18 December 2020
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Human Medicines Division

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

December 2020

The Committee for Orphan Medicinal Products held its 228th plenary meeting on 1-3 December 2020.

Orphan medicinal product designation

Positive opinions

The COMP adopted 12 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:

- Calcium oxybate, magnesium oxybate, potassium oxybate, sodium oxybate for treatment of idiopathic hypersomnia, Jazz Pharmaceuticals Ireland Limited;
- Human laminin-111, recombinant for treatment of congenital muscular dystrophy, Maxia Strategies-Europe Limited;
- Rilonacept for treatment of idiopathic pericarditis, Granzer Regulatory Consulting & Services;
- Setanaxib for treatment of primary biliary cholangitis, GenKyoTex S.A.

2. Opinions adopted at the first COMP discussion:

- Alpha galactosidase A for treatment of Fabry disease, Consejo Superior De Investigaciones Cientificas;
- Celecoxib, ciprofloxacin for treatment of amyotrophic lateral sclerosis, Morrison & Foerster;
- Cyclo-L-glycyl-L-2-allylproline for treatment of Angelman syndrome, Dlrc Pharma Services Limited;
- Cyclo-L-glycyl-L-2-allylproline for treatment of Phelan-McDermid syndrome, Dlrc Pharma Services Limited;

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- Cyclo-L-glycyl-L-2-allylproline for treatment of Pitt-Hopkins syndrome, Dlrc Pharma Services Limited;
- Humanised IgG1K monoclonal antibody against interferon beta for treatment of dermatomyositis, Pfizer Europe MA EEIG;
- Idursulfase for treatment of mucopolysaccharidosis type II (Hunter's syndrome), Shire Pharmaceuticals Ireland Limited;
- Rezafungin acetate for treatment of invasive candidiasis, 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:

None

Lists of questions

The COMP adopted 18 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

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.

[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:

- Blincyto (blinatumomab) for treatment of acute lymphoblastic leukaemia, Amgen Europe B.V. (EU/3/09/650).
- Elzonris (tagraxofusp) for treatment of blastic plasmacytoid dendritic cell neoplasm, TMC Pharma (EU) Limited (EU/3/15/1567). The opinion was adopted by written procedure after the November 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 229th meeting of the COMP will be held on 19-21 January 2021.

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

Enquiries to: AskEMA (https://www.ema.europa.eu/en/about-us/contact/send-question-european-medicines-agency)

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
(R)-tetrahydrofuran-3-yl 4-(6-(5-(4-ethoxy-1-isopropylpiperidin-4-yl)pyridin-2-yl)pyrrolo[1,2-b]pyridazin-4-yl)piperazine-1-carboxylate sesquisuccinate	Treatment of fibrodysplasia ossificans progressiva	Ipsen Pharma	08 October 2020	13 November 2020
3,5-diamino-6-chloro-N-(N-(4-(4-(2-(hexyl((2S,3R,4R,5R)-2,3,4,5,6-pentahydroxyhexyl)amino)ethoxy)phenyl)butyl)-carbamimidoyl)pyrazine-2-carboxamide, Sodium chloride solution 4.2% (w/v)	Treatment of primary ciliary dyskinesia	EUDRAC GmbH	08 October 2020	16 November 2020
Adeno-associated viral vector serotype 9 expressing codon-optimized human *GRN* gene	Treatment of frontotemporal dementia	PPD Bulgaria EOOD	08 October 2020	13 November 2020
Alisitol, retinol palmitate, zinc gluconate	Treatment of microvillus inclusion disease	Vanessa Research Magyarorszag Kft.	08 October 2020	13 November 2020
Anti-(pancreatic adenocarcinoma upregulated factor) IgG1 humanised monoclonal antibody	Treatment of pancreatic cancer	Prestige Biopharma Belgium	08 October 2020	13 November 2020
DNA plasmid encoding human transferrin gene	Treatment of retinitis pigmentosa	Eyevensys S.A.S.	08 October 2020	13 November 2020

L-pyroglutamyl-L-asparaginyl-L-prolyl-D-tyrosyl-D-tryptophan amide	Treatment of amyotrophic lateral sclerosis	Neuropath Therapeutics Limited	08 October 2020	13 November 2020
Tislelizumab	Treatment of oesophageal cancer	BeiGene Ireland Limited	08 October 2020	13 November 2020
Triheptanoin	Treatment of carnitine palmitoyltransferase I deficiency	Ultragenyx Germany GmbH	08 October 2020	13 November 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
arimoclomol	Treatment of Niemann-Pick disease, type C	Orphazyme A/S	EU/3/14/1376
avacopan	Treatment of granulomatosis with polyangiitis	Vifor Fresenius Medical Care Renal Pharma France	EU/3/14/1373
maralixibat	Treatment of progressive familial intrahepatic cholestasis	FGK Representative Service GmbH	EU/3/13/1216
odevixibat	Treatment of progressive familial intrahepatic cholestasis	Albireo	EU/3/12/1028