10 June 2021
EMA/COMP/276731/2021
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 15-17 June 2021

Chair: Violeta Stoyanova-Beninska

15 June 2021, 08:30-19:30, remote virtual meeting

16 June 2021, 08:30-19:30, remote virtual meeting

17 June 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).

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 15-17 June 2021. See (current) June 2021 COMP minutes (to be published post July 2021 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 15-17 June 2021.

1.3. Adoption of the minutes

COMP minutes for 10-12 May 2021.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000053160

Treatment of primary biliary cholangitis

Action: For adoption

2.1.2. - EMA/OD/0000053899

Treatment of follicular lymphoma

Action: For adoption

2.1.3. - EMA/OD/0000055257

Treatment of growth hormone deficiency

Action: For adoption

2.1.4. - EMA/OD/0000045468

Treatment of glioma

Action: For adoption, Oral explanation to be held on 15 June 2021 at 11:30

2.1.5. - EMA/OD/0000055663

Treatment of soft-tissue sarcoma

Action: For adoption, Oral explanation to be held on 15 June 2021 at 15:00

2.1.6. - EMA/OD/0000055340

Treatment of medulloblastoma

Action: For adoption, Oral explanation to be held on 16 June 2021 at 15:30

2.1.7. - EMA/OD/0000054743

Treatment of diffuse large B-cell lymphoma

Action: For information

Notes: Withdrawal request received on 17 May 2021.

2.1.8. - EMA/OD/0000056712

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 17 June at 09:00

2.1.9. - EMA/OD/0000056592

Treatment of amyotrophic lateral sclerosis

Action: For adoption, Oral explanation to be held on 15 June 2021 at 17:00

2.1.10. - EMA/OD/0000054015

Treatment of hereditary angioedema

Action: For adoption, Oral explanation to be held on 16 June 2021 at 13:30

2.1.11. - EMA/OD/0000055883

Treatment of nontraumatic spontaneous intracerebral hemorrhage (SICH)

Action: For adoption, Oral explanation to be held on 16 June 2021 at 17:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000033980

Treatment of biliary tract cancer

Action: For discussion/adoption

2.2.2. - EMA/OD/0000041501

Treatment of pulmonary arterial hypertension (PAH) condition

Action: For discussion/adoption

2.2.3. - EMA/OD/0000044658

Treatment of familial chylomicronaemia syndrome

Action: For discussion/adoption

2.2.4. - EMA/OD/0000053211

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000053228

Treatment of pantothenate kinase-associated neurodegeneration

Action: For discussion/adoption

2.2.6. - EMA/OD/0000053262

Treatment of propionic acidaemia

Action: For discussion/adoption

2.2.7. - EMA/OD/0000053328

Treatment of Graft versus Host Disease

Action: For discussion/adoption

2.2.8. - EMA/OD/0000054695

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.9. - EMA/OD/0000055969

Treatment of chronic myeloid leukemia

Action: For discussion/adoption

2.2.10. - EMA/OD/0000056765

Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000056412

Treatment of small cell lung cancer

Action: For discussion/adoption

2.2.12. - EMA/OD/0000057079

Treatment of GM2 gangliosidosis

Action: For discussion/adoption

2.2.13. - EMA/OD/0000057402

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.14. - EMA/OD/0000057891

Treatment of glycogen storage disease type III (GSD III)

Action: For discussion/adoption

2.2.15. - EMA/OD/0000057939

Treatment of *SLC13A5*-epileptic encephalopathy deficiencies

Action: For discussion/adoption

2.2.16. - EMA/OD/0000058120

Treatment of upper tract urothelial carcinoma

Action: For discussion/adoption

2.2.17. - EMA/OD/0000058064

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

2.2.18. - EMA/OD/0000058250

Treatment of achondroplasia

Action: For discussion/adoption

2.2.19. - EMA/OD/0000058262

Treatment of myotonic disorders

Action: For discussion/adoption

2.2.20. - EMA/OD/0000058312

Treatment of Allan-Herndon-Dudley syndrome

Action: For discussion/adoption

2.2.21. - EMA/OD/0000058314

Treatment of otoferlin gene (*OTOF*)-mediated hearing loss

Action: For discussion/adoption

2.2.22. - EMA/OD/0000058504

Treatment of adenosine triphosphate binding cassette transporter protein subfamily C member 6 (*ABCC6*) deficiency

