26 July 2019
EMA/COMP/386594/2019
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

July 2019

The Committee for Orphan Medicinal Products held its 213th plenary meeting on 16-18 July 2019.

Orphan medicinal product designation

Positive opinions

The COMP adopted 14 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:

- Gallium citrate for treatment of cystic fibrosis, Clinical Network Services (NL) B.V.;
- Peginterferon lambda-1a for treatment of hepatitis D virus infection, Eiger Biopharmaceuticals Europe Limited;
- Temozolomide for treatment of neuroblastoma, Orphelia Pharma S.A.S.

2. Opinions adopted at the first COMP discussion:

- 1-(2,2-diphenyltetrahydrofuran-3-yl)-N,N-dimethylmethanamine hydrochloride for treatment of Rett syndrome, Anavex Germany GmbH;
- 4-(2-chloro-4-methoxy-5-methylphenyl)-N-[(1S)-2-cyclopropyl-1-(3-fluoro-4-methylphenyl)ethyl]-5-methyl-N-(2-propynyl)-1,3-thiazol-2-amine for treatment of congenital adrenal hyperplasia, Neurocrine Therapeutics Limited;
- Acetazolamide for treatment of periodic paralysis, Laboratorios Tillomed Spain, S.L.U;
- Adenoviral vector serotype 5 encoding the human interleukin-12 p70 transgene under the control of activator ligand veledimex for treatment of glioma, Ziopharm Oncology Limited;
- Clofazimine for treatment of nontuberculous mycobacterial lung disease, Sebastian Canisius;

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- Poly(oxy-1,2-ethanediyl), alpha-hydro-omega-hydroxy-,15,15'-diester with N-acetyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyl-L-alpha-aspartyl-L-tryptophylglycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-L-threonyl-2-[2-(2-aminoethoxy)ethoxy]acetyl-N6-carboxy-L-lysinamide cyclic (2.fwdarw.12)-(disulfide); where two identical synthetic peptide domains are covalently linked at the ends of the polyethylene glycol chain for treatment of C3 glomerulopathy, Apellis Ireland Limited;
- Recombinant self-complementary adeno-associated viral vector serotype 9 containing the human *CLN3* gene for treatment of neuronal ceroid lipofuscinosis, Amicus Therapeutics Europe Limited;
- Recombinant self-complementary adeno-associated viral vector serotype 9 containing the human *CLN6* gene for treatment of neuronal ceroid lipofuscinosis, Amicus Therapeutics Europe Limited;
- Relacorilant for treatment of pancreatic cancer, Granzer Regulatory Consulting & Services;
- Setmelanotide for treatment of Bardet-Biedl syndrome (BBS), TMC Pharma (EU) Limited;
- Veledimex for treatment of glioma, Ziopharm Oncology Limited.

3. Opinion following appeal procedures:

None

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

- Monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E for treatment of peripheral T-cell lymphoma, Takeda Pharma 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:

- Naltrexone for treatment of fibromyalgia, Able AB.

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 16 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

9 oral hearings took place.

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

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 13 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. Opinions adopted at time of CHMP opinion:

- Epidyolex (cannabidiol) for treatment of Dravet syndrome, GW Pharma (International) B.V. (EU/3/14/1339). The opinion was adopted by written procedure after the July meeting.
- Epidyolex (cannabidiol) for treatment of Lennox-Gastaut syndrome, GW Pharma (International) B.V. (EU/3/17/1855). The opinion was adopted by written procedure after the July meeting.
- Imbruvica (ibrutinib) - Type II variation, for treatment of lymphoplasmacytic lymphoma, Janssen-Cilag International NV (EU/3/14/1264).
- Soliris (eculizumab) - Type II variation, for treatment of neuromyelitis optica spectrum disorder, Alexion Europe SAS (EU/3/13/1185). The opinion was adopted by written procedure after the July 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 214th meeting of the COMP will be held on 10-12 September 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
(S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidine-3-yl)chroman-2-carboxamide hydrochloride	Treatment of maternally-inherited diabetes and deafness	Khondrion BV	23 May 2019	28 June 2019
2-(2-{[2-(1H-benzimidazol-2-yl)ethyl]amino}ethyl)-N-[(3-fluoropyridine-2-yl)methyl]-1,3-oxazole-4-carboxamide trihydrochloride	Treatment of beta-thalassaemia intermedia and major	Vifor France S.A.	23 May 2019	28 June 2019
5'-cEtG-sp-cEt5MeU-sp-cEt5MeU-sp-dT-sp-dA-sp-dT-sp-dT-sp-dA-sp-dT-sp-dA-sp-dG-sp-dG-sp-dG-sp-cEt5MeC-sp-cEt5MeU-sp-cEt5MeU-3'	Treatment of centronuclear myopathies	Dynacure S.A.S.	23 May 2019	28 June 2019
Gaboxadol monohydrate	Treatment of Angelman syndrome	FGK Representative Service GmbH	23 May 2019	28 June 2019
Imidazolyl ethanamide pentandioic acid	Treatment of acute radiation syndrome	Myelo Therapeutics GmbH	23 May 2019	28 June 2019
Rasagiline	Treatment of Duchenne muscular dystrophy	TMC Pharma (EU) Limited	23 May 2019	28 June 2019
Recombinant adeno-associated viral vector containing a bioengineered capsid serotype AAV-rh74 and a codon-optimised expression cassette to drive the expression of a secretable	Treatment of glycogen storage disease type II (Pompe's disease)	Spark Therapeutics Ireland Limited	23 May 2019	28 June 2019

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
form of human acid alpha-glucosidase				
Recombinant human coagulation factor VIII Fc - von Willebrand factor - XTEN fusion protein	Treatment of haemophilia A	Swedish Orphan Biovitrum AB (publ)	23 May 2019	28 June 2019
Regorafenib	Treatment of glioma	Bayer AG	23 May 2019	28 June 2019
Reldesemtiv	Treatment of spinal muscular atrophy	Pharma Gateway AB	23 May 2019	28 June 2019
Sodium benzoate, sodium phenylacetate	Treatment of hyperargininaemia	Dipharma B.V.	23 May 2019	28 June 2019
Sodium benzoate, sodium phenylacetate	Treatment of argininosuccinic aciduria	Dipharma B.V.	23 May 2019	28 June 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
Crizanlizumab	Treatment of sickle cell disease	Novartis Europharm Limited	EU/3/12/1034
Obiltoxaximab	Treatment of anthrax	SFL Regulatory Services GmbH	EU/3/18/2065
Idebenone	Treatment of Duchenne muscular dystrophy	Santhera Pharmaceuticals (Deutschland) GmbH	EU/3/07/437