2 June 2020
EMA/COMP/284050/2020
Human Medicines Division

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

May 2020

The Committee for Orphan Medicinal Products held its 222nd plenary meeting on 18-20 May 2020.

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:

- (+)-epicatechin for treatment of Becker muscular dystrophy, MWB Consulting S.A.R.L.;
- Lys^{40}(NODAGA-^{68}Ga)NH_2-exendin-4 for diagnosis of insulinoma, Stichting Katholieke Universiteit;
- Magrolimab for treatment of myelodysplastic syndromes, Granzer Regulatory Consulting & Services;
- Anti-CD123 IgG1 humanised monoclonal antibody conjugated to N1-(2-(2,5-dioxo-2,5-dihydro-1H-pyrrol-1-yl)ethyl)-N6-((S)-1-(((S)-1-((3-((((S)-8-methoxy-6-oxo-11,12,12a,13-tetrahydro-6H-benzo[5,6][1,4]diazepino[1,2-a]indol-9-yl)oxy)methyl)-5-((((S)-8-methoxy-6-oxo-12a,13-dihydro-6Hbenzo[5,6][1,4]diazepino[1,2-a]indol-9-yl)oxy)methyl)phenyl)amino)-1-oxopropan-2-yl)amino)-1-oxopropan-2-yl)adipamide for treatment of blastic plasmacytoid dendritic cell neoplasm, ImmunoGen BioPharma (Ireland) Limited;
- Sodium cromoglicate for treatment of idiopathic pulmonary fibrosis, IQVIA RDS Spain S.L.

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2. Opinions adopted at the first COMP discussion:

- Adeno-associated virus serotype HSC15 expressing human arylsulfatase A gene for treatment of metachromatic leukodystrophy, YES Pharmaceutical Development Services GmbH;
- Axicabtagene ciloleucel for treatment of marginal zone lymphoma, Kite Pharma EU B.V.;
- Nomacopan for treatment of bullous pemphigoid, Akari Malta Limited;
- Onfekafusp alfa for treatment of glioma, Philogen S.p.A.;
- Stiripentol for treatment of primary hyperoxaluria, Biocodex S.A.S..

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 10 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

One oral hearing took place.

Withdrawals of applications for orphan medicinal product designation

None

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:

- Daurismo (glasdegib) for treatment of acute myeloid leukaemia, Pfizer Europe MA EEIG (EU/3/17/1923);
- Reblozyl (luspatercept) for the treatment of beta-thalassaemia intermedia and major, Celgene Europe BV (EU/3/14/1300);
- Reblozyl (luspatercept) for the treatment of myelodysplastic syndromes, Celgene Europe BV (EMA/OD/048/14).

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 223rd meeting of the COMP will be held on 16-18 June 2020.

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
Adeno-associated virus vector serotype hu37 encoding human factor VIII	Treatment of haemophilia A	Bayer AG	19 March 2020	22 April 2020
Anti-(integrin beta-3) human monoclonal antibody	Prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility	FGK Representative Service GmbH	19 March 2020	22 April 2020
Glucagon analogue linked to a human immunoglobulin Fc fragment	Treatment of insulin autoimmune syndrome	JVM Europe B.V.	19 March 2020	22 April 2020
Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R, 4R)-N1-(2-benzyl-7-(2-methyl-2H-tetrazol-5-yl)-9H-pyrimido[4,5-b]indol-4-yl)cyclohexane-1,4-diamine	Treatment in haematopoietic stem cell transplantation	CATS Consultants GmbH	19 March 2020	22 April 2020

dihydrobromide dihydrate				
Mitapivat sulfate	Treatment of pyruvate kinase deficiency	Agios Netherlands B.V.	19 March 2020	22 April 2020
Rozanolixizumab	Treatment of myasthenia gravis	UCB Pharma	19 March 2020	22 April 2020

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
Cannabidiol	Treatment of Dravet syndrome	GW Pharma (International) B.V.	EU/3/14/1339
Cannabidiol	Treatment of Lennox-Gastaut syndrome	GW Pharma (International) B.V.	EU/3/17/1855
Lonafarnib	Treatment of Hutchinson-Gilford Progeria Syndrome	EigerBio Europe Limited	EU/3/18/2118
Lumasiran	Treatment of primary hyperoxaluria type 1	Alnylam Netherlands B.V.	EU/3/16/1637