EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

1-2 June 2010

The Committee for Orphan Medicinal Products held its 113th plenary meeting on 1-2 June 2010.

Orphan medicinal product designation

The COMP adopted 20 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 5 March 2010 with an active review time of 90 days:

- 11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene[1] for treatment of primary myelofibrosis, Voisin Consulting S.A.R.L.
- 11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene for treatment of post-polycythaemia vera myelofibrosis, Voisin Consulting S.A.R.L.
- 11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene for treatment of post-essential thrombocythaemia myelofibrosis, Voisin Consulting S.A.R.L.
- Abarelix for treatment of low-flow priapism, Speciality European Pharma Ltd.
- Autologous dendritic cells pulsed with recombinant human-fusion protein (mucin 1 – glutathione S transferase) coupled to oxidised polymannose[2] for treatment of ovarian cancer, Prima Biomed Europe Ltd.
- Eflornithine for treatment of familial adenomatous polyposis, Cancer Prevention Pharma Ltd.

[1] Revised name
[2] Revised name

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For the following medicines the review began on 12 April 2010 with an active review time of 52 days:

- 1-[2-(Benzo[1,2,5]thiadiazol-5-ylamino)-6-(2,6-dichloro-phenyl)-pyrido[2,3-*d*]pyrimidin-7-yl]-3-*tert*-butyl-urea for treatment of acute myeloid leukaemia, Sanofi Aventis.
- Adenovirus-associated viral vector serotype 10 carrying the human N-sulfoglucosamine sulfohydrolase and sulfatase modifying factor 1 cDNAs for treatment of mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)[3], SANFILIPPO Therapeutics SAS.
- Allogeneic human dermal fibroblasts for treatment of epidermolysis bullosa, Intercytex Ltd.
- Allogeneic T cells encoding an exogenous TK gene for treatment of acute myeloid leukaemia, LTKFarma SAS.
- Autologous bone marrow-derived mononuclear cell fraction for treatment of thromboangiitis obliterans (Buerger's disease), t2cure GmbH.
- Cyclic pyranopterin monophosphate for treatment of molybdenum cofactor deficiency type A, Orphatec Pharmaceuticals GmbH.
- Cysteamine bitartrate (gastroresistant) for treatment of cystinosis, Raptor Pharmaceuticals Europe BV.
- Forodesine for treatment of chronic lymphocytic leukaemia, Mundipharma Research Limited.
- Glutathione-pegylated liposomal doxorubicin hydrochloride[4] for treatment of glioma, to-BBB Technologies BV.
- Heat-killed *Mycobacterium vaccae* (whole cell) for treatment of tuberculosis, Immodulon Therapeutics Ltd.
- Nafamostat mesylate for treatment of cystic fibrosis, Mucokinetica Ltd.
- Recombinant fusion protein consisting of human coagulation factor VIII attached to the Fc domain of human IgG1 for treatment of haemophilia A, Biogen Idec Limited.
- Recombinant porcine VIII (B domain deleted) for treatment of haemophilia A, Inspiration Biopharmaceutical EU Limited[5].
- Vorinostat for treatment of malignant mesothelioma, Merck Sharp & Dohme Limited.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 9 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

4 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

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

[3] Revised name
[4] Revised name
[5] Revised company name

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 Agency's website.

Article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council

No opinions were discussed under this procedure.

Upcoming meetings

- The Informal COMP meeting will be held on 17 June 2010 in Barcelona.
- The 114th meeting of the COMP will be held on 7-8 July 2010.

Other matters

The main topics addressed during the meeting related to:

- 1 protocol assistance letter was 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
2010	72	89	61 (67%)	26 (31%)	2 (2%)	29
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[6]	**1132**	**1079**	**788 (73%)**	**275 (25%)**	**16 (1%)**	**728**

[6] Revised total numbers

Annex 2

Designated orphan medicinal products that have been subject of a new Community marketing authorisation application under the centralised procedure since the May 2010 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Hydrocortisone (modified release tablet)	Plenadren	DuoCort Pharma AB - Sweden	EU/3/06/372	Treatment of adrenal insufficiency