EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

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

February 2022

The Committee for Orphan Medicinal Products held its 241st plenary meeting on 15-17 February 2022.

Orphan medicinal product designation

Positive opinions

The COMP adopted 11 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the first and second COMP discussion:

- (S)-12-fluoro-4-(2-methylpyridin-3-yl)-7a,8,13,14-tetrahydro-7H-[1,2,4]triazolo[4',3':1,6]pyrido[3,2-b]benzofuro[4,3-fg][1,4]oxazonine for treatment of sickle cell disease, Pharma Gateway AB;
- Alisporivir for treatment of collagen VI-related myopathies, Fondazione Telethon;
- Adeno-associated virus serotype HSC 15 expressing human iduronate 2-sulfatase for treatment of mucopolysaccharidosis type II (Hunter syndrome), Diamond Pharma Services Ireland Limited;
- Adeno-associated virus serotype HSC 15, containing human homology arms, expressing human phenylalanine hydroxylase for treatment of phenylalanine hydroxylase deficiency, Diamond Pharma Services Ireland Limited;
- Codergocrine mesilate, oxitriptan for treatment of fragile X syndrome, Purposeful I.K.E.;
- Dersimelagon for treatment of erythropoietic protoporphyria, Mitsubishi Tanabe Pharma GmbH;
- Emactuzumab for treatment of tenosynovial giant-cell tumour, local and diffuse type, Synox Therapeutics Limited;
- Heterologous human adult liver-derived stem cells for treatment of argininosuccinic aciduria, Unicyte S.R.L.;

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- Hymecromone for treatment of spinal cord injury, Ros Lynch;
- Tropatepine hydrochloride for treatment of narcolepsy, Laboratoires Delbert;
- Treprostinil sodium for treatment of idiopathic pulmonary fibrosis, Unither Therapeutik GmbH.

2. Opinion following appeal procedures:

None

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

None

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 4 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to the adoption of an opinion.

Oral explanations

3 oral explanations took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 3 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 medicinal products for which decisions on orphan designation have been granted by the European Commission is provided in Community Register of orphan medicinal products.

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

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

to review whether or not the medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation.

Positive opinions

1. Opinions adopted at time of CHMP opinion:

- AYVAKYT (avapritinib) for treatment of mastocytosis, Blueprint Medicines (Netherlands) B.V. EU/3/18/2074. The opinion was adopted by written procedure after the January meeting.
- KIMMTRAK (tebentafusp) for treatment of uveal melanoma, Immunocore Ireland Limited, EU/3/21/2397. The opinion was adopted by written procedure after the February meeting.

2. Opinions following appeal procedures:

None

Negative opinions

1. Opinions adopted at time of CHMP opinion:

None

2. Opinion following appeal procedures:

- Uplizna (inebilizumab) for treatment of neuromyelitis optica spectrum disorders, Viela Bio B.V. EU/3/17/1856. The opinion was adopted by written procedure after the February meeting.

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 1.

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

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 242nd meeting of the COMP will be held on 15-17 March 2022.

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

Enquiries to: AskEMA (https://www.ema.europa.eu/en/about-us/contact/send-question-european-medicines-agency

Annex 1

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
Fenfluramine	Treatment of Dravet syndrome	Zogenix ROI Limited	EU/3/13/1219