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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

December 2019

The Committee for Orphan Medicinal Products held its 217th plenary meeting on 03-05 December 2019.

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 second COMP discussion, following the sponsor's response to the COMP list of questions:

- Adeno-associated virus serotype 2/6 encoding human alpha-galactosidase A cDNA for treatment of Fabry disease, ERA Consulting GmbH;
- Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the *BCL11A* gene for treatment of sickle cell disease, Vertex Pharmaceuticals (Ireland) Limited;
- Motixafortide for treatment of pancreatic cancer, Granzer Regulatory Consulting & Services;
- Tripotassium citrate monohydrate and potassium hydrogen carbonate for treatment of cystinuria, Advicenne S.A.

2. Opinions adopted at the first COMP discussion:

- 2-(3,7-dimethyl-octa-2, 6-dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzoquinone for treatment of Huntington's disease, Emerald Health Pharmaceuticals España S.L.;
- Adeno-associated virus serotype 9 vector containing human N-acetylgalactosamine-6-sulfate sulfatase gene for treatment of mucopolysaccharidosis type IVA (Morquio A syndrome), Esteve Pharmaceuticals S.A.;

- Dimethyl fumarate for treatment of adrenoleukodystrophy, Consorcio Centro de Investigación Biomédica en Red, M.P.;
- Nicardipine for treatment of non-traumatic subarachnoid haemorrhage, Bit Pharma GmbH;
- Romilkimab for treatment of systemic sclerosis, Sanofi-Aventis Groupe;
- Setmelanotide for treatment of Alström syndrome, TMC Pharma (EU) Limited;
- Ziritaxestat for treatment of systemic sclerosis, Galapagos N.V.

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:

- Melatonin for treatment of primary hepatocellular carcinoma, Worphmed S.r.l.

2. Opinion following appeal procedures:

- None

Lists of questions

The COMP adopted 7 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

5 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 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.

[1] 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.

1. Opinions adopted at time of CHMP opinion:

- Polivy (polatuzumab vedotin) for treatment of diffuse large B-cell lymphoma, Roche Registration GmbH (EU/3/18/2013);
- Isturisa (osilodrostat) for the treatment of Cushing's syndrome, Novartis Europharm Limited (EU/3/14/1345). The opinion was adopted by written procedure after the November 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

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 218th meeting of the COMP will be held on 20-22 January 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)

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(2S,3R,4R,5S)-2-(hydroxymethyl)-1-pentylpiperidine-3,4,5-triol	Treatment of gm2 gangliosidosis	Idorsia Pharmaceuticals Deutschland GmbH	10 October 2019	13 November 2019
4-((e)-(5-(2-(2-((s)-2-((s)-1-(l-threonyl-l-lysyl)pyrrolidine-2-carboxamido)-5-guanidinopentanamido)acetamido)-2-carboxyethyl)-2-hydroxyphenyl)diazenyl)phenyl (2-(trimethylammonio)ethyl) phosphate	Treatment of non-infectious uveitis	Granzer Regulatory Consulting & Services	10 October 2019	13 November 2019
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.	10 October 2019	13 November 2019
Camsirubicin	Treatment of soft tissue sarcoma	Monopar Therapeutics S.A.R.L	10 October 2019	13 November 2019

Chimeric fibril-reactive igg1k monoclonal antibody 11-1f4	Treatment of al amyloidosis	Real Regulatory Limited	10 October 2019	13 November 2019
Exendin (9-39)	Treatment of congenital hyperinsulinism	Eigerbio Europe Limited	10 October 2019	13 November 2019
Ganaxolone	Treatment of cdkl5 deficiency disorder	Pharma Gateway AB	10 October 2019	13 November 2019
Replication-incompetent, non-integrating, herpes simplex virus 1 vector expressing the human transglutaminase-1 enzyme	Treatment of autosomal recessive congenital ichthyosis	IDEA Innovative Drug European Associates (Ireland) Limited	10 October 2019	13 November 2019

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 CD34+ cell enriched population that contains hematopoietic stem and progenitor cells transduced ex vivo using a lentiviral vector	Treatment of metachromatic leukodystrophy	Orchard Therapeutics Limited	EU/3/07/446