EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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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

March 2012

The Committee for Orphan Medicinal Products held its 132nd plenary meeting on 7-8 March 2012.

Orphan medicinal product designation

The COMP adopted 13 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:

- **Exon 45 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy, Prosensa Therapeutics B.V.
- **Exon 53 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy, Prosensa Therapeutics B.V.
- **Pegylated recombinant factor VIII** for treatment of haemophilia A, Novo Nordisk A/S.

2. Opinions adopted at the first COMP discussion:

- **(E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt** for treatment of myelodysplastic syndromes, JJGConsultancy Ltd.
- **1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H-pyrazolo[3,4 d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one** for treatment of chronic lymphocytic leukaemia, Nexus Oncology Ltd.
- **2-Allyl-1-[6-(1-hydroxy-1-methylethyl)pyridin-2-yl]-6-{[4-(4-methylpiperazin-1-yl)phenyl]amino}-1,2-dihydro-3H-pyrazolo[3,4-d]pyrimidin-3-one** for treatment of ovarian cancer, Merck Sharp & Dohme Limited.
- **Halofuginone hydrobromide** for treatment of Duchenne muscular dystrophy, Biological Consulting Europe Ltd.

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- **Heterologous human adult liver-derived stem cells** for treatment of acute liver failure, Fresenius Medical Care Deutschland GmbH.
- **N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino)benzamide** for treatment of neurofibromatosis type 2, Sirius Regulatory Consulting Limited.
- **Pomalidomide** for treatment of systemic sclerosis, Celgene Europe Limited.
- **Recombinant human methionine proinsulin** for treatment of retinitis pigmentosa, ProRetina Therapeutics S.L.
- **Vosaroxin** for treatment of acute myeloid leukaemia, Sunesis Europe Ltd.
- **Yttrium (^{90}Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10** for treatment of soft tissue sarcoma, Laboratoires OncoTherapy Science France, S.A.R.L.

Public summaries of these opinions will be available on the Agency's 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 5 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

3 oral hearings took place.

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 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 highlights on the Agency's website:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000508.jsp&mid=WC0b01ac0580028d2a

Upcoming meetings

- The 133rd meeting of the COMP will be held on 11-12 April 2012.

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/enterprise/sectors/pharmaceuticals/documents/community-register/html/index_en.htm

Other matters

The main topics addressed during the meeting related to:

- 6 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 Agency's 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	Designations granted by Commission
2012	41	45	36 (80%)	9 (20%)	0 (0%)	23
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107
2010	174	176	123 (70%)	51 (29%)	2[2] (1%)	128
2009	164	137	113 (82%)	23 (17%)	1 (1%)	106
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88
2004	108	101	75 (74%)	22 (22%)	4 (4%)	72
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55
2002	80	76	43 (57%)	30 (39%)	3 (4%)	49
2001	83	92	64 (70%)	27 (29%)	1 (1%)	64
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14
Total	**1441**	**1369**	**997 (73%)**	**354 (26%)**	**18 (1%)**	**957**

[2] One more opinion was re-adopted in 2010 following the appeal to a negative opinion from 2009

Annex 2

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

Active substance	(1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate
Orphan indication	Treatment of soft tissue sarcoma
Sponsor	Nexus Oncology Ltd
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	(1S,3S)-3-amino-4-(difluoromethylene) cyclopentanecarboxylic acid hydrochloride
Orphan indication	Treatment of West Syndrome
Sponsor	Catalent Pharma Solutions Limited
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one
Orphan indication	Treatment of amyotrophic lateral sclerosis
Sponsor	ICON Clinical Research UK Limited
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	Autologous haematopoietic cells genetically modified with a lentiviral vector containing the human *gp91(phox)* gene
Orphan indication	Treatment of X-linked chronic granulomatous disease
Sponsor	Généthon
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	Doxycycline hyclate
Orphan indication	Treatment of systemic amyloidosis caused by beta-2 microglobulin
Sponsor	Giampaolo Merlini
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	Glucagon
Orphan indication	Treatment of congenital hyperinsulinism
Sponsor	Biodel UK Limited
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	Heterologous human adult liver-derived stem cells
Orphan indication	Treatment of carbamoyl-phosphate synthase-1 deficiency
Sponsor	Fresenius Medical Care Deutschland GmbH
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	Human monoclonal antibody against Fas ligand
Orphan indication	Treatment of pemphigus
Sponsor	PinCell s.r.l.
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	Human monoclonal antibody targeting *Staphylococcus aureus* alpha-toxin
Orphan indication	Treatment of pneumonia caused by *Staphylococcus aureus*
Sponsor	Envestia Limited
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	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 and folic acid
Orphan indication	Diagnosis of folate receptor status in ovarian cancer
Sponsor	Endocyte Europe B.V.
COMP opinion date	7 October 2011
Orphan designation date	9 February 2012

Active substance	Nimorazole maleate
Orphan indication	Treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy
Sponsor	Conventia Medical LLP
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	S[+] apomorphine
Orphan indication	Treatment of amyotrophic lateral sclerosis
Sponsor	University of Sheffield
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	Sialic acid
Orphan indication	Treatment of hereditary inclusion body myopathy
Sponsor	NDA Regulatory Science Ltd
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	Sodium phenylbutyrate
Orphan indication	Treatment of carbamoyl-phosphate synthase-1 deficiency
Sponsor	Lucane Pharma SAS
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	Sodium phenylbutyrate
Orphan indication	Treatment of citrullinaemia type 1
Sponsor	Lucane Pharma SAS
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	Sodium phenylbutyrate
Orphan indication	Treatment of ornithine transcarbamylase deficiency
Sponsor	Lucane Pharma SAS
COMP opinion date	7 December 2011
Orphan designation date	9 February 2012

Active substance	Sodium nitrite
Orphan indication	Treatment of pulmonary arterial hypertension
Sponsor	FGK Representative Service GmbH
COMP opinion date	11 January 2012
Orphan designation date	5 March 2012

Active substance	Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine
Orphan indication	Treatment of ovarian cancer
Sponsor	Endocyte Europe B.V.
COMP opinion date	7 October 2011
Orphan designation date	9 February 2012