17 February 2020
EMA/COMP/51841/2020
Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 18-20 February 2020

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

18 February 2020, 09:00-19:30, room 2A

19 February 2020, 08:30-19:30, room 2A

20 February 2020, 08:30-13:00, 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 18-20 February 2020. See February 2020 COMP minutes (to be published post March 2020 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 18-20 February 2020.

1.3. Adoption of the minutes

COMP minutes for 20-22 January 2020.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000005680

Treatment of short-bowel syndrome

Action: For adoption, Oral explanation to be held on 18 February 2020 at 09:30

2.1.2. - EMA/OD/0000021072

Treatment of viral associated haemorrhagic cystitis

Action: For adoption, Oral explanation to be held on 18 February 2020 at 14:00

2.1.3. - EMA/OD/0000020924

Treatment of biliary tract cancer

Action: For adoption, Oral explanation to be held on 18 February 2020 at 15:30

2.1.4. - EMA/OD/0000020976

Treatment of pancreatic cancer

Action: For adoption

2.1.5. - EMA/OD/0000019066

Treatment of nonsquamous non-small cell lung cancer

Action: For information

Note: Withdrawal request received on 31 January 2020.

2.1.6. - EMA/OD/0000021100

Treatment of chronic myeloid leukaemia (CML)

Action: For information

Note: Withdrawal request received on 12 February 2020.

2.1.7. - EMA/OD/0000020079

Treatment of chronic myeloid leukaemia

Action: For adoption, Oral explanation to be held on 19 February 2020 at 14:00

2.1.8. - EMA/OD/0000020844

Treatment of eumycetoma

Action: For adoption, Oral explanation to be held on 19 February 2020 at 15:30

2.1.9. - EMA/OD/0000020769

Treatment of intracerebral hemorrhage

Action: For adoption, Oral explanation to be held on 19 February 2020 at 17:00

2.1.10. - EMA/OD/0000014446

Treatment of cryopyrin-associated periodic syndromes

Action: For information

Note: Withdrawal request received on 29 January 2020.

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000015410

Treatment of facioscapulohumeral muscular dystrophy

Action: For discussion/adoption

2.2.2. - EMA/OD/0000015613

Treatment of autosomal recessive congenital ichthyosis

Action: For discussion/adoption

2.2.3. - EMA/OD/0000019167

Treatment of ovarian cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000020629

Treatment of small cell lung cancer (SCLC)

Action: For discussion/adoption

2.2.5. - EMA/OD/0000021732

Treatment of hepatocellular carcinoma

Action: For discussion/adoption

2.2.6. - EMA/OD/0000022351

Prevention of graft versus host disease

Action: For discussion/adoption

2.2.7. - EMA/OD/0000022586

Treatment of von Hippel-Lindau disease

Action: For discussion/adoption

2.2.8. - EMA/OD/0000022633

Prevention of retinopathy of prematurity

Action: For discussion/adoption

2.2.9. - EMA/OD/0000022802

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.10. - EMA/OD/0000022808

Treatment of intrahepatic cholestasis of pregnancy

Action: For discussion/adoption

2.2.11. - EMA/OD/0000022918

Treatment of myasthenia gravis

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 18-20 February 2020 COMP meeting

2.7. Evaluation on-going

16 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. -

Prevention of ischaemia reperfusion injury associated with solid organ transplantation

Action: For adoption

3.2. Finalised letters

Treatment of haemophilia A

Action: For information

3.3. New requests

3.3.1. -

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

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. Trepulmix - treprostinil sodium – EMEA/H/C/005207/0000, EMA/OD/154/12, EU/3/13/1103, EMA/OD/0000025710

SciPharm Sarl; Treatment of chronic thromboembolic pulmonary hypertension

Action: For adoption, Oral explanation to be held on 18 February 2020 at 11:00

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

4.2.1. - isatuximab – EMEA/H/C/004977, EMA/OD/198/13, EU/3/14/1268, EMA/OD/0000019553

Sanofi-Aventis Groupe; Treatment of plasma cell myeloma

Action: For discussion/adoption

4.2.2. - pexidartinib – EMEA/H/C/004832, EMA/OD/279/14, EU/3/15/1457, EMA/OD/0000021360

Daiichi Sankyo Europe GmbH; Treatment of tenosynovial giant cell tumour, localised and diffuse type

Action: For discussion/adoption

4.2.3. - bulevirtide – EMEA/H/C/004854, EMA/OD/329/14, EU/3/15/1500, EMA/OD/0000018086

Accelerated assessment

MYR GmbH; Treatment of hepatitis delta virus infection

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 meeting – COMP, 12-14 February 2020, Zagreb, Croatia

Report from the meeting

Action: For information

Document(s) tabled:

Agenda and related documents

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 18 February 2020 at 18:30 in room 2D

Document tabled:
PAWG draft agenda for 18 February 2020 meeting

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 2020

7.2.2. Kick-off meeting – COMP-CAT Working Group

Proposed meeting time on 19 February 2020 at 18:30 in room 1B.

Action: For discussion

Document tabled:
Draft agenda for 19 February 2020 meeting
Presentation

7.2.3. SAWP/COMP joint membership

Election of SAWP members/alternates - call for nomination - Deadline 14 February 2020

Action: For adoption

7.2.4. COMP members nominated on EMA's recommendation

Call for nomination - Deadline 21 February 2020

Action: For information

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

Documents tabled:

Meeting Summary from the Annual PCWP/HCPWP meeting with all eligible organisations, 20 November 2019

Agenda for the PCWP/HCPWP Joint Meeting, 3-4 March 2020

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 2020

Action: For information

7.8.2. Overview of orphan marketing authorisations/applications

Action: For information

8. Any other business

8.1.1. EU NTC Training curriculum - Pharmacoepidemiology - from Real-world data to Real-world evidence

Consultation on Training Curriculum in Pharmacoepidemiology – Deadline 14th February

Action: For information

8.1.2. Update on EMA organisational aspects

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/