EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Division

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

June 2021

The Committee for Orphan Medicinal Products held its 234th plenary meeting on 15-17 June 2021.

## Orphan medicinal product designation

### Positive opinions

The COMP adopted 22 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:

- Eftansomatropin alfa for treatment of growth hormone deficiency, Parexel International (Irl) Limited;
- Lutetium ($^{177}$Lu) omburtamab barzuxetan for treatment of medulloblastoma, Y-Mabs Therapeutics A/S;
- Melatonin for treatment of non-traumatic spontaneous intracerebral haemorrhage, Worphmed S.r.l.;
- Pridopidine hydrochloride for treatment of amyotrophic lateral sclerosis, Prilenia Therapeutics B.V.;
- Saroglitazar magnesium for treatment of primary biliary cholangitis, Zydus France;
- Sirolimus for treatment of perivascular epithelioid tumours, YES Pharmaceutical Development Services GmbH;
- Tisagenlecleucel for treatment of follicular lymphoma, Novartis Europharm Limited.

2. Opinions adopted at the first COMP discussion:

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- 1-(4-(6-chloropyridazin-3-yl)piperazin-1-yl)-2-(4-cyclopropyl-3-fluorophenyl)ethan-1-one for treatment of pantothenate kinase-associated neurodegeneration, Premier Research Group S.L.;
- 1-(4-(6-chloropyridazin-3-yl)piperazin-1-yl)-2-(4-cyclopropyl-3-fluorophenyl)ethan-1-one for treatment of propionic acidaemia, Premier Research Group S.L.;
- 3,5-diiodothyropropionic acid for treatment of Allan-Herndon-Dudley syndrome, Raremoon Consulting Esp S.L.;
- Adeno-associated viral vector serotype 9 containing the human *SLC13A5* gene for treatment of SLC13A5-epileptic encephalopathy deficiencies, Raremoon Consulting Esp S.L.;
- Adeno-associated viral vector serotype Anc80 containing the 3' portion of human *OTOF* gene, adeno-associated viral vector serotype Anc80 containing the 5' portion of human *OTOF* gene for treatment of otoferlin gene-mediated hearing loss, Boyd Consultants Limited;
- Adeno-associated virus vector serotype 1 containing the human *GRN* gene for treatment of frontotemporal dementia, Pharma Gateway AB;
- Autologous CD34+ haematopoietic stem and progenitor cells genetically modified with the lentiviral vector encoding for the human palmitoyl-protein thioesterase 1 gene for treatment of neuronal ceroid lipofuscinosis, University of Padua;
- Humanised IgG2k Fc-modified bispecific monoclonal antibody against CD3 and BCMA for treatment of multiple myeloma, Pfizer Europe MA EEIG;
- Infigratinib for treatment of achondroplasia, YES Pharmaceutical Development Services GmbH;
- Itolizumab for treatment of graft-versus-host disease, Biocon Pharma Ireland Limited;
- mRNA encoding the human glycogen debranching enzyme for treatment of glycogen storage disease type III, Ultragenyx Germany GmbH;
- Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 for treatment of adenosine triphosphate binding cassette transporter protein subfamily C member 6 deficiency, Inozyme Pharma Ireland Limited;
- Synthetic double-stranded siRNA oligonucleotide directed against apolipoprotein C-III mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues for treatment of familial chylomicronaemia syndrome, Pharma Gateway AB;
- Vodobatinib for treatment of chronic myeloid leukaemia, Sun Pharmaceutical Industries Europe B.V.;
- Zanidatamab for treatment of biliary tract cancer, Voisin Consulting.

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.

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

### 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 9 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

3 oral hearings took place.

### Withdrawals of applications for orphan medicinal product designation

The COMP noted that 4 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:

- Bylvay (odevixibat) for treatment of progressive familial intrahepatic cholestasis, Albireo (EU/3/12/1028). The opinion was adopted by written procedure after the May meeting.
- Darzalex (daratumumab) for treatment of AL amyloidosis, Janssen-Cilag International NV (EU/3/18/2020). The opinion was adopted by written procedure after the May meeting.
- IMCIVREE (setmelanotide) for treatment of leptin receptor deficiency, TMC Pharma (EU) Limited (EU/3/18/2101). The opinion was adopted by written procedure after the May meeting.
- IMCIVREE (setmelanotide) for treatment of pro-opiomelanocortin deficiency, TMC Pharma (EU) Limited (EU/3/16/1703). The opinion was adopted by written procedure after the May meeting.

- Skysona (elivaldogene autotemcel) for treatment of adrenoleukodystrophy, bluebird bio (Netherlands) B.V (EU/3/12/1003). The opinion was adopted by written procedure after the May 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 235th meeting of the COMP will be held on 13-15 July 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 |
|---|---|---|---|
| Ciltacabtagene autoleucel | Treatment of multiple myeloma | Janssen-Cilag International NV | EU/3/20/2252 |
| Melphalan flufenamide | Treatment of plasma cell myeloma | Oncopeptides AB | EU/3/15/1463 |
| Omburtamab I-131 | Treatment of neuroblastoma | Y-Mabs Therapeutics A/S | EU/3/17/1839 |
| Palovarotene | Treatment of fibrodysplasia ossificans progressiva | Ipsen Pharma | EU/3/14/1368 |