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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 2019

The Committee for Orphan Medicinal Products held its 207th plenary meeting on 22-24 January 2019.

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 adult live cultured osteoblasts for treatment of non-traumatic osteonecrosis, Clinical Network Services (UK) Limited;
- Lurbinectedin for treatment of small cell lung cancer, Pharma Mar S.A.

2. Opinions adopted at the first COMP discussion:

- 9-cis, 12-cis-11,11-D2-linoleic acid ethyl ester for treatment of infantile neuroaxonal dystrophy, FGK Representative Service GmbH;
- Allogeneic cultured postnatal thymus-derived tissue for treatment of DiGeorge syndrome, Enzyvant Therapeutics Ireland Limited;
- Allogeneic cultured postnatal thymus-derived tissue for treatment of CHARGE syndrome, Enzyvant Therapeutics Ireland Limited;
- Allogeneic cultured postnatal thymus-derived tissue for treatment of severe combined immunodeficiency due to FOXN1 deficiency, Enzyvant Therapeutics Ireland Limited;
- Anti-Epstein Barr virus cytotoxic lymphocytes for treatment of post-transplant lymphoproliferative disorder, Common Services Agency (National Health Services - Scotland);

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- Humanised IGg1 monoclonal antibody targeting human transferrin receptor conjugated to human iduronate-2-sulfatase for treatment of mucopolysaccharidosis II (Hunter's syndrome), Artemida Pharma Europe Limited;
- Lentiviral vector encoding human coagulation factor IX for treatment of haemophilia B, Fondazione Telethon;
- Losartan for treatment of epidermolysis bullosa, 3R Pharma Consulting GmbH;
- Poly(N-acetyl, N-arginyl)glucosamine for treatment of cystic fibrosis, Accelsiors CRO And Consultancy Services Ltd.;
- Risdiplam for treatment of spinal muscular atrophy, Roche Registration 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 6 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

4 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 7 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.

[1] Details of all orphan designations granted to date by the European Commission are entered in the EU 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:

- Adcetris - Brentuximab vedotin – Type II variation, for treatment of Hodgkin lymphoma, Takeda Pharma A/S (EU/3/08/596).

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 208th meeting of the COMP will be held on 19-21 February 2019.

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
(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro-1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid	Treatment of biliary atresia	Albireo AB	8 November 2018	14 December 2018
(4-{(2S,4S)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid-hydrogen chloride(1/1))	Treatment of C3 glomerulopathy	Novartis Europharm Limited	8 November 2018	14 December 2018
6,8-bis(benzylthio)octanoic acid	Treatment of pancreatic cancer	IQVIA RDS Ireland Limited	8 November 2018	14 December 2018
6,8-bis(benzylthio)octanoic acid	Treatment of acute myeloid leukaemia	IQVIA RDS Ireland Limited	8 November 2018	14 December 2018
6-fluoro-9-methyl-9H-pyrido[3,4-b]-indole	Treatment of sudden sensorineural hearing loss	AudioCure Pharma GmbH	8 November 2018	14 December 2018
Acetylcysteine	Treatment of pseudomyxoma peritonei	MUCPharm Pty Ltd	8 November 2018	14 December 2018
Acetylleucine	Treatment of ataxia telangiectasia	Intrabio Limited	6 December 2018	11 January 2019
Adeno-associated viral vector expressing human 21-hydroxylase	Treatment of congenital adrenal hyperplasia	Pharma Gateway AB	8 November 2018	14 December 2018
Adeno-associated virus serotype HSC15	Treatment of phenylanaline	Yes Pharmaceutical	8 November 2018	14 December 2018

