30 November 2018
EMA/COMP/792954/2018
Inspections, Human Medicines Pharmacovigilance and Committees

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 04-06 December 2018

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

04 December 2018, 08:30-19:30, room 02-F

05 December 2018, 08:30-19:30, room 02-F

06 December 2018, 08:30-15:00, room 02-F

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 04-06 December 2018. See December 2018 COMP minutes (to be published post January 2019 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 04-06 December 2018.

1.3. Adoption of the minutes

COMP minutes for 06-08 November 2018.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000001052

Treatment of Eosinophilic Esophagitis

Action: For information

Note: Withdrawal request received 16 November 2018.

2.1.2. - EMA/OD/0000001741

Treatment of Ataxia Telangiectasia

Action: For adoption, Oral explanation to be held on 04 December 2018 at 09:00

2.1.3. - EMA/OD/0000001574

Treatment of primary IgA nephropathy

Action: For adoption, Oral explanation to be held on 04 December 2018 at 10:00

2.1.4. - EMA/OD/0000001633

Treatment of Burkitt lymphoma

Action: For adoption, Oral explanation to be held on 05 December 2018 at 11:00

2.1.5. - EMA/OD/0000002033

Treatment of acetaminophen (paracetamol) poisoning

Action: For adoption, Oral explanation to be held on 04 December 2018 at 15:30

2.1.6. - EMA/OD/0000001719

Treatment of sickle cell disease

Action: For adoption, Oral explanation to be held on 04 December 2018 at 17:00

2.1.7. - EMA/OD/0000001592

Treatment of short bowel syndrome

Action: For adoption, Oral explanation to be held on 04 December 2018 at 18:00

2.1.8. - EMA/OD/0000002050

Treatment of glycogen storage disease type II (Pompe's disease)

Action: For adoption, Oral explanation to be held on 05 December 2018 at 17:00

2.1.9. - EMA/OD/0000001558

Treatment of haemophilia A

Action: For information

Note: Withdrawal request received 19 November 2018.

2.1.10. - EMA/OD/0000001096

Treatment of soft tissue sarcoma

Action: For information

Note: Withdrawal request received 19 November 2018.

2.1.11. - EMA/OD/0000001368

Treatment of active thyroid eye disease

Action: For adoption, Oral explanation to be held on 05 December 2018 at 12:00

2.1.12. - EMA/OD/0000001039

Diagnosis of medullary thyroid carcinoma

Action: For adoption, Oral explanation to be held on 05 December 2018 at 14:30

2.1.13. - EMA/OD/0000001988

Treatment of soft tissue sarcoma

Action: For adoption, Oral explanation to be held on 05 December 2018 at 15:30

2.1.14. - EMA/OD/0000002029

Treatment of immune thrombocytopenia

Action: For adoption, Oral explanation to be held on 06 December 2018 at 09:30

2.1.15. - EMA/OD/0000001206

Treatment of small cell lung cancer

Action: For adoption, Oral explanation to be held on 05 December 2018 at 18:00

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000001317

Treatment of Small cell lung cancer

Action: For discussion/adoption

2.2.2. - EMA/OD/0000001582

Treatment of acute myeloid leukaemia (AML)

Action: For discussion/adoption

2.2.3. - EMA/OD/0000001604

Treatment of Tuberous Sclerosis Complex

Action: For discussion/adoption

2.2.4. - EMA/OD/0000001606

Treatment of Pancreatic Carcinoma

Action: For discussion/adoption

2.2.5. - EMA/OD/0000001655

Treatment of Non-traumatic osteonecrosis

Action: For discussion/adoption

2.2.6. - EMA/OD/0000001791

Treatment of follicular lymphoma

Action: For discussion/adoption

2.2.7. - EMA/OD/0000001829

Treatment of Ulcerative Proctitis

Action: For discussion/adoption

2.2.8. - EMA/OD/0000001845

Treatment of beta-thalassaemia intermedia and major

Action: For discussion/adoption

2.2.9. - EMA/OD/0000001854

Treatment of Myasthenia gravis

Action: For discussion/adoption

2.2.10. - EMA/OD/0000001861

Treatment of Cyclin-dependent kinase-like 5 deficiency disorder

Action: For discussion/adoption

2.2.11. - EMA/OD/0000001881

Treatment of Pancreatic Cancer

Action: For discussion/adoption

2.2.12. - EMA/OD/0000001921

Treatment of perinatal asphyxia

Action: For discussion/adoption

2.2.13. - EMA/OD/0000002037

Treatment of Pulmonary Arterial Hypertension (PAH)

Action: For discussion/adoption

2.2.14. - EMA/OD/0000002209

Treatment of Rett syndrome

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 04-06 December 2018 COMP meeting

2.7. Evaluation on-going

Sixteen applications for orphan designation will not be discussed as evaluation is on-going.

