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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 2019

The Committee for Orphan Medicinal Products held its 209th plenary meeting on 19-21 March 2019.

Orphan medicinal product designation

Positive opinions

The COMP adopted 6 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:

- 3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-pyrazol-1-yl)propanoic acid for treatment of Stargardt's disease, TMC Pharma (EU) Limited;
- Autologous human bone marrow-derived haemaetopoietic and mesenchymal stem cells depleted of erythrocytes, monocytes and lymphocytes for treatment of spinal cord injury, Neuroplast B.V.;
- Balipodect for treatment of fragile X syndrome, Takeda Pharma A/S.

2. Opinions adopted at the first COMP discussion:

- Human culture expanded autologous mesenchymal stromal cells for treatment of amyotrophic lateral sclerosis, IQVIA RDS Ireland Limited;
- Modified messenger ribonucleic acid encoding human propionyl-coenzyme A carboxylase alpha and beta subunits encapsulated into lipid nanoparticle for treatment of propionic acidaemia, Pharma Gateway AB;
- Sodium benzoate, sodium phenylacetate for treatment of ornithine transcarbamylase deficiency, Dipharma B.V.

3. Opinion following appeal procedures:

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None

The COMP also recommended the amendment to 1 existing orphan designation:

- Eculizumab for treatment of neuromyelitis optica spectrum disorders, Alexion Europe S.A.S.

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 8 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 4 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] Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products

1. Opinions adopted at time of CHMP opinion:

- Palynziq (pegvaliase) for treatment of hyperphenylalaninaemia, BioMarin International Limited (EU/3/09/708).
- Waylivra (volanesorsen) for treatment of familial chylomicronemia syndrome, Akcea Therapeutics UK Ltd (EU/3/14/1249). The opinion was adopted by written procedure after the February 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

Upcoming meetings

- The 210th meeting of the COMP will be held on 15-17 April 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
9-cis, 12-cis-11,11-D2-linoleic acid ethyl ester	Treatment of infantile neuroaxonal dystrophy	FGK Representative Service GmbH	24 January 2019	26 February 2019
Allogeneic cultured postnatal thymus-derived tissue	Treatment of DiGeorge syndrome	Enzyvant Therapeutics Ireland Limited	24 January 2019	26 February 2019
Allogeneic cultured postnatal thymus-derived tissue	Treatment of CHARGE syndrome	Enzyvant Therapeutics Ireland Limited	24 January 2019	26 February 2019
Allogeneic cultured postnatal thymus-derived tissue	Treatment of severe combined immunodeficiency due to FOXN1 deficiency	Enzyvant Therapeutics Ireland Limited	24 January 2019	26 February 2019
Anti-Epstein Barr virus cytotoxic lymphocytes	Treatment of post-transplant lymphoproliferative disorder	Common Services Agency (National Health Services - Scotland)	24 January 2019	26 February 2019
Autologous adult live cultured osteoblasts	Treatment of non-traumatic osteonecrosis	Clinical Network Services (UK) Limited	24 January 2019	26 February 2019
Humanised IGg1 monoclonal antibody targeting human transferrin receptor conjugated to human iduronate-2-sulfatase	Treatment of mucopolysaccharidosis II (Hunter's syndrome)	Artemida Pharma Europe Limited	24 January 2019	26 February 2019
Lentiviral vector encoding human coagulation	Treatment of haemophilia B	Fondazione Telethon	24 January 2019	26 February 2019

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
factor IX				
Losartan	Treatment of epidermolysis bullosa	3R Pharma Consulting GmbH	24 January 2019	26 February 2019
Lurbinectedin	Treatment of small cell lung cancer	Pharma Mar S.A.	24 January 2019	26 February 2019
Poly(N-acetyl, N-arginyl)glucosamine	Treatment of cystic fibrosis	Accelsiors CRO And Consultancy Services Ltd.	24 January 2019	26 February 2019
Risdiplam	Treatment of spinal muscular atrophy	Roche Registration GmbH	24 January 2019	26 February 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

Please also refer to the Community Register of orphan medicinal products for human use.

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Diclofenamide	Treatment of periodic paralysis	Sun Pharmaceutical Industries Europe B.V.	EU/3/16/1616
Fenfluramin	Treatment of Dravet syndrome	Zogenix GmbH	EU/3/13/1219
Imlifidase	Prevention of graft rejection following solid organ transplantation	Hansa Biopharma AB	EU/3/16/1826
Treprostinil sodium	Treatment of chronic thromboembolic pulmonary hypertension	SciPharm Sarl	EU/3/13/1103
Gilteritinib	Treatment of acute myeloid leukaemia	Astellas Pharma Europe B.V.	EU/3/17/1961
Nintedanib	Treatment of idiopathic pulmonary fibrosis	Boehringer Ingelheim International GmbH	EU/3/13/1123