24 February 2021
EMA/COMP/109507/2021
Human Medicines Division

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

February 2021

The Committee for Orphan Medicinal Products held its 230th plenary meeting on 16-18 February 2021.

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 virus serotype hu68 containing the human *GALC* gene for treatment of Krabbe disease, Pharma Gateway AB;
- Ilixadencel for treatment of gastrointestinal stromal tumours, Immunicum AB;
- Lorcaserin hydrochloride for treatment of Dravet syndrome, Premier Research Group S.L.;
- Messenger RNA encoding Cas9, single guide RNA targeting the human *TTR* gene for treatment of ATTR amyloidosis, Voisin Consulting S.A.R.L..

2. Opinions adopted at the first COMP discussion:

- 5-fluoro-4-(7'-fluoro-2'-methylspiro[cyclopentane-1,3'-indol]-5'-yl)-N-(5-(1-methylpiperidin-4-yl)pyridin-2-yl)pyrimidin-2-amine for treatment of glioma, Rapport Global Strategic Services Ireland Limited;
- Alpelisib for treatment of PIK3CA-related overgrowth spectrum, Novartis Europharm Limited;
- Alpha-L-iduronidase fused to Fab fragment of a humanised monoclonal antibody targeting human transferrin receptor for treatment of mucopolysaccharidosis type I, Artemida Pharma Europe Limited;

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

- Herpes simplex virus 1 expressing the human *CFTR* gene for treatment of cystic fibrosis, IDEA Innovative Drug European Associates (Ireland) Limited;
- Lefitolimod for treatment of small cell lung cancer, Molecular Biology And Integral Biomathics;
- Vatiquinone for treatment of Friedreich's ataxia, Ptc Therapeutics International 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 8 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

6 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 3 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

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

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:

- Pemazyre (pemigatinib) for treatment of biliary tract cancer, Incyte Biosciences Distribution B.V. (EU/3/18/2066). The opinion was adopted by written procedure after the January 2021 meeting.
- Sogroya (somapacitan) for treatment of growth hormone deficiency, Novo Nordisk A/S (EU/3/18/2068).

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 231st meeting of the COMP will be held on 16-18 March 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
None				

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
voxelotor	Treatment of sickle cell disease	Global Blood Therapeutics Netherlands B.V.	EU/3/16/1769