15 July 2019
EMA/COMP/361001/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 16-18 July 2019

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

16 July 2019, 08:30-19:30, room 2A

17 July 2019, 08:00-19:30, room 2A

18 July 2019, 08:00-17:00, room 2A

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 16-18 July 2019. See July 2019 COMP minutes (to be published post September 2019 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 16-18 July 2019.

1.3. Adoption of the minutes

COMP minutes for 18-20 June 2019.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/00000029

Treatment of amyotrophic lateral sclerosis (ALS)

Action: For adoption, Oral explanation to be held on 16 July 2019 at 09:00

2.1.2. - EMA/OD/0000003546

Treatment of aneurysmal subarachnoid haemorrhage

Action: For adoption, Oral explanation to be held on 16 July 2019 at 10:00

2.1.3. - EMA/OD/0000003683

Treatment of beta-thalassaemia intermedia and major

Action: For information

Note: Withdrawal request received on 28 June 2019.

2.1.4. - EMA/OD/0000004755

Treatment of neuroblastoma

Action: For adoption, Oral explanation to be held on 16 July 2019 at 12:30

2.1.5. - EMA/OD/0000005579

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 16 July 2019 at 14:30

2.1.6. - EMA/OD/0000006192

Treatment of pneumonia caused by Pseudomonas aeruginosa

Action: For adoption, Oral explanation to be held on 16 July 2019 at 15:45

2.1.7. - EMA/OD/0000003566

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 16 July 2019 at 17:00

2.1.8. - EMA/OD/0000006345

Treatment of West syndrome

Action: For adoption, Oral explanation to be held on 16 July 2019 at 18:00

2.1.9. - EMA/OD/0000005689

Treatment of haemophilia B

Action: For adoption, Oral explanation to be held on 17 July 2019 at 09:00

2.1.10. - EMA/OD/0000005861

Treatment of myelodysplastic syndromes

Action: For adoption, Oral explanation to be held on 17 July 2019 at 14:00

2.1.11. - EMA/OD/0000006618

Treatment of haematopoietic stem cell transplantation

Action: For information

Note: Withdrawal request received on 3 July 2019.

2.1.12. - EMA/OD/0000004919

Treatment of graft loss in solid organ transplantation

Action: For information

Note: Withdrawal request received on 27 June 2019.

2.1.13. - EMA/OD/0000006189

Treatment of subarachnoid haemorrhage

Action: For information

Note: Withdrawal request received on 28 June 2019.

2.1.14. - EMA/OD/0000006379

Treatment of hepatitis D virus infection

Action: For adoption, Oral explanation to be held on 18 July 2019 at 09:00

2.1.15. - EMA/OD/0000006314

Treatment of fibromyalgia

Action: For adoption, Oral explanation to be held on 18 July 2019 at 10:00

2.1.16. - EMA/OD/0000004401

Treatment of sarcoidosis

Action: For information

Note: Withdrawal request received on 28 June 2019.

2.1.17. - EMA/OD/0000005898

Treatment of Huntington's disease

Action: For adoption, Oral explanation to be held on 17 July 2019 at 16:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000002080

Treatment of hypoparathyroidism

Action: For discussion/adoption

2.2.2. - EMA/OD/0000003541

Prevention of haemolytic disease of the foetus and newborn (HDFN)

Action: For discussion/adoption

2.2.3. - EMA/OD/0000004356

Treatment of progressive supranuclear palsy

Action: For discussion/adoption

2.2.4. - EMA/OD/0000004428

Treatment of conditioning treatment prior to haematopoietic stem cell transplantation

Action: For discussion/adoption

2.2.5. - EMA/OD/0000004857

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.6. - EMA/OD/0000005753

Treatment of myeloid or lymphoid neoplasm associated with FGFR1 rearrangement

Action: For discussion/adoption

2.2.7. - EMA/OD/0000005878

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.8. - EMA/OD/0000006325

Treatment of angiosarcoma

Action: For discussion/adoption

2.2.9. - EMA/OD/0000006352

Treatment of C3 Glomerulopathy

Action: For discussion/adoption

2.2.10. - EMA/OD/0000006386

Treatment of angiosarcoma

Action: For discussion/adoption

2.2.11. - EMA/OD/0000006955

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

2.2.12. - EMA/OD/0000007359

Treatment of Bardet Biedl syndrome (BBS).

Action: For discussion/adoption

2.2.13. - EMA/OD/0000007386

Treatment of periodic paralysis

Action: For discussion/adoption

2.2.14. - EMA/OD/0000007519

Treatment of congenital adrenal hyperplasia

Action: For discussion/adoption

2.2.15. - EMA/OD/0000007945

Treatment of Rett syndrome

Action: For discussion/adoption

2.2.16. - EMA/OD/0000008395

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

2.2.17. - EMA/OD/0000008785

Treatment of neuronal ceroid lipofuscinosis

Action: For discussion/adoption

2.2.18. - EMA/OD/0000008878

Treatment of acute myeloid leukemia

Action: For discussion/adoption

2.2.19. - EMA/OD/0000009156

Treatment of endophthalmitis

Action: For discussion/adoption

2.2.20. - EMA/OD/0000009203

Treatment of soft tissue sarcoma

Action: For discussion/adoption

2.2.21. - EMA/OD/0000009406

Treatment of glioma

Action: For discussion/adoption

2.2.22. - EMA/OD/0000009454

Treatment of glioma

Action: For discussion/adoption

2.2.23. - EMA/OD/0000009805

Treatment of multiple myeloma

Action: For discussion/adoption

2.2.24. - EMA/OD/0000009840

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.25. - EMA/OD/0000009964

Treatment of nontuberculous mycobacterial lung disease

Action: For discussion/adoption

2.2.26. - EMA/OD/0000009969

Prevention of complications in end-stage renal disease patients on peritoneal dialysis

