



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

20 July 2022
EMA/COMP/646272/2022
Human Medicines Division

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

July 2022

The Committee for Orphan Medicinal Products held its 246th plenary meeting on 12-14 July 2022.

Orphan medicinal product designation

Positive opinions

The COMP adopted 21 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the first and second COMP discussion, following the sponsor's response to the COMP list of questions:

- (7S)-8,8-dimethyl-7-[[[(2E)-3-phenyl-2-propen-1-yl]oxy]-7,8-dihydro-2H,6H-pyrano[3,2-g]chromen-2-on for treatment of Hutchinson-Gilford progeria syndrome, Global Medical Services Sp. z o.o.;
- Allogeneic adult liver-derived stem cells for treatment of urea cycle disorders , Unicyte S.r.l.;
- Allogeneic placenta-derived decidual stromal cells for treatment of graft-versus-host-disease, MDC RegAffairs GmbH;
- Amphotericin B for treatment of cryptococcosis, Insight Drug Regulatory;
- Autologous CD34+ haematopoietic stem and progenitor cells genetically modified with a lentiviral vector encoding for the N-acetylgalactosamine 6-sulfatase cDNA for treatment of mucopolysaccharidosis type IV A, (Morquio A syndrome), Fondazione Telethon;
- Autologous human bone marrow-derived haematopoietic and mesenchymal stem cells depleted of erythrocytes, monocytes and lymphocytes for treatment of frontotemporal dementia, Neuroplast B.V.;
- Batoclimab for treatment of myasthenia gravis, Pharma Gateway AB;
- Bezafibrate for treatment of primary sclerosing cholangitis, Amsterdam UMC;

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- Calmangafodipir for prevention of acute liver failure, Egetis Therapeutics AB;
- Clarithromycin, clofazimine, rifabutin for treatment of nontuberculous mycobacterial lung disease, Regintel Limited;
- Epetraborole for treatment of nontuberculous mycobacterial lung disease, Dirc Pharma Services Limited;
- Gold for treatment of amyotrophic lateral sclerosis, Clene Netherlands B.V.;
- H-Lys-Lys-Gly-Asp-Asn-Ile-Met-Val-Thr-Phe-Arg-Asn-Gln-Ala-Ser-Arg-Pro-Tyr-Gly-Lys-Lys-OH for treatment of haemophilia A, S-Cubed Pharmaceutical Services ApS;
- Humanized IgG1 tetravalent monoclonal antibody against death receptor 5 for treatment of chondrosarcoma, TMC Pharma (EU) Limited;
- Lithium carbonate for treatment of familial adenomatous polyposis, Amsterdam UMC;
- Mazindol for treatment of idiopathic hypersomnia, Propharma Group The Netherlands B.V.;
- Nanatinostat, valganciclovir for treatment of peripheral T-cell lymphoma, Pharma Gateway AB;
- Sotuletinib for treatment of amyotrophic lateral sclerosis, Novartis Europharm Limited;
- Tamibarotene for treatment of myelodysplastic syndrome, Syros Pharmaceuticals (Ireland) Limited;
- Toll-like receptor 4 agonist for treatment of osteosarcoma, Hephaistos-Pharma;
- Vutrisiran for treatment of Stargardt's disease, Alnylam Netherlands B.V.

2. Opinions following appeal procedures:

None

Public summaries of opinions will be available on the [EMA website](#) following adoption of the respective decisions on orphan designation¹ by the European Commission. Please also refer to the Community Register of orphan medicinal products for human use.

Negative opinions

1. Opinion adopted following the sponsor's response to the COMP list of questions:

- Melatonin for treatment of retinopathy of prematurity, Industria Farmaceutica Galenica Senese S.r.l.

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

4 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 list of 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.

Positive opinions

1. Opinions adopted at time of CHMP opinion:

- Roctavian (valoctocogene roxaparvovec) for treatment of haemophilia A, BioMarin International Limited, EU/3/16/1622.
- Scemblix (asciminib) for treatment of chronic myeloid leukaemia, Novartis Europharm Limited, EU/3/20/2261.
- Vyvgart (efgartigimod alfa) for treatment of myasthenia gravis, Argenx, EU/3/18/1992. The opinion was adopted by written procedure after the June meeting.

2. Opinion following appeal procedures:

None

Negative opinions

1. Opinions adopted at time of CHMP opinion:

None

2. Opinions 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

None

Upcoming meetings

- The 247th meeting of the COMP will be held on 6-8 September 2022.

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 ([Send a question to the European Medicines Agency | European Medicines Agency \(europa.eu\)](#))

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
pirtobrutinib	Treatment of mantle cell lymphoma	Eli Lilly Nederland B.V.	EU/3/21/2450
treprostinil diolamine	Treatment of pulmonary arterial hypertension	Ferrer Internacional S.A	EU/3/05/310