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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

6-7 October 2010

The Committee for Orphan Medicinal Products held its 116th plenary meeting on 6-7 October 2010.

Orphan medicinal product designation

The COMP adopted 15 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 12 July 2010 with an active review time of 88 days:

- **Ovine anti-colchicine polyclonal antibody fragments** for treatment of colchicine poisoning; Laboratoires SERB.
- **Para-aminosalicylic acid** for treatment of tuberculosis; Lucane Pharma SAS.
- **Lomitapide** for treatment of familial chylomicronaemia; Dimensione Ricerca S.r.l.

For the following medicines the review began on 13 August 2010 with an active review time of 56 days:

- **1H-Benzimidazole-7-carboxamide, 2-[(2R)-2-methyl-2-pyrrolidinyl]** for treatment of ovarian cancer; Abbott Laboratories.
- **7-beta-hydroxycholesteryl-3-beta-oleate** for treatment of glioma; Intsel Chimos SA.
- **Adeno-associated viral vector containing DNA encoding an RNAi targeting rhodopsin / adeno-associated viral vector containing a rhodopsin gene**; treatment of rhodopsin-linked retinitis pigmentosa, Genable Technologies Ltd.
- **Human heterologous liver cells (for infusion)**, treatment of hyperargininaemia; Cytonet GmbH & Co. KG.
- **Human heterologous liver cells (for infusion)**, treatment of argininosuccinic aciduria; Cytonet GmbH & Co. KG.

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- **Human heterologous liver cells (for infusion)**, treatment of citrullinaemia type 1; Cytonet GmbH & Co. KG.
- **Human heterologous liver cells (for infusion)**, treatment of carbamoyl-phosphate synthetase-1 deficiency; Cytonet GmbH & Co. KG.
- **Lentiviral vector carrying the Fanconi anaemia-A (*FANCA*) gene**, treatment of Fanconi anaemia type A; Center for Biomedical Network Research on Rare Diseases (CIBERER).
- **N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride**, treatment of acute myeloid leukaemia; Merck KGaA.
- **Recombinant human lysosomal acid lipase**, treatment of lysosomal acid lipase deficiency; HungaroTrial Ltd.
- **Silibinin-C-2',3-dihydrogensuccinate, disodium salt**, prevention of recurrent hepatitis C in liver transplant recipients; Rottapharm S.p.A.
- **Tesetaxel**, treatment of gastric cancer; Genta Development Ltd.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 9 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to adoption of the opinion.

Oral hearings

5 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 3 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.

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 monthly report on the Agency's website.

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

Article 5 (12) 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 registry of orphan medicinal products:

- **TOBI Podhaler (tobramycin)** for treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis; Novartis Europharm Limited.

Upcoming meetings

- The 117th meeting of the COMP will be held on 9-10 November 2010.

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 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
2010	136	131	105 (80%)	40 (31%)	3 (2%)	86
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	**1196**	**1121**	**832 (74%)**	**289 (26%)**	**17 (2%)**	**785**

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the September 2010 COMP monthly report

Active substance	1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-d]pyrimidin-7-yl]-3-tert-butyl-urea
Sponsor	Sanofi Aventis
Orphan indication	Treatment of acute myeloid leukaemia
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Abarelix
Sponsor	Speciality European Pharma Ltd
Orphan indication	Treatment of low-flow priapism
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Adenovirus-associated viral vector serotype 10 carrying the human N-sulfoglucosamine sulfohydrolase and sulfatase modifying factor 1 cDNAs
Sponsor	SANFILIPPO Therapeutics SAS
Orphan Indication	Treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)
COMP opinion date	02/06/2010
Orphan Designation date	20/09/2010

Active substance	Allogeneic T cells encoding an exogenous TK gene
Sponsor	LTKFarma SAS
Orphan indication	Treatment of acute myeloid leukaemia
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Allogeneic human dermal fibroblasts
Sponsor	Intercytex Ltd
Orphan indication	Treatment of epidermolysis bullosa
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Autologous bone marrow-derived mononuclear cell fraction
Sponsor	t2cure GmbH
Orphan indication	Treatment of thromboangiitis obliterans (Buerger's disease)
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 - glutathione S transferase) coupled to oxidised polymannose
Sponsor	Prima Biomed Europe Ltd
Orphan indication	Treatment of ovarian cancer
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Cyclic pyranopterin monophosphate
Sponsor	Orphatec Pharmaceuticals GmbH
Orphan indication	Treatment of molybdenum cofactor deficiency type A
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Cysteamine bitartrate (gastroresistant)
Sponsor	Raptor Pharmaceuticals Europe BV
Orphan indication	Treatment of cystinosis
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Eflornithine
Sponsor	Cancer Prevention Pharma Ltd
Orphan indication	Treatment of familial adenomatous polyposis
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Forodesine
Sponsor	Mundipharma Research Limited
Orphan indication	Treatment of chronic lymphocytic leukaemia
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Glutathione-pegylated liposomal doxorubicin hydrochloride
Sponsor	to-BBB Technologies BV
Orphan indication	Treatment of glioma
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Nafamostat mesilate
Sponsor	Mucokinetica Ltd
Orphan indication	Treatment of cystic fibrosis
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1
Sponsor	Biogen Idec Limited
Orphan indication	Treatment of haemophilia A
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Recombinant porcine factor VIII (B domain deleted)
Sponsor	Inspiration Biopharmaceuticals EU Limited
Orphan indication	Treatment of haemophilia A
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Vorinostat
Sponsor	Merck Sharp & Dohme Limited
Orphan indication	Treatment of malignant mesothelioma
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

Active substance	Heat-killed *Mycobacterium vaccae* (whole cell)
Sponsor	Immodulon Therapeutics Ltd
Orphan indication	Treatment of tuberculosis
COMP opinion date	02/06/2010
Orphan designation date	20/09/2010

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 2010 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
N-methyl D-(2,3,4,5,6-pentahydroxy-hexyl)-ammonium; 2-(3,5-dichloro-phenyl)-benzoxazole-6-carboxylate	Tafamidis meglumine	FoldRx Pharmaceuticals Limited	EU/3/06/401	Treatment of familial amyloid polyneuropathy