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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

11-12 January 2011

The Committee for Orphan Medicinal Products held its 119th plenary meeting on 11-12 January 2011.

During this meeting the Committee adopted positive opinions on the designation of active substances for the treatment of mitochondrial neurogastrointestinal encephalomyopathy (MNGIE) due to thymidine phosphorylase deficiency and fragile X syndrome. This is the first time an active substance receives orphan designation in the EU for these conditions. At present there is no satisfactory treatment authorised in the European Union for none of them.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of its potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

Orphan medicinal product designation

The COMP adopted 10 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 15 October 2010 with an active review time of 90 days:

- **Darinaparsin** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), Ziopharm Oncology Limited.
- **Glufosfamide** for treatment of pancreatic cancer, Theradex (Europe) Ltd.

For the following medicines the review began on 12 November 2010 with an active review time of 62 days:

- **(S)-{8-fluoro-2-2[4-(3-methoxyphenyl)-1-piperazinyl]-3-[2-methoxy-5-(trifluoromethyl)-phenyl]-3,4-dihydro-4-quinazolinyl}acetic acid** for prevention of

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cytomegalovirus (CMV) disease in patients with impaired cell mediated immunity deemed at risk, AiCuris GmbH & Co. KG.

- **Human anthrax monoclonal antibody** for treatment of inhalation anthrax disease, Emergent Sales and Marketing Germany GmbH.
- **Ombrabulin** for treatment of soft tissue sarcoma, Sanofi Aventis.
- **R-baclofen** for treatment of fragile X syndrome, Lakeside Regulatory Consulting Services Ltd.
- **Recombinant fusion protein linking human coagulation factor VIIa with human albumin** for treatment of haemophilia A, CSL Behring GmbH.
- **Recombinant thymidine phosphorylase encapsulated in autologous erythrocytes** for treatment of mitochondrial neurogastrointestinal encephalomyopathy (MNGIE) due to thymidine phosphorylase deficiency, St George's University of London.
- **Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA** for treatment of familial amyloid polyneuropathy, Voisin Consulting SARL.
- **Vorinostat** for treatment of multiple myeloma, Merck Sharp & Dohme Limited.

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 adoption of the opinion.

Oral hearings

3 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 1 application for orphan medicinal product designation was 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.

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:

- **Orphacol (Cholic acid)** for treatment of inborn errors of primary bile acid synthesis, Laboratoires CTRS.

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

Upcoming meetings

- The 120th meeting of the COMP will be held on 8-9 February 2011.

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
2011	1	11	10 (91%)	1 (9%)	0 (0%)	0
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	**1235**	**1177**	**860 (73%)**	**301 (26%)**	**16 (1%)**	**827**

[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 December 2010 COMP monthly report

Active substance	**7-beta-hydroxycholesteryl-3-beta-oleate**
Sponsor	Intsel Chimos SA
Orphan indication	Treatment of glioma
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	**Adeno-associated viral vector containing DNA encoding an RNAi targeting rhodopsin / adeno-associated viral vector containing a rhodopsin gene**
Sponsor	Genable Technologies Ltd.
Orphan Indication	Treatment of rhodopsin-linked retinitis pigmentosa
COMP opinion date	7 October 2010
Orphan Designation date	17 December 2010

Active substance	**Human heterologous liver cells (for infusion)**
Sponsor	Cytonet GmbH & Co. KG.
Orphan indication	Treatment of argininosuccinic aciduria
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	**Human heterologous liver cells (for infusion)**
Sponsor	Cytonet GmbH & Co. KG.
Orphan indication	Treatment of carbamoyl-phosphate synthetase-1 deficiency
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	**Human heterologous liver cells (for infusion)**
Sponsor	Cytonet GmbH & Co. KG
Orphan indication	Treatment of citrullinaemia type 1
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	**Human heterologous liver cells (for infusion)**
Sponsor	Cytonet GmbH & Co. KG
Orphan indication	Treatment of hyperargininaemia
COMP opinion date	7 October 2010

Active substance	Human heterologous liver cells (for infusion)
Orphan designation date	17 December 2010

Active substance	Lentiviral vector carrying the Fanconi anaemia-A (*FANCA*) gene
Sponsor	Center for Biomedical Network Research on Rare Diseases (CIBERER)
Orphan indication	Treatment of Fanconi anaemia type A
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	Lomitapide
Sponsor	Dimensione Ricerca S.r.l.
Orphan indication	Treatment of familial chylomicronaemia
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride
Sponsor	Merck KGaA
Orphan indication	Treatment of acute myeloid leukaemia
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	Ovine anti-colchicine polyclonal antibody fragments
Sponsor	Laboratoires SERB
Orphan indication	Treatment of colchicine poisoning
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	Para-aminosalicylic acid
Sponsor	Lucane Pharma SAS
Orphan indication	Treatment of tuberculosis
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	Recombinant human lysosomal acid lipase
Sponsor	HungaroTrial Ltd.
Orphan indication	Treatment of lysosomal acid lipase deficiency
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	Silibinin-C-2′,3-dihydrogensuccinate, disodium salt
Sponsor	Rottapharm S.p.A.
Orphan indication	Prevention of recurrent hepatitis C in liver transplant recipients
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	Tesetaxel
Sponsor	Genta Development Ltd.
Orphan indication	Treatment of gastric cancer
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010

Active substance	Veliparib
Sponsor	Abbott Laboratories
Orphan indication	Treatment of ovarian cancer
COMP opinion date	7 October 2010
Orphan designation date	17 December 2010