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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

January 2020

The Committee for Orphan Medicinal Products held its 218th plenary meeting on 20-22 January 2020.

Orphan medicinal product designation

Positive opinions

The COMP adopted 12 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:

- Autologous skin equivalent graft composed of keratinocytes and fibroblasts genetically corrected by CRISPR/Cas9-mediated excision of mutation-carrying *COL7A1* exon 80 for treatment of epidermolysis bullosa, Consorcio Centro de Investigación Biomédica en Red, M.P.;
- Combination of three adeno-associated viral vectors of serotype 8 containing the 5'-, the body- and the 3'- coding sequences of human CEP290 fused to inteins for treatment of inherited retinal dystrophies, Fondazione Telethon;
- Reldesemtiv for treatment of amyotrophic lateral sclerosis, Pharma Gateway AB.

2. Opinions adopted at the first COMP discussion:

- 2-((4S)-6-(4-chlorophenyl)-1-methyl-4H-benzo[C]isoxazolo[4,5-e]azepin-4-yl)acetamide monohydrate for treatment of myelofibrosis, IQVIA RDS Ireland Limited;
- Adeno-associated viral vector serotype S3 encoding human alpha-galactosidase A cDNA for treatment of Fabry disease, Freeline Therapeutics (Ireland) Limited;

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- Adeno-associated virus serotype 8 containing the human RdCVF sequence and the human RdCVFL sequence for treatment of inherited retinal dystrophies, SparingVision;
- Adeno-associated virus serotype rh74 containing the human micro-dystrophin gene for treatment of Duchenne muscular dystrophy, Sarepta Therapeutics Ireland Limited;
- Artesunate for treatment of malaria, YES Pharmaceutical Development Services GmbH;
- Autologous human T-cells genetically modified ex vivo with a lentiviral vector encoding a chimeric antigen receptor for B-cell maturation antigen for treatment of multiple myeloma, Janssen-Cilag International N.V.;
- Luspatercept for treatment of myelofibrosis, Celgene Europe B.V.;
- Sintilimab for treatment of peripheral T-cell lymphoma, Parexel International GmbH;
- Sutimlimab for treatment of immune thrombocytopenia, Celerion Austria 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.

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 10 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 4 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

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

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:

- Darzalex (daratumumab) for treatment of plasma cell myeloma, Janssen-Cilag International NV (EU/3/13/1153). The opinion was adopted by written procedure after the December meeting.
- Vyndaqel (tafamidis) for treatment of familial amyloid polyneuropathy, Pfizer Europe MA EEIG (EU/3/06/401). The opinion was adopted by written procedure after the December meeting.
- Vyndaqel (tafamidis) for treatment of senile systemic amyloidosis, Pfizer Europe MA EEIG (EU/3/12/1066). 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 2.

Details on the authorised orphan medicinal products can be found on the EMA website.

Other matters

None

Upcoming meetings

- The 219th meeting of the COMP will be held on 18-20 February 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
(E)-2-((2S,4S)-4-(((2R,4S,5S,6S)-4-amino-5-hydroxy-6-methyltetrahydro-2H-pyran-2-yl)oxy)-2,5,12-trihydroxy-7-methoxy-6,11-dioxo-1,2,3,4,6,11-hexahydrotetracen-2-yl)-10-(carboxymethyl)-1-hydroxy-13-(2-(2-(2-((E)-3-(3-((3-hydroxy-3,3 diphosphonopropyl)(methyl)amino)propoxy)benzylidene)hydrazine-1-carbonothioyl)hydrazineyl)-2-oxoethyl)-8-oxo-5-thioxo-3,4,6,7,10,13-hexaazapentadec-2-en-15-oic acid	Treatment of osteosarcoma	Atlanthera	07 November 2019	16 December 2019
2-(3,7-dimethyl-octa-2, 6-dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzoquinone	Treatment of Huntington's disease	Emerald Health Pharmaceuticals Espana S.L.	05 December 2019	09 January 2020
2-(isopropylamino)-3-methyl-5-(6-methyl-5-((2-(1-methyl-1H-pyrazol-4-yl)pyridin-4-yl)oxy)pyridin-2-yl)pyrimidin-4(3H)-one	Treatment of tenosynovial giant cell tumour, localised and diffuse type	Pharma Gateway AB	07 November 2019	16 December 2019
5,7-dichloro-2-((ethylamino)methyl)-8-hydroxy-3-methylquinazolin-4(3H)-one mesilate	Treatment of multiple system atrophy	Alterity Therapeutics UK Limited	07 November 2019	16 December 2019

