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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

April 2017

The Committee for Orphan Medicinal Products held its 188th plenary meeting on 10-11 April 2017.

Orphan medicinal product designation

Positive opinion(s)

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:

- ^{225}Ac-lintuzumab for treatment of acute myeloid leukaemia, Voisin Consulting S.A.R.L.;
- Ursodeoxycholic acid for treatment of Niemann-Pick disease, IntraBio Ltd.

2. Opinions adopted at the first COMP discussion:

- Chimeric locked nucleic acid deoxynucleoside phosphorothioate-linked oligonucleotide inhibitor directed against microRNA-155-5p for treatment of cutaneous T-cell lymphoma, Miragen Therapeutics Europe Ltd;
- Poly(oxy-1,2-ethanediyl),.alpha.-hydro-.omega.-hydroxy-,15,15'-diester with N-acetyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyl-L-.alpha.-aspartyl-L-tryptophylglycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-L-threonyl-2-[2-(2-aminoethoxy)ethoxy]acetyl-N6-carboxy-L-lysinamide cyclic (2.fwdarw.12)-(disulfide); where two identical synthetic peptide domains are covalently linked at the ends of the polyethylene glycol chain for treatment of paroxysmal nocturnal haemoglobinuria, Best Regulatory Consulting Ltd;
- Recombinant adeno-associated viral vector serotype 6 encoding the B-domain-deleted human factor VIII for treatment of haemophilia A, Coté Orphan Consulting UK Limited;

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- Recombinant human interleukin-7 fused to a hybrid crystallisable fragment region of a human antibody for treatment of idiopathic CD4 lymphocytopenia, NeoImmuneTech, INC., Spółka Akcyjna, Oddział w Polsce;
- Sodium (1R,3R,4R,5S)-3-({2-N-acetylamino-2-deoxy-3-O-[(1S)-1-carboxylato-2-cyclohexylethyl]-beta-D-galactopyranosyl}oxy)-4-({6-deoxy-alpha-L-galactopyranosyl}oxy)-5-ethyl-cyclohexan-1-yl-(38-oxo-2,5,8,11,14,17,20,23,26,29,32,35-dodecaoxa-39-azahentetracontan-41-yl) carboxamide for treatment of acute myeloid leukaemia, TMC Pharma Services Ltd;
- Tamoxifen citrate for treatment of cystic fibrosis, GB Pharma Srl.

3. Opinions following appeal procedures

None

The COMP also recommended the amendment to 1 existing orphan designation:

- Ciclosporin for treatment of bronchiolitis obliterans syndrome, PARI Pharma GmbH (initially for treatment of graft rejection after lung transplantation);

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 15 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

4 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 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)

In line with its responsibility to review whether or not a designated orphan medicinal product still fulfils the designation criteria prior to the granting of a marketing authorisation, the COMP adopted 1 opinion recommending to the European Commission that the following orphan medicinal product be kept in the Community Register of Orphan Medicinal Products for human use:

1. Opinion(s) adopted at time of CHMP opinion

- Dinutuximab beta Apeiron (chimeric monoclonal antibody against GD2) for treatment of neuroblastoma, APEIRON Biologics AG (EU/3/12/1062). The final opinion was adopted by written procedure after the April meeting.

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 189th meeting of the COMP will be held on 10-12 May 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	56	84	51 (61%)	36 (41%)	1 (1%)	53	3	3
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
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	**2771**	**2627**	**1878 (71%)**	**725 (28%)**	**24 (1%)[6]**	**1858**	**131**	**145**

[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
[6] Total number corrected

Annex 2

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
(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	Treatment of soft tissue sarcoma	Daiichi Sankyo Europe GmbH	16 February 2017	20 March 2017
Acetylleucine	Treatment of Niemann-Pick disease	IntraBio Ltd	16 February 2017	20 March 2017
Adeno-associated viral vector serotype 8 containing the human alpha-galactosidase A gene	Treatment of Fabry disease	Freeline Therapeutics Ltd	16 February 2017	20 March 2017
Adeno-associated viral vector serotype LK03 encoding human ornithine transcarbamylase	Treatment of ornithine transcarbamylase deficiency	Dr Julien Baruteau	16 February 2017	20 March 2017
Adeno-associated viral vector serotype rh.10 expressing beta-galactosidase	Treatment of GM1 gangliosidosis	Lysogene	16 February 2017	20 March 2017
Allogeneic ex–vivo-expanded umbilical cord blood-derived haematopoietic CD34+ progenitor cells and allogeneic non-expanded umbilical cord blood-derived haematopoietic mature	treatment in haematopoietic stem cell transplantation	Regulatory Resources Group Ltd	16 February 2017	20 March 2017

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
myeloid and lymphoid cells				
Antisense oligonucleotide targeting the *USH2A* gene	Treatment of retinitis pigmentosa	ProQR Therapeutics IV BV	16 February 2017	20 March 2017
Autologous adipose tissue-derived mesenchymal stem cells	Treatment of thromboangiitis obliterans (Buerger's disease)	SPC GmbH	16 February 2017	20 March 2017
Cannabidiol	Treatment of Lennox-Gastaut syndrome	GW Research Ltd	16 February 2017	20 March 2017
Inebilizumab	Treatment of neuromyelitis optica spectrum disorders	AstraZeneca AB	16 February 2017	20 March 2017
Ketoconazole	Treatment of granulosa cell tumours	Grupo Español de Tumores Huérfanos e Infrecuentes (GETHI)	16 February 2017	20 March 2017
Megestrol acetate	Treatment of granulosa cell tumours	Grupo Español de Tumores Huérfanos e Infrecuentes (GETHI)	16 February 2017	20 March 2017
Phosphoinositide 3-kinase gamma peptide	Treatment of cystic fibrosis	Kither Biotech s.r.l.	16 February 2017	20 March 2017
Poly-cyclodextrin-bis-cysteine-PEG_{3400}-camptothecin-conjugate	Treatment of ovarian cancer	Viadoc Business Solutions Limited	16 February 2017	20 March 2017

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.

None