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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

March 2020

The Committee for Orphan Medicinal Products held its 220th plenary meeting on 17-19 March 2020.

Orphan medicinal product designation

Positive opinions

The COMP adopted 11 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:

- DNA plasmid encoding IL-12 p35 and p40 genes for treatment of ovarian cancer, FGK Representative Service GmbH;
- Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R, 4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate for treatment in haematopoietic stem cell transplantation, CATS Consultants GmbH;
- Rozanolixizumab for treatment of myasthenia gravis, UCB Pharma;
- Tislelizumab for treatment of hepatocellular carcinoma, BeiGene Ireland Limited.

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 3B encoding human multidrug resistance protein 3A for treatment of progressive familial intrahepatic cholestasis, Vivet Therapeutics S.A.S.;
- Adeno-associated virus vector serotype hu37 encoding human factor VIII for treatment of haemophilia A, Bayer AG;

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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- Anti-(integrin beta-3) human monoclonal antibody for prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility, FGK Representative Service GmbH;
- Cusatuzumab for treatment of acute myeloid leukaemia, Janssen-Cilag International N.V.;
- Florbetaben (^{18}F) for diagnosis of AL amyloidosis, Life Molecular Imaging GmbH;
- Glucagon analogue linked to a human immunoglobulin Fc fragment for treatment of insulin autoimmune syndrome, JVM Europe B.V.;
- Mitapivat sulfate for treatment of pyruvate kinase deficiency, Agios Netherlands B.V..

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:

The following two opinions were revised after the February meeting and adopted in March 2020.

- Benzyl benzoate, beta-caryophyllene, cineole, cinnamaldehyde, cinnamyl acetate, linalool, trans-2-methoxycinnamaldehyde for treatment of eumycetoma, Septeos S.A.S.;
- Melatonin treatment of intracerebral haemorrhage, Worphmed S.r.l..

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 9 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 5 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:

- Hepcludex (bulevirtide) for treatment of hepatitis delta virus infection, MYR GmbH (EU/3/15/1500).

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 221st meeting of the COMP will be held on 21-23 April 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

Contact details of our press officer

Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: press@ema.europa.eu

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-((4S)-6-(4-chlorophenyl)-1-methyl-4H-benzo[C]isoxazolo[4,5-e]azepin-4-yl)acetamide monohydrate	Treatment of myelofibrosis	IQVIA RDS Ireland Limited	22 January 2020	28 February 2020
Adeno-associated virus serotype 2/6 encoding human alpha-galactosidase A cDNA	Treatment of Fabry disease	Freeline Therapeutics (Ireland) Limited	22 January 2020	28 February 2020
Adeno-associated virus serotype 8 containing the human RdCVF sequence and the human RdCVFL sequence	Treatment of inherited retinal dystrophies	SparingVision	22 January 2020	28 February 2020
Adeno-associated virus serotype rh74 containing the human micro-dystrophin gene	Treatment of Duchenne muscular dystrophy	Sarepta Therapeutics Ireland Limited	22 January 2020	28 February 2020
Artesunate	Treatment of malaria	YES Pharmaceutical Development Services GmbH	22 January 2020	28 February 2020

Autologous human T cells genetically modified ex-vivo with a lentiviral vector encoding a chimeric antigen receptor for B-cell maturation antigen	Treatment of multiple myeloma	Janssen-Cilag International N.V.	22 January 2020	28 February 2020
Autologous skin equivalent graft composed of keratinocytes and fibroblasts genetically corrected by CRISPR/Cas9-mediated excision of mutation-carrying *COL7A1* exon 80	Treatment of epidermolysis bullosa	Consorcio Centro de Investigación Biomédica en Red, M.P.	22 January 2020	28 February 2020
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	Treatment of inherited retinal dystrophies	Fondazione Telethon	22 January 2020	28 February 2020
Luspatercept	Treatment of myelofibrosis	Celgene Europe B.V.	22 January 2020	28 February 2020
Reldesemtiv	Treatment of amyotrophic lateral sclerosis	Pharma Gateway AB	22 January 2020	28 February 2020
Sintilimab	Treatment of peripheral T-cell lymphoma	Parexel International GmbH	22 January 2020	28 February 2020
Sutimlimab	Treatment of immune thrombocytopenia	Celerion Austria GmbH	22 January 2020	28 February 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
None			