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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

May 2012

The Committee for Orphan Medicinal Products held its 134th plenary meeting on 10-11 May 2012.

Orphan medicinal product designation

The COMP adopted 16 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:

- Eculizumab for treatment of infection-associated haemolytic uraemic syndrome, Alexion Europe SAS.
- Levoglutamide for treatment of sickle cell disease, Emmaus Medical Europe Limited.
- Recombinant human interleukin-7 for treatment of progressive multifocal leukoencephalopathy, CYTHERIS SA.
- Talarozole for treatment of autosomal recessive congenital ichthyosis, Stiefel Laboratories (Maidenhead) Limited.
- Talarozole for treatment of keratinopathic ichthyosis, Stiefel Laboratories (Maidenhead) Limited.
- Talarozole for treatment of recessive X-linked ichthyosis, Stiefel Laboratories (Maidenhead) Limited.

2. Opinions adopted at the first COMP discussion:

- 16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide for treatment of neuroblastoma, Biogenera srl.
- 17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5) for treatment of retinitis pigmentosa, Avena Therapeutics Ltd.
- 2S, 4R ketoconazole for treatment of Cushing's syndrome, Cortendo AB.

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- Ataluren for treatment of Becker muscular dystrophy, PTC Therapeutics Limited.
- Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine) for treatment of glioma, Avena Therapeutics Ltd.
- Givinostat for treatment of Duchenne muscular dystrophy, Italfarmaco S.p.A.
- Human erythrocytes encapsulating inositol hexaphosphate for treatment of sickle cell disease, ERYtech Pharma S.A.
- Ramucirumab for treatment of gastric cancer, Eli Lilly Nederland B.V.
- Ramucirumab for treatment of hepatocellular carcinoma, Eli Lilly Nederland B.V.
- Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene for treatment of glycogen storage disease type II (Pompe's disease), TMC Pharma Services Ltd.

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 10 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

6 oral hearings took place.

Appeal

1 sponsor submitted a letter of intention to appeal after the negative opinion was adopted on 11-12 April 2012. Detailed grounds for appeal must be submitted within 90 days of receipt of the opinion.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 3 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:

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

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 2 opinions recommending to the European Commission that the following orphan medicinal products be kept in the EU registry of orphan medicinal products:

- Jakavi ((R)-3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate) for treatment of chronic idiopathic myelofibrosis, Novartis Europharm Limited.
- Jakavi ((R)-3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate) for treatment of myelofibrosis secondary to polycythaemia vera or essential thrombocythaemia, Novartis Europharm Limited.

Upcoming meetings

- The 135th meeting of the COMP will be held on 12-13 June 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	54	75	60 (80%)	15 (20%)	0 (0%)	54
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	**1454**	**1399**	**1021 (73%)**	**360 (26%)**	**18 (1%)**	**989**

[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
(E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt	Treatment of myelodysplastic syndromes	JJGConsultancy Ltd	8 March 2012	26 April 2012
1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H-pyrazolo[3,4 d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one	Treatment of chronic lymphocytic leukaemia	Nexus Oncology Ltd	8 March 2012	26 April 2012
2-Allyl-1-[6-(1-hydroxy-1-methylethyl)pyridin-2-yl]-6-{[4-(4-methylpiperazin-1-yl)phenyl]amino}-1,2-dihydro-3H-pyrazolo[3,4-d]pyrimidin-3-one	Treatment of ovarian cancer	Merck Sharp & Dohme Limited	8 March 2012	26 April 2012
Exon 45 specific phosphorothioate oligonucleotide	Treatment of Duchenne muscular dystrophy	Prosensa Therapeutics B.V.	8 March 2012	26 April 2012
Exon 53 specific phosphorothioate oligonucleotide	Treatment of Duchenne muscular dystrophy	Prosensa Therapeutics B.V.	8 March 2012	26 April 2012
Halofuginone hydrobromide	Treatment of Duchenne muscular dystrophy	Biological Consulting Europe Ltd.	8 March 2012	26 April 2012
Heterologous human adult liver-derived stem cells	Treatment of acute liver failure	Fresenius Medical Care Deutschland GmbH	8 March 2012	26 April 2012
Ketoconazole	Treatment of Cushing's syndrome	Laboratoire HRA Pharma	11 January 2012 (revision adopted on 5 March 2012)	23 April 2012

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino)benzamide	Treatment of neurofibromatosis type 2	Sirius Regulatory Consulting Limited	8 March 2012	26 April 2012
Oleylphosphocholine	Treatment of leishmaniasis	Dafra Pharma International NV	11 January 2012 (revision adopted on 5 March 2012)	23 April 2012
Pegylated recombinant factor VIII	Treatment of haemophilia A	Novo Nordisk A/S	8 March 2012	26 April 2012
Pomalidomide	Treatment of systemic sclerosis	Celgene Europe Limited	8 March 2012	26 April 2012
Recombinant human methionine proinsulin	Treatment of retinitis pigmentosa	ProRetina Therapeutics S.L.	8 March 2012	26 April 2012
Vosaroxin	Treatment of acute myeloid leukaemia	Sunesis Europe Ltd	8 March 2012	26 April 2012