12 February 2021
EMA/COMP/52622/2021
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 16-18 February 2021

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

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

17 February 2021, 08:30-19:30, remote virtual meeting

18 February 2021, 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. 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 16-18 February 2021. See February 2021 COMP minutes (to be published post March 2021 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 16-18 February 2021.

1.3. Adoption of the minutes

COMP minutes for 19-21 January 2021.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000046077

Treatment of Krabbe disease

Action: For discussion/adoption

2.1.2. - EMA/OD/0000041484

Treatment of fulminant hypermetabolic crisis secondary to calcium dysregulation in skeletal muscle

Action: For adoption, Oral explanation to be held on 16 February 2021 at 11:00

2.1.3. - EMA/OD/0000043808

Treatment of bronchopulmonary dysplasia

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

2.1.4. - EMA/OD/0000046325

Prevention of bronchopulmonary dysplasia

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

2.1.5. - EMA/OD/0000046448

Treatment of ATTR amyloidosis

Action: For adoption, Oral explanation to be held on 17 February 2021 at 13:30

2.1.6. - EMA/OD/0000048780

Treatment of Dravet syndrome

Action: For adoption, Oral explanation to be held on 17 February 2021 at 18:00

2.1.7. - EMA/OD/0000046254

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 18 February 2021 at 09:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000038966

Treatment of pulmonary hypertension associated with interstitial lung disease

Action: For discussion/adoption

2.2.2. - EMA/OD/0000041696

Treatment of mucopolysaccharidosis type I

Action: For discussion/adoption

2.2.3. - EMA/OD/0000041862

Treatment of small cell lung cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000042673

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000044231

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.6. - EMA/OD/0000045910

Treatment of glioma

Action: For discussion/adoption

2.2.7. - EMA/OD/0000047280

Treatment of PIK3CA related overgrowth spectrum

Action: For discussion/adoption

2.2.8. - EMA/OD/0000047634

Treatment of ovarian cancer

Action: For discussion/adoption

2.2.9. - EMA/OD/0000047694

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000048121

Treatment of cutaneous T-cell lymphoma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000048469

Treatment of non-functioning pituitary adenomas

Action: For discussion/adoption

2.2.12. - EMA/OD/0000048721

Treatment of non-small cell lung cancer with EGFR alterations

Action: For discussion/adoption

2.2.13. - EMA/OD/0000052192

Treatment of Friedreich's ataxia

Action: For discussion/adoption

2.2.14. - EMA/OD/0000052275

Treatment of eosinophilic oesophagitis

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 16-18 February 2021 COMP meeting

2.7. Evaluation on-going

14 applications for orphan designation will not be discussed as evaluation is ongoing.

Action: For information

Notes: See 7.8.1. table

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of Fabry disease

Action: For adoption

3.1.2. –

Treatment of relapsed or refractory multiple myeloma

Action: For adoption

3.1.3. -

Treatment of sickle cell disease

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 of multiple myeloma

Action: For information

3.2.3. -

Treatment of ATTR amyloidosis-polyneuropathy

Action: For information

3.2.4. -

Treatment of ATTR amyloidosis-cardiomyopathy

Action: For information

3.3. New requests

3.3.1. -

Treatment of pancreatic cancer

Action: For information

3.3.2. -

Treatment of Gaucher disease

Action: For information

3.3.3. -

Treatment of growth hormone deficiency

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. Nexpovio - selinexor - EMEA/H/C/005127, EMA/OD/087/14, EU/3/14/1355, EMA/OD/0000043722

Karyopharm Europe GmbH; Treatment of plasma cell myeloma

CHMP Rapporteur: Jorge Camarero Jiménez; CHMP Co-Rapporteur: Sinan B. Sarac

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

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

Novo Nordisk A/S; Treatment of growth hormone deficiency

Action: For adoption, Oral explanation to be held on 17 February 2021 at 11:30

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

4.2.1. 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

Action: For information

4.2.2. – duvelisib

Verastem Europe GmbH

a) Treatment of Follicular lymphoma, EMEA/H/C/005381/0000, EMA/OD/047/13, EU/3/13/1157, EMA/OD/0000024085

b) Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma EMEA/H/C/005381/0000, EMA/OD/196/12, EU/3/13/1125, EMA/OD/0000026423

Action: For information

4.2.3. – hydrocortisone - EMEA/H/C/005105/0000, EMA/OD/020/05, EU/3/05/296, EMA/OD/0000032128

Diurnal Europe BV; Treatment of congenital adrenal hyperplasia

Action: For discussion

4.2.4. – setmelanotide

Accelerated assessment

TMC Pharma (EU) Limited

a) Treatment of leptin receptor deficiency, EMEA/H/C/005089/0000, EU/3/18/2101, EMA/OD/0000040440

b) Treatment of pro-opiomelanocortin deficiency, EMEA/H/C/005089/0000, EMA/OD/063/16, EU/3/16/1703, EMA/OD/0000040443

Action: For discussion

4.2.5. – satralizumab - EMEA/H/C/004788, EMA/OD/014/16, EU/3/16/1680, EMA/OD/0000016001

Roche Registration GmbH; Treatment of neuromyelitis optica spectrum disorders

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

Document(s) tabled:

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. Kaftrio - ivacaftor/tezacaftor/elexacaftor - EMEA/H/C/005269/II/0001, EMA/OD/0000001208, EU/3/18/2116, EMA/OD/0000042077

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP Rapporteur: Johann Lodewijk Hillege

Action: For information

5.2.2. Kalydeco - ivacaftor - EMEA/H/C/002494/II/0089, EMA/OD/010/08, EU/3/08/556, EMA/OD/0000042076

Vertex Pharmaceuticals (Ireland) Limited; Treatment of cystic fibrosis

CHMP Rapporteur: Maria Concepcion Prieto Yerro

Action: For information

5.2.3. Darzalex - daratumumab - EMEA/H/C/004077/II/0044, EMA/OD/038/13, EU/3/13/1153, EMA/OD/0000049818

Janssen-Cilag International NV; Treatment of plasma cell myeloma

CHMP Rapporteur: Sinan B. Sarac

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 meetings

COMP-SRLM of the Portuguese Presidency will be held on 11th February 2021 – outcome of the meeting

Action: For information

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 12 February 2021 at 11:30

Document tabled:
PAWG draft agenda for 12 February 2021

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 January 2021

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 for the PCWP/HCPWP meeting on 2-3 March

7.4. Cooperation within the EU regulatory network

7.4.1. COMP Working Group on the orphan regulation

Conclusions of the COMP WG on the orphan regulation

Action: For discussion/adoption

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 2021

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1.

8.2. Roll-out of WebEx for COMP

Action: For information

8.3. Re-engineered ITF - presentation to COMP

Action: For information

8.4. EMA draft pregnancy strategy

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/