10 October 2011
EMA/COMP/811210/2011
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

5-7 October 2011

The Committee for Orphan Medicinal Products held its 127th plenary meeting on 5-7 October 2011.

This month the COMP delivered for a first time ever a positive opinion on orphan designation for the treatment of Leigh syndrome, for which no authorised treatments exist in the EU. This syndrome is a very rare and severe disease caused by mutations in mitochondrial respiratory enzymes, leading mainly to neurological deficits and a poor survival for these patients.

Orphan medicinal product designation

The COMP adopted 12 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 11 July 2011 with an active review time of 89 days:

- **Plerixafor** for adjunctive treatment to cytotoxic therapy in acute myeloid leukaemia, Genzyme Europe B.V.
- **Alpha-tocotrienol quinone** for treatment of Leigh syndrome, Edison Orphan Pharma BV.
- **Pegylated proline-interferon alpha-2b** for treatment of polycythaemia vera, AOP Orphan Pharmaceuticals AG.

For the following medicines the review began on 12 August 2011 with an active review time of 57 days:

- **4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol** for treatment of idiopathic pulmonary fibrosis, Celgene Europe Limited.
- **Adeno-associated viral vector serotype 8 containing the human *AIPL1* gene** for treatment of Leber's congenital amaurosis type 4**.**

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- **Cysteamine** for treatment of cystic fibrosis, NovaBiotics Ltd.
- **Human haptoglobin** for treatment of sickle cell disease, Bio Products Laboratory Ltd.
- **Interferon gamma** for treatment of Friedreich's ataxia, Prof. Roberto Testi.
- **N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine and folic acid** for diagnosis of folate receptor status in ovarian cancer, Endocyte Europe B.V.
- **Nanoliposomal irinotecan** for treatment of pancreatic cancer, Merrimack Pharmaceuticals UK Limited.
- **Resminostat** for treatment of Hodgkin's lymphoma, 4 SC AG.
- **Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine** for treatment of ovarian cancer, Endocyte Europe B.V.

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

5 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 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 monthly report on the Agency's website.

1 Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products http://ec.europa.eu/enterprise/sectors/pharmaceuticals/documents/community-register/html/index_en.htm

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:

- **Soliris** (Eculizumab) for treatment of atypical haemolytic uremic syndrome; Alexion Europe SAS.

Upcoming meetings

- The 128th meeting of the COMP will be held on 8-9 November 2011.
- Joint DIA/EMA/FDA Orphan Drug Designation Workshop will be held on 10 November 2011 in London.

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	117	128	91	36	1	84
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	**1351**	**1294**	**941 (73%)**	**336 (26%)**	**17 (1%)**	**911**

[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 September 2011 COMP monthly report

Active substance	**2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid**
Sponsor	Takeda Global Research and Development Centre (Europe) Ltd
Orphan indication	Treatment of multiple myeloma
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	**20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecine 10-[1,4'-bipiperidine]-1'-carboxylate**
Sponsor	Nektar Therapeutics UK Ltd
Orphan indication	Treatment of ovarian cancer
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	**Dinaciclib**
Sponsor	Merck Sharp & Dohme Limited
Orphan Indication	Treatment of chronic lymphocytic leukaemia
COMP opinion date	7 July 2011
Orphan Designation date	27 September 2011

Active substance	**Eflornithine**
Sponsor	Cancer Prevention Pharma Ltd
Orphan indication	Treatment of neuroblastoma
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	**Genetically modified *Lactococcus lactis* bacteria containing the human *trefoil factor 1* gene**
Sponsor	ActoGeniX N.V.
Orphan indication	Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Heterologous human adult liver-derived stem cells
Sponsor	Fresenius Medical Care Deutschland GmbH
Orphan indication	Treatment of ornithine transcarbamylase deficiency
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Kifunensine
Sponsor	Généthon
Orphan indication	Treatment of alpha-sarcoglycanopathy
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Kifunensine
Sponsor	Généthon
Orphan indication	Treatment of beta-sarcoglycanopathy
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Kifunensine
Sponsor	Généthon
Orphan indication	Treatment of delta-sarcoglycanopathy
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Kifunensine
Sponsor	Généthon
Orphan indication	Treatment of gamma-sarcoglycanopathy
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Macitentan
Sponsor	Actelion Registration Limited
Orphan indication	Treatment of pulmonary arterial hypertension
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	NH_2-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$
Sponsor	PHARMAXON
Orphan indication	Treatment of traumatic spinal cord injury
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Recombinant human galactocerebrosidase
Sponsor	ACE Biosciences A/S
Orphan indication	Treatment of globoid cell leukodystrophy (Krabbe disease)
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Reparixin
Sponsor	Dompé S.p.A.
Orphan indication	Prevention of graft rejection in pancreatic islet transplantation
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Resminostat
Sponsor	4SC AG
Orphan indication	Treatment of hepatocellular carcinoma
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011

Active substance	Smilagenin
Sponsor	Phytopharm plc
Orphan indication	Treatment of amyotrophic lateral sclerosis
COMP opinion date	7 July 2011
Orphan designation date	27 September 2011