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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 2017

The Committee for Orphan Medicinal Products held its 190th plenary meeting on 13-15 June 2017.

Orphan medicinal product designation

Positive opinion(s)

The COMP adopted 9 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:

- Oxymetazoline hydrochloride for treatment of spinal cord injury, RDD Pharma Limited;
- Recombinant human antibody directed against misfolded human superoxide dismutase 1 for treatment of amyotrophic lateral sclerosis, The Medical & Regulatory Partnership Limited;
- Sirolimus for treatment of pachyonychia congenita, Raremoon Consulting Ltd.

2. Opinions adopted at the first COMP discussion:

- (S)-1-(4-fluorophenyl)-1-(2-(4-(6-(1-methyl-1H-pyrazol-4-yl)pyrrolo[2,1-f][1,2,4]triazin-4-yl)piperazin-yl)pyrimidin-5-yl)ethan-1-amine for treatment of gastrointestinal stromal tumours, PhaRA bvba;
- Autologous CD4+ and CD8+ T cells expressing a CD19-specific chimeric antigen receptor for treatment of diffuse large B-cell lymphoma, Celgene Europe Limited;
- *Bacillus subtilis* oxalate decarboxylase for treatment of primary hyperoxaluria, Allena Pharmaceuticals Ireland Limited;
- Polyphenyl(disodium 3-O-sulfo-beta-D-glucopyranuronate)-(1→3)-beta-D-galactopyranoside for treatment of anti-MAG neuropathy, SFL Regulatory Affairs Consulting Ltd;

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- Retinol for prevention of retinopathy of prematurity, Orphanix GmbH;
- Tirapazamine for treatment of hepatocellular carcinoma, PhaRA bvba.

3. Opinion(s) 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(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 12 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

6 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 7 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)

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

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

- Oxervate (cenegermin) for treatment of neurotrophic keratitis, Dompé farmaceutici S.p.A. (EU/3/15/1586). The opinion was adopted by written procedure after the May meeting.

2. Opinion(s) 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 191th meeting of the COMP will be held on 11-13 July 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[4]
2017	105	125	70 (56%)	54 (43%)	1 (1%)	71	7	7
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] 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
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	**2820**	**2664**	**1897 (71%)**	**743 (28%)**	**24 (1%)**	**1876**	**135**	**149**

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
^{225}Ac-lintuzumab	Treatment of acute myeloid leukaemia	Voisin Consulting S.A.R.L.	11 April 2017	22 May 2017
Ciclosporin*	Treatment of bronchiolitis obliterans syndrome	PARI Pharma GmbH	11 April 2017	24 May 2017
Chimeric locked nucleic acid deoxynucleoside phosphorothioate-linked oligonucleotide inhibitor directed against microRNA-155-5p	Treatment of cutaneous T-cell lymphoma	Miragen Therapeutics Europe Ltd	11 April 2017	22 May 2017
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	Treatment of paroxysmal nocturnal haemoglobinuria	Best Regulatory Consulting Ltd	11 April 2017	22 May 2017
Recombinant adeno-associated viral vector serotype 6 encoding the B-domain-deleted	Treatment of haemophilia A	Coté Orphan Consulting UK Limited	11 April 2017	22 May 2017

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
human factor VIII				
Recombinant human interleukin-7 fused to a hybrid crystallisable fragment region of a human antibody	Treatment of idiopathic CD4 lymphocytopenia	NeoImmuneTech, INC., Spółka Akcyjna, Oddział w Polsce	11 April 2017	22 May 2017
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	Treatment of acute myeloid leukaemia	TMC Pharma Services Ltd	11 April 2017	22 May 2017
Tamoxifen citrate	Treatment of cystic fibrosis	GB Pharma Srl	11 April 2017	22 May 2017
Ursodeoxycholic acid	Treatment of Niemann-Pick disease	IntraBio Ltd	11 April 2017	22 May 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.

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Masitinib	Treatment of amyotrophic lateral sclerosis	AB Science	EU/3/16/1722
Viable t-cells	a)Treatment in haematopoietic stem cell transplantation b) Treatment of acute myeloid leukaemia c) Prevention of Graft-versus-Host Disease	Kiadis Pharma Netherlands B.V.	EU/3/16/1678 EU/3/14/1356 EU/3/08/561