EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

2-3 March 2010

The Committee for Orphan Medicinal Products held its 110th plenary meeting on 2-3 March 2010.

On occasion of the last World Rare Disease Day (the last day of February), the FDA and the EMA announced the agreement on accepting the submission of a single annual report from sponsors of orphan products designated for both the US and the EU. More information can be found in the public press release http://www.ema.europa.eu/htms/human/orphans/annualreport.htm.

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 4 December 2009 with an active review time of 90 days:

- **Dexamethasone (40 mg tablet)** for treatment of multiple myeloma, Laboratoires C.T.R.S.
- **N-{2-Chloro-4-[(6,7-dimethoxy-4-quinolyl)oxy]phenyl}-N'-(5-methyl-3-isoxazolyl) urea hydrochloride monohydrate** for treatment of renal cell carcinoma, Aveo Pharma Ltd.
- **Rifapentine** for treatment of tuberculosis, Sanofi Aventis.
- **Synthetic double-stranded siRNA oligonucleotide directed against p53 mRNA** for prevention of delayed graft function after renal transplantation[1], Verius Limited.

For the following medicines the review began on 11 January 2010 with an active review time of 52 days:

- **Adrenomedullin** for treatment of acute lung injury, Prof Dr Stefan Hippenstiel.

[1] Indication revision

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- **Maytansinoid-conjugated humanised monoclonal antibody against CD56** for treatment of Merkel cell carcinoma, ImmunoGen Europe Limited.
- **Pagibaximab** for prevention of sepsis caused by gram positive pathogens in premature infants less than or equal to 34 weeks of gestational age, Omnicare Clinical Research GmbH.
- **Pravastatin[2] / zoledronic acid** for treatment of Hutchinson-Gilford progeria, Prenyl BIO SAS.
- **Recombinant human anti-interferon gamma monoclonal antibody** for treatment of haemophagocytic lymphohistiocytosis, NovImmune B.V.
- **Velaglucerase alfa** for treatment of Gaucher disease, Shire Pharmaceuticals Ireland Limited.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 9 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to adoption of the opinion.

Five oral hearings took place.

Appeal

No appeal procedures are currently ongoing.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 1 application for orphan medicinal product designation was 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[3] 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 community 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.

[2] Name revision
[3] 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

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 list of questions. The responses will be discussed at the next meeting.

Upcoming meetings

- The 111th meeting of the COMP will be held on 7-8 April 2010.

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 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	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	Designations granted by Commission
2010	24	26	13	2	25
2009	164	113	23	1	106
2008	119	86	31	1	73
2007	125	97	19	1	98
2006	104	81	20	2	80
2005	118	88	30	0	88
2004	108	75	22	4	72
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14
Total	**1084**	**753**	**262**	**16**	**724**

Annex 2

Medicinal products granted a community designation as orphan medicinal product by the European Commission since the February 2010 COMP monthly report

Active substance	**2-iminobiotin**
Sponsor	Neurophyxia B.V.
Orphan indication	Treatment of perinatal asphyxia
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	**Beta-artemether / lumefantrine (powder for oral suspension)**
Sponsor	Dafra Pharma International NV
Orphan indication	Treatment of malaria
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	**Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]**
Sponsor	ARIAD Pharma Ltd
Orphan indication	Treatment of chronic myeloid leukaemia
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	**Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]**
Sponsor	ARIAD Pharma Ltd
Orphan indication	Treatment of acute lymphoblastic leukaemia
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	**Brivudine**
Sponsor	RESprotect GmbH
Orphan Indication	Treatment of pancreatic cancer
COMP opinion date	05/11/2009
Orphan Designation date	28/01/2010

Active substance	Ecopipam
Sponsor	Dr Alain Munoz
Orphan indication	Treatment of Lesch-Nyhan disease
COMP opinion date	03/12/2009
Orphan designation date	03/02/2010

