20 May 2021
EMA/COMP/279124/2021
Human Medicines Division

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

May 2021

The Committee for Orphan Medicinal Products held its 233rd plenary meeting on 10-12 May 2021.

Orphan medicinal product designation

Positive opinions

The COMP adopted 14 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:

- Bomedemstat ditosilate for treatment of essential thrombocythaemia, Imago Biosciences B.V.;
- Imatinib for treatment of pulmonary arterial hypertension, MDC RegAffairs GmbH;
- L-ergothioneine for treatment of cystinuria, Consorcio Centro de Investigación Biomédica en Red, M.P.;
- Tislelizumab for treatment of nasopharyngeal cancer, BeiGene Ireland Limited.

2. Opinions adopted at the first COMP discussion:

- (S)-5-amino-3-(4-((5-fluoro-2-methoxybenzamido)methyl)phenyl)-1-(1,1,1-trifluoropropane-2-yl)-1H-pyrazole-4-carboxamide for treatment of mantle cell lymphoma, Eli Lilly Nederland B.V.;
- Adeno-associated viral vector LK03 encoding human methylmalonyl-CoA mutase for treatment of methylmalonic acidaemia, Parexel International (Irl) Limited;
- Adeno-associated virus serotype 9 containing the human *FXN* gene isoform 1 for treatment of Friedreich's ataxia, Novartis Gene Therapies EU 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
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- Adeno-associated virus serotype 9 expressing human CLN5 for treatment of neuronal ceroid lipofuscinosis, Real Regulatory Limited;
- Allogenic placenta-derived mesenchymal stromal cells secretome for treatment of pre-eclampsia, Corion Biotech S.r.l.;
- H-D-valyl1-D-alanyl-D-glutamyl-D-alanyl-D-arginyl5-D-glutamyl-D-glutamyl-D-leucyl-D-glutamyl-D-arginyl10-D-leucyl-D-glutamyl-D-alanyl-D-arginyl-D-leucyl15-glycyl-D-glutaminyl-D-alanyl-D-arginyl-glycyl20-D-glutamyl-D-leucyl-D-lysyl-D-lysyl-D-tryptophyl25-D-lysyl-D-methionyl-D-arginyl-D-arginyl-D-asparaginyl30-D-glutaminyl-D-phenylalanyl-D-tryptophyl-D-leucyl-D-lysyl35-D-leucyl-D-glutaminyl-D-arginine for treatment of glioma, Sapience Therapeutics Limited;
- Humanised IgG2 monoclonal antibody against TNFSF13 for treatment of primary IgA nephropathy, Otsuka Pharmaceutical Netherlands B.V.;
- Humanised monoclonal antibody derivative against fibroblast growth factor receptor 3 for treatment of achondroplasia, Genzyme Europe B.V.;
- Hydrocortisone hydrogen succinate for treatment of bronchopulmonary dysplasia, Laboratoire Aguettant;
- Melatonin for prevention of retinopathy of prematurity, Worphmed S.r.l.

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 11 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

4 oral hearings took place.

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

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 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:

- Enspryng (satralizumab) for treatment of neuromyelitis optica spectrum disorders, Roche Registration GmbH (EU/3/16/1680). The opinion was adopted by written procedure after the April meeting.
- Koselugo (selumetinib) for treatment of neurofibromatosis type 1, AstraZeneca AB (EU/3/18/2050). The opinion was adopted by written procedure after the April 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 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 234th meeting of the COMP will be held on 15-17 June 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
betulae cortex dry extract (5-10 : 1); extraction solvent: n-heptane 95% (w/w)	Treatment of epidermolysis bullusa	Amryt Pharmaceuticals DAC	EU/3/10/845