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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
NOVEMBER 2008 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its ninety-fifth plenary meeting on 4-5 November 2008. The Committee thanked warmly Dr V. Boudinova (COMP member for Bulgaria), who has left the COMP, for her contribution to the work of the Committee.

The director of the FDA's Office of Orphan Drug Development visits the COMP
The Committee welcomed Dr Tim Coté, director of the FDA's Office of Orphan Drug Development. The Committee discussed with him the future collaboration in the field of rare diseases, particularly the encouragement for cross submissions for orphan designation to the FDA and EMEA, the strengthening of communication between agencies, and the future collaboration for annual reports on development. Both agencies are currently working in collaboration to simplify procedures related to orphan designation and will continue their work in this field.

Dr Coté presented to the COMP three FDA initiatives in the field of Orphan Product Development: an internal analysis of the databases of orphan designations, a new effort to retrospectively find "abandoned orphans of great promise" and a project aimed at stimulating the development of therapies for tropical diseases. This last one seeks to secure FDA and European orphan designations for drug candidates for tropical diseases. While holding fast to the established designation criteria of both agencies, this effort holds much potential for enhancing the attraction of these compounds for further development by industrial partners.

Dialogue between Industry representatives and the COMP
The Committee had the opportunity to meet an Industry delegation from the EBE – EuropaBio Task Force on Orphan Medicinal Products for their second meeting with the COMP. The delegation presented their views on a number of topics related to orphan designations such as protocol assistance procedures, international collaboration in the field of rare diseases and the availability of orphan drugs. The Committee thanked the Industry delegation for the comments and suggestions and will explore any actions that can be taken in relation to these topics. The Chair of the COMP reassured the EBE – EuropaBio Task Force on Orphan Medicinal Products that regular meetings with stakeholders will be maintained and used as a communication channel to address any topics that need to be discussed between Industry and the EMEA-COMP.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted ten 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 8 August 2008 with an active review time of 90 days.

- **2,3,4,5 tetrahydro-2,8-dimethyl-5-[2-(6-methyl-3-pyridinyl)ethyl]-1H-pyrido[4,3-b]indole dihydrochloride,** from Innovative Drug, for treatment of Huntington disease

- **Milatuzumab,** from Immunomedics GmbH, for treatment of chronic lymphocytic leukaemia.

- **Milatuzumab,** from Immunomedics GmbH, for treatment of multiple myeloma.

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- **Recombinant human residue 41 glutamic acid to glutamine variant of Interferon-alfa-2b,** from Creabilis Therapeutics S.p.A. for treatment of Behçet's Disease.

For the following medicines the EMEA review began on 12 September 2008 with an active review time of 55 days.

- **Exon 44 specific phosphorothioate oligonucleotide*,** from Prosensa Therapeutics B.V., for treatment of Duchenne muscular dystrophy.

- **Exon 51 specific phosphorothioate oligonucleotide*,** from Prosensa Therapeutics B.V., for treatment of Duchenne muscular dystrophy.

- **Human anti-intercellular adhesion molecule-1 monoclonal antibody,** from BioInvent International AB, for treatment of multiple myeloma.

- **Pralatrexate,** from European Medical Advisory Services Limited, for treatment of non-papillary transitional cell carcinoma of the urinary bladder.

- **Recombinant human minibody against complement component C5 fused with RGD-motif,** from Adienne S.r.l., for prevention of the ischaemia/reperfusion injury associated with solid organ transplantation.

- **Recombinant human monoclonal antibody to human Nogo-A protein of the IgG4/kappa class,** from Novartis Europharm Limited, for treatment of spinal cord injury.

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 seven 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 hearings

Four oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that four of 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 plenary meeting is provided in **Annex 2**.

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* Name of the active ingredient was revised and adopted at the COMP meeting held on 7 January 2009 (corr.)

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/pharmaceuticals/index_en.htm)

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 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 EMEA website.

UPCOMING MEETINGS FOLLOWING THE NOVEMBER 2008 COMP PLENARY MEETING

- Rare Disease task Force Meeting will be held in Luxembourg on the 13 November 2008.
- The ninety-sixth meeting of the COMP will be held on 9-10 December 2008.

OTHER MATTERS

Other topics addressed during the November COMP meeting related to:

- Dr V. Boudinova (COMP member for Bulgaria), has left the COMP.
- Discussion on the Workshop on Clinical Outcome measures and endpoints for efficacy assessment in spinal muscular atrophy held at the EMEA on 13 October 2008.
- Two 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	98	80	26	-	50
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

MEDICINAL PRODUCTS GRANTED A COMMUNITY DESIGNATION AS ORPHAN MEDICINAL PRODUCT SINCE THE OCTOBER 2008 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	Drotrecogin alfa (activated)
Sponsor	Drugrecure Aps
Orphan Indication	Treatment of acute respiratory distress syndrome
COMP Opinion date	09/07/2008
Orphan Designation date	22/9/2008

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

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Autologous Tumor-Derived gp96 Heat Shock Protein-peptide Complex	Oncophage	Antigenics Therapeutics Limited	EU/3/05/270	Treatment of renal cell carcinoma