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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

May 2019

The Committee for Orphan Medicinal Products held its 211th plenary meeting on 21-23 May 2019.

Orphan medicinal product designation

Positive opinions

The COMP adopted 12 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:

- (S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidine-3-yl)chroman-2-carboxamide hydrochloride for treatment of maternally-inherited diabetes and deafness, Khondrion B.V.;
- Gaboxadol monohydrate for treatment of Angelman syndrome, FGK Representative Service GmbH;
- Recombinant human coagulation factor VIII Fc - von Willebrand factor - XTEN fusion protein for treatment of haemophilia A, Swedish Orphan Biovitrum AB (publ).

2. Opinions adopted at the first COMP discussion:

- Reldesemtiv for treatment of spinal muscular atrophy, Pharma Gateway AB;
- 2-(2-{[2-(1H-benzimidazol-2-yl)ethyl]amino}ethyl)-N-[(3-fluoropyridine-2-yl)methyl]-1,3-oxazole-4-carboxamide trihydrochloride for treatment of beta-thalassaemia intermedia and major, Vifor France S.A.;
- 5'-cEtG-sp-cEt5MeU-sp-cEt5MeU-sp-dT-sp-dA-sp-dT-sp-dT-sp-dA-sp-dT-sp-dA-sp-dG-sp-dG-sp-dG-sp-cEt5MeC-sp-cEt5MeU-sp-cEt5MeU-3' for treatment of centronuclear myopathies, Dynacure S.A.S.;

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- Imidazolyl ethanamide pentandioic acid for treatment of acute radiation syndrome, Myelo Therapeutics GmbH;
- Rasagiline for treatment of Duchenne muscular dystrophy, TMC Pharma (EU) Limited;
- Recombinant adeno-associated viral vector containing a bioengineered capsid serotype AAV-Rh74 and a codon-optimised expression cassette to drive the expression of a secretable form of human acid alpha-glucosidase for treatment of glycogen storage disease type II (Pompe's disease), Spark Therapeutics Ireland Limited;
- Regorafenib for treatment of glioma, Bayer AG;
- Sodium benzoate, sodium phenylacetate for treatment of argininosuccinic aciduria, Dipharma B.V.;
- Sodium benzoate, sodium phenylacetate for treatment of hyperargininaemia, Dipharma B.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

2. Opinion following appeal procedures:

None

Lists of questions

The COMP adopted 7 lists of questions on initial applications. These applications will be discussed again at the next COMP meting prior to the adoption of an opinion.

Oral hearings

9 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 list of medicinal products for which decisions on orphan designation have been granted by the European Commission since the last COMP meeting is provided in Annex 1.

[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.

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 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 212th meeting of the COMP will be held on 18-20 June 2019.

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

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Annex 1

Designations granted by the European Commission following COMP opinion on the fulfilment of the orphan designation criteria since last COMP plenary meeting

No new designations were granted by the European Commission since last COMP plenary meeting.

Annex 2

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

No new designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since last COMP plenary meeting.