16 April 2010
EMA/COMP/159501/2010
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

7-8 April 2010

The Committee for Orphan Medicinal Products held its 111th plenary meeting on 7-8 April 2010.

To increase transparency, the Agency will from May 2010 make public the outcomes of reviews of orphan designation. When an orphan medicine receives marketing authorisation, the Agency will publish a document that will summarise the COMP position on whether the orphan designation should be maintained or revoked. The document will also include a discussion on the justification of significant benefit over other authorised treatments.

These 'review of orphan designation' documents will be published on the Agency's website, linked to the public summary of opinion on orphan designation and the European public assessment report (EPAR).

Orphan medicinal product designation

The COMP adopted 7 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 11 January 2010 with an active review time of 88 days:

- **Octenidine dihydrochloride** for prevention of late-onset sepsis in premature infants of less than or equal to 32 weeks of gestational age, Schülke & Mayr GmbH.
- **Pomalidomide** for treatment of primary myelofibrosis, Celgene Europe Limited.
- **Pomalidomide** for treatment of post-polycythaemia vera myelofibrosis, Celgene Europe Limited.
- **Pomalidomide** for treatment of post-essential thrombocythaemia myelofibrosis, Celgene Europe Limited.

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For the following medicines the review began on 8 February 2010 with an active review time of 60 days:

- **6alpha-ethyl-chenodeoxycholic acid** for treatment of primary biliary cirrhosis, Intercept Pharma.
- **Heparin-activated recombinant human fibroblast growth factor 1 (on a biodegradable device made from alpha-calcium sulphate hemihydrate)** for treatment of traumatic spinal cord injury, BioArctic Neuroscience AB.
- **Tranilast** for prevention of scarring post glaucoma filtration surgery, Altacor Ltd.

Negative opinions

No negative opinions were adopted recommending the refusal of the orphan medicinal product designation

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 3 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

8 oral hearings took place.

Appeal

Currently no appeal procedures are ongoing.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 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 meeting is provided in Annex 2.

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 Agency's website.

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

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

No opinions were adopted under this procedure.

Upcoming meetings

- 10 Years of the Orphan Regulation in Europe Conference will be held on 3-4 May 2010 http://www.ema.europa.eu/pdfs/conferenceflyers/Conference_announcement3-4May2010.pdf
- The 112th meeting of the COMP will be held on 5-6 May 2010.

Other matters

The main topics addressed during the meeting related to:

- The Committee discussed the topics to be included in the agenda of the upcoming informal COMP meeting to be held on 16-17 June 2010 in Barcelona.

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	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	Designations granted by Commission
2010	47	33	20	2	29
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	**1107**	**760**	**269**	**16**	**728**

Annex 2

Medicinal products granted a community designation as orphan medicinal product by the European Commission since the March 2010 COMP monthly report

Active substance	**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**
Sponsor	Merck KGaA
Orphan indication	Treatment of acute myeloid leukaemia
COMP opinion date	06/01/2010
Orphan designation date	23/03/2010

Active substance	**Davunetide**
Sponsor	FGK Representative Service GmbH
Orphan Indication	Treatment of progressive supranuclear palsy
COMP opinion date	06/01/2010
Orphan Designation date	23/03/2010

Active substance	**Lentiviral vector containing the human *MYO7A* gene**
Sponsor	Oxford Biomedica (UK) Ltd
Orphan indication	Treatment of retinitis pigmentosa in Usher syndrome 1B
COMP opinion date	06/01/2010
Orphan designation date	23/03/2010

Active substance	**Taliglucerase alfa**
Sponsor	Protalix B.V.
Orphan indication	Treatment of Gaucher disease
COMP opinion date	06/01/2010
Orphan designation date	23/03/2010

Annex 3

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

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Human C1 inhibitor	Cinryze	ViroPharma SPRL	EU/3/09/668	Treatment of angioedema caused by C1 inhibitor deficiency
Pirfenidone	Esbriet	Intermune Europe Limited	EU/3/04/241	Treatment of idiopathic pulmonary fibrosis