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Human Medicines Division

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

July 2021

The Committee for Orphan Medicinal Products held its 235th plenary meeting on 13-15 July 2021.

Orphan medicinal product designation

Positive opinions

The COMP adopted 17 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:

- Adeno-associated viral vector serotype 9 containing the human *HEXA* and *HEXB* genes for treatment of GM2 gangliosidosis, Raremoon Consulting Esp S.L.;
- Humanised IgG1 monoclonal antibody against TfR1 conjugated to double stranded siRNA oligonucleotide against DMPK via a non-cleavable linker for treatment of myotonic disorders, MWB Consulting S.A.R.L.;
- Loncastuximab tesirine for treatment of diffuse large B-cell lymphoma, FGK Representative Service GmbH.

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 9 containing the human *MECP2* gene for treatment of Rett syndrome, Raremoon Consulting Esp S.L.;
- Adeno-associated viral vector serotype S3 containing codon-optimised expression cassette encoding human beta-glucocerebrosidase variant for treatment of Gaucher disease, Freeline Therapeutics (Ireland) Limited;

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- Adeno-associated virus serotype 9 encoding human *NGLY*1 gene for treatment of NGLY1 deficiency, Voisin Consulting;
- Adeno-associated virus serotype PTC3 expressing the human *UBE3A* gene for treatment of Angelman syndrome, PTC Therapeutics International Limited;
- Autologous CD34+ cell enriched population containing haematopoietic stem and progenitor cells transduced ex vivo with a lentiviral vector encoding the human *ADA2* gene for treatment of adenosine deaminase 2 deficiency (DADA2), Fondazione Telethon;
- Autologous CD34+ cells transfected with a lentiviral vector containing codon optimised *RPS19* gene for treatment of Diamond-Blackfan anaemia, Premier Research Group S.L.;
- Autologous CD34+ haematopoietic stem and progenitor cells genetically modified with the lentiviral vector encoding for the human iduronate 2-sulfatase gene for treatment of mucopolysaccharidosis type II (Hunter's syndrome), University Of Padua;
- Bardoxolone methyl for treatment of autosomal dominant polycystic kidney disease, Pharma Gateway AB;
- Cemdisiran for treatment of primary IgA nephropathy, Alnylam Netherlands B.V.;
- Human keratinocytes for treatment of partial deep dermal and full thickness burns, Dizg Deutsches Institut Für Zell- Und Gewebeersatz gGmbH;
- Lurbinectedin for treatment of malignant mesothelioma, Pharma Mar S.A.;
- Recombinant human apolipoprotein A-I for treatment of lecithin-cholesterol acyltransferase deficiency, Abionyx Pharma;
- Sabatolimab for treatment of myelodysplastic syndromes, Novartis Europharm Limited;
- Talquetamab for treatment of multiple myeloma, Janssen-Cilag International N.V.

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

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

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 12 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

5 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 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:

- Abecma (idecabtagene vicleucel) for treatment of multiple myeloma, Celgene Europe BV (EU/3/17/1863). The opinion was adopted by written procedure after the June meeting.
- Minjuvi (tafasitamab) for treatment of diffuse large B-cell lymphoma, Incyte Biosciences Distribution B.V. (EU/3/14/1424).
- Voxzogo (vosoritide) for treatment of achondroplasia, BioMarin International Limited (EU/3/12/1094). The opinion was adopted by written procedure after the June 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 236th meeting of the COMP will be held on 7-9 September 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
Budesonide, micronised	Treatment of primary IgA nephropathy	Calliditas Therapeutics AB	EU/3/16/1778
Copanlisib	Treatment of marginal zone lymphoma	Bayer AG	EU/3/18/2064
Maribavir	Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity	Shire Pharmaceuticals Ireland Limited	EU/3/13/1133
Maribavir	Prevention of cytomegalovirus disease in patients with impaired cell mediated immunity deemed at risk	Shire Pharmaceuticals Ireland Limited	EU/3/07/519