9 September 2019
EMA/COMP/416435/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 10-12 September 2019

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

10 September 2019, 09:00-19:30, room 2A

11 September 2019, 08:30-19:30, room 2A

12 September 2019, 08:30-17:00, 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).

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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 September 2019. See September 2019 COMP minutes (to be published post October 2019 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 10-12 September 2019.

1.3. Adoption of the minutes

COMP minutes for 16-18 July 2019.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000009805

Treatment of multiple myeloma

Action: For adoption, Oral explanation to be held on 10 September 2019 at 14:00

2.1.2. - EMA/OD/0000004356

Treatment of progressive supranuclear palsy

Action: For information

Note: Withdrawal request received on 26 August 2019.

2.1.3. - EMA/OD/0000009156

Treatment of endophthalmitis

Action: For information

Note: Withdrawal request received on 9 August 2019.

2.1.4. - EMA/OD/0000004428

Treatment in haematopoietic stem cell transplantation

Action: For adoption, Oral explanation to be held on 10 September 2019 at 15:30

2.1.5. - EMA/OD/0000003541

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

Action: For adoption, Oral explanation to be held on 10 September 2019 at 17:00

2.1.6. - EMA/OD/0000005753

Treatment of myeloid or lymphoid neoplasm associated with FGFR1 rearrangement

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

2.1.7. - EMA/OD/0000004857

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 11 September 2019 at 10:30

2.1.8. - EMA/OD/0000008878

Treatment of acute myeloid leukaemia

Action: For adoption, Oral explanation to be held on 11 September 2019 at 12:00

2.1.9. - EMA/OD/0000006325

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 11 September 2019 at 14:00

2.1.10. - EMA/OD/0000006386

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 11 September 2019 at 14:00

2.1.11. - EMA/OD/0000009203

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 11 September 2019 at 15:30

2.1.12. - EMA/OD/0000009840

Treatment of cystic fibrosis

Action: For adoption, Oral explanation to be held on 11 September 2019 at 17:00

2.1.13. - EMA/OD/0000006955

Treatment of hepatocellular carcinoma

Action: For adoption, Oral explanation to be held on 11 September 2019 at 18:00

2.1.14. - EMA/OD/0000010152

Treatment of beta thalassemia intermedia and major

Action: For adoption, Oral explanation to be held on 12 September 2019 at 09:00

2.1.15. - EMA/OD/0000002080

Treatment of hypoparathyroidism

Action: For information

Note: Withdrawal request received on 13 August 2019.

2.1.16. - EMA/OD/0000009969

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

Action: For information

Note: Withdrawal request received on 27 August 2019.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000006190

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.2. - EMA/OD/0000007487

Treatment of growth hormone deficiency

Action: For discussion/adoption

2.2.3. - EMA/OD/0000007627

Treatment of retinopathy of prematurity

Action: For discussion/adoption

2.2.4. - EMA/OD/0000007659

Treatment of glioma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000007780

Treatment of mantle cell lymphoma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000008501

Treatment of Stargardt disease

Action: For discussion/adoption

2.2.7. - EMA/OD/0000009633

Treatment of autosomal recessive congenital ichthyosis (ARCI)

Action: For discussion/adoption

2.2.8. - EMA/OD/0000009997

Treatment of non-infectious uveitis

Action: For discussion/adoption

2.2.9. - EMA/OD/0000010168

Treatment of soft-tissue sarcoma

Action: For discussion/adoption

2.2.10. - EMA/OD/0000010228

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.11. - EMA/OD/0000011311

Treatment of CDKL5 deficiency disorder

Action: For discussion/adoption

2.2.12. - EMA/OD/0000012038

Treatment of graft versus host disease

Action: For discussion/adoption

2.2.13. - EMA/OD/0000012140

Treatment of Friedreich's ataxia

Action: For discussion/adoption

2.2.14. - EMA/OD/0000012303

Treatment of Duchenne muscular dystrophy

Action: For discussion/adoption

2.2.15. - EMA/OD/0000012576

Treatment of Netherton syndrome

Action: For discussion/adoption

2.2.16. - EMA/OD/0000012626

Treatment of ATTR amyloidosis

Action: For discussion/adoption

2.2.17. - EMA/OD/0000012628

Treatment of Alexander disease

Action: For discussion/adoption

2.2.18. - EMA/OD/0000012715

Treatment of invasive aspergillosis

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 September 2019 COMP meeting

