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Human Medicines Division

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

June 2020

The Committee for Orphan Medicinal Products held its 223rd plenary meeting on 16-18 June 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:

- Adeno-associated viral vector expressing acid alpha-glucosidase gene for treatment of glycogen storage disease type II (Pompe's disease), Audentes Therapeutics Netherlands B.V.;
- Pegylated adrenomedullin for treatment of acute respiratory distress syndrome, Bayer AG;
- Triheptanoin for treatment of carnitine acylcarnitine translocase deficiency, Ultragenyx Germany GmbH.

2. Opinions adopted at the first COMP discussion:

- C-type natriuretic peptide conjugated to multi-arm polyethylene glycol carrier through a cleavable linker for treatment of achondroplasia, Ascendis Pharma Growth Disorders A/S;
- Fasudil hydrochloride for treatment of non-traumatic subarachnoid haemorrhage, Aneuryst (Ireland) Limited;
- Hemopexin, human for treatment of sickle cell disease, CSL Behring GmbH;
- Imetelstat sodium for treatment of myelodysplastic syndromes, Parexel International GmbH.
- Maralixibat chloride for treatment of biliary atresia, Granzer Regulatory Consulting & Services;

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- Retinol palmitate for prevention of bronchopulmonary dysplasia; Provepharm S.A.S;
- Tinostamustine for treatment of T-cell prolymphocytic leukaemia, Mundipharma Corporation (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 adopted following the sponsor's response to the COMP list of questions:

None

2. Opinion following appeal procedures:

- Benzyl benzoate, beta-caryophyllene, cineole, cinnamaldehyde, cinnamyl acetate, linalool, trans-2-methoxycinnamaldehyde for treatment of eumycetoma, Septeos S.A.S.;
- Melatonin, treatment of intracerebral haemorrhage, Worphmed S.r.l..

Lists of questions

The COMP adopted 14 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

8 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 8 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)

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

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:

- Hepcludex (bulevirtide) for treatment of hepatitis delta virus infection, MYR GmbH (EU/3/15/1500). The opinion was adopted by written procedure after the May 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 224th meeting of the COMP will be held on 14-16 July 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
(4-{(2S,4S)-4-ethoxy-1-[(5-methoxy-7-methyl-1H-indol-4-yl)methyl]piperidin-2-yl}benzoic acid-hydrogen chloride(1/1))	Treatment of paroxysmal nocturnal haemoglobinuria	Novartis Europharm Limited	23 April 2020	04 June 2020
Adeno-associated virus serotype 9 containing the human ASPA gene	Treatment of Canavan disease	Raremoon Consulting Limited	23 April 2020	04 June 2020
Autologous CD4+ and CD8+ T cells transduced with a lentiviral vector encoding an affinity enhanced T cell receptor specific to MAGE-A4	Treatment of soft tissue sarcoma	Adaptimmune Limited	23 April 2020	04 June 2020
Autologous T lymphocyte-enriched population of cells transduced with a lentiviral vector encoding a chimeric antigen receptor targeting human B cell maturation	Treatment of multiple myeloma	FGK Representative Service GmbH	23 April 2020	04 June 2020

antigen with 4-1BB and CD3-zeta intracellular signalling domains				
Ile-Ala-Leu-Ile-Leu-Glu-Pro-Ile-Cys-Cys-Gln-Glu-Arg-Ala-Ala-(discrete-polyethylene glycol)24	Treatment of neonatal encephalopathy	Clinipace GmbH	23 April 2020	04 June 2020
Lumacaftor	Treatment of non-traumatic subarachnoid haemorrhage	Qanatpharma GmbH	23 April 2020	04 June 2020
Lutetium (^{177}Lu) lilotomab satetraxetan	Treatment of marginal zone lymphoma	Nordic Nanovector ASA	23 April 2020	04 June 2020
Methotrexate	Treatment of retinal detachment	Helio Vision Germany GmbH	23 April 2020	04 June 2020
Rilzabrutinib	Treatment of immune thrombocytopenia	Clinical Network Services (NL) B.V.	23 April 2020	04 June 2020
Sodium phenylbutyrate, tauroursodeoxycholic acid	Treatment of amyotrophic lateral sclerosis	Drug Development and Regulation S.L.	23 April 2020	04 June 2020
Viltolarsen	Treatment of Duchenne muscular dystrophy	Medpace Finland Oy	23 April 2020	04 June 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
idecabtagene vicleucel	Treatment of multiple myeloma	Celgene Europe BV	EU/3/17/1863
tafasitamab	Treatment of diffuse large B-cell lymphoma	Morphosys AG	EU/3/14/1424