EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

July 2012

The Committee for Orphan Medicinal Products held its 136th plenary meeting on 10-11 July 2012 in Uppsala, Sweden.

Orphan medicinal product designation

The COMP adopted 14 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- **(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro- 1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid** for treatment of Alagille syndrome, Albireo AB
- **(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro- 1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid** for treatment of primary biliary cirrhosis, Albireo AB.
- **Covalently closed DNA plasmids coding for cytomegalovirus *phosphoprotein 65* and *glycoprotein B* genes** for prevention of cytomegalovirus disease in patients with impaired cell mediated immunity deemed at risk, Astellas Pharma Europe B.V.
- **Humanised monoclonal antibody against epidermal growth factor receptor** for treatment of glioma, Abbott Laboratories.
- **Humanised monoclonal antibody against P-selectin** for treatment of sickle cell disease, Quintiles Ireland Ltd.
- **N-Butyldeoxygalactonojirimycin** for treatment of Fabry disease, Actelion Registration Limited.

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- **Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), AOP Orphan Pharmaceuticals AG.
- **Recombinant human monoclonal antibody against activin receptor type IIB** for treatment of inclusion body myositis, Novartis Europharm Limited.

2. Opinions adopted at the first COMP discussion:

- **Elotuzumab** for treatment of multiple myeloma, Bristol-Myers Squibb Pharma EEIG.
- **Ketoconazole** for treatment of Cushing's syndrome, Agenzia Industrie Difesa-Stabilimento Chimico Farmaceutico Militare.
- **Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein** for treatment of cutaneous T-cell lymphoma, AOP Orphan Pharmaceuticals AG.
- **Trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexane acetic acid sodium salt** for treatment of familial chylomicronaemia, Novartis Europharm Limited.
- **Vatreptacog alfa (activated)** for treatment of haemophilia A, Novo Nordisk A/S.
- **Vatreptacog alfa (activated)** for treatment of haemophilia B, Novo Nordisk A/S.

Negative opinion

The Committee noted the withdrawal of the sponsor's intent to appeal to the negative opinion adopted by the COMP on 3 March 2012, recommending the refusal of the orphan medicinal product designation for the following medicine:

- **Tariquidar** for treatment of P-gp positive breast cancer, Avaant Holdings Ltd. The review began on 12 December 2011 with an active review time of 88 days.

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions by the European Commission.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 11 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to the adoption of an 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.

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.

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/health/documents/community-register/html/index_en.htm

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 meeting reports on the EMA website: http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000508.jsp&mid=WC0b01ac0580028d2a.

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:

- **Revestive ([gly2]-recombinant human glucagon-like peptide)** for treatment of short bowel syndrome, NYCOMED DANMARK APS.

Upcoming meetings

- The 137th meeting of the COMP will be held on 4-5 September 2012.

Other matters

The main topics addressed during the meeting related to:

- 2 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 EMA 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	EC designations
2012	103	108	83 (77%)	24 (22%)	1 (1%)	80
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128
2009	164	137	113 (83%)	23 (17%)	0[2] (0%)	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%)	73
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55
2002	80	76	43 (57%)	30 (40%)	2[3] (3%)	49
2001	83	92	64 (70%)	27 (29%)	1 (1%)	64
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14
Total	**1503**	**1432**	**1044 (73%)**	**369 (26%)**	**17 (1%)**	**1015**

[2] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[3] Following a quality assurance exercise it was identified that this figure needed correction

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the April 2012 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide	Treatment of neuroblastoma	Biogenera srl	11 May 2012	4 June 2012
2S, 4R ketoconazole	Treatment of Cushing's syndrome	Cortendo AB	11 May 2012	4 June 2012
Ataluren	Treatment of Becker muscular dystrophy	PTC Therapeutics Limited	11 May 2012	4 June 2012
Eculizumab	Treatment of infection-associated haemolytic uraemic syndrome	Alexion Europe SAS	11 May 2012	4 June 2012
Givinostat	Treatment of Duchenne muscular dystrophy	Italfarmaco S.p.A.	11 May 2012	4 June 2012
Human erythrocytes encapsulating inositol hexaphosphate	Treatment of sickle cell disease	ERYtech Pharma S.A.	11 May 2012	4 June 2012
Levoglutamide	Treatment of sickle cell disease	Emmaus Medical Europe Limited	11 May 2012	4 June 2012
Ramucirumab	Treatment of gastric cancer	Eli Lilly Nederland B.V.	11 May 2012	4 June 2012
Ramucirumab	Treatment of hepatocellular carcinoma	Eli Lilly Nederland B.V.	11 May 2012	4 June 2012
Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene	Treatment of glycogen storage disease type II (Pompe's disease)	TMC Pharma Services Ltd	11 May 2012	4 June 2012
Recombinant human interleukin-7	Treatment of progressive multifocal leukoencephalopathy	CYTHERIS SA	11 May 2012	4 June 2012
Talarozole	Treatment of autosomal recessive congenital ichthyosis	Stiefel Laboratories (Maidenhead) Limited	11 May 2012	4 June 2012
Talarozole	Treatment of keratinopathic ichthyosis	Stiefel Laboratories (Maidenhead) Limited	11 May 2012	4 June 2012
Talarozole	Treatment of recessive X-linked ichthyosis	Stiefel Laboratories (Maidenhead) Limited	11 May 2012	4 June 2012

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the June 2012 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Pomalidomide	Pomalidomide Celgene	Celgene Europe	EU/3/09/672	Treatment of multiple myeloma