20 July 2020
EMA/COMP/382197/2020
Human Medicines Division

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

July 2020

The Committee for Orphan Medicinal Products held its 224th plenary meeting on 14-16 July 2020.

Orphan medicinal product designation

Positive opinions

The COMP adopted 22 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:

- 2-(2-(18F)fluoropyridin-4-yl)-9H-pyrrolo[2,3-b:4,5-c']dipyridine for treatment of progressive supranuclear palsy, Life Molecular Imaging GmbH;
- Adeno-associated virus serotype 2/8 vector containing the human *PDE6A* gene for treatment of retinitis pigmentosa, Institute For Ophthalmic Research;
- Allogeneic T-cell precursors, mobilised peripheral blood-derived, ex vivo cultured for treatment in haematopoietic stem cell transplantation, Smart Immune;
- Allogeneic umbilical cord tissue-derived mesenchymal stromal cells ex vivo expanded for prevention of bronchopulmonary dysplasia, MDTB Cells GmbH;
- Bis-(3-deoxy-3-(4-(3-fluorophenyl)-1h-1,2,3-triazol-1-yl)-beta-d-galactopyranosyl) sulfane for treatment of idiopathic pulmonary fibrosis, Galecto Biotech AB;
- Dextran sulfate low molecular weight for treatment of amyotrophic lateral sclerosis, TikoMed AB;
- Humanised IgG1 monoclonal antibody against human eotaxin-2 for treatment of primary sclerosing cholangitis, Granzer Regulatory Consulting & Services;

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- Protein-based delivery vector carrying a DNA payload encoding an RNA-guided nuclease that targets *stx* genes of Shiga toxin-producing *Escherichia coli* for treatment of haemolytic uraemic syndrome, Eligo Bioscience;
- Sodium (4-{(E)-3-(4-fluorophenyl)-3-[4-(3-morpholin-4-yl-prop1ynyl)phenyl]allyloxy}-2-methylphenoxy)acetate for treatment of mitochondrial encephalomyopathy, lactic acidosis and stroke-like episodes, Scendea (NL) B.V..

2. Opinions adopted at the first COMP discussion:

- 3-(((1S,2S,3R)-2,3-difluoro-1-hydroxy-7-(methylsulfonyl)-2,3-dihydro-1H-inden-4-yl)oxy)-5-fluorobenzonitrile for treatment of von Hippel-Lindau disease, Merck Sharp & Dohme B.V.;
- 6-[(3S,4S)-4-methyl-1-(pyrimidin-2-ylmethyl)pyrrolidin-3-yl]-3-tetrahydropyran-4-yl-7H-imidazo[1,5-a]pyrazin-8-one for treatment of sickle cell disease, TMC Pharma (EU) Limited;
- Adeno-associated viral vector serotype 3B encoding shortened human ATP7B for treatment of Wilson's disease, Vivet Therapeutics S.A.S.;
- Allogeneic hepatoblastoma cells encapsulated in alginate, ex vivo expanded for treatment of acute liver failure, ESPL Regulatory Consulting Limited;
- Autologous CD34+ cells transduced with a lentiviral vector encoding glucosylceramidase beta for treatment of Gaucher disease, Clinical Technology Centre (Ireland) Limited;
- Autologous T cells transduced with lentiviral vector containing a tandem chimeric antigen receptor directed against CD20 and CD19 for treatment of diffuse large B-cell lymphoma, Miltenyi Biomedicine GmbH;
- Copper histidinate for treatment of Menkes disease, CambPharma Solutions (CY) Limited;
- Human frataxin fused to TAT cell-penetrating peptide for treatment of Friedreich's ataxia, YES Pharmaceutical Development Services GmbH;
- Infigratinib for treatment of cholangiocarcinoma, YES Pharmaceutical Development Services GmbH.
- Pentosan polysulfate sodium for treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome), Paradigm Biopharmaceuticals (Ireland) Limited;
- Sodium (4-{(E)-3-(4-fluorophenyl)-3-[4-(3-morpholin-4-yl-prop1ynyl)phenyl]allyloxy}-2-methylphenoxy)acetate for t Ttreatment of long-chain 3-hydroxyacyl-coenzyme A dehydrogenase deficiency, Scendea (NL) B.V.;
- Trehalose for treatment of mucopolysaccharidosis type III (Sanfilippo syndrome), FGK Representative Service GmbH;
- Venglustat for treatment of GM2 gangliosidosis, Genzyme Europe B.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:

None

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 16 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 5 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:

- Kaftrio (elexacaftor/tezacaftor/ivacaftor) for treatment of cystic fibrosis, Vertex Pharmaceuticals (Ireland) Limited (EU/3/18/2116).

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

- Idefirix (imlifidase) for the prevention of graft rejection following solid organ transplantation, Hansa Biopharma AB (EU/3/16/1826). The opinion was adopted by written procedure after the June 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 225th meeting of the COMP will be held on 8-10 September 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
(+)-epicatechin	Treatment of Becker muscular dystrophy	MWB Consulting S.A.R.L.	20 May 2020	26 June 2020
1-((2S,4S)-2-((((S)-(4-bromophenoxy)(((S)-1-oxo-1-(((S)-pentan-2-yl)oxy)propan-2-yl)amino)phosphoryl)oxy)methyl)-1,3-dioxolan-4-yl)-2-oxo-1,2-dihydropyrimidin-4-aminium chloride	Treatment of hepatocellular carcinoma	Medivir AB	23 April 2020	04 June 2020
Adeno-associated virus serotype HSC15 expressing human arylsulfatase A gene	Treatment of metachromatic leukodystrophy	YES Pharmaceutical Development Services GmbH	20 May 2020	26 June 2020
Axicabtagene ciloleucel	Treatment of marginal zone lymphoma	Kite Pharma EU B.V.	20 May 2020	26 June 2020
Lys^{40}(NODAGA-^{68}Ga)NH_2-exendin-4	Diagnosis of insulinoma	Stichting Katholieke Universiteit	20 May 2020	26 June 2020
Magrolimab	Treatment of myelodysplastic syndromes	Granzer Regulatory Consulting & Services	20 May 2020	26 June 2020

Nomacopan	Treatment of bullous pemphigoid	Akari Malta Limited	20 May 2020	26 June 2020
Onfekafusp alfa	Treatment of glioma	Philogen S.p.A.	20 May 2020	26 June 2020
Sodium cromoglicate	Treatment of idiopathic pulmonary fibrosis	IQVIA RDS Spain S.L.	20 May 2020	26 June 2020
Stiripentol	Treatment of primary hyperoxaluria	Biocodex S.A.S.	20 May 2020	26 June 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
Eflornithine / sulindac	Treatment of familial adenomatous polyposis Treatment of familial adenomatous polyposis	Cancer Prevention Pharma (Ireland) Limited	EU/3/12/1086 EU/3/10/779
Lisocabtagene maraleucel	Treatment of diffuse large B-cell lymphoma Treatment of follicular lymphoma Treatment of primary mediastinal large-B-cell lymphoma	Celgene Europe BV	EU/3/17/1890 EU/3/18/2018 EU/3/18/2099
Setmelanotide	Treatment of leptin receptor deficiency Treatment of pro-opiomelanocortin deficiency	TMC Pharma (EU) Limited	EU/3/18/2101 EU/3/16/1703
Zanubrutinib	Treatment of lymphoplasmacytic lymphoma	BeiGene Ireland Ltd	EU/3/19/2167