1 October 2021
EMA/COMP/513657/2021
Human Medicines Division

Committee for Orphan Medicinal Products (COMP)

Draft agenda for the meeting on 5-7 October 2021

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

05 October 2021, 08:30-19:30, remote virtual meeting

06 October 2021, 08:30-19:30, remote virtual meeting

07 October 2021, 08:30-17:00, 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 5-7 October 2021. See (current) October 2021 COMP minutes (to be published post November 2021 COMP meeting).

1.2. Adoption of agenda

COMP agenda for 5-7 October 2021.

1.3. Adoption of the minutes

COMP minutes for 7-9 September 2021.

2. Applications for orphan medicinal product designation

2.1. For opinion

2.1.1. - EMA/OD/0000062364

Treatment of solid organ transplantation

Action: For adoption, Oral explanation to be held on 05 October 2021 at 09:15

2.1.2. - EMA/OD/0000057352

Treatment of primary biliary cholangitis

Action: For adoption, Oral explanation to be held on 05 October 2021 at 11:00

2.1.3. - EMA/OD/0000061146

Treatment of retinal detachment

Action: For adoption, Oral explanation to be held on 05 October 2021 at 15:00

2.1.4. - EMA/OD/0000064329

Treatment of diffuse large B-cell lymphoma

Action: For adoption, Oral explanation to be held on 05 October 2021 at 16:45

2.1.5. - EMA/OD/0000064903

Treatment of systemic sclerosis

Action: For adoption, Oral explanation to be held on 06 October 2021 at 09:00

2.1.6. - EMA/OD/0000058336

Treatment of pancreatic cancer

Action: For adoption, Oral explanation to be held on 06 October 2021 at 10:30

2.1.7. - EMA/OD/0000064772

Treatment of ovarian cancer

Action: For adoption, Oral explanation to be held on 06 October 2021 at 13:45

2.1.8. - EMA/OD/0000060407

Treatment of Diamond-Blackfan anemia

Action: For adoption, Oral explanation to be held on 06 October 2021 at 15:30

2.1.9. - EMA/OD/0000065329

Treatment of idiopathic pulmonary fibrosis

Action: For adoption, Oral explanation to be held on 06 October 2021 at 17:15

2.1.10. - EMA/OD/0000062259

Treatment of glioma

Action: For information

Notes: Withdrawal request received on 17 September 2021.

2.1.11. - EMA/OD/0000058552

Treatment of follicular lymphoma

Action: For adoption, Oral explanation to be held on 05 October 2021 at 13:30

2.2. For discussion / preparation for an opinion

2.2.1. - EMA/OD/0000036998

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.2. - EMA/OD/0000057224

Treatment of chronic thromboembolic pulmonary hypertension

Action: For discussion/adoption

2.2.3. - EMA/OD/0000060140

Treatment of pancreatic cancer

Action: For discussion/adoption

2.2.4. - EMA/OD/0000061806

Treatment of aneurysmal subarachnoid haemorrhage

Action: For discussion/adoption

2.2.5. - EMA/OD/0000062237

Treatment of adrenoleukodystrophy

Action: For discussion/adoption

2.2.6. - EMA/OD/0000062530

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.7. - EMA/OD/0000062804

Treatment of acute lymphoblastic leukaemia/lymphoblastic lymphoma

Action: For discussion/adoption

2.2.8. - EMA/OD/0000063093

Treatment of Complex Regional Pain Syndrome (CRPS)

Action: For discussion/adoption

2.2.9. - EMA/OD/0000064296

Treatment of narcolepsy

Action: For discussion/adoption

2.2.10. - EMA/OD/0000064393

Treatment of Dravet syndrome

Action: For discussion/adoption

2.2.11. - EMA/OD/0000064736

Treatment of pulmonary arterial hypertension

Action: For discussion/adoption

2.2.12. - EMA/OD/0000064849

Treatment of invasive candidiasis

Action: For discussion/adoption

2.2.13. - EMA/OD/0000065454

Treatment of cystic fibrosis

Action: For discussion/adoption

2.2.14. - EMA/OD/0000065981

Treatment of amyotrophic lateral sclerosis

Action: For discussion/adoption

2.2.15. - EMA/OD/0000066191

Treatment of acute lymphoblastic leukaemia

Action: For discussion/adoption

2.2.16. - EMA/OD/0000066312

Treatment of Lennox-Gastaut syndrome

Action: For discussion/adoption

2.2.17. - EMA/OD/0000066630

Treatment of primary sclerosing cholangitis

Action: For discussion/adoption

2.2.18. - EMA/OD/0000066742

Treatment of acute myeloid leukaemia

Action: For discussion/adoption

2.2.19. - EMA/OD/0000066935

Treatment of epilepsy with myoclonic-atonic seizures

Action: For discussion/adoption

2.2.20. - EMA/OD/0000066944

Treatment of chronic myeloid leukaemia

Action: For discussion/adoption

2.2.21. - EMA/OD/0000067046

Treatment of Leigh 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 05-07 October 2021 COMP meeting

