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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

4-5 May 2011

The Committee for Orphan Medicinal Products held its 123rd plenary meeting on 4-5 May 2011.

Orphan medicinal product designation

The COMP adopted 10 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 7 February 2011 with an active review time of 88 days:

- **Fresolimumab** for treatment of focal segmental glomerulosclerosis, Genzyme Europe BV.
- **Pegylated recombinant *Erwinia chrysanthemi* L-asparaginase** for treatment of acute lymphoblastic leukaemia, Alize Pharma II.
- **Peretinoin** for treatment of hepatocellular carcinoma, Kowa Pharmaceutical Europe Co. Ltd.

For the following medicines the review began on 14 March 2011 with an active review time of 53 days:

- **Acadesine** for treatment of multiple myeloma, Advancell - Advanced In Vitro Cell Technologies S.A.
- **Low molecular weight dextran sulfate** for treatment for mobilisation of progenitor cells prior to stem cell transplantation, TikoMed AB.
- **Methyl *O*-4-*O*-[2-[2-[2-[2-[[*N*-[(1*R*)-1-[[4-(aminoiminomethyl)phenyl]methyl]-2-oxo-2-(1-piperidinyl)ethyl]-N^2-[(4-methoxy-2,3,6-trimethylphenyl)sulfonyl]-L-α-asparaginyl-4-aminobutanoyl-N^6-[5-[(3a*S*,4*S*,6a*R*)-hexahydro-2-oxo-1*H*-thieno[3,4-*d*]imidazol-4-yl]-1-oxopentyl]-L-lysyl]amino]ethoxy]ethoxy]ethoxy]ethyl]-2,3-di-*O*-methyl-6-*O*-sulfo-α-D-glucopyranosyl-(1→4)-*O*-2,3-di-*O*-methyl-β-D-glucopyranuronosyl-(1→4)-*O*-2,3,6-tri-*O*-sulfo-α-D-glucopyranosyl-(1→4)-*O*-2,3-di-*O*-methyl-α-L-idopyranuronosyl-(1→4)-3-*O*-methyl-α-D-glucopyranoside 2,6-bis(hydrogen sulfate) octasodium salt** for prevention of ischaemia/reperfusion injury associated with solid organ transplantation, Endotis Pharma.

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- **Mixture of seven synthetic fragments consisting of p21 RAS peptides** for treatment of pancreatic cancer, Targovax AS.
- **N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide dihydrochloride salt** for treatment of post-essential thrombocythaemia myelofibrosis, Cres Pharmaceuticals Limited.
- **N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide dihydrochloride salt** for treatment of post-polycythaemia vera myelofibrosis, Cres Pharmaceuticals Limited.
- **N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide, dihydrochloride salt** for treatment of primary myelofibrosis, Cres Pharmaceuticals Limited.

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 5 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.

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 European Union 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.

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

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

- **Carbaglu (Carglumic acid)** for treatment of isovaleric acidaemia, Orphan Europe S.A.R.L.
- **Carbaglu (Carglumic acid)** for treatment of methylmalonic acidaemia, Orphan Europe S.A.R.L.
- **Carbaglu (Carglumic acid)** for treatment of propionic acidaemia, Orphan Europe S.A.R.L.

Upcoming meetings

- The Informal COMP meeting will be held on 30 May-1 June 2011 in Budapest.
- The 124th meeting of the COMP will be held on 8-9 June 2011.

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	37	53	43 (81%)	10 (19%)	0 (0%)	28
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	**1271**	**1219**	**893 (74%)**	**310 (25%)**	**16 (1%)**	**855**

[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 2011 COMP monthly report

Active substance	**(S)-{8-fluoro-2-2[4-(3-methoxyphenyl)-1-piperazinyl]-3-[2-methoxy-5-(trifluoromethyl)-phenyl]-3,4-dihydro-4-quinazolinyl}acetic acid**
Sponsor	AiCuris GmbH & Co. KG.
Orphan indication	Prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	**Darinaparsin**
Sponsor	Ziopharm Oncology Limited
Orphan indication	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	**Glufosfamide**
Sponsor	Theradex (Europe) Ltd.
Orphan indication	Treatment of pancreatic cancer
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	**Human anthrax monoclonal antibody**
Sponsor	Emergent Sales and Marketing Germany GmbH
Orphan Indication	Treatment of inhalation anthrax disease
COMP opinion date	12 January 2011
Orphan Designation date	15 April 2011

Active substance	**Ombrabulin**
Sponsor	Sanofi Aventis
Orphan indication	Treatment of soft tissue sarcoma
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	R-baclofen
Sponsor	Lakeside Regulatory Consulting Services Ltd
Orphan indication	Treatment of fragile X syndrome
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	Recombinant fusion protein linking human coagulation factor VIIa with human albumin
Sponsor	CSL Behring GmbH
Orphan indication	Treatment of haemophilia A
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	Recombinant thymidine phosphorylase encapsulated in autologous erythrocytes
Sponsor	St George's University of London
Orphan indication	Treatment of mitochondrial neurogastrointestinal encephalomyopathy (MNGIE) due to thymidine phosphorylase deficiency
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA
Sponsor	Voisin Consulting SARL
Orphan indication	Treatment of familial amyloid polyneuropathy
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Active substance	Vorinostat
Sponsor	Merck Sharp & Dohme Limited
Orphan indication	Treatment of multiple myeloma
COMP opinion date	12 January 2011
Orphan designation date	15 April 2011

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the April 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Axitinib	INLYTA	Pfizer Limited	EU/3/10/844	Treatment of renal cell carcinoma