14 September 2021
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Human Medicines Division

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

September 2021

The Committee for Orphan Medicinal Products held its 236th plenary meeting on 7-9 September 2021.

Orphan medicinal product designation

Positive opinions

The COMP adopted 17 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 of serotype rh10 encoding human *MLC1* under the control of GFAP promoter for treatment of megalencephalic leukoencephalopathy with subcortical cysts, Consorcio Centro de Investigación Biomédica en Red, M.P.;
- Devimistat for treatment of Burkitt's lymphoma, IQVIA RDS Ireland Limited;
- Ganaxolone for treatment of tuberous sclerosis, Marinus Pharmaceuticals Emerald Limited;
- Human IgG1 monoclonal antibody against sortilin for treatment of frontotemporal dementia, Pharma Gateway AB.

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 5 expressing the human cone-rod homeobox gene for treatment of cone-rod dystrophy, Variant;
- Adeno-associated virus serotype rh10 containing the human *GALC* gene for treatment of Krabbe disease, Diamond Pharma Services Ireland Limited;

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- Allogeneic retinal pigment epithelial cells genetically modified with a non-viral vector to express human alpha-L-iduronidase for treatment of mucopolysaccharidosis type I, TMC Pharma (EU) Limited;
- Allogeneic umbilical cord mesenchymal cells-derived extracellular vesicles for treatment of bronchopulmonary dysplasia, Exo Biologics;
- Autologous haematopoietic stem and progenitor cell population containing CD34+ cells transduced with a lentiviral vector encoding the *TCIRG1* cDNA ex vivo expanded for treatment of osteopetrosis, Fondazione Telethon;
- Batiraxcept for treatment of ovarian cancer, Kinesys Consulting NL B.V.;
- Encaleret sulfate for treatment of hypoparathyroidism, Voisin Consulting Life Sciences;
- Glofitamab for treatment of diffuse large B-cell lymphoma, Roche Registration GmbH;
- Humanised IgG1 monoclonal antibody against SEZ6 linked to N-acetyl-calicheamicin for treatment of small cell lung cancer, AbbVie Deutschland GmbH & Co. KG;
- Idursulfase beta for treatment of mucopolysaccharidosis type II (Hunter's syndrome), Parexel International (Irl) Limited;
- Mocravimod for treatment in haematopoietic stem cell transplantation, Priothera;
- Psilocybine for treatment of fragile X syndrome, Comac Medical Ltd.;
- Selinexor for treatment of glioma, Karyopharm Europe GmbH.

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:

- Enterovirus b, echovirus 7, live for treatment of uveal melanoma, Latima SIA. The opinion was adopted by written procedure after the September meeting.

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 11 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to the adoption of an opinion.

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

Oral hearings

8 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 7 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 medicinal products for which decisions on orphan designation have been granted by the European Commission is provided in Community 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:

None

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 1.

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 237th meeting of the COMP will be held on 5-7 October 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

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
asciminib	Treatment of chronic myeloid leukaemia	Novartis Europharm Limited	EU/3/20/2261
axicabtagene ciloleucel	Treatment of primary mediastinal large B-cell lymphoma	Kite Pharma EU B.V.	EU/3/15/1553
axicabtagene ciloleucel	Treatment of follicular lymphoma	Kite Pharma EU B.V.	EU/3/15/1579
axicabtagene ciloleucel	Treatment of diffuse large B cell lymphoma	Kite Pharma EU B.V.	EU/3/14/1393
efgartigimod alfa	Treatment of myasthenia gravis	Argenx	EU/3/18/1992
mitapivat	Treatment of pyruvate kinase deficiency	Agios Netherlands B.V.	EU/3/20/2270
octreotide	Treatment of acromegaly	FGK Representative Service GmbH	EU/3/13/1170
tebentafusp	Treatment of uveal melanoma	Immunocore Ireland Limited	EU/3/21/2397