8 December 2011
EMA/COMP/811367/2011
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

6-7 December 2011

The Committee for Orphan Medicinal Products held its 129th plenary meeting on 6-7 December 2011.

Orphan medicinal product designation

The COMP adopted 9 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 September 2011 with an active review time of 90 days:

- **(1S,3S)-3-amino-4-(difluoromethylene) cyclopentanecarboxylic acid hydrochloride** for treatment of West Syndrome, Catalent Pharma Solutions Limited.
- **Nimorazole maleate** for treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy, Conventia Medical LLP.
- **S[+] apomorphine** for treatment of amyotrophic lateral sclerosis, University of Sheffield.
- **Sodium phenylbutyrate** for treatment of carbamoyl-phosphate synthase-1 deficiency, Lucane Pharma SAS.
- **Sodium phenylbutyrate** for treatment of citrullinaemia type 1, Lucane Pharma SAS.
- **Sodium phenylbutyrate** for treatment of ornithine transcarbamylase deficiency, Lucane Pharma SAS.

For the following medicines the review began on 14 October 2011 with an active review time of 55 days:

- **Autologous haematopoietic cells genetically modified with a lentiviral vector containing the human *gp91(phox)* gene** for treatment of X-linked chronic granulomatous disease, Généthon.

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- **Doxycycline hyclate** for treatment of familial amyloid polyneuropathy, Giampaolo Merlini.
- **Human monoclonal antibody against Fas ligand** for treatment of pemphigus, PinCell s.r.l.

Public summaries of opinions will be available on the Agency's website 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 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

2 oral hearings took place.

Detailed information on the orphan designation procedure

An overview of orphan designation procedures since 2000 is provided in Annex 1.

No orphan designation[1] decisions have been given by the European Commission since the last COMP meeting.

Applications for marketing authorisation for orphan medicinal products

Details of those designated orphan medicinal products that have been subject of a new European Union 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.

Upcoming meetings

- The 130th meeting of the COMP will be held on 10-11 January 2012.

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

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/sectors/pharmaceuticals/documents/community-register/html/index_en.htm

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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
2011	150	156	111 (71%)	43 (28%)	2 (1%)	97
2010	174	176	123 (70%)	51 (29%)	2[2] (1%)	128
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	**1384**	**1322**	**961 (73%)**	**343 (26%)**	**18 (1%)**	**924**

[2] One more opinion was re-adopted in 2010 following the appeal to a negative opinion from 2009

Annex 2

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the November 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide	Kalydeco	Vertex Pharmaceuticals (U.K.) Limited	EU/3/08/556	Treatment of cystic fibrosis