18 October 2018
EMA/COMP/679187/2018
Inspections, Human Medicines Pharmacovigilance and Committees Division

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

October 2018

The Committee for Orphan Medicinal Products held its 204th plenary meeting on 9-11 October 2018.

Orphan medicinal product designation

Positive opinions

The COMP adopted 22 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:

- 3-(3-(3,5-dimethyl-1H-pyrazol-4-yl)propoxy)-4-fluorobenzoic acid for treatment of ATTR amyloidosis, Pharma Gateway AB;
- 5-{(1R,2R)-2-[(cyclopropylmethyl)amino]cyclopropyl}-N-(tetrahydro-2H-pyran-4-yl)thiophene-3-carboxamide monohydrochloride for treatment of Kabuki syndrome, Takeda Pharma A/S;
- Anetumab ravtansine for treatment of ovarian cancer, Bayer AG;
- Apraglutide for treatment of short bowel syndrome, IQVIA RDS Ireland Limited;
- Autologous human adipose perivascular stromal cells genetically modified to secrete soluble tumour necrosis factor-related apoptosis-inducing ligand for treatment of pancreatic cancer, Rigenerand S.r.l.;
- Cyclo[L-alanyl-L-seryl-L-isoleucyl-L-prolyl-L-prolyl-L-glutaminyl-L-lysyl-L-tyrosyl-D-prolyl-L-prolyl-(2S)-2-aminodecanoyl-L-glutamyl-L-threonyl]acetate for treatment of cystic fibrosis, Santhera Pharmaceuticals (Deutschland) GmbH;
- Glucagon for treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome, Pharma Gateway AB;

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- Humanised IgG1 monoclonal antibody against GD2 for treatment of neuroblastoma, Y-mAbs Therapeutics A/S;
- Imlifidase for treatment of anti-glomerular basement membrane disease, Hansa Medical AB;
- Larotrectinib for treatment of glioma, Bayer AG;
- Larotrectinib for treatment of papillary thyroid cancer, Bayer AG;
- Lisocabtagene maraleucel for treatment of primary mediastinal large B-cell lymphoma, Celgene Europe Limited;
- Propagermanium for treatment of focal segmental glomerulosclerosis, Quality Regulatory Clinical Ireland Limited.

2. Opinions adopted at the first COMP discussion:

- Allogeneic faecal microbiota, pooled for treatment of graft-versus-host disease, MaaT PHARMA;
- Etamsylate for treatment of hereditary heamorrhagic telangiectasia, Consejo Superior de Investigaciones Cientificas (CSIC);
- Ex vivo fused normal allogeneic human myoblast with another normal allogeneic human myoblast for treatment of Duchenne muscular dystrophy, Dystrogen Therapeutics S.A.;
- Ex vivo fused normal allogeneic human myoblast with autologous human myoblast derived from Duchenne muscular dystrophy affected donor for treatment of Duchenne muscular dystrophy, Dystrogen Therapeutics S.A.;
- H-Arg-Pro-Lys-Pro-Gln-Gln-Phe-2Thi-Gly-Leu-Met(O_2)-NH_2-DOTA-213-bismuth for treatment of glioma, Dr. Regenold GmbH;
- Human apotransferrin for treatment of beta-thalassaemia intermedia and major, Sanquin Plasma Products B.V.;
- Ile-Ser-Ile-Thr-Glu-Ile-Lys-Gly-Val-Ile-Val-His-Arg-Ile-Glu-Thr-Ile-Leu-Phe-Lys-Lys-Lys-Lys-Glu-Met-Pro-Ser-Glu-Glu-Gly-Tyr-Gln-Asp for treatment of multiple system atrophy, United Neuroscience Limited;
- Fidanacogene elaparvovec for treatment of haemophilia B, Pfizer Europe MA EEIG;
- Setmelanotide for treatment of leptin receptor deficiency, TMC Pharma Services Ltd.

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 10 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

18 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 12 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

Detailed information on the orphan designation procedures

An overview of orphan designation procedures since 2000 is provided in Annex 1.

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 2.

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:

- Luxturna - voretigene neparvovec for treatment of Leber's congenital amaurosis, Spark Therapeutics Ireland Ltd (EU/3/12/981).
- Luxturna - voretigene neparvovec for treatment of retinitis pigmentosa, Spark Therapeutics Ireland Ltd (EU/3/15/1518).
- Poteligeo (mogamulizumab) for treatment of cutaneous T-cell lymphoma, Kyowa Kirin Limited (EU/3/16/1756).

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 3.

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 205th meeting of the COMP will be held on 6-8 November 2018.

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

Overview for orphan medicinal product designation procedure since 2000

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

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn[2]	Negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication[4]
2018	187	210	131 (62%)	77 (37%)	2 (1%)	115	11	14
2017	260	245	144 (59%)	100 (41%)	2 (1%)	147	14	15
2016	330	304	220 (72%)	82 (27%)	2 (1%)	209	14	14
2015	258	272	177 (65%)	94 (35%)	1 (1%)	190	14	21
2014	329	259	196 (76%)	62 (24%)	2 (1%)	187	15	16
2013	201	197	136 (69%)	60 (30%)	1 (1%)	136	7	8
2012	197	192	139 (72%)	52 (27%)	1 (1%)	148	10	12
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4	4
2009	164	136	113 (83%)	23 (17%)	0 (0%)	106	9	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6	7
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9	11
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6	6
2003	87	96	54 (56%)	37 (40%)	1 (1%)	55	5	5

[2] Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000
[3] The number of orphan medicinal products authorised includes the products for which the market exclusivity has expired.
[4] The market authorisation of an orphan medicinal product may cover more than one orphan designation.

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	EC designations	Orphan medicinal products authorised	Orphan designations included in authorised therapeutic indication
2002	80	75	43 (57%)	32 (42%)	2 (3%)	49	4	4
2001	83	90	62 (70%)	26 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	3 (10%)	0 (0%)	14	0	0
Total	**3162**	**2995**	**2102 (70%)**	**866 (29%)**	**27 (1%)**	**2067**	**153**	**171**

Annex 2

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 3

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
Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human *beta*$^{A\text{-}T87Q}$*-globin* gene	Treatment of beta-thalassemia intermedia and major	bluebird bio GmbH	EU/3/12/1091
Larotrectinib	Treatment of salivary gland cancer	Bayer AG	EU/3/18/1995
Larotrectinib	Treatment of soft tissue sarcoma	Bayer AG	EU/3/15/1606