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
expressing human phenylalanine hydroxylase	hydroxylase deficiency	Development Services GmbH		
Afatinib	Treatment of Fanconi anaemia	Consorcio Centro de Investigación Biomédica en Red, M.P.	8 November 2018	14 December 2018
Allogeneic ABCB5-positive limbal stem cells	Treatment of limbal stem cell deficiency	Rheacell GmbH & Co. KG	8 November 2018	14 December 2018
Benserazide hydrochloride	Treatment of sickle cell disease	Isabelle Ramirez	6 December 2018	11 January 2019
Bifunctional fusion protein composed of two extracellular domains of transforming growth factor beta receptor II fused with a human immunoglobulin G1 monoclonal antibody against programmed death ligand 1	Treatment of biliary tract cancer	Merck Europe B.V.	8 November 2018	14 December 2018
Bromelain	Treatment of pseudomyxoma peritonei	MUCPharm Pty Ltd	8 November 2018	14 December 2018
C1 esterase inhibitor (human)	Treatment in solid organ transplantation	Shire Pharmaceuticals Ireland Limited	8 November 2018	14 December 2018
Human anti-promyostatin monoclonal antibody	Treatment of spinal muscular atrophy	Yes Pharmaceutical Development Services GmbH	8 November 2018	14 December 2018
Human glucagon-like peptide-2 analogue linked to a human immunoglobulin Fc fragment	Treatment of short bowel syndrome	Hanmi Europe Limited	6 December 2018	11 January 2019
Ivacaftor, N-(1,3-dimethyl-1H-pyrazole-4-sulfonyl)-6-[3-(3,3,3-trifluoro-2,2-dimethylpropoxy)-1H-pyrazol-1-yl]-2-[(4S)-2,2,4-trimethylpyrrolidin-1-yl]pyridine-3-carboxamide, tezacaftor	Treatment of cystic fibrosis	Vertex Pharmaceuticals (Europe) Limited	8 November 2018	14 December 2018

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Ivacaftor, potassium(benzenesulfonyl)({[6-(3-{2-[1-(trifluoromethyl) cyclopropyl]ethoxy}-1H-pyrazol-1-yl)-2-[(4S)-2,2,4-trimethylpyrrolidin-1-yl]pyridin-3-yl]carbonyl})azanide, tezacaftor	Treatment of cystic fibrosis	Vertex Pharmaceuticals (Europe) Limited	8 November 2018	14 December 2018
Lonafarnib	Treatment of Hutchinson-Gilford progeria syndrome	Eiger Biopharmaceuticals Europe Limited	8 November 2018	14 December 2018
Marizomib	Treatment of glioma	Celgene Europe B.V.	8 November 2018	14 December 2018
Melatonin	Treatment of perinatal asphyxia	Therapicon S.r.l.	6 December 2018	11 January 2019
Mercaptamine-pantetheine disulfide	Treatment of Rett syndrome	Thiogenesis Therapeutics S.A.R.L.	6 December 2018	11 January 2019
Miglustat	Treatment of glycogen storage disease type II (Pompe's disease)	Amicus Therapeutics UK Limited	6 December 2018	11 January 2019
Pevonedistat	Treatment of myelodysplastic syndromes	Takeda Pharma A/S	8 November 2018	14 December 2018
Ralinepag	Treatment of pulmonary arterial hypertension	Arena Pharmaceuticals Limited	6 December 2018	11 January 2019
Rozanolixizumab	Treatment of immune thrombocytopenia	UCB Pharma	6 December 2018	11 January 2019
Sodium 2-hydroxylinoleate	Treatment of biliary tract cancer	Ability Pharmaceuticals SL	8 November 2018	14 December 2018
Synthetic double-stranded siRNA oligonucleotide directed against *TMPRSS6* mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues	Treatment of beta-thalassaemia intermedia and major	Silence Therapeutics AG	6 December 2018	11 January 2019
Venglustat	Treatment of autosomal dominant polycystic kidney disease	Genzyme Europe BV	8 November 2018	14 December 2018

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Vinorelbine tartrate	Treatment of soft tissue sarcoma	TLC Biopharmaceuticals B.V.	6 December 2018	11 January 2019

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

No new designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since last COMP plenary meeting.