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Human Medicines Division

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

December 2021

The Committee for Orphan Medicinal Products held its 239th plenary meeting on 7-9 December 2021.

Orphan medicinal product designation

Positive opinions

The COMP adopted 17 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:

- 5' moe^{m}C-(sp)- moe^{m}C-(p)-moeA-(p)-moe^{m}C-(p)-moeG-(p)-moeA-(p)-d^{m}C-(sp)-dA-(sp)-dT-(sp)-dA-(sp)-dT-(sp)-dT-(sp)-dT-(sp)-dT-(sp)-dT-(sp)-d^{m}C-(sp)-moeT-(p)-moeA-(sp)-moe^{m}C-(sp)-moeA 3' for treatment of *SCN2A* developmental and epileptic encephalopathy, Real Regulatory Limited;
- 5-((4'-(3,3-difluorocyclobutyl)-[1,1'-biphenyl]-4-yl)oxy)-1h-1,2,3-triazole-4-carboxylic acid for treatment of primary hyperoxaluria, Voisin Consulting Life Sciences;
- Adeno-associated virus serotype 9 expressing the human fukutin related protein and target sequence of the miR-208a for treatment of limb girdle muscular dystrophy, Atamyo Therapeutics;
- Adeno-associated virus vector serotype 2 expressing the human MT-*ND4* codon-optimised gene for treatment of Leber's hereditary optic neuropathy, IQVIA RDS Spain S.L.;
- Azithromycin dihydrate for prevention of bronchopulmonary dysplasia, Vale Pharmaceuticals Limited;
- Chimeric peptide of human glucagon-like peptide-1, glucagon and gastric inhibitory polypeptide analogues linked to a human immunoglobulin fc fragment for treatment of primary biliary cholangitis, JVM Europe B.V.;

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- Efgartigimod alfa for treatment of chronic inflammatory demyelinating polyneuropathy, Argenx;
- Fasudil hydrochloride for treatment of amyotrophic lateral sclerosis, Granzer Regulatory Consulting & Services;
- Gadolinium-chelated polysiloxane nanoparticles for treatment of glioma, Nh Theraguix;
- Insulin human for prevention of retinopathy of prematurity, Sirius Regulatory Consulting EU Limited;
- Melatonin for treatment of pre-eclampsia, Worphmed S.r.l.;
- mRNA encoding human glucose-6-phosphatase variant S298C for treatment of glycogen storage disease type Ia, Moderna Biotech Spain S.L.;
- Navtemadlin for treatment of Merkel cell carcinoma, Yes Pharmaceutical Development Services GmbH;
- N-sulfoglucosamine sulfohydrolase fused to a humanised monoclonal antibody Fab targeting the human transferrin receptor for treatment of mucopolysaccharidosis type IIIa (Sanfilippo A syndrome), Artemida Pharma Europe Limited;
- Olorofim for treatment of invasive scopulariopsis, F2G Biotech GmbH;
- Sirolimus for treatment of bronchiolitis obliterans syndrome, Maxia Strategies-Europe Limited;
- Trans N-ethyl-2-((4-(7-((4-(ethylsulfonamido)cyclohexyl)methyl)-2,7-diazaspiro[3.5]nonan-2-yl)pyrimidin-5-yl)oxy)-5-fluoro-N-isopropylbenzamide sesquifumarate for treatment of acute myeloid leukaemia, Syndax Europe B.V.

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

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

Oral hearings

2 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 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 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:

- Lonapegsomatropin Ascendis Pharma (lonapegsomatropin) for treatment of growth hormone deficiency, Ascendis Pharma Endocrinology Division A/S, EU/3/19/2213. The opinion was adopted by written procedure after the November meeting.
- Tavneos (avacopan) for treatment of granulomatosis with polyangiitis, Vifor Fresenius Medical Care Renal Pharma France, EU/3/14/1373. The opinion was adopted by written procedure after the November meeting.
- Tavneos (avacopan) for treatment of microscopic polyangiitis, Vifor Fresenius Medical Care Renal Pharma France, EU/3/14/1372. The opinion was adopted by written procedure after the November meeting.
- Voraxaze (glucarpidase) adjunctive treatment in patients at risk of methotrexate toxicity, Protherics Medicines Development Europe B.V., EU/3/02/128. The opinion was adopted by written procedure after the November meeting.

2. Opinions following appeal procedures:

None

Negative opinions

1. Opinions adopted at time of CHMP opinion:

- Nexviadyme (avalglucosidase alfa) for treatment of Pompe's disease, Genzyme Europe B.V. EU/3/14/1251. The opinion was adopted by written procedure after the December meeting.

- 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 December 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 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 240th meeting of the COMP will be held on 18-20 January 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
Cipaglucosidase alfa	Treatment of glycogen storage disease type II (Pompe's disease)	Amicus Therapeutics Europe Limited	EU/3/18/2000
Fosdenopterin	Treatment of molybdenum cofactor deficiency type A	Comharsa Life Sciences Ltd	EU/3/10/777
Infigratinib	Treatment of cholangiocarcinoma	Helsinn Birex	EU/3/20/2329
Miglustat	Treatment of glycogen storage disease type II (Pompe's disease)	Amicus Therapeutics Europe Limited	EU/3/18/2129
Mozunetuzumab	Treatment of follicular lymphoma	Roche Registration GmbH	EU/3/21/2517
Olipudase alfa	Treatment of Niemann-Pick disease	Genzyme Europe BV	EU/3/01/056
Setmelanotide	Treatment of leptin receptor deficiency	Rhythm Pharmaceuticals Netherlands B.V.	EU/3/18/2101
Setmelanotide	Treatment of pro-opiomelanocortin deficiency	Rhythm Pharmaceuticals Netherlands B.V.	EU/3/16/1703
Tabelecleucel	Treatment of post-transplant lymphoproliferative disorder	Atara Biotherapeutics Ireland Limited	EU/3/16/1627