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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

June 2012

The Committee for Orphan Medicinal Products held its 135th plenary meeting on 12-13 June 2012.

Orphan medicinal product designation

The COMP adopted 9 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:

- **1-[(2-Chloro-4-methoxyphenoxy)methyl]-4-[(2,6-dichlorophenoxy)methyl]benzene** for prevention of poliomyelitis in patients with immunodeficiencies deemed at risk, ProPhase Development Ltd.
- **Metreleptin** for treatment of Barraquer-Simons syndrome, Aptiv Solutions (UK) Limited.
- **Metreleptin** for treatment of Berardinelli-Seip syndrome, Aptiv Solutions (UK) Limited.
- **Metreleptin** for treatment of familial partial lipodystrophy, Aptiv Solutions (UK) Limited.
- **Metreleptin** for treatment of Lawrence syndrome, Aptiv Solutions (UK) Limited.

2. Opinions adopted at the first COMP discussion:

- **(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 progressive familial intrahepatic cholestasis, Albireo AB.
- **Hexasodium phytate** for treatment of calciphylaxis, Sanifit Laboratoris, S.L.
- **Human apotransferrin** for treatment of congenital hypotransferrinaemia, Sanquin Blood Supply Foundation.

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- **Recombinant human pentraxin-2** for treatment of idiopathic pulmonary fibrosis, Appletree Europe S.à.r.l.

Public summaries of opinions will be available on the EMA 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 8 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

5 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 5 applications for orphan medicinal product designation were withdrawn.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 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 products be kept in the EU registry of orphan medicinal products:

- **Kalydeco** (N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide) for treatment of cystic fibrosis, Vertex Pharmaceuticals (U.K.).

Upcoming meetings

- The 136th meeting of the COMP will be held on 10-11 July 2012 in Uppsala, Sweden.

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

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	91	92	69 (75%)	23 (25%)	0 (0%)	66
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107
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%)	73
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	**1491**	**1416**	**1030 (73%)**	**368 (26%)**	**18 (1%)**	**1001**

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

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
1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea	Treatment of ovarian cancer	Abbott Laboratories	12 April 2012	6 June 2012
Adenovirus-associated vector containing human *Fas-c* gene	Treatment of glioma	Gregory Fryer Associates Ltd	12 April 2012	6 June 2012
Allogeneic human dendritic cells derived from a CD34+ progenitor cell line	Treatment of acute myeloid leukaemia	DCPrime BV	11 January 2012	22 May 2012
Autologous CD34+ cells transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene	Treatment of Wiskott-Aldrich syndrome	Fondazione Telethon	12 April 2012	6 June 2012
Autologous haematopoietic stem cells transduced with lentiviral vector Lenti-D encoding the human *ABCD1* cDNA	Treatment of adrenoleukodystrophy	bluebird bio France	12 April 2012	6 June 2012
Chimeric monoclonal antibody against kappa myeloma antigen	Treatment of multiple myeloma	Gregory Fryer Associates Ltd	11 January 2012	22 May 2012
Chlormethine	Treatment of cutaneous T-cell lymphoma	TMC Pharma Services Ltd	11 January 2012	22 May 2012
Letermovir	Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity	AiCuris GmbH & Co. KG.	12 April 2012	6 June 2012
N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide	Treatment of meningioma	Sirius Regulatory Consulting Limited	12 April 2012	6 June 2012
N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide	Treatment of schwannoma	Sirius Regulatory Consulting Limited	12 April 2012	6 June 2012

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine	Treatment of pancreatic cancer	Bioncotech Therapeutics S.L.	12 April 2012	6 June 2012
Yttrium (^{90}Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10	Treatment of soft tissue sarcoma	Laboratoires OncoTherapy Science France, S.A.R.L	8 March 2012	25 May 2012