European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
JANUARY 2009 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its ninety-seventh plenary meeting on 7 January 2009. The Committee thanked Dr Ulla Närhi who will leave the European Commission, for her contribution to the work with COMP-EMEA activities at the European Commission, DG Enterprise, Pharmaceuticals.

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

For the following medicines the EMEA review began on 13 October 2008 with an active review time of 62 days.

- **(6R)-4, 5, 6, 7-tetrahydro-N6-propyl-2, 6-benzothiazolediamine dihydrochloride monohydrate,** from Knopp Neurosciences Sub Ltd, for treatment of amyotrophic lateral sclerosis.
- **2,2-dimethylbutyric acid, sodium salt,** from Isabelle Ramírez, for treatment of beta-thalassaemia intermedia and major.
- **Allogeneic *ex vivo* expanded umbilical cord blood cells,** from Teva Pharma GmbH, for treatment of acute myeloid leukaemia.
- **Allogeneic *ex vivo* expanded umbilical cord blood cells,** from Teva Pharma GmbH, for treatment of acute lymphoblastic leukaemia.
- **Mifepristone,** from EXELGYN, for treatment of hypercortisolism (Cushing's syndrome) of endogenous origin.
- **N-terminal hexaglutamine-tagged recombinant human N-acetylgalactosamine-6-sulfate sulfatase,** from Prof. Dr Arndt Rolfs, for treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome).
- **Tobramycin (inhalation use),** from PARI Pharma GmbH, for treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis.

Negative opinion

The COMP adopted one negative opinion recommending that the following medicine will not be granted designation as orphan medicinal product:

- **4-[[[4-(4-Chlorophenoxy)phenyl]sulfonyl]-methyl]tetrahydro-N-hydroxy-2H-pyran-4-carboxamide**, from Anthony William Fox, for prevention of graft rejection after liver transplantation. EMEA review began on 13 June 2008 with an active review time of 90 days.

Public summaries of opinion will be available on the EMEA website which the Agency updates following adoption of the respective decisions on orphan designation by the European Commission.

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OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

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

Oral hearing

One oral hearing took place.

Withdrawal of application for orphan medicinal product designation

The COMP noted that one 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**.

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new community marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in **Annex 2**.

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 EMEA website.

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 one opinion recommending to the European Commission that the following orphan medicinal product be kept in the Community registry of orphan medicinal products:

- **Mepact** (muramyl tripeptide phosphatidyl ethanolamine, mifamurtide)**,** from Immuno-Designed Molecules SA, for treatment of osteosarcoma.

UPCOMING MEETINGS FOLLOWING THE JANUARY 2009 COMP PLENARY MEETING

- The ninety-eighth meeting of the COMP will be held on 3 February 2009.

OTHER MATTERS

The main topics addressed during the January 2009 COMP meeting related to:

- One Protocol Assistance letter was adopted.

NOTE: This Monthly Report and other documents may be found on the internet at the following location: http://www.emea.europa.eu

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OVERVIEW FOR ORPHAN MEDICINAL PRODUCT DESIGNATION PROCEDURE SINCE 2000

Year	Applications submitted	Positive COMP Opinions	Applications withdrawn	Final negative COMP Opinions	Designations granted by Commission
2009	-	7	1	-	-
2008	119	86	31	1	73
2007	125	97	19	1	98
2006	104	81	20	2	80
2005	118	88	30	0	88
2004	108	75	22	4	72
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14

DESIGNATED ORPHAN MEDICINAL PRODUCTS THAT HAVE BEEN SUBJECT OF A NEW COMMUNITY MARKETING AUTHORISATION APPLICATION UNDER THE CENTRALISED PROCEDURE SINCE THE DECEMBER 2008 COMP MONTHLY REPORT

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Recombinant human monoclonal antibody to human IL-1 beta of the IgG1/K class	Ilaris	Novartis Europharm Limited	EU/3/07/439	Treatment of cryopirin-associated periodic syndromes (Familial Cold Urticaria Syndrome (FCUS), Muckle-Wells Syndrome (MWS), and Neonatal Onset Multisystem Inflammatory Disease (NOMID), also known as Chronic Infantile Neurological Cutaneous Articular Syndrome (CINCA))
Eltrombopag olamine	Revolade	GlaxoSmithKline Trading Services Limited	EU/3/07/467	Treatment of idiopathic thrombocytopenic purpura
Recombinant human acid alpha-glucosidase	Myozyme	Genzyme Europe BV	EU/3/00/018	Treatment of glycogen storage disease type II (Pompe's disease)
Imatinib mesylate	Glivec	Novartis Europharm Ltd	EU/3/01/061	Treatment of malignant gastrointestinal stromal tumours
Sildenafil citrate	Revatio	Pfizer Limited - UK	EU/3/03/178	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Sitaxentan sodium	Thelin	Encysive (UK) Ltd - UK	EU/3/04/234	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Trabectedin	Yondelis	PharmaMar SA Sociedad Unipersonal	EU/3/03/171	Treatment of ovarian cancer