7 May 2021
EMA/COMP/221232/2021
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 10-12 May 2021

Chair: Violeta Stoyanova-Beninska

10 May 2021, 08:30-19:30, remote virtual meeting

11 May 2021, 08:30-19:30, remote virtual meeting

12 May 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
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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 10-12 May 2021. See May 2021 minutes (to be published post June 2021 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 10-12 May 2021.

1.3. Adoption of the minutes

COMP minutes for 13-15 April 2021.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000052866

Treatment of essential thrombocythaemia

Action: For adoption

2.1.2. - EMA/OD/0000049844

Treatment of solid organ transplantation

Action: For adoption, Oral explanation to be held on 10 May 2021 at 11:30

2.1.3. - EMA/OD/0000037664

Treatment of cystinuria

Action: For adoption, Oral explanation to be held on 10 May 2021 at 16:00

2.1.4. - EMA/OD/0000051199

Treatment of Huntington's disease

Action: For adoption, Oral explanation to be held on 11 May 2021 at 09:00

2.1.5. - EMA/OD/0000045928

Treatment of nasopharyngeal cancer

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

2.1.6. - EMA/OD/0000051869

Treatment of pulmonary arterial hypertension

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

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000024142

Treatment of primary IgA nephropathy

Action: For discussion/adoption

2.2.2. - EMA/OD/0000045468

Treatment of glioma

Action: For discussion/adoption

2.2.3. - EMA/OD/0000047485

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

2.2.4. - EMA/OD/0000049662

Treatment of pre-eclampsia

Action: For discussion/adoption

2.2.5. - EMA/OD/0000052133

Treatment of mantle cell lymphoma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000053160

Treatment of primary biliary cholangitis

Action: For discussion/adoption

2.2.7. - EMA/OD/0000053493

Prevention of bronchopulmonary dysplasia

Action: For discussion/adoption

2.2.8. - EMA/OD/0000053556

Prevention of retinopathy of prematurity

Action: For discussion/adoption

2.2.9. - EMA/OD/0000053899

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.10. - EMA/OD/0000054015

Treatment of hereditary angioedema

Action: For discussion/adoption

2.2.11. - EMA/OD/0000054743

Treatment of diffuse large B-cell lymphoma

Action: For discussion/adoption

2.2.12. - EMA/OD/0000055239

Treatment of achondroplasia

Action: For discussion/adoption

2.2.13. - EMA/OD/0000055257

Treatment of growth hormone deficiency

Action: For discussion/adoption

2.2.14. - EMA/OD/0000055289

Treatment of glioma

Action: For discussion/adoption

2.2.15. - EMA/OD/0000055340

Treatment of medulloblastoma

Action: For discussion/adoption

2.2.16. - EMA/OD/0000055345

Treatment of Friedreich`s ataxia

Action: For discussion/adoption

2.2.17. - EMA/OD/0000055663

Treatment of soft-tissue sarcoma

Action: For discussion/adoption

2.2.18. - EMA/OD/0000055883

Treatment of nontraumatic spontaneous intracerebral hemorrhage (SICH)

Action: For discussion/adoption

2.2.19. - EMA/OD/0000056297

Treatment of methylmalonic acidaemia

Action: For discussion/adoption

2.2.20. - EMA/OD/0000056592

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.21. - EMA/OD/0000056712

Treatment of amyotrophic lateral sclerosis

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 10-12 May 2021 COMP meeting

2.7. Evaluation on-going

24 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 transthyretin-mediated amyloidosis in patients with cardiomyopathy

Action: For adoption

3.1.2. -

Treatment of mucopolysaccharidosis type I

Action: For adoption

3.1.3. -

Treatment of glioma

Action: For adoption

3.2. Finalised letters

3.2.1. -

Diagnosis of AL amyloidosis

Action: For information

3.2.2. -

Treatment of Gaucher disease

Action: For information

3.3. New requests

3.3.1. -

Treatment of pancreatic cancer

Action: For information

3.3.2. -

Treatment in haematopoietic stem cell transplantation

Action: For information

3.3.3. -

Treatment of myelodysplastic syndromes

Action: For information

3.3.4. -

Treatment of acute myeloid leukemia

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. – elivaldogene autotemcel - EMEA/H/C/003690/0000, EMA/OD/009/12, EU/3/12/1003, EMA/OD/0000044429

Accelerated Assessment

bluebird bio (Netherlands) B.V; Treatment of adrenoleukodystrophy

Action: For information

4.2.2. – odevixibat - EMEA/H/C/004691/0000, EMA/OD/022/12, EU/3/12/1028, EMA/OD/0000048989

Accelerated Assessment

Albireo; Treatment of progressive familial intrahepatic cholestasis

Action: For discussion/adoption

4.2.3. Darzalex - daratumumab - EMEA/H/C/004077/II/0043 EMA/OD/207/17, EU/3/18/2020, EMA/OD/0000049819

Janssen-Cilag International NV; Treatment of AL amyloidosis

CHMP Rapporteur: Sinan B. Sarac; CHMP Co-Rapporteur: Blanca Garcia Ochoa

Action: For discussion/adoption

4.2.4. – setmelanotide - EMEA/H/C/005089/0000

Accelerated assessment

TMC Pharma (EU) Limited

a) Treatment of leptin receptor deficiency, EU/3/18/2101, EMA/OD/0000040440

b) Treatment of pro-opiomelanocortin deficiency, EMA/OD/063/16, EU/3/16/1703, EMA/OD/0000040443

Action: For discussion/adoption

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

Genzyme Europe BV; Treatment of Pompe's disease

Action: For discussion

4.2.6. – 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 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 meetings

None

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 7 May 2021 at 12.00

Document tabled:

PAWG draft agenda for 7 May 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 April 2021

7.2.2. CAT-COMP Working Group

Proposed meeting time on 7 May 2021 at 13:00

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)

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. Update from the Research and Innovation (RNI) workstream

Action: For information

8.2. Marketing Authorisation Applications 3-year forecast report

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/