19 May 2022
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Human Medicines Division

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

May 2022

The Committee for Orphan Medicinal Products held its 244th plenary meeting on 10-12 May 2022.

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 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)-5-methyl-P-thiouridylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiouridylyl-(3'-O->5'-O)-2'-O-(2-methoxyethyl)-5-methyl-P-thiocytidylyl-(3'-O->5'-O)-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)-5-methyl-P-thiouridylyl -(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)-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-thiouridylyl-(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-thioadenine, sodium salt for treatment of cystic fibrosis, AdRes EU B.V.;

- 2'-O-(2-methoxyethyl) modified antisense oligonucleotide targeting UBE3A antisense transcript RNA for treatment of Angelman syndrome, Ionis Development (Ireland) Limited;

- 3-(1-(2',3'-dimethoxy-[1,1'-biphenyl]-4-yl)-1H-1,2,3-triazol-4-yl)benzoic acid treatment of gain-of-function mutations of STIM1 and ORAI1 related diseases, ChemICare S.R.L.;

- 4-[[(3S)-1-benzylpyrrolidin-3-yl]-methylamino]-2-fluoro-5-methyl-N-(1,3-thiazol-4-yl)benzenesulfonamide for treatment of *SCN8A* developmental and epileptic encephalopathy, Neurocrine Therapeutics Limited;

- Amitriptyline for treatment of erythromelalgia, AlgoTherapeutix;

- Diflunisal for treatment of ATTR amyloidosis, Turnkey Pharmaconsulting Ireland Limited;
- Doxorubicin for treatment of soft tissue sarcoma, Thermosome GmbH;
- Eltanexor for treatment of myelodysplastic syndromes, Karyopharm Europe GmbH;
- Epcoritamab for treatment of follicular lymphoma, AbbVie Deutschland GmbH & Co. KG;
- *Escherichia coli*, strain Nissle 1917, expressing high affinity phenylalanine transporter, phenylalanine ammonia lyase and l-amino acid deaminase for treatment of hyperphenylalaninaemia, Orphix Consulting GmbH;
- Govorestat for treatment of galactosaemia, Drug Development and Regulation S.L.;
- Human allogeneic keratinocytes for treatment of partial deep dermal and full thickness burns, Evomedis GmbH;
- Humanised IgG4 monoclonal antibody against proliferation-inducing ligand for treatment of primary IgA nephropathy, TMC Pharma (EU) Limited;
- Lithium carbonate for treatment of *TBR1*-related disorder, Centre Hospitalier Universitaire Dijon Bourgogne;
- Losartan for treatment of osteogenesis imperfecta, 3R Pharma Consulting GmbH;
- Modified mRNA encoding human methylmalonyl-coenzyme A mutase containing a polymorphism at position 671 for treatment of methylmalonic acidemia, Moderna Biotech Spain S.L.;
- Pirfenidone for treatment of idiopathic pulmonary fibrosis, Regintel Limited;
- Ropeginterferon alfa-2b for treatment of chronic myeloid leukaemia, Aop Orphan Pharmaceuticals GmbH;
- Sebetralstat for treatment of hereditary angioedema, Kalvista Pharmaceuticals (Ireland) Limited;
- Sirolimus for treatment of lymphatic malformations, Raremoon Consulting Esp S.L.;
- Sodium (4Z,7Z,10R,11E,13E,15Z,17S,19Z)10,17-dihydroxy-docosa-4,7,11,13,15,19-hexaenoate for treatment of retinopathy of prematurity, Granzer Regulatory Consulting & Services GmbH;
- Tamoxifen citrate for treatment of neuronal ceroid lipofuscinosis, Fondazione Telethon.

2. Opinions 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 opinions

1. Opinions adopted following the sponsor's response to the COMP list of questions:

None

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

2. Opinions following appeal procedures:

None

Lists of questions

The COMP adopted 18 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 2 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:

- Yescarta (axicabtagene ciloleucel) for treatment of follicular lymphoma, EU/3/15/1579, Kite Pharma EU B.V.

2. Opinions following appeal procedures:

None

Negative opinions

1. Opinions adopted at time of CHMP opinion:

None

2. Opinions 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 245th meeting of the COMP will be held on 14-16 June 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
None			