23 March 2021
EMA/COMP/170529/2021
Human Medicines Division

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

March 2021

The Committee for Orphan Medicinal Products held its 231st plenary meeting on 16-18 March 2021.

Orphan medicinal product designation

Positive opinions

The COMP adopted 3 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:

- Cevostamab for treatment of multiple myeloma, Roche Registration GmbH;
- S-[5-(omega-methoxypoly(oxyethylene)-2-oxopentyl)]-L-cysteinylglycyl-L-serinylglycylgylcyl-L-isoleucyl-L-lysyl-L-glutamyl-L-phenylalanyl-L-leucyl-L-glutaminyl-L-arginyl-L-phenylalanyl-L-isoleucyl-L-histyl-L-isoleucyl-L-valyl-L-glutaminyl-L-serinyl-L-isoleucyl-L-isoleucyl-L-asparaginyl-L-threonyl-L-serinamide, acetate salt for treatment of cutaneous T-cell lymphoma, Almirall S.A.

2. Opinions adopted at the first COMP discussion:

- Ganglioside GM1 for treatment of amyotrophic lateral sclerosis, 3R Pharma Consulting GmbH.

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.

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

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Negative opinion

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

- Amivantamab for treatment of non-small cell lung cancer with EGFR alterations, Janssen-Cilag International N.V.

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 6 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

6 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The COMP noted that 5 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.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:

- Epidyolex (cannabidiol) for treatment of tuberous sclerosis, GW Pharma (International) B.V. (EU/3/17/1959).

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 232nd meeting of the COMP will be held on 13-15 April 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

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
(1R,3S,5R)-2-(2-(3-acetyl-5-(2-methylpyrimidin-5-yl)-1H-indazol-1-yl)acetyl)-N-(6-bromo-3-methylpyridin-2-yl)-5-methyl-2-azabicyclo[3.1.0]hexane-3-carboxamide	Treatment of paroxysmal nocturnal haemoglobinuria	Alexion Europe S.A.S.	21 January 2021	19 February 2021
(2S)-2-[(2S)-4-cyclohexyl-2-{[(2S)-1-(4-fluorobenzoyl)pyrrolidin-2-yl]formamido}butanamido]-N-(1-{[(1S)-1-{[(1S)-1-({1-[(1-{[2-({1-[(dimethylamino)methyl]cyclobutyl}carbamoyl)ethyl]carbamoyl}-1-methylethyl)carbamoyl]-1-methylethyl}carbamoyl)-3-methylbutyl]carbamoyl}-3-methylbutyl]carbamoyl}-1-methylethyl)-4-methylpentanamide acetate	Treatment of leishmaniasis	Biopharma Excellence GmbH	21 January 2021	19 February 2021
18-(p-[^{131}I]-iodophenyl)octadecyl phosphocholine	Treatment of lymphoplasmacytic lymphoma	Scendea (NL) B.V.	21 January 2021	19 February 2021
2-(2-{[2-(1H-benzimidazol-2-yl)ethyl]amino}ethyl)-N-[(3-fluoropyridin-2-yl)methyl]-1,3-oxazole-4-carboxamide trihydrochloride	Treatment of sickle cell disease	Vifor France	21 January 2021	19 February 2021

2-[6-(6,7-dimethoxyquinolin-3-yl)pyridin-3-yl]-N-[3-(1,1,1-trifluoro-2-methylpropan-2-yl)-1,2-oxazol-5-yl]acetamide	Treatment of medullary thyroid carcinoma	Southwood Research Limited	21 January 2021	19 February 2021
2'-O-methyl phosphorothioate RNA oligonucleotide, 5'-m^5CUGm^5CUGm^5CUGm^5CUGm^5CUGm^5CUGm^5CUG-3'	Treatment of spinocerebellar ataxia	Vico Therapeutics B.V.	21 January 2021	19 February 2021
Adeno-associated viral vector serotype 9 expressing codon-optimized human *GBA* gene	Treatment of Gaucher disease	PPD Bulgaria EOOD	21 January 2021	19 February 2021
Adeno-associated virus serotype 5 containing the human *NR2E3* gene	Treatment of Leber's congenital amaurosis	Ocugen Limited	21 January 2021	19 February 2021
Adeno-associated virus serotype 5 containing the human *NR2E3* gene	Treatment of retinitis pigmentosa	Ocugen Limited	21 January 2021	19 February 2021
Anti-SIGLEC8 IgG1 humanised monoclonal antibody	Treatment of eosinophilic gastroenteritis	Turnkey Pharmaconsulting Ireland Limited	21 January 2021	19 February 2021
Autologous CD34+ cells transduced with a lentiviral RNA vector that results in integrated cDNA encoding for functional cystinosin	Treatment of cystinosis	Clinical Technology Centre (Ireland) Limited	21 January 2021	19 February 2021
Autologous CD34+ hematopoietic stem and progenitor cells transfected with zinc finger nuclease mRNAs SB-mRENH1 and SB-mRENH2	Treatment of sickle cell disease	Genzyme Europe B.V.	21 January 2021	19 February 2021
Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human claudin 18.2 antigen with CD28 and CD3-zeta intracellular signalling domains	Treatment of gastric cancer	ICON Clinical Research Limited	21 January 2021	19 February 2021
Dodecyl creatine ester, dodecyl creatine ester hydrochloride	Treatment of creatine deficiency syndromes	Ceres Brain Therapeutics S.A.S.	21 January 2021	19 February 2021

Gly-Arg-Lys-Lys-Arg-Arg-Gln-Arg-Arg-Arg-Cys-Asp-Met-Ala-Glu-His-Thr-Glu-Arg-Leu-Lys-Ala-Asn-Asp-Ser-Leu-Lys-Leu-Ser-Gln-Glu-Tyr-Glu-Ser-Ile-NH_2	Treatment of spinal cord injury	Raremoon Consulting Esp S.L.	21 January 2021	19 February 2021
Rintatolimod	Treatment of pancreatic cancer	Hemispherx Biopharma Europe	21 January 2021	19 February 2021
Tebentafusp	Treatment of uveal melanoma	Pharma Gateway AB	21 January 2021	19 February 2021

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
Avapritinib	Treatment of gastrointestinal stromal tumours	Blueprint Medicines (Netherlands) B.V.	EU/3/17/1889
Avapritinib	Treatment of mastocytosis	Blueprint Medicines (Netherlands) B.V.	EU/3/18/2074
Retifanlimab	Treatment of anal cancer	Incyte Biosciences Distribution B.V.	EU/3/20/2343
Somatrogon	Treatment of growth hormone deficiency	Pfizer Europe MA EEIG	EU/3/12/1087