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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

6-8 September 2011

The Committee for Orphan Medicinal Products held its 126th plenary meeting on 6-8 September 2011.

During the meeting the Committee adopted opinions recommending the maintenance of three products as orphan medicinal products at the time of its authorisation. Vyndaqel will be the first medicinal product to be authorised for treatment of familial amyloid polyneuropathy. Mercaptopurine Nova Laboratories is expected to provide significant benefit by addressing the specific needs of the paediatric population. Additional information on these products and the maintenance of their orphan designation at the time of marketing authorisation will be published by the Agency in the form of public summary of opinions after the Decision on Marketing Authorisation is adopted by the European Commission.

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 10 June 2011:

- **2-hydroxyoleic acid** for treatment of glioma, Lipopharma Therapeutics SL (with an active review time of 91 days).
- **Recombinant human minibody against complement component C5** for treatment of primary membranoproliferative glomerulonephritis, ADIENNE S.r.l. (with an active review time of 99 days).

For the following medicines the review began on 11 July 2011:

- **1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1*H*-pyrazol-4-yl]thieno[3,2-*c*]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea** for treatment of acute myeloid leukaemia; Abbott Laboratories (with an active review time of 60 days).

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- **Adeno-associated viral vector containing the human alpha-N-acetylglucosaminidase gene** for treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome); Institut Pasteur (with an active review time of 60 days).
- **Brivanib alaninate** for treatment of hepatocellular carcinoma; Bristol-Myers Squibb Pharma EEIG (with an active review time of 60 days).
- **Clonidine hydrochloride** for prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy; Bioalliance Pharma (with an active review time of 60 days).
- **Gallium (^{68}Ga)-pasireotide tetraxetan** for diagnosis of gastro-entero-pancreatic neuroendocrine tumours; OctreoPharm Sciences GmbH (with an active review time of 60 days).
- **Glycosylation independent lysosomal targeting tagged recombinant human acid alpha glucosidase** for treatment of glycogen storage disease type II (Pompe's disease); BioMarin Europe Ltd (with an active review time of 60 days).
- **Human platelet antigen-1a immunoglobulin** for prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility; Prophylix Pharma AS (with an active review time of 60 days).
- **L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyl-L-tyrosyl-L-valyl-L-valyl-L-threonyl-L-alpha-aspartyl-L-histidyl-S-[1-[(4-carboxycyclohexyl)methyl]-2,5-dioxo-3-pyrrolidinyl]-complex with keyhole limpet hemocyanin** for treatment of glioma; Orphix Consulting GmbH (with an active review time of 60 days).
- **Lenalidomide** for treatment of mantle cell lymphoma; Celgene Europe Limited (with an active review time of 60 days).
- **Mifepristone** for treatment of hypercortisolism (Cushing's syndrome) of endogenous origin; Voisin Consulting S.A.R.L (with an active review time of 68 days).
- **Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid** for treatment of cystic fibrosis; Pharm Research Associates (UK) Limited (with an active review time of 60 days).

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 7 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

9 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 8 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 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:

- **Mercaptopurine Nova Laboratories (mercaptopurine (oral suspension))** for treatment of acute lymphoblastic leukaemia, Nova Laboratories Limited – UK.
- **Plenadren (hydrocortisone (modified release tablet))** for treatment of adrenal insufficiency; DuoCort Pharma AB – Sweden.
- **Vyndaqel (N-methyl D-(2,3,4,5,6-pentahydroxy-hexyl)-ammonium; 2-(3,5-dichloro-phenyl)-benzoxazole-6-carboxylate)** for treatment of familial amyloid polyneuropathy; Pfizer Speciality UK Limited.

Upcoming meetings

- EU Committee of Experts on Rare Diseases (EUCERD) Registry Workshop will be held on 4 October 2011 at the EMA.
- The 127th meeting of the COMP will be held on 5-7 October 2011.

Other matters

The main topics addressed during the meeting related to:

- 2 Protocol Assistance letters were adopted.