2.7. Evaluation on-going

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

Action: For information

3. Requests for protocol assistance with significant benefit question

3.1. Ongoing procedures

3.1.1. -

Treatment of soft tissue sarcoma

Action: For adoption

3.1.2. -

Treatment of glioma

Action: For adoption

3.1.3. -

Treatment of haemophilia A

Action: For adoption

3.1.4. -

Treatment of glioma

Action: For adoption

3.2. Finalised letters

3.2.1. -

Treatment of hyperphenylalaninemia

Action: For information

3.2.2. -

Treatment of polycythaemia vera

Action: For information

3.3. New requests

3.3.1. -

Treatment of multiple myeloma

Action: For information

3.3.2. -

Treatment of primary hyperoxaluria

Action: For information

3.3.3. -

Treatment of primary IgA nephropathy

Action: For information

3.3.4. -

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

4.1.1. QINLOCK – ripretinib - EMEA/H/C/005614, EU/3/17/1936, EMA/OD/0000057360

Deciphera Pharmaceuticals (Netherlands) B.V; Treatment of gastrointestinal stromal tumours

Action: For discussion/adoption

4.1.2. Brukinsa - zanubrutinib - EMEA/H/C/004978/0000, EU/3/19/2167, EMA/OD/0000058248

BeiGene Ireland Limited; Treatment of lymphoplasmacytic lymphoma

Action: For information

Notes: Orphan designation withdrawal request received on 28 September 2021.

4.1.3. Artesunate Amivas – artesunate - EMEA/H/C/005550, EU/3/20/2251, EMA/OD/0000060998

Amivas Ireland Ltd; Treatment of malaria

Action: For discussion/adoption

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

4.2.1. – glucarpidase - EMEA/H/C/005467/0000, EMA/OD/049/02, EU/3/02/128, EMA/OD/0000042598

Protherics Medicines Development Europe B.V.; Adjunctive treatment in patients at risk of methotrexate toxicity

Action: For discussion

4.2.2. – pegcetacoplan - EMEA/H/C/005553, EU/3/17/1873, EMA/OD/0000051430

Apellis Ireland Limited; Treatment of paroxysmal nocturnal haemoglobinuria

Action: For information

4.2.3. – artesunate - EMEA/H/C/005718/0000, EMA/OD/043/15, EU/3/15/1521, EMA/OD/0000063220

B And O Pharm; Treatment of malaria

Action: For information

4.2.4. - lonapegsomatropin - EMEA/H/C/005367, EU/3/19/2213, EMA/OD/0000059751

Ascendis Pharma Endocrinology Division A/S; Treatment of growth hormone deficiency

Action: For discussion

4.2.5. Flynpovi – eflornithine / sulindac - EMEA/H/C/005043/0000, EMA/OD/130/12, EU/3/12/1086, EMA/OD/0000061571

Cancer Prevention Pharma (Ireland) Limited; Treatment of familial adenomatous polyposis

Action: For discussion/adoption

CHMP negative opinion was adopted in June 2021, CHMP re-examination of initial application procedure under Article 9(2) of Regulation no 726/2004.

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 – joint COMP/PDCO, 19 November 2021, Lisbon, Portugal

Action: For discussion

7.1.2. Protocol Assistance Working Group (PAWG)

Proposed meeting time on 1 October 2021 at 12:15

Document tabled:

PAWG draft agenda for 1 October 2021 meeting

7.2. Coordination with EMA Scientific Committees or CMDh-v

7.2.1. Recommendation on eligibility to PRIME – report

Action: For information

Document(s) tabled:

PRIME eligibility requests - list of adopted outcomes September 2021

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

Document(s) tabled:

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

Action: For information

Document(s) tabled:

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)

None

7.5.2. Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

None

7.5.3. Therapeutic Goods Administration (TGA), Australia

None

7.5.4. Health Canada

None

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. Update on the development of the database of principal decision to the agenda

Action: For discussion

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/