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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

April 2019

The Committee for Orphan Medicinal Products held its 210th plenary meeting on 15-17 April 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:

- Relacorilant for treatment of Cushing's syndrome, Granzer Regulatory Consulting & Services;
- Zanubrutinib for treatment of lymphoplasmacytic lymphoma, BeiGene Ireland Limited.

2. Opinions adopted at the first COMP discussion:

- Allogeneic skin-derived ABCD5-positive mesenchymal stem cells for treatment of epidermolysis bullosa, Rheacell GmbH & Co. KG;
- Diacerein for treatment of epidermolysis bullosa, WORPHMED World Orphan Medicines Limited;
- Emixustat hydrochloride for treatment of Stargardt's disease, Pharma Gateway AB;
- (S)-5-(1-(6-chloro-2-oxo-1,2-dihydroquinolin-3-yl)ethylamino)-1-methyl-6-oxo-1,6-dihydropyridine-2-carbonitrile for treatment of acute myeloid leukaemia, Pharma Gateway AB;
- N-(trans-3-(5-((R)-1-hydroxyethyl)-1,3,4-oxadiazol-2-yl)cyclobutyl)-3-phenylisoxazole-5-carboxamide for treatment of cystic fibrosis, Voisin Consulting S.A.R.L.;
- (S)-3-((3-(1-((6-(3,4-dimethoxyphenyl)pryazin-2-yl)amino)ethyl)phenyl)carbamoyl)-5-methylpridin-1-ium for treatment of pulmonary arterial hypertension, MWB Consulting S.A.R.L.;

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- Sodium benzoate, sodium phenylacetate for treatment of citrullinaemia type 1, Dipharma B.V.;
- Sodium benzoate, sodium phenylacetate for treatment of carbamoyl-phosphate synthase-1 deficiency, Dipharma 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:

None

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 12 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 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 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:

- Imnovid (pomalidomide) for treatment of multiple myeloma, Celgene Europe Limited (EU/3/09/672).

2. Opinion following appeal procedures:

Following an appeal procedure, the COMP adopted an opinion recommending the following orphan medicinal product to be removed from the Community Register of orphan medicinal products for human use:

- Trecondi (treosulfan) for conditioning treatment prior to haematopoietic progenitor cell transplantation, medac Gesellschaft fur klinische Spezialpraparate mbH (EU/3/04/186). 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 211th meeting of the COMP will be held on 21-23 May 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
1-[(3S)-3-{4-amino-3-[(3,5-dimethoxyphenyl)ethynyl]-1H-pyrazolo[3,4-d]pyrimidin-1-yl}pyrrolidin-1-yl]-2-propen-1-one	Treatment of biliary tract cancer	Taiho Pharma Europe Limited	21 February 2019	1 April 2019
2-[3-(2-chloro-4-{[5-cyclopropyl-3-(2,6-dichlorophenyl)-1,2-oxazol-4-yl]methoxy}phenyl)-3-hydroxyazetidin-1-yl]pyridine-4-carboxylic acid-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1)	Treatment of primary sclerosing cholangitis	Gilead Sciences Ireland UC	21 February 2019	1 April 2019
3-(3-(3,5-bis(trifluoromethyl)phenyl)-1H-pyrazol-1-yl)propanoic acid	Treatment of Stargardt's disease	TMC Pharma (EU) Limited	21 March 2019	24 April 2019
4-hydroxy-6-{2-[4-(trifluoromethyl)phenyl]ethyl}pyridazin-3(2H)-one	Treatment of Friedreich’s ataxia	Takeda Pharma A/S	21 February 2019	1 April 2019
Adeno-associated viral vector serotype rh10 containing the human cholesterol 24-hydroxylase gene	Treatment of Huntington's disease	Brainvectis	21 February 2019	1 April 2019
Autologous human bone marrow-derived haemaetopoietic and mesenchymal stem cells	Treatment of spinal cord injury	Neuroplast B.V.	21 March 2019	24 April 2019

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
depleted of erythrocytes, monocytes and lymphocytes				
Balipodect	Treatment of fragile X syndrome	Takeda Pharma A/S	21 March 2019	24 April 2019
Codon-optimised human cystic fibrosis transmembrane conductance regulator messenger ribonucleic acid complexed with lipid-based nanoparticles	Treatment of cystic fibrosis	Real Regulatory Limited	21 February 2019	1 April 2019
Human culture expanded autologous mesenchymal stromal cells	Treatment of amyotrophic lateral sclerosis	IQVIA RDS Ireland Limited	21 March 2019	24 April 2019
Marzeptacog alfa (activated)	Treatment of haemophilia B	Voisin Consulting S.A.R.L.	21 February 2019	1 April 2019
Modified messenger ribonucleic acid encoding human propionyl-coenzyme A carboxylase alpha and beta subunits encapsulated into lipid nanoparticle	Treatment of propionic acidaemia	Pharma Gateway AB	21 March 2019	24 April 2019
Sodium benzoate, sodium phenylacetate	Treatment of ornithine transcarbamylase deficiency	Dipharma B.V.	21 March 2019	24 April 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
Pretomanid	Treatment of tuberculosis	FGK Representative Service GmbH	EU/3/07/513
Lenalidomide	Treatment of marginal zone lymphoma	Celgene Europe BV	EU/3/15/1473
Lenalidomide	Treatment of follicular lymphoma	Celgene Europe BV	EU/3/12/1097
Pexidartinib	Treatment of tenosynovial giant cell tumour, localised and diffuse type	Daiichi Sankyo Europe GmbH	EU/3/15/1457