EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Monthly Report

Committee for Orphan Medicinal Products (COMP)

5-6 January 2010

The Committee for Orphan Medicinal Products held its 108th meeting on 5-6 January 2010.

Orphan medicinal product designation

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

For the following medicines the review began on 9 October 2009 with an active review time of 90 days:

- **Taliglucerase alfa** for treatment of Gaucher disease, Protalix B.V

For the following medicines the review began on 6 November 2009 with an active review time of 62 days:

- **(1S, 2S, 3R, 4R)-3-(5-Fluoro-2-(3-methyl-4-(4-methylpiperazin-1-yl)-phenylamino)-pyrimidin-4-ylamino)-bicyclo[2.2.1]hept-5-ene-2-carboxamide benzoate)** for treatment of acute myeloid leukaemia, Merck KGaA.
- **Davunetide** for treatment of progressive supranuclear palsy, FGK Representative Service GmbH.
- **Lentiviral vector containing the human *MYO7A* gene** for treatment of retinitis pigmentosa in Usher syndrome 1B, Oxford Biomedica.

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

[1] Correction of 2009 figures, page 4

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted eight 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

Four oral hearings took place.

Appeal

One sponsor submitted the ground for appeal after the negative opinion was adopted on 2 September 2009. The Committee will discuss the grounds at the next COMP meeting prior to adoption of the opinion.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that four applications for orphan medicinal product designation were withdrawn.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[2] 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 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.

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 two opinions recommending to the European Commission that the following orphan medicinal products be kept in the Community registry of orphan medicinal products:

- **Revolade (eltrombopag olamine)** for treatment of idiopathic thrombocytopenic purpura, GlaxoSmithKline Trading Services Limited.

[2] 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)

- **Tepadina (thiotepa)** for conditioning treatment prior to haematopoietic progenitor cell transplantation, Adienne S.r.l.

Upcoming meetings

- The 109th meeting of the COMP will be held on 2-3 February 2010.

Other matters

The main topics addressed during the meeting related to:

- Discussion on the incentives in the paediatric and orphan regulations.
- Two 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

Contact our press officers

Martin Harvey Allchurch or Monika Benstetter

Tel. +44 (0)20 7418 8427

E-mail: press@ema.europa.eu

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
2010	-	4	4	-	-
2009	164	113	23	1	106
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
Total	1059	730	253	14	696

Medicinal products granted a community designation as orphan medicinal product by the European Commission since the December 2009 COMP monthly report

Active substance	6-thioguanine (oral liquid)
Sponsor	Only For Children Pharmaceuticals
Orphan Indication	Treatment of acute lymphoblastic leukaemia
COMP Opinion date	07/10/2009
Orphan Designation date	26/11/2009

Active substance	8-[4-(1-aminocyclobutyl)phenyl]-9-phenyl1,2,4-triazolo[3,4-f][1,6]naphthyridin-3(2H)-one mono-hydrochloride
Sponsor	Merck Sharp & Dohme Limited
Orphan Indication	Treatment of ovarian cancer
COMP Opinion date	07/10/2009
Orphan Designation date	30/11/2009

Active substance	Human MHC non-restricted cytotoxic T-cell line
Sponsor	Abiogen Pharma S.p.A.
Orphan Indication	Treatment of ovarian cancer
COMP Opinion date	07/10/2009
Orphan Designation date	30/11/2009

Active substance	Pegylated carboxyhaemoglobin
Sponsor	Voisin Consulting S.A.R.L.
Orphan Indication	Treatment of sickle cell disease
COMP Opinion date	07/10/2009
Orphan Designation date	26/11/2009

Active substance	Recombinant chimeric monoclonal antibody against CD20
Sponsor	LFB-Biotechnologies
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP Opinion date	07/10/2009
Orphan Designation date	26/11/2009

Active substance	Vaccinia GM-CSF/TK-deactivated virus
Sponsor	Sirius Regulatory Consulting Limited
Orphan Indication	Treatment of hepatocellular carcinoma
COMP Opinion date	07/10/2009
Orphan Designation date	26/11/2009