10 November 2021
EMA/COMP/639056/2021
Human Medicines Division

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

November 2021

The Committee for Orphan Medicinal Products held its 238th plenary meeting on 3-5 November 2021.

Orphan medicinal product designation

Positive opinions

The COMP adopted 23 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:

- 2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-5-methyl-p-thiouridylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioguanylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioadenylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioadenylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioadenylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioguanoylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioadenylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiouridylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioguanylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioguanylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioadenylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioadenylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioguanylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-P-thioadenylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'-O->5'-O)-2′-O-(2-methoxyethyl)-5-methyl-uridine sodium salt for treatment of cystic fibrosis, AdRes EU B.V.;
- 3-(ethoxydifluoromethyl)-6-(5-fluoro-6-(2,2,2-trifluoroethoxy)pyridin-3-yl)-[1,2,4]triazolo[4,3-a]pyrazine for treatment of *SCN2A* developmental and epileptic encephalopathy, Real Regulatory Limited;

- 3-(ethoxydifluoromethyl)-6-(5-fluoro-6-(2,2,2-trifluoroethoxy)pyridin-3-yl)-[1,2,4]triazolo[4,3-a]pyrazine for treatment of *SCN8A* developmental and epileptic encephalopathy, Real Regulatory Limited;
- 6-(4-(tert-butyl)phenoxy)pyridin-3-amine for treatment of acute lymphoblastic leukaemia, Yes Pharmaceutical Development Services GmbH;
- Adeno-associated virus vector serotype 9 encoding the human *GRN* gene for treatment of frontotemporal dementia, Scendea (NL) B.V.;
- Allogeneic foetal mesenchymal stem cells for treatment of osteogenesis imperfecta, Boost Pharma ApS;
- Anti-(endothelin-1 receptor subtype A) IgG4 humanised monoclonal antibody for treatment of pulmonary arterial hypertension, Gmax Biopharm Belgium;
- Atrasentan for treatment of primary IgA nephropathy, Voisin Consulting Life Sciences;
- Autologous T cells ex vivo modified with a lentiviral vector encoding a chimeric antigen receptor specific for CD1a for treatment of acute lymphoblastic leukaemia, Onechain Immunotherapeutics S.L.;
- Cedazuridine, decitabine for treatment of acute myeloid leukaemia, Otsuka Pharmaceutical Netherlands B.V.;
- 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 sclerosing cholangitis, JVM Europe B.V.;
- Copper (^{64}Cu) chloride for treatment of glioma, Advanced Center Oncology Macerata - S.r.l.;
- Crofelemer for treatment of short bowel syndrome, Napo EU S.p.A.;
- Garadacimab for treatment of hereditary angioedema, CSL Behring GmbH;
- Macitentan for treatment of chronic thromboembolic pulmonary hypertension, Janssen-Cilag International N.V.;
- N-[1-((5-cyanopyridin-2-yl)methyl)-1h-pyrazol-3-yl]-2-[4-(1-(trifluoromethyl)cyclopropyl)phenyl]acetamide for treatment of epileptic encephalopathy with continuous spike-and-wave during sleep, Neurocrine Therapeutics Limited;
- Norrin (25-133), Lys86Pro for treatment of familial exudative vitreoretinopathy, Maxia Strategies-Europe Limited;
- Octreotide acetate for treatment of idiopathic intracranial hypertension, Granzer Regulatory Consulting & Services;
- Retinol palmitate for prevention of bronchopulmonary dysplasia, Real Regulatory Limited;
- Ribonucleoprotein complex composed of two sgRNA and a Cas9 nuclease targeting the human *COL7A1* gene for treatment of epidermolysis bullosa, Branca Bunus Limited;
- Troriluzole hydrochloride for treatment of spinocerebellar ataxia, Biohaven Pharmaceutical Ireland DAC;
- Unesbulin for treatment of soft tissue sarcoma, PTC Therapeutics International Limited;

- Vatiquinone for treatment of Alpers-Huttenlocher syndrome, 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[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

2 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 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.

1. Opinions adopted at time of CHMP opinion:

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

- Aspaveli (pegcetacoplan) for treatment of a paroxysmal nocturnal haemoglobinuria, Swedish Orphan Biovitrum AB (publ), EU/3/17/1873.

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 239th meeting of the COMP will be held on 7-9 December 2021.

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
bardoxolone methyl	Treatment of Alport syndrome	Reata Ireland Limited	EU/3/18/2019
ganaxolone	Treatment of CDKL5 deficiency disorder	Marinus Pharmaceuticals Emerald Limited	EU/3/19/2224
loncastuximab tesirine	Treatment of diffuse large B-cell lymphoma	FGK Representative Service GmbH	EU/3/21/2481
maralixibat	Treatment of Alagille syndrome	Mirum Pharmaceuticals International B.V.	EU/3/13/1214
sutimlimab	Treatment of autoimmune haemolytic anaemia	Genzyme Europe BV	EU/3/16/1609