EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

13 October 2021
EMA/COMP/576496/2021
Human Medicines Division

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

October 2021

The Committee for Orphan Medicinal Products held its 237th plenary meeting on 5-7 October 2021.

Orphan medicinal product designation

Positive opinions

The COMP adopted 18 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:

- 2-[1-(3-{6-[(1E)-(hydroxyimino)methyl]-5-methyl-4-oxo-7-propyl-3H,4H-pyrrolo[2,1-f][1,2,4]triazin-2-yl}-4-propoxybenzenesulfonyl)piperidin-4-yl]ethyl nitrate for treatment of systemic sclerosis, Topadur Pharma Deutschland GmbH;
- 5-fluoro-4-(4-fluoro-2-methoxyphenyl)-N-{4-[(S-methylsulfonimidoyl)methyl]pyridin-2-yl}pyridin-2-amine for treatment of diffuse large B-cell lymphoma, Vincerx Pharma GmbH;
- Autologous CD34+ enriched cells transduced with a self-inactivating lentiviral vector containing the codon-optimized *RPS19* gene for treatment of Diamond-Blackfan anemia, Consorcio Centro de Investigación Biomédica en Red, M.P.;
- Linerixibat for treatment of primary biliary cholangitis, GlaxoSmithKline (Ireland) Limited;
- Mosunetuzumab for treatment of follicular lymphoma, Roche Registration GmbH;
- Nadunolimab for treatment of pancreatic cancer, Cantargia AB;
- Rebastinib for treatment of ovarian cancer, Deciphera Pharmaceuticals (Netherlands) B.V.;
- Udonitrectag lysine for treatment in solid organ transplantation, Mimetech S.r.l.

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

2. Opinions adopted at the first COMP discussion:

- (14S)-8-[3-(2-{dispiro[2.0.24.13]heptan-7-yl}ethoxy)-1H-pyrazol-1-yl]-12,12-dimethyl-2lamba6-thia-3,9,11,18,23-penta-azatetracyclo[17.3.1.111,14.05,10]tetracosa-1(22),5,7,9,19(23),20-hexaene-2,2,4-trione calcium salt hydrate, deutivacaftor, tezacaftor for treatment of cystic fibrosis, Vertex Pharmaceuticals (Ireland) Limited;
- Adeno-associated viral vector serotype 9 containing the human *SURF1* gene for treatment of Leigh syndrome, Raremoon Consulting Esp S.L.;
- Anti-CD38 IgG4 human monoclonal antibody for treatment of amyotrophic lateral sclerosis, Encefa;
- Cannabidiol, dronabinol for treatment of complex regional pain syndrome, Tetra Bio-Pharma Europe Limited;
- D-lactic acid, glycolic acid for treatment of amyotrophic lateral sclerosis, Neurevo GmbH;
- Fingolimod for treatment of adrenoleukodystrophy, Consorcio Centro de Investigación Biomédica en Red, M.P.;
- Ibrexafungerp for treatment of invasive candidiasis, Dlrc Pharma Services Limited;
- Olverembatinib for treatment of chronic myeloid leukaemia, Ascentage Pharma Europe Limited.
- Paclitaxel, polyoligo(ethylene glycol)methacrylate-co-poly(vinylbenzyldithiodibutyric acid-gemcitabine) for treatment of pancreatic cancer, Karma Oncology B.V.;
- Soticlestat for treatment of Lennox-Gastaut syndrome, Takeda Pharma A/S.

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 meeting prior to the adoption of an opinion.

Oral hearings

7 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 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 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:

- Qinlock (ripretinib) for treatment of gastrointestinal stromal tumours, Deciphera Pharmaceuticals (Netherlands) B.V, EU/3/17/1936.
- Artesunate Amivas (artesunate) for treatment of achondroplasia, Amivas Ireland Ltd, EU/3/20/2251.

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 238th meeting of the COMP will be held on 3-5 November 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
tisagenlecleucel	Treatment of follicular lymphoma	Novartis Europharm Limited	EU/3/21/2464
vutrisiran	Treatment of transthyretin-mediated amyloidosis	Alnylam Netherlands B.V.	EU/3/18/2026