Adeno-associated virus serotype 2/6 encoding human alpha-galactosidase A cDNA	Treatment of Fabry disease	ERA Consulting GmbH	05 December 2019	09 January 2020
Adeno-associated virus serotype 9 vector containing human N-acetylgalactosamine-6-sulfate sulfatase gene	Treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome)	Esteve Pharmaceuticals S.A.	05 December 2019	09 January 2020
Adeno-associated virus vector encoding human phenylalanine hydroxylase	Treatment of phenylalanine hydroxylase deficiency	BioMarin International Limited	07 November 2019	16 December 2019
Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the *BCL11A* gene	Treatment of sickle cell disease	Vertex Pharmaceuticals (Ireland) Limited	05 December 2019	09 January 2020
Dimethyl fumarate	Treatment of adrenoleukodystrophy	Consorcio Centro de Investigación Biomédica en Red, M.P.	05 December 2019	09 January 2020
Efgartigimod alfa	Treatment of immune thrombocytopenia	Argenx B.V.B.A.	07 November 2019	16 December 2019
H-Leu-Pro-Pro-Leu-Pro-Tyr-Pro-OH	Treatment of amyotrophic lateral sclerosis	AdRes EU B.V.	07 November 2019	16 December 2019
Lactobacillus plantarum	Treatment of amyotrophic lateral sclerosis	MDC RegAffairs GmbH	07 November 2019	16 December 2019

Motixafortide	Treatment of pancreatic cancer	Granzer Regulatory Consulting & Services	05 December 2019	09 January 2020
Navitoclax	Treatment of myelofibrosis	AbbVie Deutschland GmbH & Co. KG	07 November 2019	16 December 2019
Nicardipine	Treatment of non-traumatic subarachnoid haemorrhage	Bit Pharma GmbH	05 December 2019	09 January 2020
Pamrevlumab	Treatment of Duchenne muscular dystrophy	Voisin Consulting S.A.R.L.	07 November 2019	16 December 2019
Romilkimab	Treatment of systemic sclerosis	Sanofi-Aventis Groupe	05 December 2019	09 January 2020
Setmelanotide	Treatment of Alström syndrome	TMC Pharma (EU) Limited	05 December 2019	09 January 2020
Synthetic double-stranded siRNA oligonucleotide directed against SERPINA1 mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues	Treatment of congenital alpha-1 antitrypsin deficiency	Dicerna Ireland Limited	07 November 2019	16 December 2019
Tripotassium citrate monohydrate and potassium hydrogen carbonate	Treatment of cystinuria	Advicenne S.A.	05 December 2019	09 January 2020
Ziritaxestat	Treatment of systemic sclerosis	Galapagos N.V.	05 December 2019	09 January 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
Duvelisib	Treatment of Follicular lymphoma	Verastem Europe GmbH	EU/3/13/1157
Moxetumomab pasudotox	Treatment of hairy cell leukaemia	AstraZeneca AB	EU/3/08/592
Pemigatinib	Treatment of biliary tract cancer	Incyte Biosciences Distribution B.V.	EU/3/18/2066
Potassium	Treatment of distal renal tubular acidosis	Advicenne Pharma S.A.	EU/3/17/1888
Valoctocogene roxaparvovec	Treatment of Haemophilia A	BioMarin International Limited	EU/3/16/1622