11 November 2019
EMA/COMP/557148/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

November 2019

The Committee for Orphan Medicinal Products held its 216th plenary meeting on 5-7 November 2019.

Orphan medicinal product designation

Positive opinions

The COMP adopted 10 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:

- Efgartigimod alfa for treatment of immune thrombocytopenia, Argenx B.V.B.A.;
- *Lactobacillus plantarum* for treatment of amyotrophic lateral sclerosis, MDC RegAffairs GmbH. The opinion was adopted by written procedure after the November meeting;
- Pamrevlumab for treatment of Duchenne muscular dystrophy, Voisin Consulting S.A.R.L.

2. Opinions adopted at the first COMP discussion:

- (E)-2-((2S,4S)-4-(((2R,4S,5S,6S)-4-amino-5-hydroxy-6-methyltetrahydro-2H-pyran-2-yl)oxy)-2,5,12-trihydroxy-7-methoxy-6,11-dioxo-1,2,3,4,6,11-hexahydrotetracen-2-yl)-10-(carboxymethyl)-1-hydroxy-13-(2-(2-(2-((E)-3-(3-((3-hydroxy-3,3 diphosphonopropyl)(methyl)amino)propoxy)benzylidene)hydrazine-1-carbonothioyl)hydrazineyl)-2-oxoethyl)-8-oxo-5-thioxo-3,4,6,7,10,13-hexaazapentadec-2-en-15-oic acid, for treatment of osteosarcoma, Atlanthera;
- 2-(isopropylamino)-3-methyl-5-(6-methyl-5-((2-(1-methyl-1H-pyrazol-4-yl)pyridin-4-yl)oxy)pyridin-2-yl)pyrimidin-4(3H)-one for treatment of tenosynovial giant cell tumour, localised and diffuse type, Pharma Gateway AB;

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
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

- 5,7-dichloro-2-((ethylamino)methyl)-8-hydroxy-3-methylquinazolin-4(3H)-one mesilate for treatment of multiple system atrophy, Alterity Therapeutics UK Limited;
- Adeno-associated virus vector encoding human phenylalanine hydroxylase for treatment of phenylalanine hydroxylase deficiency, BioMarin International Limited. The opinion was adopted by written procedure after the November meeting;
- H-Leu-Pro-Pro-Leu-Pro-Tyr-Pro-OH for treatment of amyotrophic lateral sclerosis, AdRes EU B.V.;
- Navitoclax for treatment of myelofibrosis, AbbVie Deutschland GmbH & Co. KG;
- Synthetic double-stranded siRNA oligonucleotide directed against SERPINA1 mRNA and containing four modified nucleosides which form a ligand cluster of four N-acetylgalactosamine residues for treatment of congenital alpha-1 antitrypsin deficiency, Dicerna Ireland Limited.

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(s) adopted following the sponsor's response to the COMP list of questions:

- None

2. Opinion following appeal procedures:

- Naltrexone for treatment of fibromyalgia, Able AB.

Lists of questions

The COMP adopted 11 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting 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 5 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:

- None

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:

- None

Upcoming meetings

- The 217th meeting of the COMP will be held on 3-5 December 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

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
(16E)-14-methyl-20-oxa-5,7,14,26-tetraaza-tetracyclo[19.3.1.1(2,6).1(8,12)]heptacosa-1(25),2(26),3,5,8(27),9,11,16,21,23-decaene–citric acid	Treatment of glioma	Clinical Network Services (NL) B.V.	12 September 2019	17 October 2019
(S)-2-isobutyrylamino-pentanedioic acid 5-amide 1-{[(2S,5S,8S,11R,12S,15S,18S,21R)-2,8-bis-((S)-sec-butyl)-21-hydroxy-5-(4-hydroxy-benzyl)-15-isobutyl-4,11-dimethyl-3,6,9,13,16,22-hexaoxo-10-oxa-1,4,7,14,17-pentaaza-bicyclo[16.3.1]docos-12-yl]-amide}	Treatment of Netherton syndrome	MDC RegAffairs GmbH	12 September 2019	17 October 2019
18-(p-(131I)-iodophenyl)octadecyl phosphocholine	Treatment of multiple myeloma	Voisin Consulting S.A.R.L.	12 September 2019	17 October 2019
2-(3-(4-(1H-indazol-5-ylamino)quinazolin-2-yl)phenoxy)-N-isopropylacetamide-methane sulfonic acid salt	Treatment of graft-versus-host disease	Quality Regulatory Clinical Ireland Limited	12 September 2019	17 October 2019
2'-O-(2-methoxyethyl)-D-ribose antisense oligonucleotide targeting glial fibrillary acidic protein messenger ribonucleic acid	Treatment of Alexander disease	Ionis Development (Ireland) Limited	12 September 2019	17 October 2019
4-oxo-4H-chromene-2-carboxylic acid (2-(2-4-(2-(6,7-dimethoxy-3,4-dihydro-1H-isoquinolin-2-yl)-ethyl)-phenyl-2H-tetrazol-5-yl)-4,5-dimethoxy-phenyl)-amide	Treatment of soft tissue sarcoma	Boyd Consultants Limited	12 September 2019	17 October 2019
Anti-neonatal Fc receptor human monoclonal antibody	Prevention of haemolytic disease of the foetus and newborn	Biopharma Excellence GmbH	12 September 2019	17 October 2019
Autologous CD34+ haematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene	Treatment of beta-thalassaemia intermedia and major	Vertex Pharmaceuticals (Ireland) Limited	12 September 2019	17 October 2019

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Besilesomab	Treatment in haematopoietic stem cell transplantation	Therapharm Deutschland GmbH	12 September 2019	17 October 2019
Combination of two adeno-associated viral vectors of serotype 8 containing the 5'- and the 3'- half coding sequences of human ABCA4 fused to inteins	Treatment of Stargardt's disease	Fondazione Telethon	12 September 2019	17 October 2019
Leriglitazone	Treatment of Friedreich’s ataxia	Minoryx Therapeutics S.L.	12 September 2019	17 October 2019
Lonapegsomatropin	Treatment of growth hormone deficiency	Ascendis Pharma Endocrinology Division A/S	12 September 2019	17 October 2019
Nirogacestat	Treatment of soft tissue sarcoma	Voisin Consulting S.A.R.L.	12 September 2019	17 October 2019
Paclitaxel	Treatment of soft tissue sarcoma	Boyd Consultants Limited	12 September 2019	17 October 2019
Pemigatinib	Treatment of myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2	Incyte Biosciences Distribution B.V.	12 September 2019	17 October 2019
Propranolol hydrochloride	Treatment of retinopathy of prematurity	Recordati Rare Diseases	12 September 2019	17 October 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
Acalabrutinib	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma	AstraZeneca AB	EU/3/16/1624
Bulevirtide	Treatment of hepatitis delta virus infection	MYR GmbH	EU/3/15/1500
Elexacaftor/Tezacaftor/Ivacaftor	Treatment of cystic fibrosis	Vertex Pharmaceuticals (Ireland) Limited	EU/3/18/2116