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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

8-9 November 2011

The Committee for Orphan Medicinal Products held its 128th plenary meeting on 8-9 November 2011.

Orphan medicinal product designation

The COMP adopted 11 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 12 August 2011 with an active review time of 90 days:

- **N,N'-bis(2-mercaptoethyl)isophthalamide** for treatment of mercury toxicity, CTI Science Ltd[1].
- **Sodium phenylbutyrate** for treatment of 5q spinal muscular atrophy, GMP-Orphan SAS.

For the following medicines the review began on 9 September 2011 with an active review time of 62 days:

- **Adeno-associated viral vector containing the human factor IX gene** for treatment of haemophilia B, Amsterdam Molecular Therapeutics BV.
- **Brentuximab vedotin** for treatment of cutaneous T-cell lymphoma, Takeda Global Research and Development Centre (Europe) Ltd**.**
- **Chimeric locked nucleic acid-deoxynucleoside phosphorothioate-linked oligonucleotide directed against microRNA-451** for treatment of polycythaemia vera, Miragen Therapeutics Europe Ltd.
- **Recombinant homodimer of the human annexin V** for prevention of the ischaemia/reperfusion injury associated with solid organ transplantation, Astellas Pharma Europe B.V.
- **Lipopolysaccharide of *Ochrobactrum intermedium*** for prevention of sepsis in at-risk premature infants of less than or equal to 32 weeks of gestational age, Diomune, S.L.

[1] Corrected sponsor's name

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- **Liposomal combination of cytarabine and daunorubicin** for treatment of acute myeloid leukaemia, Celator UK (Ltd).
- **Mogamulizumab** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), Gregory Fryer Associates Ltd.
- **Ornithine phenylacetate** for treatment of acute liver failure, Dr Ulrich Granzer.
- **Recombinant protein consisting of modified human growth hormone releasing hormone and the translocation and endopeptidase domains of botulinum toxin serotype D** for treatment of acromegaly, Syntaxin 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

2 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[2] 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.

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:

- **Bronchitol** for treatment of cystic fibrosis, Pharmaxis Pharmaceuticals Ltd.

2 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

Upcoming meetings

- The 129th meeting of the COMP will be held on 6-7 December 2011.

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	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	Designations granted by Commission
2011	139	143	102 (71%)	40 (28%)	1 (1%)	97
2010	174	176	123 (70%)	51 (29%)	2[3] (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	**1373**	**1309**	**952 (73%)**	**340 (26%)**	**17 (1%)**	**924**

[3] 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 October 2011 COMP monthly report

Active substance	**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**
Sponsor	Abbott Laboratories
Orphan indication	treatment of acute myeloid leukaemia
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	**2-hydroxyoleic acid**
Sponsor	Lipopharma Therapeutics SL
Orphan indication	Treatment of glioma
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	**Adeno-associated viral vector containing the human alpha-N-acetylglucosaminidase gene**
Sponsor	Institut Pasteur
Orphan Indication	Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome)
COMP opinion date	8 September 2011
Orphan Designation date	27 October 2011

Active substance	**Brivanib alaninate**
Sponsor	Bristol-Myers Squibb Pharma EEIG
Orphan indication	Treatment of hepatocellular carcinoma
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	**Clonidine hydrochloride**
Sponsor	Bioalliance Pharma
Orphan indication	Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	Gallium (^{68}Ga)-pasireotide tetraxetan
Sponsor	OctreoPharm Sciences GmbH
Orphan indication	Diagnosis of gastro-entero-pancreatic neuroendocrine tumours
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	Glycosylation independent lysosomal targeting tagged recombinant human acid alpha glucosidase
Sponsor	BioMarin Europe Ltd
Orphan indication	Treatment of glycogen storage disease type II (Pompe's disease)
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	Human platelet antigen-1a immunoglobulin
Sponsor	Prophylix Pharma AS
Orphan indication	Prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	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
Sponsor	Orphix Consulting GmbH
Orphan indication	Treatment of glioma
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	Lenalidomide
Sponsor	Celgene Europe Limited
Orphan indication	Treatment of mantle cell lymphoma
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	Mifepristone
Sponsor	Voisin Consulting S.A.R.L
Orphan indication	Treatment of hypercortisolism (Cushing's syndrome) of endogenous origin
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	Recombinant human minibody against complement component C5
Sponsor	ADIENNE S.r.l.
Orphan indication	Treatment of primary membranoproliferative glomerulonephritis
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011

Active substance	Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid
Sponsor	Pharm Research Associates
Orphan indication	Treatment of cystic fibrosis
COMP opinion date	8 September 2011
Orphan designation date	27 October 2011