



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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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 February 2019

The Committee for Orphan Medicinal Products held its 208th plenary meeting on 19-21 February 2019.

Orphan medicinal product designation

Positive opinions

The COMP adopted 6 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 rh10 containing the human cholesterol 24-hydroxylase gene for treatment of Huntington's disease, Brainvectis;
- Codon-optimised human cystic fibrosis transmembrane conductance regulator messenger ribonucleic acid complexed with lipid-based nanoparticles for treatment of cystic fibrosis, Real Regulatory Limited.

2. Opinions adopted at the first COMP discussion:

- 1-[(3S)-3-{4-amino-3-[(3,5-dimethoxyphenyl)ethynyl]-1H-pyrazolo[3,4-d]pyrimidin-1-yl}pyrrolidin-1-yl]-2-propen-1-one for treatment of biliary tract cancer, Taiho Pharma Europe Limited;
- 2-[3-(2-chloro-4-{[5-cyclopropyl-3-(2,6-dichlorophenyl)-1,2-oxazol-4-yl]methoxy}phenyl)-3-hydroxyazetid-1-yl]pyridine-4-carboxylic acid-2-amino-2-(hydroxymethyl)propane-1,3-diol (1/1) for treatment of primary sclerosing cholangitis, Gilead Sciences Ireland UC;
- 4-hydroxy-6-{2-[4-(trifluoromethyl)phenyl]ethyl}pyridazin-3(2H)-one for treatment of Friedreich's ataxia, Takeda Pharma A/S;
- Marzeptacog alfa (activated) for treatment of haemophilia B, Voisin Consulting S.A.R.L.



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 designation¹ 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 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 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.

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

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 209th meeting of the COMP will be held on 19-21 March 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

Please also refer to the Community Register of orphan medicinal products for human use.

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
XPOVIO	Treatment of plasma cell myeloma	Karyopharm Europe GmbH	EU/3/14/1355
Tibsovo	Treatment of acute myeloid leukaemia	FGK Representative Service GmbH	EU/3/16/1802
Polivy	Treatment of diffuse large B-cell lymphoma	Roche Registration GmbH	EU/3/18/2013
ELZONRIS	Treatment of blastic plasmacytoid dendritic cell neoplasm	TMC Pharma (EU) Limited	EU/3/15/1567