17 February 2022
EMA/COMP/46465/2022 Corr[1]
Human Medicines Division

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

January 2022

The Committee for Orphan Medicinal Products held its 240th plenary meeting on 18-20 January 2022.

Orphan medicinal product designation

Positive opinions

The COMP adopted 12 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:

- [Ala1,3,12,Gln10,Arg11,Trp14]PTH(1-14)/[Ala18,22, Lys26]PTHrP(15-36)COOH for treatment of hypoparathyroidism, Amolyt Pharma;
- Aldesleukin for treatment of amyotrophic lateral sclerosis, Iltoo Pharma;
- Autologous CD34+ cells edited with CRISPR/CAS9 and transduced with an adeno-associated virus vector serotype 6 containing the codon-optimized version of *PKLR* gene for treatment of pyruvate kinase deficiency, Consorcio Centro de Investigación Biomédica en Red, M.P.;
- Bovactant for treatment of respiratory distress syndrome, Aerogen Pharma Limited;
- Cannabidiol for treatment of fragile X syndrome, EUDRAC GmbH;
- Enzastaurin hydrochloride for treatment of Ehlers-Danlos syndrome, Dlrc Pharma Services Limited;
- Epcoritamab for treatment of diffuse large B-cell lymphoma, AbbVie Deutschland GmbH & Co. KG;
- Gadolinium-chelated polysiloxane nanoparticles for treatment of pancreatic cancer, Nh Theraguix;

[1] Change in active substance name

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- Pyridoxal 5'-phosphate for treatment of pyridoxamine 5'-phosphate oxidase deficiency, Amsterdam UMC;
- 18-mer antisense oligonucleotide complementary to SCN1A mRNA, sodium salt for treatment of Dravet syndrome, Insidereg Limited;
- Valemetostat tosilate for treatment of peripheral T-cell lymphoma, Daiichi Sankyo Europe GmbH;
- Vatiquinone for treatment of mitochondrial encephalomyopathy, lactic acidosis and stroke-like episodes, PTC Therapeutics International Limited.

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[2] 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 5 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 hearings

2 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 1 application for orphan medicinal product designation was 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.

[2] Details of all orphan designations granted to date by the European Commission are entered in the EU 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 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:

- Ngenla (somatrogon) for treatment of growth hormone deficiency, Pfizer Europe MA EEIG, EU/3/12/1087. The opinion was adopted by written procedure after the December meeting.
- Oxbryta (2-hydroxy-6-((2-(1-isopropyl-1h-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde) for treatment of sickle cell disease, Global Blood Therapeutics Netherlands B.V., EU/3/16/1769. The opinion was adopted by written procedure after the December meeting.

2. Opinions following appeal procedures:

None

Negative opinions

1. Opinions adopted at time of CHMP opinion:

None

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 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 241st meeting of the COMP will be held on 15-17 February 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
Parsaclisib	Treatment of marginal zone lymphoma	Incyte Biosciences Distribution B.V.	EU/3/19/1285
Sirolimus	Treatment of tuberous sclerosis	Plusultra pharma GmbH	EU/3/17/1910