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Inspections, Human Medicines Pharmacovigilance and Committees Division

Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

February 2020

The Committee for Orphan Medicinal Products held its 219th plenary meeting on 18-20 February 2020.

Orphan medicinal product designation

Positive opinions

The COMP adopted 6 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- Allogeneic multi-virus specific T lymphocytes targeting BK virus, cytomegalovirus, human herpesvirus-6, Epstein-Barr virus and adenovirus for treatment in haematopoietic stem cell transplantation, TMC Pharma (EU) Limited;
- Asciminib for treatment of chronic myeloid leukaemia, Novartis Europharm Limited;
- Fosgemcitabine palabenamide for treatment of biliary tract cancer, Pharma Gateway AB.

2. Opinions adopted at the first COMP discussion:

- 2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-ylidene)methyl)furan-2-yl)benzoate for treatment of pancreatic cancer, MWB Consulting S.A.R.L.;
- Losmapimod for treatment of facioscapulohumeral muscular dystrophy, Pharma Gateway AB;
- Trifarotene for treatment of autosomal recessive congenital ichthyosis, Premier Research Group S.L..

3. Opinion following appeal procedures:

None

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Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation[1] by the European Commission. Please also refer to the Community Register of orphan medicinal products for human use.

Negative opinion

1. Opinions adopted following the sponsor's response to the COMP list of questions:

- Benzyl benzoate, beta-caryophyllene, cineole, cinnamaldehyde, cinnamyl acetate, linalool, trans-2-methoxycinnamaldehyde for treatment of eumycetoma, Septeos S.A.S.;
- Melatonin for treatment of intracerebral haemorrhage, Worphmed S.r.l..

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 9 lists of questions on initial applications. These applications will be discussed again at the next COMP meting prior to the adoption of an opinion.

Oral hearings

4 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that four applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

Detailed information on the orphan designation procedures

The list of medicinal products for which decisions on orphan designation have been granted by the European Commission since the last COMP meeting is provided in Annex 1.

Re-assessment of orphan designation at time of marketing authorisation

(Article 5(12) (b) of Regulation (EC) No 141/2000 of the European Parliament and of the Council)

When a designated orphan medicinal product receives a positive opinion for marketing authorisation from EMA's Committee for Medicinal Products for Human Use (CHMP), the COMP has the responsibility to review whether or not the medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation.

1. Opinions adopted at time of CHMP opinion:

[1] Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products

- Trepulmix (treprostininl sodium) for treatment of chronic thromboembolic pulmonary hypertension, SciPharm S.a.r.l (EU/3/13/1103);
- Givlaari (givosiran) for treatment of treatment of acute hepatic porphyria, Alnylam Netherlands B.V. (EU/3/16/1731). The opinion was adopted by written procedure after the January meeting.

2. Opinion following appeal procedures:

None

Details of the designated orphan medicinal products that have been subject of a new European Union (EU) marketing authorisation application since the last COMP monthly report are provided in Annex 2.

Details on the authorised orphan medicinal products can be found on the EMA website.

Other matters

None

Upcoming meetings

- The 220th meeting of the COMP will be held on 17-19 March 2020.

Note

This monthly report, together with other information on the work of the European Medicines Agency, can be found on the EMA website: www.ema.europa.eu

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Annex 1

Designations granted by the European Commission following COMP opinion on the fulfilment of the orphan designation criteria since last COMP plenary meeting

Please also refer to the Community Register of orphan medicinal product for human use.

The list includes designation decisions that were revised following the amendment of an existing designated condition (identified by * when applicable)Click or tap here to enter text.

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
None				

Annex 2

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the last COMP monthly report

Please also refer to the Community Register of orphan medicinal products for human use.

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured	Treatment of mantle cell lymphoma	Kite Pharma EU B.V.	EU/3/19/2220
Belantamab mafodotin	Treatment of multiple myeloma	GlaxoSmithKline (Ireland) Limited	EU/3/17/1925
Eladocagene exuparvovec	Treatment of aromatic L-amino acid decarboxylase deficiency	PTC Therapeutics International Limited	EU/3/16/1786
Fedratinib	Treatment of primary myelofibrosis Treatment of post-essential thrombocythaemia myelofibrosis Treatment of post-polycythaemia vera myelofibrosis	Celgene Europe BV	EU/3/10/794 EU/3/10/810 EU/3/10/811