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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

October 2019

The Committee for Orphan Medicinal Products held its 215th plenary meeting on 8-10 October 2019.

Orphan medicinal product designation

Positive opinions

The COMP adopted 8 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:

- 4-((E)-(5-(2-(2-((S)-2-((S)-1-(L-threonyl-L-lysyl)pyrrolidine-2-carboxamido)-5-guanidinopentanamido)acetamido)-2-carboxyethyl)-2-hydroxyphenyl)diazenyl)phenyl (2-(trimethylammonio)ethyl) phosphate for treatment of non-infectious uveitis, Granzer Regulatory Consulting & Services;
- Camsirubicin for treatment of soft tissue sarcoma, Monopar Therapeutics S.A.R.L;
- Ganaxolone for treatment of CDKL5 deficiency disorder, Pharma Gateway AB;
- Replication-incompetent, non-integrating, herpes simplex virus 1 vector expressing the human transglutaminase-1 enzyme for treatment of autosomal recessive congenital ichthyosis, IDEA Innovative Drug European Associates (Ireland) Limited.

2. Opinions adopted at the first COMP discussion:

- (2S,3R,4R,5S)-2-(hydroxymethyl)-1-pentylpiperidine-3,4,5-triol for treatment of GM2 gangliosidosis, Idorsia Pharmaceuticals Deutschland GmbH;
- Autologous peripheral blood T cells CD4 and CD8 selected and CD3 and CD28 activated transduced with retroviral vector expressing anti CD19 CD28/CD3-zeta chimeric antigen receptor and cultured for treatment of mantle cell lymphoma, Kite Pharma EU B.V.;

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- Chimeric fibril-reactive IgG1k monoclonal antibody 11-1F4 for treatment of AL amyloidosis, Real Regulatory Limited;
- Exendin (9-39) for treatment of congenital hyperinsulinism, Eigerbio Europe 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 meeting prior to the adoption of an opinion.

Oral hearings

5 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:

- Xospata (gilteritinib) for treatment of acute myeloid leukaemia, Astellas Pharma Europe B.V. (EU/3/17/1961). The opinion was adopted by written procedure after the September 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 216th meeting of the COMP will be held on 5-7 November 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

No new designations were granted by the European Commission since last COMP plenary meeting

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
Satralizumab	Treatment of neuromyelitis optica spectrum disorders	Roche Registration GmbH	EU/3/16/1680
Avapritinib	Treatment of gastrointestinal stromal tumours	Blueprint Medicines (Netherlands) B.V.	EU/3/17/1889
Somapacitan	Treatment of growth hormone deficiency	Novo Nordisk A/S	EU/3/18/2068