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Human Medicines Division

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

April 2020

The Committee for Orphan Medicinal Products held its 221st plenary meeting on 21-23 April 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 T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation antigen with 4-1BB and CD3-zeta intracellular signalling domains for treatment of multiple myeloma, FGK Representative Service GmbH;
- Lutetium (^{177}Lu) lilotomab satetraxetan for treatment of marginal zone lymphoma, Nordic Nanovector ASA;
- Methotrexate for treatment of retinal detachment, Helio Vision Germany GmbH.

2. Opinions adopted at the first COMP discussion:

- (4-{(2S,4S)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid-hydrogen chloride(1/1)) for treatment of paroxysmal nocturnal haemoglobinuria, Novartis Europharm Limited;
- 1-((2S,4S)-2-((((S)-(4-bromophenoxy)(((S)-1-oxo-1-(((S)-pentan-2-yl)oxy)propan-2-yl)amino)phosphoryl)oxy)methyl)-1,3-dioxolan-4-yl)-2-oxo-1,2-dihydropyrimidin-4-aminium chloride for treatment of hepatocellular carcinoma, Medivir AB;

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- Adeno-associated virus serotype 9 containing the human ASPA gene for treatment of Canavan disease, Raremoon Consulting Limited;
- Autologous CD4+ and CD8+ T cells transduced with a lentiviral vector encoding an affinity enhanced T cell receptor specific to MAGE-A4 for treatment of soft tissue sarcoma, Adaptimmune Limited;
- Ile-Ala-Leu-Ile-Leu-Glu-Pro-Ile-Cys-Cys-Gln-Glu-Arg-Ala-Ala-(discrete-polyethylene glycol)24 for treatment of neonatal encephalopathy, Clinipace GmbH;
- Lumacaftor for treatment of non-traumatic aneurysmal subarachnoid haemorrhage, Qanatpharma GmbH;
- Rilzabrutinib for treatment of immune thrombocytopenia, Clinical Network Services (NL) B.V.;
- Sodium phenylbutyrate, tauroursodeoxycholic acid for treatment of amyotrophic lateral sclerosis, Drug Development and Regulation S.L.;
- Viltolarsen for treatment of Duchenne muscular dystrophy, Medpace Finland Oy.

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 5 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 7 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:

- Adcetris (brentuximab vedotin) for treatment of peripheral T-cell lymphoma, Takeda Pharma A/S (EU/3/08/595).
- Zolgensma (onasemnogene abeparvovec) for treatment of spinal muscular atrophy, AveXis Netherlands B.V (EU/3/15/1509). The opinion was adopted by written procedure after the March meeting.
- Pretomanid FGK (pretomanid) for the treatment of tuberculosis, FGK Representative Service GmbH (EU/3/07/513). The opinion was adopted by written procedure after the March 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

The main topics addressed during the meeting related to:

- Protocol assistance advice
- Adoption of the updated COMP Rules of Procedure

Upcoming meetings

- The 222nd meeting of the COMP will be held on 18-20 May 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
2-hydroxy-N,N,N-trimethylethan-1-aminium (Z)-4-(5-((3-benzyl-4-oxo-2-thioxothiazolidin-5-ylidene)methyl)furan-2-yl)benzoate	Treatment of pancreatic cancer	MWB Consulting S.A.R.L.	20 February 2020	24 March 2020
Adeno-associated viral vector serotype 3B encoding human multidrug resistance protein 3A	Treatment of progressive familial intrahepatic cholestasis	Vivet Therapeutics S.A.S.	19 March 2020	22 April 2020
Allogeneic multi-virus specific T lymphocytes targeting BK virus, cytomegalovirus, human herpesvirus-6, Epstein-Barr virus and adenovirus	Treatment in haematopoietic stem cell transplantation	TMC Pharma (EU) Limited	20 February 2020	24 March 2020
Asciminib	Treatment of chronic myeloid leukaemia	Novartis Europharm Limited	20 February 2020	24 March 2020

Cusatuzumab	Treatment of acute myeloid leukaemia	Janssen-Cilag International NV	19 March 2020	22 April 2020
DNA plasmid encoding IL-12 p35 and p40 genes	Treatment of ovarian cancer	FGK Representative Service GmbH	19 March 2020	22 April 2020
Florbetaben (^{18}F)	Diagnosis of AL amyloidosis	Life Molecular Imaging GmbH	19 March 2020	22 Apr 2020
Fosgemcitabine palabenamide	Treatment of biliary tract cancer	Pharma Gateway AB	20 February 2020	24 March 2020
Losmapimod	Treatment of facioscapulohumeral muscular dystrophy	Pharma Gateway AB	20 February 2020	24 March 2020
Trifarotene	Treatment of autosomal recessive congenital ichthyosis	Premier Research Group S.L.	20 February 2020	24 March 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
Berotralstat	Treatment of hereditary angioedema	BioCryst Ireland Limited	EU/3/18/2028
Hydrocortisone	Treatment of congenital adrenal hyperplasia	Diurnal Europe BV	EU/3/05/296
Selumetinib	Treatment of neurofibromatosis type 1	AstraZeneca AB	EU/3/18/2050