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Human Medicines Division

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

April 2022

The Committee for Orphan Medicinal Products held its 243rd plenary meeting on 11-13 April 2022.

Orphan medicinal product designation

Positive opinions

The COMP adopted 13 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the first and second COMP discussion:

- Adeno-associated virus serotype 8 expressing the human gamma-sarcoglycan gene for treatment of limb-girdle muscular dystrophy, Atamyo Therapeutics;
- Adeno-associated virus serotype R100 containing the human *RPGRorf15* gene isoform for treatment of inherited retinal dystrophies due to defects in the *RPGR* gene, Pharma Gateway AB;
- Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD123 for treatment of blastic plasmacytoid dendritic cell neoplasm, INSERM UMR 1098;
- Autologous peripheral blood-derived CD4 T-cells CRISPR-edited at the CD40LG locus for treatment of hyper IgM syndromes, Fondazione Telethon;
- Cannabidiol for treatment of epidermolysis bullosa, Tetra Bio-Pharma Europe Limited;
- Chimeric peptide of human glucagon-like peptide-1, glucagon and gastric inhibitory polypeptide analogues linked to a human immunoglobulin Fc fragment for treatment of idiopathic pulmonary fibrosis, JVM Europe B.V.;
- Elezanumab for treatment of spinal cord injury, AbbVie Deutschland GmbH & Co. KG;
- Elamipretide for treatment of myopathic mitochondrial DNA depletion syndrome, Scendea (NL) B.V.;
- Fusion protein composed of the first 2 immunoglobulin-like domains of the human roundabout guidance receptor 2 fused to a human IgG1 crystallised fragment for treatment of focal segmental glomerulosclerosis., Pfizer Europe MA EEIG;
- Ibudilast for treatment of fragile X syndrome, Healx Technology Limited;
- Icerguastat acetate for treatment of amyotrophic lateral sclerosis, Inflectis Bioscience S.A.S;
- Pasireotide for treatment of noninsulinoma pancreatogenous hypoglycemia syndrome, Recordati Rare Diseases;

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- Streptococcus pyogenes, group A, type 3, strain Su, inactivated for treatment of primary lymphatic malformations, Pharma Gateway AB.

2. Opinions 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 opinions

1. Opinions adopted following the sponsor's response to the COMP list of questions:

- Melatonin for prevention of spaceflight-related radiation and microgravity, Worphmed S.r.l.

2. Opinions 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

6 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 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 medicinal products for which decisions on orphan designation have been granted by the European Commission is provided in Community 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] Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products

Positive opinions

1. Opinions adopted at time of CHMP opinion:

- Polivy (polatuzumab vedotin) for treatment of diffuse large B-cell lymphoma, EU/3/18/2013, Roche Registration GmbH. The opinion was adopted by written procedure after the March meeting.
- Lunsumio (mosunetuzumab) for treatment of follicular lymphoma, EU/3/21/2517, Roche Registration GmbH. The opinion was adopted by written procedure after the April meeting.
- Filsuvez (betulae cortex dry extract (DER 5-10: 1), extraction solvent n-heptane 95% (w/w)) for treatment of epidermolysis bullosa, EU/3/10/845, Amryt Pharmaceuticals Designated Activity Company. The opinion was adopted by written procedure after the April meeting.

2. Opinions following appeal procedures:

None

Negative opinions

1. Opinions adopted at time of CHMP opinion:

None

2. Opinions following appeal procedures:

- Nexviadyme (avalglucosidase alfa) for treatment of Pompe's disease, Genzyme Europe B.V. EU/3/14/1251. The opinion was adopted by written procedure after the April meeting.

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 244th meeting of the COMP will be held on 10-12 May 2022.

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

Enquiries to: AskEMA (https://www.ema.europa.eu/en/about-us/contact/send-question-european-medicines-agency

Annex 1

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
Etranacogene dezaparvovec	Treatment of haemophilia B	CLS Behring GmbH	EU/3/18/1999
Ivosidenib	Treatment of biliary tract cancer Treatment of acute myeloid leukaemia	Les Laboratoires Servier	EU/3/18/1994 EU/3/16/1802
Tislelizumab	Treatment of oeshphageal cancer	Novartis Europharm Limited	EU/3/20/2357
Tremelimumab	Treatment of hepatocellular carcinoma	Astra ZenecaAB	EU/3/20/2370