21 October 2020
EMA/COMP/546547/2020
Human Medicines Division

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

October 2020

The Committee for Orphan Medicinal Products held its 226th plenary meeting on 06-08 October 2020.

Orphan medicinal product designation

Positive opinions

The COMP adopted 13 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:

- Adeno-associated viral vector serotype 9 expressing codon-optimized human *GRN* gene for treatment of frontotemporal dementia, PPD Bulgaria EOOD;
- Anti-(pancreatic adenocarcinoma upregulated factor) IgG1 humanised monoclonal antibody for treatment of pancreatic cancer, Prestige Biopharma Belgium;
- Perflubron for treatment of congenital pulmonary hypoplasia, Boyd Consultants Limited;
- Tislelizumab for treatment of oesophageal cancer, BeiGene Ireland Limited;
- Zanidatamab for treatment of gastric cancer, Voisin Consulting S.A.R.L.

2. Opinions adopted at the first COMP discussion:

- (R)-tetrahydrofuran-3-yl 4-(6-(5-(4-ethoxy-1-isopropylpiperidin-4-yl)pyridin-2-yl)pyrrolo[1,2-b]pyridazin-4-yl)piperazine-1-carboxylate sesquisuccinate for treatment of fibrodysplasia ossificans progressiva, Ipsen Pharma;
- 3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide, sodium chloride solution 4.2% (w/v) for treatment of primary ciliary dyskinesia, EUDRAC GmbH;

- Alisitol, retinol palmitate, zinc gluconate for treatment of microvillus inclusion disease, Vanessa Research Magyarorszag Kft.;
- Autologous bone marrow derived CD34+ cells transduced ex vivo with a self-inactivating lentiviral vector containing a normal version of the coding region of the *IL2RG* gene for treatment of X-linked severe combined immunodeficiency, Real Regulatory Limited;
- Autologous CD34+ cells transduced ex vivo with a lentiviral vector containing a modified gamma-globin gene for treatment of sickle cell disease, Clinical Technology Centre (Ireland) Limited;
- DNA plasmid encoding human transferrin gene for treatment of retinitis pigmentosa, Eyevensys S.A.S.;
- L-pyroglutamyl-L-asparaginyl-L-prolyl-D-tyrosyl-D-tryptophan amide for treatment of amyotrophic lateral sclerosis, Neuropath Therapeutics Limited;
- Triheptanoin for treatment of carnitine palmitoyltransferase I deficiency, Ultragenyx Germany GmbH.

3. 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 8 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

6 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 5 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

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

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:

- Obiltoxaximab SFL (obiltoxaximab) for anthrax, SFL Pharmaceuticals Deutschland GmbH (EU/3/18/2065). The opinion was adopted by written procedure after the September meeting.
- Zejula (niraparib) for treatment of ovarian cancer, Novartis Europharm Limited (EU/3/10/760).

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

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 227th meeting of the COMP will be held on 03-05 November 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

Contact details of our press officer

Monika Benstetter

Tel. +31 (0) 88 781 8427

E-mail: press@ema.europa.eu

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)

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Trehalose	Treatment of mucopolysaccharidosis type III (Sanfilippo syndrome)	FGK Representative Service GmbH	16 July 2020	21 August 2020

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 glioma tumor cells, inactivated/autologous glioma tumor cell lysates, inactivated/allogeneic glioma tumor cells, inactivated/allogeneic glioma tumor cell lysates, inactivated	Treatment of glioma	Epitopoietic Research Corporation-Belgium (E.R.C.)	EU/3/13/1211
Elivaldogene autotemcel	Treatment of adrenoleukodystrophy	bluebird bio (Netherlands) B.V	EU/3/12/1003