Active substance	Fingolimod
Sponsor	Novartis Europharm Limited -
Orphan indication	Treatment of chronic inflammatory demyelinating polyneuropathy
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	Givinostat
Sponsor	Italfarmaco S.p.A.
Orphan indication	Treatment of systemic-onset juvenile idiopathic arthritis
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	Givinostat
Sponsor	Italfarmaco S.p.A.
Orphan indication	Treatment of polycythaemia vera
COMP opinion date	03/12/2009
Orphan designation date	03/02/2010

Active substance	Human monoclonal antibody against *Pseudomonas aeruginosa* IATS-O1
Sponsor	Envestia Limited
Orphan indication	Treatment of pneumonia caused by serotype O1 *Pseudomonas aeruginosa*
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	Lentiviral vector containing the human *ABCA4* gene
Sponsor	Oxford Biomedica (UK) Ltd
Orphan indication	Treatment of Stargardt's disease
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	Lithium citrate tetrahydrate (in reverse-micelle formulation)
Sponsor	Medesis Pharma
Orphan indication	Treatment of Huntington's disease
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	Macitentan
Sponsor	Actelion Registration Limited
Orphan indication	Treatment of idiopathic pulmonary fibrosis
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	Panobinostat
Sponsor	Novartis Europharm Limited
Orphan indication	Treatment of Hodgkin's lymphoma
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	Pegylated recombinant phenylalanine ammonia lyase
Sponsor	BioMarin Europe Ltd.
Orphan indication	Treatment of hyperphenylalaninaemia
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	Pixantrone dimaleate
Sponsor	CTI Life Sciences Ltd
Orphan indication	Treatment of diffuse large B-cell lymphoma
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	Recombinant fusion protein consisting of the extracellular portion of CD95 fused to the Fc part of a human IgG1 molecule
Sponsor	Apogenix GmbH
Orphan indication	Treatment of glioma
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	Recombinant fusion protein linking human coagulation factor IX with human albumin
Sponsor	CSL Behring GmbH
Orphan indication	Treatment of haemophilia B
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	Recombinant human elafin
Sponsor	Proteo Biotech AG
Orphan indication	Treatment of oesophagus carcinoma
COMP opinion date	05/11/2009
Orphan designation date	28/01/2010

Active substance	Recombinant human monoclonal antibody to human interleukin (IL)-17A of the IgG1/k class
Sponsor	Novartis Europharm Limited
Orphan indication	Treatment of chronic non-infectious uveitis
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	Recombinant human vascular endothelial growth factor
Sponsor	NeuroNova AB
Orphan indication	Treatment of amyotrophic lateral sclerosis
COMP opinion date	05/11/2009
Orphan designation date	29/01/2010

Active substance	Recombinant kallikrein inhibitor
Sponsor	Voisin Consulting S.A.R.L.
Orphan indication	Treatment of Netherton syndrome
COMP opinion date	05/11/2009
Orphan designation date	29/01/2010

Active substance	RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a□5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride
Sponsor	AVI BioPharma International Ltd
Orphan indication	Treatment of Duchenne muscular dystrophy
COMP opinion date	03/12/2009
Orphan designation date	02/02/2010

Active substance	*Streptococcus pyogenes* Su strain cells treated with benzylpenicillin
Sponsor	Theradex (Europe) Ltd.
Orphan indication	Treatment of congenital lymphatic malformations
COMP opinion date	05/11/2009
Orphan designation date	29/01/2010

Active substance	Veltuzumab
Sponsor	Immunomedics GmbH
Orphan indication	Treatment of chronic lymphocytic leukaemia
COMP opinion date	05/11/2009
Orphan designation date	29/01/2010

Annex 3

Designated orphan medicinal products that have been subject of a new Community marketing authorisation application under the centralised procedure since the February 2010 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Cyclo {{(E,Z)-(2S,3R,4R)-3-hydroxy-4-methyl-2-(methylamino)nona-6,8-dienoyl}-L-2-aminobutyryl-N-methyl-glycyl-N-methyl-L-leucyl-L-valyl-N-methyl-L-leucyl-L-alanyl-D-alanyl-N-methyl-L-leucyl-N-methyl-L-leucyl-N-methyl-L-valyl}	Luveniq	Lux Biosciences GmbH	EU/3/07/472	Treatment of chronic non-infectious uveitis