6 September 2021
EMA/COMP/490692/2021
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 7-9 September 2021

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

07 September 2021, 08:30-19:30, remote virtual meeting

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

09 September 2021, 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 7-9 September 2021. See (current) September 2021 COMP minutes (to be published post October 2021 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 7-9 September 2021.

1.3. Adoption of the minutes

COMP minutes for 13-15 July 2021.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000055853

Treatment of frontotemporal dementia

Action: For adoption

2.1.2. - EMA/OD/0000059436

Treatment of megalencephalic leukoencephalopathy with subcortical cysts

Action: For adoption, Oral explanation to be held on 07 September 2021 at 09:15

2.1.3. - EMA/OD/0000058277

Treatment of dermatomyositis

Action: For adoption, Oral explanation to be held on 08 September 2021 at 17:00

2.1.4. - EMA/OD/0000058281

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Action: For adoption, Oral explanation to be held on 08 September 2021 at 17:00

2.1.5. - EMA/OD/0000061301

Treatment of invasive aspergillosis

Action: For adoption, Oral explanation to be held on 07 September 2021 at 16:30

2.1.6. - EMA/OD/0000061466

Treatment of transthyretin-mediated amyloidosis (ATTR)

Action: For adoption, Oral explanation to be held on 08 September 2021 at 09:00

2.1.7. - EMA/OD/0000057849

Treatment of uveal melanoma

Action: For adoption, Oral explanation to be held on 07 September 2021 at 11:00

2.1.8. - EMA/OD/0000058053

Treatment of gastric cancer

Action: For adoption, Oral explanation to be held on 08 September 2021 at 11:45

2.1.9. - EMA/OD/0000058526

Treatment of acute liver failure

Action: For adoption, Oral explanation to be held on 08 September 2021 at 13:45

2.1.10. - EMA/OD/0000061671

Treatment of tuberous sclerosis

Action: For adoption, Oral explanation to be held on 08 September 2021 at 15:30

2.1.11. - EMA/OD/0000062387

Treatment of optic neuritis

Action: For information

Notes: Withdrawal request received on 28 July 2021.

2.1.12. - EMA/OD/0000059143

Treatment of Burkitt's lymphoma

Action: For adoption, Oral explanation to be held on 07 September 2021 at 14:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000053713

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.2. - EMA/OD/0000057352

Treatment of primary biliary cholangitis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000058336

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000058552

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000060407

Treatment of Diamond-Blackfan anemia

Action: For discussion/adoption

2.2.6. - EMA/OD/0000061114

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Action: For discussion/adoption

2.2.7. - EMA/OD/0000061146

Treatment of retinal detachment

Action: For discussion/adoption

2.2.8. - EMA/OD/0000061663

Treatment of small cell lung cancer (SCLC)

Action: For discussion/adoption

2.2.9. - EMA/OD/0000061869

Treatment in hematopoietic stem cell transplantation (HSCT)

Action: For discussion/adoption

2.2.10. - EMA/OD/0000062259

Treatment of glioma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000062364

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Action: For discussion/adoption

2.2.12. - EMA/OD/0000063084

Treatment of mucopolysaccharidosis I

Action: For discussion/adoption

2.2.13. - EMA/OD/0000064329

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.14. - EMA/OD/0000064480

Treatment of cone-rod dystrophy

Action: For discussion/adoption

2.2.15. - EMA/OD/0000064772

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Action: For discussion/adoption

2.2.16. - EMA/OD/0000064903

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Action: For discussion/adoption

2.2.17. - EMA/OD/0000064944

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Action: For discussion/adoption

2.2.18. - EMA/OD/0000065116

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Action: For discussion/adoption

2.2.19. - EMA/OD/0000065221

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Action: For discussion/adoption

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Action: For discussion/adoption

2.2.21. - EMA/OD/0000065329

Treatment of idiopathic pulmonary fibrosis

Action: For discussion/adoption

2.2.22. - EMA/OD/0000065353

Treatment of Krabbe's disease

Action: For discussion/adoption

2.2.23. - EMA/OD/0000065415

Treatment of mucopolysaccharidosis type II (Hunter's syndrome)

Action: For discussion/adoption

2.2.24. - EMA/OD/0000065562

Treatment of fragile X syndrome

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(s) tabled:
OMPD applications - appointment of rapporteurs at the 07-09 September 2021 COMP meeting

