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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
JULY 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its ninety-second plenary meeting on 8-9 July 2008. The COMP welcomed the new COMP members, Dr János Borvendég as CHMP representative and Mr Agnis Zvaigzne as the new COMP member from Latvia. The Committee discussed further new ways to improve the work of the COMP, namely a revision of the structure of the reports. The Committee also discussed the basis for defining valid conditions and subsets for orphan designation.

In addition, the future of the COMP Working Group with Interested Parties (COMP-WGIP) was discussed. It was agreed that after years of work the group's structure should be considered. The proposal to transform it into dedicated and project focused groups was accepted.

The COMP adopted its 27th positive opinion for treatment of cystic fibrosis. Despite the high number of orphan designations for this condition no orphan products have been authorised up to date. The EMEA has recently published the Guideline on the Clinical Development of Medicinal Products for the Treatment of Cystic Fibrosis (EMEA/CHMP/EWP/9147/2008) (http://www.emea.europa.eu/pdfs/human/ewp/914708en.pdf).

ORPHAN MEDICINAL PRODUCT DESIGNATION

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

- **Avian polyclonal IgY antibody against *Pseudomonas aeruginosa*,** Immunsystem I.M.S. AB, for treatment of cystic fibrosis. EMEA review began on 13 June 2008 with an active review time of 27 days.
- **Drotrecogin alfa (activated),** Drugrecure Aps, for treatment of acute respiratory distress syndrome. EMEA review began on 13 June 2008 with an active review time of 27 days.
- **Levofloxacin hemihydrate,** Mpex London Ltd, for treatment of cystic fibrosis. EMEA review began on 11 April 2008 with an active review time of 90 days.
- **Miltefosine,** ExperGen Drug Development GmbH, for treatment of cutaneous T-cell lymphoma. EMEA review began on 13 June 2008 with an active review time of 27 days.
- **N'-(5-chloro-2-hydroxy-3-methylbenzylidene)-2,4-dihydroxybenzhydrazide,** Innate Pharmaceuticals AB, for treatment of partial deep dermal and full thickness burn wounds. EMEA review began on 11 April 2008 with an active review time of 90 days.
- **Pegylated L-asparaginase,** Enzon (UK) Limited, for treatment of acute lymphoblastic leukaemia. EMEA review began on 13 June 2008 with an active review time of 27 days.
- **Recombinant human CXCL8 mutant**, ProtAffin Biotechnologie AG, for prevention of delayed graft function after solid organ transplantation. EMEA review began on 11 April 2008 with an active review time of 90 days.

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- **Recombinant human minibody against complement component C5,** Adienne S.r.l, for treatment of atypical haemolytic uraemic syndrome (aHUS) associated with an inherited abnormality of the complement system. EMEA review began on 13 June 2008 with an active review time of 27 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.

OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

Lists of questions

The COMP adopted 4 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.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that one of 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.

UPCOMING MEETINGS FOLLOWING THE JULY 2008 COMP PLENARY MEETING

- The ninety-third meeting of the COMP will be held on 9-10 September 2008.

ORGANISATIONAL MATTERS

The main topics addressed during the June 2008 COMP meeting related to:

- The appointment of Dr János Borvendég and Dr Bruno Sepodes as new COMP members nominated by the European Commission on the EMEA's recommendation.
- Discussion on work improvements of the COMP was presented.
- Discussion on valid conditions and subsets.
- Discussion on COMP's Working Group with Interested Parties to be adapted to a new concept of COMP Focus Groups.
- Discussion with the significant benefit ad-hoc group on the revision on the draft Guideline on the 'Elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation' (COMP/1527/03).

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- Three Protocol Assistance letters were 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
2008	54	45	20	-	32
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

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

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
1, 1'-[1,4- phenylenebis (methylene)]-bis-1,4,8,11-tetraazacyclotetradecane	Mozobil	Genzyme BV	EU/3/04/227	Treatment to mobilize progenitor cells prior to stem cell transplantation
3,4 diaminopyridine phosphate	Zenas	EUSA PHARMA SAS	EU/3/02/124	Treatment of Lambert-Eaton myasthenic syndrome

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