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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 2017

The Committee for Orphan Medicinal Products held its 186th plenary meeting on 14-16 February 2017.

Orphan medicinal product designation

Positive opinion(s)

The COMP adopted 14 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinion(s) adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- Acetylleucine for treatment of Niemann-Pick disease, IntraBio Ltd;
- Antisense oligonucleotide targeting the *USH2A* gene for treatment of retinitis pigmentosa, ProQR Therapeutics IV BV;
- Cannabidiol for treatment of Lennox-Gastaut syndrome, GW Research Ltd;
- Inebilizumab for treatment of neuromyelitis optica spectrum disorders, AstraZeneca AB;
- Ketoconazole for treatment of granulosa cell tumours, Grupo Español de Tumores Huérfanos e Infrecuentes (GETHI);
- Megestrol acetate for treatment of granulosa cell tumours, Grupo Español de Tumores Huérfanos e Infrecuentes (GETHI).

2. Opinions adopted at the first COMP discussion:

- (3'R,4'S,5'R)-N-[(3R,6S)-6-carbamoyltetrahydro-2H-pyran-3-yl]-6''-chloro-4'-(2-chloro-3-fluoropyridin-4-yl)-4,4-dimethyl-2''-oxo-1'',2''-dihydrodispiro[cyclohexane-1,2'-pyrrolidine-3',3''-indole]-5'-carboxamide mono(4-methylbenzenesulfonate) monohydrate for treatment of soft tissue sarcoma, Daiichi Sankyo Europe GmbH;

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- Adeno-associated viral vector serotype 8 containing the human alpha-galactosidase A gene for treatment of Fabry disease, Freeline Therapeutics Ltd;
- Adeno-associated viral vector serotype LK03 encoding human ornithine transcarbamylase for treatment of ornithine transcarbamylase deficiency, Dr Julien Baruteau;
- Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase for treatment of GM1 gangliosidosis, Lysogene;
- Allogeneic ex–vivo-expanded umbilical cord blood-derived haematopoietic CD34+ progenitor cells and allogeneic non-expanded umbilical cord blood-derived haematopoietic mature myeloid and lymphoid cells for treatment in haematopoietic stem cell transplantation, Regulatory Resources Group Ltd;
- Autologous adipose tissue-derived mesenchymal stem cells for treatment of thromboangiitis obliterans (Buerger's disease), SPC GmbH;
- Phosphoinositide 3-kinase gamma peptide for treatment of cystic fibrosis, Kither Biotech s.r.l.;
- Poly-cyclodextrin-bis-cysteine-PEG_{3400}-camptothecin-conjugate for treatment of ovarian cancer, Viadoc Business Solutions Limited.

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(s)

The COMP did not adopt any negative opinions recommending the refusal of orphan medicinal product designations to the European Commission (EC).

Lists of questions

The COMP adopted 14 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

10 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.

[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)

The COMP did not adopt any opinion relating to the maintenance of orphan designated medicinal products in the EU registry of orphan medicinal products.

Details of the designated orphan medicinal products that have been subject of a 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 187th meeting of the COMP will be held on 14-15 March 2017.

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]	Final negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication
2017	19	50	33 (66%)	17 (34%)	0	20	1	1
2016	330	304	220 (72%)	82 (27%)	2	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
2002	80	75	43 (57%)	32 (42%)	2 (3%)	49	4	4

[2] Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000
[3] Number of authorised orphan medicinal products may cover more than one orphan designation

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn[4]	Final negative COMP opinions	EC designations	Orphan medicinal products[5] authorised	Orphan designations included in authorised therapeutic indication
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	**2734**	**2589**	**1860 (72%)**	**706 (27%)**	**23(1%)**	**1825**	**129**	**143**

[4] Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000
[5] Number of authorised orphan medicinal products may cover more than one orphan designation

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
Metreleptin	a) Treatment of familial partial lipodystrophy	Aegerion Pharmaceuticals Limited	EU/3/12/1022
	b) Treatment of Barraquer-Simons syndrome		EU/3/12/1023
	c) Treatment of Lawrence syndrome		EU/3/12/1024
	d) Treatment of Berardinelli-Seip syndrome		EU/3/12/1025
Ciclosporin	Treatment of vernal keratoconjunctivitis	Santen Oy	EU/3/06/360