2.7. Evaluation ongoing

Twelve 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 post-polycythaemia vera myelofibrosis

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of biliary tract cancer

Action: For information

3.2.2. -

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

Action: For information

3.2.3. -

Treatment of medullary thyroid carcinoma

Action: For information

3.3. New requests

3.3.1. -

Treatment of gastrointestinal stromal tumours

Action: For information

3.3.2. -

Treatment of graft-versus-host disease

Action: For information

3.3.3. -

Treatment of Duchenne muscular dystrophy

Action: For information

3.3.4. -

Treatment of amyotrophic lateral sclerosis

Action: For information

3.3.5. -

Treatment of congenital adrenal hyperplasia

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

Accelerated assessment

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

Action: For adoption, Oral explanation to be held on 11 September 2019 at time 09:00

4.2.2. - 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

4.2.3. – enasidenib - EMEA/H/C/004324, EMA/OD/253/15, EU/3/16/1640, EMA/OD/0000007422

Celgene Europe B.V.; Treatment of acute myeloid leukaemia

Action: For information

4.2.4. – glutamine – EMEA/H/C/004734, EMA/OD/016/12, EU/3/12/1011

Emmaus Medical Europe Limited; Treatment of sickle cell 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

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

None

5.2. Prior to adoption of CHMP opinion

5.2.1. Adcetris - brentuximab vedotin - Type II variation – EMEA/H/C/002455/II/0070

Takeda Pharma A/S;

a) Treatment of cutaneous T-cell lymphoma, EMA/OD/100/11, EU/3/11/939

b) Treatment of anaplastic large cell lymphoma, EMEA/OD/072/08, EU/3/08/595

c) Treatment of Hodgkin lymphoma, EMEA/OD/073/08, EU/3/08/596

CHMP rapporteur: Paula Boudewina van Hennik

Action: For discussion

Document(s) tabled:
Draft report on review of OMPD
Sponsor's report

5.2.2. Vyndaqel – tafamidis – EMEA/H/C/002294/X/0049/G, EMEA/OD/032/06, EU/3/06/401, EMA/OD/0000003853

Pfizer Europe MA EEIG; Treatment of familial amyloid polyneuropathy

Action: For discussion

5.2.3. Jorveza – budesonide – EMEA/H/C/004655/X/0007/G, EMA/OD/078/13, EU/3/13/1181, EMA/OD/0000013431

Dr. Falk Pharma GmbH; Treatment of eosinophilic oesophagitis

Action: For discussion

5.3. Appeal

None

5.4. Ongoing procedures

Action: For information

Document(s) tabled:
Review of orphan designation for OMP for MA extension - Ongoing 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, 27-28 May 2019, Rome, Italy

Romanian presidency meeting held in Rome on 27-28 May 2019.

Action: For adoption

Document tabled:
COMP SRLM minutes May 2019

7.1.2. Strategic Review & Learning meeting– joint COMP/CAT/PDCO, 21-22 November 2019, Helsinki, Finland

Action: For information

Document(s) tabled:
Draft Joint program 21.11.2019 – PDCO/COMP/CAT

7.1.3. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 10 September 2019 at 19:00

Document tabled:
PAWG draft agenda for 10 September 2019 meeting

7.2. Coordination 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 July 2019

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 adoption

Document tabled:
PCWP HCPWP Work plan 2019-2022

PCWP/HCPWP joint meeting – 25 September 2019

Action: For information

Document(s) tabled:
Draft agenda Joint PCWP/HCPWP meeting 25 September 2019

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

Action: For information

Document(s) tabled:
Draft Agenda PCWP meeting 24 September 2019

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

Action: For information

Document(s) tabled:
Draft Agenda HCPWP meeting 24 September 2019

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 Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q3/2019 Update of the Business Pipeline report for the human scientific committees

8.2. IRIS

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/