14 June 2011
EMA/COMP/399950/2011
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

8-10 June 2011

The Committee for Orphan Medicinal Products held its 124th plenary meeting on 8-10 June 2011.

Mr Yann Le Cam (EURORDIS) and Ms Pascale Auge (Ernst and Young) briefed the Committee on the latest developments of the "CAVOD" project. This is currently subject of discussions in the context of the call for tender concerning the creation of a mechanism for the exchange of knowledge between Member States and European authorities on the scientific assessment of the clinical added value for orphan medicines. The Committee received with great interest the information on the different workshops celebrated in the context of this project and commented on the high interest of advancing in this filed for the benefit of orphan medicinal products.

On the third day the Committee welcomed Ms Virginie Hivert (Orphanet) who presented the last developments of Orphanet and the future projects of improvement and expansion of this exceptional EU tool for rare diseases.

Orphan medicinal product designation

The COMP adopted 7 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 14 March 2011 with an active review time of 89 days:

- **5-[1-(2,6-dichlorobenzyl)piperidin-4-ylmethoxy]quinazoline-2,4-diamine dihydrochloride** for treatment of 5q spinal muscular atrophy; Repligen Europe Limited.

For the following medicines the review began on 11 April 2011 with an active review time of 61 days:

- **Cardiotrophin-1** for treatment of acute liver failure; Digna Biotech S.L.
- **Everolimus** for treatment of gastric cancer; Novartis Europharm Limited.
- **Hydroxy-propyl-beta-cyclodextrin** for treatment of Niemann-Pick disease, type C; Susan French.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

- **Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phosphatidylinositol and cholesterol** for treatment of cystic fibrosis; Lamellar Biomedical Ltd.
- **N-{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide** for treatment of tuberculosis; Pfizer Limited.
- **Sirolimus (Rapamycin)** for treatment of chronic non-infectious uveitis; Santen Oy.

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 11 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

2 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 4 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.

Upcoming meetings

- The 125th meeting of the COMP will be held on 6-8 July 2011.

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

Contact our press officer

Monika Benstetter

Tel. +44 (0)20 7418 8427

E-mail: press@ema.europa.eu

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	60	65	50 (77%)	14 (22%)	1 (1%)	40
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	**1294**	**1231**	**900 (73%)**	**314 (26%)**	**17 (1%)**	**867**

[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 May 2011 COMP monthly report

Active substance	**[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'*H*,3*H*-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride]**
Sponsor	Voisin Consulting S.A.R.L.
Orphan indication	Treatment of chondrosarcoma
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	**9-*cis*-Retinyl acetate**
Sponsor	ORS Oxford Ltd
Orphan indication	Treatment of Leber's congenital amaurosis
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	**9-*cis*-Retinyl acetate**
Sponsor	ORS Oxford Ltd
Orphan Indication	Treatment of retinitis pigmentosa
COMP opinion date	9 February 2011
Orphan Designation date	13 May 2011

Active substance	**Adeno-associated viral vector containing the human *ARSB* gene**
Sponsor	Fondazione Telethon
Orphan indication	Treatment of mucopolysaccharidosis type VI (Maroteux-Lamy syndrome)
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	**Adeno-associated viral vector containing the human *NADH dehydrogenase 4* gene**
Sponsor	Institut de la Vision
Orphan indication	Treatment of Leber's hereditary optic neuropathy
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	Allogeneic bone marrow stem cells treated *ex vivo* with 16,16-dimethyl prostaglandin E2
Sponsor	Fate Therapeutics LTD
Orphan indication	Treatment of acute myeloid leukaemia
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	Allogeneic umbilical cord blood cells treated *ex vivo* with 16,16-dimethyl prostaglandin E2
Sponsor	Fate Therapeutics LTD
Orphan indication	Treatment of acute myeloid leukaemia
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	Apomorphine hydrochloride
Sponsor	Dr Elkan Raphael Gamzu
Orphan indication	Treatment of moderate and severe traumatic brain injury
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	Lenalidomide
Sponsor	Celgene Europe Limited
Orphan indication	Treatment of diffuse large B-cell lymphoma
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	Lisuride hydrogenmaleate
Sponsor	Sinoxa Pharma UG
Orphan indication	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	Recombinant fusion protein linking human coagulation factor VIIa with human albumin
Sponsor	CSL Behring GmbH
Orphan indication	Treatment of haemophilia B
COMP opinion date	9 February 2011
Orphan designation date	13 May 2011

Active substance	S-Nitrosoglutathione
Sponsor	Salupont Consulting Ltd
Orphan indication	Treatment of pre-eclampsia
COMP opinion date	9 February 2011
Orphan designation date	13 May 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 May 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Decitabine	**Dacogen**	Janssen-Cilag International NV	EU/3/06/370	Treatment of acute myeloid leukaemia
Defibrotide	**Defitelio**	Gentium S.p.A.	EU/3/04/211	Prevention of hepatic veno-occlusive disease
Defibrotide	**Defitelio**	Gentium S.p.A.	EU/3/04/212	Treatment of hepatic veno-occlusive disease
Recombinant human factor XIII (composed of two A subunits)	**NovoThirteen**	Novo Nordisk A/S	EU/3/03/179	Treatment of hereditary factor XIII deficiency