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

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	108	112	79 (71%)	32 (28%)	1 (1%)	68
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	**1342**	**1278**	**929 (73%)**	**332 (26%)**	**17 (1%)**	**895**

[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 July 2011 COMP monthly report

Active substance	**5-[1-(2,6-dichlorobenzyl)piperidin-4-ylmethoxy]quinazoline-2,4-diamine dihydrochloride**
Sponsor	Repligen Europe Limited
Orphan indication	Treatment of 5q spinal muscular atrophy
COMP opinion date	10 June 2011
Orphan designation date	30 August 2011

Active substance	**Acadesine**
Sponsor	Advancell - Advanced In Vitro Cell Technologies S.A.
Orphan indication	Treatment of multiple myeloma
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	**Cardiotrophin-1**
Sponsor	Digna Biotech S.L.
Orphan indication	Treatment of acute liver failure
COMP opinion date	10 June 2011
Orphan designation date	30 August 2011

Active substance	**Everolimus**
Sponsor	Novartis Europharm Limited
Orphan indication	Treatment of gastric cancer
COMP opinion date	10 June 2011
Orphan designation date	30 August 2011

Active substance	**Fresolimumab**
Sponsor	Genzyme Europe BV
Orphan indication	Treatment of focal segmental glomerulosclerosis
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	**Human anthrax monoclonal antibody**
Sponsor	Emergent Sales and Marketing Germany GmbH
Orphan indication	Post-exposure prophylaxis of inhalation anthrax disease
COMP opinion date	9 February 2011
Orphan designation date	5 August 2011

Active substance	Hydroxy-propyl-beta-cyclodextrin
Sponsor	Susan French
Orphan indication	Treatment of Niemann-Pick disease, type C
COMP opinion date	10 June 2011
Orphan designation date	30 August 2011

Active substance	Low molecular weight dextran sulfate
Sponsor	TikoMed AB
Orphan Indication	Treatment for mobilisation of progenitor cells prior to stem cell transplantation
COMP opinion date	5 May 2011
Orphan Designation date	5 August 2011

Active substance	**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**
Sponsor	Endotis Pharma
Orphan indication	Prevention of ischaemia/reperfusion injury associated with solid organ transplantation
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	Mixture of seven synthetic fragments consisting of p21 RAS peptides
Sponsor	Targovax AS
Orphan indication	Treatment of pancreatic cancer
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phosphatidylinositol and cholesterol
Sponsor	Lamellar Biomedical Ltd
Orphan indication	Treatment of cystic fibrosis
COMP opinion date	10 June 2011
Orphan designation date	30 August 2011

Active substance	N-{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide
Sponsor	Pfizer Limited
Orphan indication	Treatment of tuberculosis
COMP opinion date	10 June 2011
Orphan designation date	30 August 2011

Active substance	N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide dihydrochloride salt
Sponsor	Cres Pharmaceuticals Limited
Orphan indication	Treatment of post-essential thrombocythaemia myelofibrosis
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide dihydrochloride salt
Sponsor	Cres Pharmaceuticals Limited
Orphan indication	Treatment of post-polycythaemia vera myelofibrosis
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	N-(cyanomethyl)-4-(2-{[4-(morpholin-4-yl)phenyl]amino}pyrimidin-4-yl)benzamide dihydrochloride salt
Sponsor	Cres Pharmaceuticals Limited
Orphan indication	Treatment of primary myelofibrosis
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	Pegylated recombinant *Erwinia chrysanthemi* L-asparaginase
Sponsor	Alize Pharma II
Orphan indication	Treatment of acute lymphoblastic leukaemia
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	Peretinoin
Sponsor	Kowa Pharmaceutical Europe Co. Ltd.
Orphan indication	Treatment of hepatocellular carcinoma
COMP opinion date	5 May 2011
Orphan designation date	5 August 2011

Active substance	Sirolimus
Sponsor	Santen Oy
Orphan indication	Treatment of chronic non-infectious uveitis
COMP opinion date	10 June 2011
Orphan designation date	30 August 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 July 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Bosutinib	Bosulif	Pfizer Limited	EU/3/10/762	Treatment of chronic myeloid leukaemia