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Human Medicines Division

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

June 2022

The Committee for Orphan Medicinal Products held its 245th plenary meeting on 14-16 June 2022.

Orphan medicinal product designation

Positive opinions

The COMP adopted 28 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:

- (R)-3-(2,3-dihydroxypropyl)-6-fluoro-5-(2-fluoro-4-iodophenylamino)-8-methylpyrido[2,3-d]pyrimidine-4,7(3H,8H)-dione for treatment of familial adenomatous polyposis, Diamond Pharma Services Ireland Limited;
- (S)-1-(4-(1-(3,4,5-trimethoxyphenyl)-1H-imidazol-4-ylamino)thieno[2,3-d]pyrimidin-2-yl)pyrrolidine-2-carboxamide for treatment of fibrodysplasia ossificans progressive, Biocryst Ireland Limited;
- 2,4,6,7,8,9-hexahydro-4-((2-methylphenyl)methyl)-7-(phenylmethyl)imidazo(1,2-a)pyrido(3,4-e)pyrimidin-5(1H)-one for treatment of glioma, Chimerix IRL Limited;
- 3-(1,3-benzodioxol-5-yl)-5-(3-bromophenyl)-1H-pyrazole for treatment of multiple system atrophy, Teva B.V.;
- Autologous naive regulatory T cells transduced with a lentiviral vector encoding for a chimeric antigen receptor to recognise the HLA-A*02 antigen for treatment in solid organ transplantation, Sangamo Therapeutics France S.A.S.;
- Berzosertib for treatment of small-cell lung cancer, Merck Europe B.V.;
- Efgartigimod alfa for treatment of pemphigus, Argenx;

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- Erlotinib for treatment of pachyonychia congenita, Imagine Institut Des Maladies Genetiques Necker Enfants Malades;
- Fosmanogepix for treatment of invasive aspergillosis, Pfizer Europe MA EEIG;
- Fosmanogepix for treatment of invasive candidiasis, Pfizer Europe MA EEIG;
- Heterologous swine glyco-humanised polyclonal antibody against T lymphocytes for treatment in solid organ transplantation, Xenothera;
- Human decorin fused to the truncated homing peptide CRK for treatment of epidermolysis bullosa, Tampereen Korkeakoulusäätiö Sr;
- Humanised IgG4 monoclonal antibody against active complement component 1, subcomponent s for treatment of autoimmune haemolytic anaemia, Genzyme Europe B.V.
- Hydroquinidine hydrochloride for treatment of Brugada syndrome, Teofarma S.r.l.;
- Liraglutide for treatment of Wolfram syndrome, Pietro Maffei;
- mRNA encoding modified human ornithine transcarbamylase for treatment of ornithine transcarbamylase deficiency, Arcturus Therapeutics Europe B.V.;
- Odronextamab for treatment of diffuse large B-cell lymphoma, Regeneron Ireland Designated Activity Company;
- Odronextamab for treatment of follicular lymphoma, Regeneron Ireland Designated Activity Company;
- Panobinostat for treatment of glioma, Scendea (NL) B.V.;
- Parsaclisib for treatment of autoimmune haemolytic anaemia, Incyte Biosciences Distribution B.V.;
- Pyridoxal 5'-phosphate for treatment of pyridoxal 5'-phosphate homeostasis protein deficiency, Amsterdam UMC;
- *Salmonella enterica*, subsp. *enterica*, serovar *Typhimurium*, strain YS1646, live for treatment of schwannoma, Premier Research Group S.L.;
- Sodium phenylbutyrate for treatment of maple syrup urine disease, Renantos Pharmavertriebsgesellschaft mbH;
- Thiostrepton for treatment of malignant mesothelioma, EMA Regulatory Submissions Expediter Limited;
- Toripalimab for treatment of nasopharyngeal cancer, TMC Pharma (EU) Limited;
- Vilobelimab for treatment of pyoderma gangrenosum, InflaRx GmbH;
- W253R/R275S tissue plasminogen activator for treatment of non-traumatic spontaneous intracerebral haemorrhage, Op2Lysis;
- Zilucoplan for treatment of myasthenia gravis, UCB Pharma.

2. Opinions following appeal procedures:

None

Click or tap here to enter text.

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 opinions

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 10 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

10 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 14 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:

- Crysvita (burosumab) for treatment of phosphaturic mesenchymal tumour, Kyowa Kirin Holdings B.V., EU/3/18/2011. The opinion was adopted by written procedure after the June meeting.
- Kinpeygo (budesonide) for treatment of primary IgA nephropathy, Calliditas Therapeutics AB, EU/3/16/1778. The opinion was adopted by written procedure after the May meeting.

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

- Upstaza (eladocagene exuparvovec) for treatment of aromatic L-amino acid decarboxylase deficiency, PTC Therapeutics International Limited, EU/3/16/1786. The opinion was adopted by written procedure after the May meeting.
- Xenpozyme (olipudase alfa) for treatment of Niemann-Pick disease, Genzyme Europe B.V, EU/3/01/056. The opinion was adopted by written procedure after the May meeting.
- Zokinvy (lonafarnib) for treatment of Hutchinson-Gilford Progeria Syndrome, Eigerbio Europe Limited, EU/3/18/2118. The opinion was adopted by written procedure after the May 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

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 246th meeting of the COMP will be held on 12-14 July 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
glofitamab	Treatment of diffuse large B-cell lymphoma	Roche Registration GmbH	EU/3/21/2497
polihexanide	Treatment of acanthamoeba keratitis	SIFI SPA	EU/3/07/498
futibatinib	Treatment of biliary tract cancer	Taiho Pharma Netherlands B.V.	EU/3/19/2146