EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

8-9 February 2011

The Committee for Orphan Medicinal Products held its 120th plenary meeting on 8-9 February 2011.

Orphan medicinal product designation

The COMP adopted 13 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 12 November 2010 with an active review time of 90 days:

- **[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'*H*,3*H*-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride]** for treatment of chondrosarcoma, Voisin Consulting S.A.R.L.
- **Apomorphine hydrochloride** for treatment of moderate and severe traumatic brain injury; Dr Elkan Raphael Gamzu.
- **Human anthrax monoclonal antibody** for post-exposure prophylaxis of inhalation anthrax disease; Emergent Sales and Marketing Germany GmbH.
- **Lisuride hydrogenmaleate** for treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension; Sinoxa Pharma UG.
- **S-Nitrosoglutathione** for treatment of pre-eclampsia; Salupont Consulting Ltd.

For the following medicines the review began on 10 December 2010 with an active review time of 62 days:

- **9-*cis*-Retinyl acetate** for treatment of Leber's congenital amaurosis; ORS Oxford Ltd.
- **9-*cis*-Retinyl acetate** for treatment of retinitis pigmentosa; ORS Oxford Ltd.

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- **Adeno-associated viral vector containing the human *ARSB* gene** for treatment of mucopolysaccharidosis type VI (Maroteux-Lamy syndrome); Fondazione Telethon.
- **Adeno-associated viral vector containing the human *NADH dehydrogenase 4* gene** for treatment of Leber's hereditary optic neuropathy; Institut de la Vision.
- **Allogeneic bone marrow stem cells treated *ex vivo* with 16,16-dimethyl prostaglandin E2** for treatment of acute myeloid leukaemia; Fate Therapeutics LTD.
- **Allogeneic umbilical cord blood cells treated *ex vivo* with 16,16-dimethyl prostaglandin E2** for treatment of acute myeloid leukaemia; Fate Therapeutics LTD.
- **Lenalidomide** for treatment of diffuse large B-cell lymphoma; Celgene Europe Limited.
- **Recombinant fusion protein linking human coagulation factor VIIa with human albumin** for treatment of haemophilia B; CSL Behring GmbH.

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 4 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

3 oral hearings took place.

Detailed information on the orphan designation procedure

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

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 1 opinion recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal products:

- **Esbriet (pirfenidone[1])** for treatment of idiopathic pulmonary fibrosis; Intermune Europe Limited.

Upcoming meetings

- The 121st meeting of the COMP will be held on 8-9 March 2011.

Other matters

The main topics addressed during the meeting related to:

- 2 Protocol Assistance letters were adopted.

[1] Name corrected

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
2011	9	27	23 (85%)	4 (15%)	0 (0%)	0
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	**1243**	**1193**	**873 (73%)**	**304 (26%)**	**16 (1%)**	**827**

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