7 September 2020
EMA/COMP/393282/2020
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 08-10 September 2020

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

08 September 2020, 08:30-19:30, remote virtual meeting

09 September 2020, 08:30-19:30, remote virtual meeting

10 September 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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1.1. 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 8-10 September 2020. See September 2020 COMP minutes (to be published post October 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 08-10 September 2020.

1.3. Adoption of the minutes

COMP minutes for 14-16 July 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000034330

Treatment of sickle cell disease

Action: For adoption

2.1.2. - EMA/OD/0000035407

Treatment of Fabry Disease

Action: For adoption

2.1.3. - EMA/OD/0000035212

Treatment of Graft versus Host Disease

Action: For adoption, Oral explanation to be held on 08 September 2020 at 09:00

2.1.4. - EMA/OD/0000031078

Treatment of soft tissue sarcomas

Action: For adoption, Oral explanation to be held on 08 September 2020 at 10:30

2.1.5. - EMA/OD/0000033107

Treatment of sickle cell disease

Action: For adoption, Oral explanation to be held on 08 September 2020 at 11:30

2.1.6. - EMA/OD/0000030272

Treatment of uveal melanoma

Action: For adoption, Oral explanation to be held on 08 September 2020 at 13:30

2.1.7. - EMA/OD/0000030264

Treatment of KCNQ2 encephalopathy

Action: For adoption, Oral explanation to be held on 08 September 2020 at 16:30

2.1.8. - EMA/OD/0000032059

Treatment of Charcot-Marie-Tooth (CMT) disease

Action: For adoption, Oral explanation to be held on 08 September 2020 at 18:00

2.1.9. - EMA/OD/0000028614

Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 09 September 2020 at 10:30

2.1.10. - EMA/OD/0000035563

Treatment of anal cancer

Action: For adoption, Oral explanation to be held on 09 September 2020 at 11:30

2.1.11. - EMA/OD/0000030250

Treatment of solid organ transplantation

Action: For adoption, Oral explanation to be held on 09 September 2020 at 13:30

2.1.12. - EMA/OD/0000009949

Treatment of polycythaemia vera

Action: For adoption, Oral explanation to be held on 09 September 2020 at 15:30

2.1.13. - EMA/OD/0000034496

Treatment of COVID-19 related ARDS

Action: For adoption, Oral explanation to be held on 09 September 2020 at 18:00

2.1.14. - EMA/OD/0000033552

Treatment of IgA nephropathy

Action: For adoption, Oral explanation to be held on 10 September 2020 at 09:00

2.1.15. - EMA/OD/0000035618

Treatment of acute lymphoblastic leukaemia

Action: For adoption, Oral explanation to be held on 10 September 2020 at 10:00

2.1.16. - EMA/OD/0000035180

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Action: For adoption

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000030636

Treatment of unclassifiable interstitial lung disease

Action: For discussion/adoption

2.2.2. - EMA/OD/0000031910

Treatment of aspartylglucosaminuria (AGU)

Action: For discussion/adoption

2.2.3. - EMA/OD/0000032268

Treatment of gastric cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000033863

Treatment of Limb-Girdle muscular dystrophy

Action: For discussion/adoption

2.2.5. - EMA/OD/0000034572

Treatment of hereditary angioedema

Action: For discussion/adoption

2.2.6. - EMA/OD/0000034870

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.7. - EMA/OD/0000034892

Treatment of activated phosphoinositide 3-kinase delta syndrome (APDS)

Action: For discussion/adoption

2.2.8. - EMA/OD/0000035451

Treatment of epidermolysis bullosa (EB)

Action: For discussion/adoption

2.2.9. - EMA/OD/0000036055

Treatment of oesophageal cancer

Action: For discussion/adoption

2.2.10. - EMA/OD/0000036435

Treatment of primary immunoglobulin A nephropathy

Action: For discussion/adoption

2.2.11. - EMA/OD/0000037095

Treatment of GM1 gangliosidosis

Action: For discussion/adoption

2.2.12. - EMA/OD/0000037166

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

2.2.13. - EMA/OD/0000037386

Treatment of homocystinuria

Action: For discussion/adoption

2.2.14. - EMA/OD/0000037416

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

Action: For discussion/adoption

2.2.15. - EMA/OD/0000037799

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

2.2.16. - EMA/OD/0000037822

Treatment of frontotemporal dementia (FTD)

