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Human Medicines Division

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

March 2022

The Committee for Orphan Medicinal Products held its 242nd plenary meeting on 15-17 March 2022.

Orphan medicinal product designation

Positive opinions

The COMP adopted 13 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:

- (2S)-4-[2-methoxyethyl-[4-(5,6,7,8-tetrahydro-1,8-naphthyridin-2-yl)butyl]amino]-2-(quinazolin-4-ylamino)butanoic acid for treatment of primary sclerosing cholangitis, Pharma Gateway AB;
- Adeno-associated viral vector serotype 8 encoding B-domain deleted liver specific codon optimized bioengineered chimeric human porcine factor VIII, under a synthetic hepatic combinatorial bundle promoter for treatment of haemophilia A, MDC RegAffairs GmbH;
- Adeno-associated virus serotype 9 containing human *MYBPC3* gene for treatment of hypertrophic cardiomyopathy due to mutations in the *MYBPC3* gene encoding cardiac myosin-binding protein C, Yes Pharmaceutical Development Services GmbH;
- Adeno-associated virus vector serotype 9 encoding human gigaxonin gene for treatment of giant axonal neuropathy, Raremoon Consulting Esp S.L.;
- Adeno-associated virus serotype C102 containing the human *GLA* gene for treatment of Fabry disease, Pharma Gateway AB;
- Cannabidiol for treatment of epilepsy with myoclonic-atonic seizures, GW Pharma (International) B.V.;
- Devimistat for treatment of biliary tract cancer, IQVIA RDS Ireland Limited;
- Glofitamab for treatment of mantle cell lymphoma, Roche Registration GmbH;

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- Human papillomavirus type 16-derived empty nanoparticle conjugated to approximately 200 molecules of a phthalocyanine-based photosensitizer for treatment of uveal melanoma, FGK Representative Service GmbH;
- N-(methoxypolyethylene glycol 5000 carbamoyl)-1,2-dipalmitoyl-sn-glycero-3-phosphatidylethanolamine for treatment in solid organ transplantation, Icoat Medical AB;
- Norucholic acid for treatment of primary biliary cholangitis, Dr. Falk Pharma GmbH;
- Obecabtagene autoleucel for treatment of acute lymphoblastic leukaemia, Autolus GmbH;
- Tiratricol for treatment of resistance to thyroid hormone type beta, Rare Thyroid Therapeutics International AB;

2. 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 13 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

No oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that no 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.

[1] Details of all orphan designations granted to date by the European Commission are entered in the EU 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:

None

2. Opinion following appeal procedures:

None

Negative opinions

1. Opinions adopted at time of CHMP opinion:

None

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 243rd meeting of the COMP will be held on 11-13 April 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 (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
Teclistamab	Treatment of multiple myeloma	Janssen-Cilag International N.V.	EU/3/20/2331
Sodium phenylbutyrate/ ursodoxicoltaurine	Treatment of amyotrophic lateral sclerosis	Amylyx Pharmaceuticals EMEA	EU/3/20/2284
Pegunigalsidase alfa	Treatment of Fabry disease	Chiesi Farmaceutici S.p.A.	EU/3/17/1953