EMA/OD/0000024920

Zogenix GmbH; Treatment of Dravet syndrome

Action: For discussion

4.2.3. – Autologous CD34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector - EMEA/H/C/005321/0000, EMA/OD/102/06, EU/3/07/446, EMA/OD/0000023359

Accelerated assessment

Orchard Therapeutics Limited; Treatment of metachromatic leukodystrophy

Action: For discussion

4.2.4. – avapritinib - EMA/OD/037/17, EU/3/17/1889, EMA/OD/0000030630

Blueprint Medicines (Netherlands) B.V.; Treatment of gastrointestinal stromal tumours

Action: For discussion

4.2.5. – amikacin - EMEA/H/C/005264, EMA/OD/191/13, EU/3/14/1259, EMA/OD/0000030955

Insmed Netherlands B.V.; Treatment of nontuberculous mycobacterial lung

Action: For discussion

4.2.6. - valoctocogene roxaparvovec - EMEA/H/C/004749, EMA/OD/230/15, EU/3/16/1622, EMA/OD/0000024177

Accelerated assessment

BioMarin International Limited; Treatment of Haemophilia A

Action: For discussion

4.2.7. – acalabrutinib - EMEA/H/C/005299, EMA/OD/196/15, EU/3/16/1624, EMA/OD/0000021547

AstraZeneca AB; Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma

Action: For discussion

4.2.8. – belantamab mafodotin - EMEA/H/C/004935/0000, EMA/OD/077/17, EU/3/17/1925, EMA/OD/0000028779

Accelerated assessment

GlaxoSmithKline (Ireland) Limited; Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 15 July 2020 at 14:00

4.2.9. - 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, EMA/OD/0000013608, EU/3/19/2220, EMA/OD/0000026061

Accelerated assessment

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

Action: For information

4.2.10. – tagraxofusp - EMEA/H/C/005031, EMA/OD/064/15, EU/3/15/1567, EMA/OD/0000004627

TMC Pharma (EU) Limited; Treatment of blastic plasmacytoid dendritic cell neoplasm

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

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. Kalydeco – ivacaftor - Type II variation - EMEA/H/C/002494/II/0085, EMA/OD/010/08, EU/3/08/556, EMA/OD/0000036247

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP rapporteur: Maria Concepcion Prieto Yerro

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 Meeting – COMP, 24-25 September 2020, Germany

Action: For information

Document(s) tabled: Background information

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 10 July 2020 at 11:00

Action: For information

Document tabled:
PAWG draft agenda for 10 July 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 June 2020

7.2.2. COMP-CAT Working Group

Proposed meeting time on 13 July 2020 at 17:30

Action: For discussion

Document(s) tabled: Agenda and related documents

7.2.3. Final proposal for fostering collaboration between CHMP and COMP

Final proposal for fostering the scientific collaboration between CHMP and COMP taking into account comments from both Committees.

Action: For discussion

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

Update on EU Pharmaceutical Strategy and Orphan Evaluation and ongoing consultation

Action: For information

Documents: Background information

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

8.1.1.

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/