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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

June 2019

The Committee for Orphan Medicinal Products held its 212th plenary meeting on 18-20 June 2019.

## Orphan medicinal product designation

### Positive opinions

The COMP adopted 8 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:

- 7-ethyl-10-hydroxycamptothecin for treatment of soft tissue sarcoma, Cebiotex S.L.;
- Recombinant mutated extracellular domain of the human acetylcholine receptor subunit alpha1 for treatment of myasthenia gravis, Toleranzia AB.

2. Opinions adopted at the first COMP discussion:

- 2-(hydroxymethyl)-2-(methoxymethyl)-1-azabicyclo[2.2.2]octan-3-one for treatment of myelodysplastic syndromes, Aprea Therapeutics AB;
- Elafibranor for treatment of primary biliary cholangitis, Genfit;
- Mavorixafor for treatment of WHIM syndrome, Voisin Consulting S.A.R.L.;
- N-((R)-2,3-dihydroxypropoxyl)-3,4-difluro-2-(2-fluoro-4-iodo-phenylamino)-benzamide for treatment of neurofibromatosis type 1, Voisin Consulting S.A.R.L.;
- Parsaclisib for treatment of marginal zone lymphoma, Incyte Biosciences Distribution B.V.;
- Pevonedistat for treatment of acute myeloid leukaemia, Takeda Pharma A/S.

3. Opinion following appeal procedures:

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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 17 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

6 oral hearings took place.

### Withdrawals of applications for orphan medicinal product designation

The COMP noted that 5 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.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. Opinions adopted at time of CHMP opinion:

None

2. Opinion following appeal procedures:

None

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

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 213th meeting of the COMP will be held on 16-18 July 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**

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

The list includes designation decisions that were revised following the amendment of an existing designated condition (identified by * when applicable)

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| (S)-3-((3-(1-((6-(3,4-dimethoxyphenyl)pryazin-2-yl)amino)ethyl)phenyl)carbamoyl)-5-methylpridin-1-ium | Treatment of pulmonary arterial hypertension | MWB Consulting S.A.R.L. | 17 April 2019 | 29 May 2019 |
| (S)-5-(1-(6-chloro-2-oxo-1,2-dihydroquinolin-3-yl)ethylamino)-1-methyl-6-oxo-1,6-dihydropyridine-2-carbonitrile | Treatment of acute myeloid leukaemia | Pharma Gateway AB | 17 April 2019 | 29 May 2019 |
| Allogeneic skin-derived ABCD5-positive mesenchymal stem cells | Treatment of epidermolysis bullosa | Rheacell GmbH & Co. KG | 17 April 2019 | 29 May 2019 |
| Diacerein | Treatment of epidermolysis bullosa | WORPHMED World Orphan Medicines Limited | 17 April 2019 | 29 May 2019 |
| Emixustat hydrochloride | Treatment of Stargardt's disease | Pharma Gateway AB | 17 April 2019 | 29 May 2019 |
| N-(trans-3-(5-((R)-1-hydroxyethyl)-1,3,4-oxadiazol-2-yl)cyclobutyl)-3-phenylisoxazole-5-carboxamide | Treatment of cystic fibrosis | Voisin Consulting S.A.R.L. | 17 April 2019 | 29 May 2019 |
| Relacorilant | Treatment of Cushing's syndrome | Granzer Regulatory Consulting & Services | 17 April 2019 | 29 May 2019 |
| Sodium benzoate, sodium phenylacetate | Treatment of citrullinaemia type 1 | Dipharma B.V. | 17 April 2019 | 29 May 2019 |

| Active substance | Orphan indication | Sponsor | COMP opinion date | EC designation date |
|---|---|---|---|---|
| Sodium benzoate, sodium phenylacetate | Treatment of carbamoyl-phosphate synthase-1 deficiency | Dipharma B.V. | 17 April 2019 | 29 May 2019 |
| Zanubrutinib | Treatment of lymphoplasmacytic lymphoma | BeiGene Ireland Limited | 17 April 2019 | 29 May 2019 |

# 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 |
|---|---|---|---|
| Glasdegib | Treatment of acute myeloid leukaemia | Pfizer Europe MA EEIG | EU/3/17/1923 |
| Luspatercept | Treatment of beta-thalassaemia intermedia and major | Celgene Europe BV | EU/3/14/1300 |
| Luspatercept | Treatment of myelodysplastic syndromes | Celgene Europe BV | EU/3/14/1331 |
| Deferiprone | Treatment of neurodegeneration with brain iron accumulation | Apotex B.V. | EU/3/18/2034 |
| Isatuximab | Treatment of plasma cell myeloma | Sanofi-Aventis Groupe | EU/3/14/1268 |