Action: For discussion/adoption

2.2.23. - EMA/OD/0000058488

Treatment of frontotemporal dementia

Action: For discussion/adoption

2.2.24. - EMA/OD/0000058580

Treatment of idiopathic pulmonary fibrosis (IPF)

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 15-17 June 2021 COMP meeting

2.7. Evaluation on-going

1 application 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 in haematopoietic stem cell transplantation

Action: For adoption

3.1.2. -

Treatment of pancreatic cancer

Action: For adoption

3.1.3. -

Treatment of myelodysplastic syndromes

Action: For adoption

3.1.4. -

Treatment of acute myeloid leukemia

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of transthyretin-mediated amyloidosis in patients with cardiomyopathy

Action: For information

3.2.2. -

Treatment of mucopolysaccharidosis type I

Action: For information

3.2.3. -

Treatment of glioma

Action: For information

3.3. New requests

3.3.1. -

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

Action: For information

3.3.2. -

Treatment of Duchenne muscular dystrophy

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. – eflornithine / sulindac - EMEA/H/C/005043/0000, EMA/OD/130/12, EU/3/12/1086, EMA/OD/0000061571

Cancer Prevention Pharma (Ireland) Limited; Treatment of familial adenomatous polyposis

Action: For information

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

Celgene Europe BV; Treatment of multiple myeloma

Action: For discussion/adoption

4.2.3. – vosoritide - EMEA/H/C/005475/0000, EMA/OD/149/12, EU/3/12/1094, EMA/OD/0000032549

BioMarin International Limited; Treatment of achondroplasia

Action: For discussion/adoption

4.2.4. – tafasitamab - EMEA/H/C/005436/0000, EMA/OD/215/14, EU/3/14/1424, EMA/OD/0000047254

Morphosys AG; Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

4.2.5. – zanubrutinib - EMEA/H/C/004978/0000, EMA/OD/0000004269, EU/3/19/2167, EMA/OD/0000058248

BeiGene Ireland Ltd; Treatment of lymphoplasmacytic lymphoma

Action: For discussion/adoption

4.2.6. – avalglucosidase alfa - EMEA/H/C/005501, EU/3/14/1251, EMA/OD/0000048959

Genzyme Europe BV; Treatment of Pompe's disease

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 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 meetings

None

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 11 June 2021 at 13:00

Document tabled:

PAWG draft agenda for 11 June 2021 meeting

7.1.3. Election of COMP Vice-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 May 2021

7.2.2. CAT-COMP Working Group

Proposed meeting time on 14 June 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, 1-2 June 2021

7.3.2. Satisfactory Methods Working Group

Action: For discussion

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

Public consultation for the revision of the Orphan Regulation

Action: For discussion

Updated Q&A related to the assessment of similarity for Advanced Therapy Medicinal products in the context of the orphan legislation

Action: For information

The updated version of the EC Q&A is published: https://ec.europa.eu/health/human-use/advanced-therapies_en (see at the bottom of the page).

7.5. Cooperation with International Regulators

7.5.1. Food and Drug Administration (FDA)

Action: For information

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. Horizon Scanning report on Genome Editing

A report on genome editing has been finalised by the EU Innovation network (EU IN; EMA Rapporteur; contributions by experts from the EU Network, Committees and Working parties).

Action: For information

Document tabled:
Genome editing, EU-IN Horizon Scanning Report

8.2. Real World Data use cases

Action: For discussion

8.3. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q2/2021 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/