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 glioma

Action: For adoption

3.1.2. -

Treatment of glycogen storage disease type II (Pompe's disease)

Action: For adoption

3.1.3. -

Treatment of neurofibromatosis type 1

Action: For adoption

3.1.4. -

Diagnosis of glioma

Action: For adoption

3.1.5. -

Treatment of multiple myeloma

Action: For adoption

3.2. Finalised letters

3.2.1. -

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

Action: For information

3.2.2. -

Treatment of spinal cord injury

Action: For information

3.2.3. -

Treatment of neurotrophic keratitis

Action: For information

3.3. New requests

3.3.1. -

Treatment of diffuse large B-cell lymphoma

Action: For information

3.3.2. - -

Treatment of multiple myeloma

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. - treosulfan - EMEA/H/C/004751, EMEA/OD/075/03, EU/3/04/186

medac Gesellschaft fur klinische Spezialpraparate mbH; Conditioning treatment prior to haematopoietic progenitor cell transplantation

Action: For adoption, Oral explanation to be held on 4 December 2018 at 11:00

4.2.2. - ropeginterferon alfa-2b - EMA/OD/055/11, EU/3/11/932, EMEA/H/C/004128

AOP Orphan Pharmaceuticals AG; Treatment of polycythaemia vera

Action: For adoption, Oral explanation to be held on 5 December 2018 at 10:00

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

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

5.1.1. Blincyto (blinatumomab) - Type II variation – EMEA/OD/029/09, EU/3/09/650, EMEA/H/C/003731/II/0011

Amgen Europe BV - The Netherlands; Treatment of acute lymphoblastic leukaemia

CHMP rapporteur: Alexandre Moreau; CHMP co-rapporteur: Daniela Melchiorri

Action: For adoption

Documents tabled:
Draft report on review of OMPD
Sponsor's report

5.2. Prior to adoption of CHMP opinion

5.2.1. Rubraca - rucaparib - Type II variation – EMEA/H/C/004272/II/0001, EMA/OD/085/12, EU/3/12/1049

Clovis Oncology UK Limited; Treatment of ovarian cancer

CHMP rapporteur: Jorge Camarero Jiménez

Action: For information

Document tabled:
Withdrawal request received 27 November 2018

5.2.2. Imbruvica – ibrutinib - Type II variation – EMEA/H/C/003791/II/0046, EMA/OD/0000002783

Janssen-Cilag International NV;

a) Treatment of chronic lymphocytic leukaemia EMA/OD/156/11, EU/3/12/984

b) Treatment of mantle cell lymphoma EMA/OD/171/12, EU/3/13/1115

c) Treatment of lymphoplasmacytic lymphoma EMA/OD/185/13, EU/3/14/1264

CHMP rapporteur: Filip Josephson

Action: For discussion

5.2.3. Imbruvica – ibrutinib - Type II variation – EMEA/H/C/003791/II/0047, EMA/OD/0000002367

Janssen-Cilag International NV;

a) Treatment of chronic lymphocytic leukaemia EMA/OD/156/11, EU/3/12/984

b) Treatment of mantle cell lymphoma EMA/OD/171/12, EU/3/13/1115

c) Treatment of lymphoplasmacytic lymphoma EMA/OD/185/13, EU/3/14/1264

CHMP rapporteur: Filip Josephson

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, 23-24 October 2018, Vienna, Austria

Action: For information

Documents tabled:
Presentations

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 4 December 2018 at 13:00

Document tabled:
PAWG draft agenda for 4 December 2018 meeting

7.1.3. Revision of "Points to Consider on the calculation and reporting of the prevalence of a condition for orphan designation" (COMP/436/01)

Action: For discussion

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 November 2018

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:
Meeting Summary PCWP Plenary Meeting 25 Sep 2018
Meeting Summary PCWP/HCPWP Joint Meeting 25 Sep 2018

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

COMP representative at HCPWG

Action: For adoption

7.4. Cooperation within the EU regulatory network

7.4.1. European Commission

None

7.4.2. The European Network of Centres for Pharmacoepidemiology & Pharmacovigilance (ENCePP)

COMP representative at ENCePP

Action: For 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. The 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 2018

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1. Concepts of significant benefit (follow-up to COMP Work Plan 2017)

Action: For discussion

8.2. IRIS: a new way of supporting COMP procedures with a CRM (Customer Relationship Management software)

Action: For discussion

8.3. EMA Business Pipeline activity and Horizon scanning

Action: For information

Document tabled:
Q4/2018 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/