Action: For discussion/adoption

2.2.27. - EMA/OD/0000010152

Treatment of Beta thalassemia

Action: For discussion/adoption

2.3. Revision of the COMP opinions

None

2.4. Amendment of existing orphan designations

2.4.1. – EMA/OD/0000010120

Takeda Pharma A/S; Treatment of anaplastic large cell lymphoma;

Action: For adoption

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 16-18 July 2019 COMP meeting

2.7. Evaluation on-going

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

Action: For information

Notes: See 7.8.1. Table 6. Evaluation Ongoing.

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of biliary tract cancer

Action: For adoption

3.1.2. -

Treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome)

Action: For adoption

3.1.3. -

Treatment of medullary thyroid carcinoma

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of diffuse large B-cell lymphoma

Action: For information

3.2.2. - -

Treatment of beta-thalassaemia intermedia and major

Action: For information

3.2.3. -

Treatment of spinal muscular atrophy

Action: For information

3.2.4. -

Treatment of immune thrombocytopenia

Action: For information

3.2.5. -

Treatment of multiple myeloma

Action: For information

3.2.6. -

Treatment of transthyretin-mediated amyloidosis

Action: For information

3.2.7. -

Treatment of acute myeloid leukaemia

Action: For information

3.3. New requests

3.3.1. -

Treatment of post-polycythaemia vera myelofibrosis

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. - polatuzumab vedotin – EMEA/H/C/004870, EMA/OD/231/17, EU/3/18/2013, EMA/OD/0000003161

Accelerated assessment

Roche Registration GmbH; Treatment of diffuse large B-cell lymphoma

Action: For information

4.2.2. – cannabidiol - EMEA/H/C/004675

GW Pharma (International) B.V;

a) Treatment of Dravet syndrome EMA/OD/083/14, EU/3/14/1339

b) Treatment of Lennox-Gastaut syndrome EMA/OD/275/16, EU/3/17/1855

Action: For discussion/adoption

4.2.3. Soliris - eculizumab – Type II variation – EMEA/H/C/000791/II/0105, EMA/OD/087/13, EU/3/13/1185, EMA/OD/0000004454

Alexion Europe SAS; Treatment of neuromyelitis optica spectrum disorder

CHMP rapporteur: Jorge Camarero Jiménez; CHMP co-rapporteur: Alexandre Moreau

Action: For discussion/adoption

4.2.4. - gilteritinib - EMEA/H/C/004752, EMA/OD/175/17, EU/3/17/1961, EMA/OD/0000006592

Astellas Pharma Europe B.V.; Treatment of acute myeloid leukaemia

Action: For discussion/adoption

4.2.5. - larotrectinib - EMEA/H/C/004919

Bayer AG;

a) Treatment of salivary gland cancer EMA/OD/213/17, EU/3/18/1995

b) Treatment of soft tissue sarcoma EMA/OD/184/15, EU/3/15/1606

c) Treatment of glioma EMA/OD/116/18, EU/3/18/2097

d) Treatment of papillary thyroid cancer EMA/OD/117/18, EU/3/18/2098

Action: For information

Withdrawal requests received on 2 July 2019.

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 marketing authorisation extension

5.1. After adoption of CHMP opinion

5.1.1. Imbruvica – ibrutinib - EMEA/H/C/003791/II/0046, EMA/OD/185/13, EU/3/14/1264, EMA/OD/0000002783

Janssen-Cilag International NV; Treatment of lymphoplasmacytic lymphoma

Action: For adoption, Oral explanation to be held on 17 July 2019 at 12:15

Notes: Status of the procedure at the CHMP: CHMP positive opinion adopted in June 2019.

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

Romanian presidency meeting held in Rome on 27-28 May.

Action: For adoption

Document(s) tabled:
COMP SRLM minutes May 2019

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 16 July 2019 at 13:30

Document tabled:
PAWG draft agenda for 16 July 2019 meeting

7.2. Document tabled: Minutes from the meetingCoordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendations on eligibility to PRIME – report from CHMP

Action: For information

Document(s) tabled:
PRIME eligibility requests - list of adopted outcomes June 2019

7.3. Coordination with EMA Working Parties/Working Groups/Drafting Groups

7.3.1. Working Party with Patients' and Consumers' Organisations (PCWP)

Action: For information

7.3.2. Working Party with Healthcare Professionals' Organisations (HCPWP)

Action: For information

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 2019

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. EMA's move to the permanent building

Update

Action: For information

8.2.

Action: For information

Document(s) tabled:

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/