2.7. Evaluation on-going

22 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 acute myeloid leukaemia

Action: For adoption

3.1.2. -

Treatment of hyperphenylalaninemia

Action: For adoption

3.1.3. -

Treatment of polycythaemia vera

Action: For adoption

3.1.4. -

Treatment of soft tissue sarcoma

Action: For adoption

3.2. Finalised letters

3.2.1. -

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

Action: For information

3.2.2. -

Treatment in haematopoietic stem cell transplantation

Action: For information

3.3. New requests

3.3.1. -

Treatment of glioma

Action: For information

3.3.2. -

Treatment of haemophilia A

Action: For information

3.3.3. -

Treatment of acute myeloid leukaemia

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. - zanubrutinib - EMEA/H/C/004978/0000, EU/3/19/2167, EMA/OD/0000058248

BeiGene Ireland Limited; Treatment of lymphoplasmacytic lymphoma

Action: For discussion/adoption

4.2.2. – glucarpidase - EMEA/H/C/005467/0000, EMA/OD/049/02, EU/3/02/128, EMA/OD/0000042598

Protherics Medicines Development Europe B.V.; Adjunctive treatment in patients at risk of methotrexate toxicity

Action: For discussion/adoption

4.2.3. – artesunate - EMEA/H/C/005550, EU/3/20/2251, EMA/OD/0000060998

Amivas Ireland Ltd; Treatment of malaria

Action: For discussion/adoption

4.2.4. – lonafarnib - EMA/OD/0000001643, EU/3/18/2118, EMA/OD/0000067500

EigerBio Europe Limited; Treatment of Hutchinson-Gilford progeria syndrome

Action: For discussion/adoption

4.2.5. – pegcetacoplan - EMEA/H/C/005553, EU/3/17/1873, EMA/OD/0000051430

Apellis Ireland Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Action: For discussion/adoption

4.2.6. – ripretinib - EMEA/H/C/005614, EU/3/17/1936, EMA/OD/0000057360

Deciphera Pharmaceuticals (Netherlands) B.V; Treatment of gastrointestinal stromal tumours

Action: For discussion/adoption

4.2.7. Tecartus – 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/II/0008/G, EU/3/20/2344, EMA/OD/0000063560

Kite Pharma EU B.V.; Treatment of acute lymphoblastic leukaemia

CHMP Rapporteur: Jan Mueller-Berghaus; CHMP Co-Rapporteur: Rune Kjeken

Action: For discussion/adoption

4.2.8. - lonapegsomatropin - EMEA/H/C/005367/0000, EU/3/19/2213, EMA/OD/0000059751

Ascendis Pharma Endocrinology Division A/S; Treatment of growth hormone deficiency

Action: For discussion/adoption

4.2.9. – artesunate - EMEA/H/C/005718/0000, EMA/OD/043/15, EU/3/15/1521, EMA/OD/0000063220

B And O Pharm; Treatment of malaria

Action: For discussion/adoption

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 marketing authorisation extension

5.1. After adoption of CHMP opinion

None

5.2. Prior to adoption of CHMP opinion

None

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 – joint COMP/PDCO, 19 November 2021, Lisbon, Portugal

Action: For information

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 3 September 2021 at 11:00

Document(s) tabled:

PAWG draft agenda for 3 September 2021 meeting

7.1.3. COMP Rules of Procedure - revision

Action: For adoption

Document(s) tabled:
COMP Rules of Procedure Rev. 6

7.1.4. Pilot – Relaunch of face to face Scientific Committee Meetings

Action: For discussion

7.1.5. Election of COMP Chairperson

Action: For adoption

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

Action: For information

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes July 2021

7.2.2. CAT-COMP Working Group

Proposed meeting time on 6 September 2021 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) and Working Party with Healthcare Professionals' Organisations (HCPWP)

Action: For information

Document(s) tabled:

Draft Agenda - PCWP and HCPWP Joint meeting, 21-22 September 2021

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)

None

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

7.5.3. Therapeutic Goods Administration (TGA), Australia

None

7.5.4. Health Canada

None

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 2021

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Updated Guideline on registry-based studies

Action: For information

8.2. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document(s) tabled:
Q3/2021 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/