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Human Medicines Development and Evaluation

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

October 2012

The Committee for Orphan Medicinal Products held its 138th plenary meeting on 3-5 October 2012.

Orphan medicinal product designation

The COMP adopted 16 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:

- **Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotropic factor** for treatment of macular telangiectasia type 2, Enpharma Ltd.
- **IL-12-secreting dendritic cells, loaded with autologous tumour lysate** for treatment of glioma, Activartis Biotech GmbH.
- **Milciclib maleate** for treatment of malignant thymoma, Nerviano Medical Science Srl.

2. Opinions adopted at the first COMP discussion:

- **Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-dimethylaminoethylamino-17-demethocygeldanamycin)** for treatment of retinitis pigmentosa, Avena Therapeutics Ltd.
- **Alisertib** for treatment of ovarian cancer, Takeda Global Research and Development Centre (Europe) Ltd.
- **Canakinumab** for treatment of tumour necrosis factor receptor-associated periodic syndrome, Novartis Europharm Limited.
- **Chimeric monoclonal antibody against GD2** for treatment of neuroblastoma, APEIRON Biologics AG.

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- **Erdosteine** for treatment of mercury toxicity, Rafifarm SRL.
- **Ixazomib** for treatment of systemic light chain amyloidosis, Takeda Global Research and Development Centre (Europe) Ltd.
- **Melarsoprol** for treatment of African trypanosomiasis, Pr. Peter Kennedy.
- **Naloxone hydrochloride dihydrate** for treatment of cutaneous T-cell lymphoma, Winston Laboratories Ltd.
- **Panobinostat** for treatment of multiple myeloma, Novartis Europharm Limited.
- **Recombinant human dyskerin** for treatment of dyskeratosis congenita, Advanced Medical Projects.
- **Synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin)** for treatment of glioma, Avena Therapeutics Ltd.
- **Tafamidis** for treatment of senile systemic amyloidosis, Pfizer Limited.
- **Tralokinumab** for treatment of idiopathic pulmonary fibrosis, MedImmune Ltd.

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation by the European Commission.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 18 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

7 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.

Detailed information on the orphan designation procedure

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

The list of medicinal products for which decisions on orphan designation[1] have been given by the European Commission since the last COMP meeting is provided in Annex 2.

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new European Union marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 3.

1 Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products http://ec.europa.eu/health/documents/community-register/html/index_en.htm

Details on the opinions for marketing authorisation for orphan medicinal products adopted by the Committee for Medicinal Products for Human Use (CHMP) can be found in the CHMP meeting reports on the EMA website: http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000508.jsp&mid=WC0b01ac0580028d2a.

Upcoming meetings

- The 139th meeting of the COMP will be held on 6-7 November 2012.

Other matters

The main topics addressed during the meeting related to:

- 2 Protocol Assistance letters were adopted.

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

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	EC designations	Orphan medicinal products authorised
2012	155	149	113 (67%)	35 (23%)	1 (1%)	104	7
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4
2009	164	136	113 (83%)	23 (17%)	0[2] (0%)	106	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55	5
2002	80	75	43 (57%)	30 (40%)	2[3] (3%)	49	4
2001	83	90	62[4] (70%)	27 (29%)	1 (1%)	64	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0
Total	**1555**	**1469**	**1072 (73%)**	**380 (26%)**	**17 (1%)**	**1039**	**75**

[2] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[3] Following a quality assurance exercise it was identified that this figure needed correction

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the September 2012 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Folic acid to be used with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine	Diagnosis of positive folate receptor status in ovarian cancer	Endocyte Europe B.V.	23 July 2012[4]	10 September 2012
N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine to be used with folic acid	Diagnosis of positive folate receptor status in ovarian cancer	Endocyte Europe B.V.	23 July 2012[5]	10 September 2012

[4] Opinion adopted via written procedure following the 10-11 July 2012 COMP 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 September 2012 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
(1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethano	Bedaquiline	Janssen-Cilag International N.V.	EU/3/05/314	Treatment of tuberculosis
Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]	Iclusig	ARIAD Pharma Ltd	EU/3/09/716	Treatment of chronic myeloid leukaemia
			EU/3/09/715	Treatment of acute lymphoblastic leukaemia
Masitinib mesilate	Kinaction	AB Science	EU/3/09/684	Treatment of pancreatic cancer