Action: For discussion/adoption

2.2.17. - EMA/OD/0000037830

Treatment of hypoparathyroidism

Action: For discussion/adoption

2.2.18. - EMA/OD/0000037899

Treatment of congenital pulmonary hypoplasia in infancy

Action: For discussion/adoption

2.2.19. - EMA/OD/0000038026

Treatment of RDH12 mutation associated retinal dystrophy

Action: For discussion/adoption

2.2.20. - EMA/OD/0000030309

Treatment of angioimmunoblastic T-cell lymphoma (AITL) and other nodal T-cell lymphomas of follicular helper T-cell (TFH) origin

Action: For discussion/adoption

2.2.21. - EMA/OD/0000031771

Treatment of traumatic brain injury with development of oedema

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 08-10 September 2020 COMP meeting

2.7. Evaluation on-going

16 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 bullous pemphigoid

Action: For adoption

3.1.2. -

Treatment of amyotrophic lateral sclerosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of short bowel syndrome

Action: For information

3.2.2. -

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

Action: For information

3.3. New requests

3.3.1. -

Treatment of systemic mastocytosis

Action: For information

3.3.2. -

Treatment of neuroblastoma

Action: For information

3.3.3. -

Treatment of immune thrombocytopenia

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. Calquence – 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 adoption, Oral explanation to be held on 08 September 2020 at 15:00

4.1.2. Adakveo – Crizanlizumab - EMEA/H/C/004874, EMA/OD/026/12, EU/3/12/1034, EMA/OD/0000009984

Novartis Europharm Limited; Treatment of sickle cell disease

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

4.1.3. ARIKAYCE liposomal - amikacin - EMEA/H/C/005264/0000, EU/3/14/1259, EMA/OD/0000030955

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

Action: For adoption

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. - 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 discussion

4.2.3. - obiltoxaximab - EMEA/H/C/005169/0000, EU/3/18/2065, EMA/OD/0000037218

SFL Pharmaceuticals Deutschland GmbH; Treatment of anthrax

Action: For discussion

4.2.4. - valoctocogene roxaparvovec - EMEA/H/C/004749/0000, EU/3/16/1622, EMA/OD/0000024177

Accelerated assessment

BioMarin International Limited; Treatment of haemophilia A

Action: For discussion

4.2.5. - deferiprone - EMEA/H/C/005004/0000, EU/3/18/2034, EMA/OD/0000011266

Apotex B.V.; Treatment of neurodegeneration with brain iron accumulation

Action: For information

4.2.6. - somapacitan - EMEA/H/C/005030/0000, EU/3/18/2068, EMA/OD/0000033719

Novo Nordisk A/S; Treatment of growth hormone deficiency

Action: For discussion

4.2.7. - 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

4.2.8. - 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

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

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

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. 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 discussion

5.2.2. - delamanid - EMEA/H/C/002552/X/0046/G, EMA/OD/094/07, EU/3/07/524

Otsuka Novel Products GmbH; Treatment of tuberculosis

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 discussion

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 4 September 2020 at 11:00

Document tabled:
PAWG draft agenda for 4 September 2020 meeting

7.1.3. COMP Workshop 2020

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 July 2020

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)

Action: For information

Document(s) tabled:

Meeting report PCWP HCPWP meeting 2 June

Meeting report PCWP HCPWP meeting on ICH 3 June

Meeting report PCWP HCPWP meeting 24 June

Draft Agenda Workshop on the application of the General Data Protection Regulation (GDPR) in the area of health and Secondary Use of Data for Medicines and Public Health Purposes - 23/09/2020

Draft Agenda Workshop on benefit-risk of medicines used during pregnancy and breastfeeding - 22/09/2020

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. Reactivation of EMA Working parties

Action: For information

8.2. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q3/2020 Update of the Business Pipeline report for the human scientific committees

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/