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Committee for Orphan Medicinal Products (COMP)

Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation

January 2012

The Committee for Orphan Medicinal Products held its 130th plenary meeting on 10-11 January 2012.

Orphan medicinal product designation

The COMP adopted 16 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 October 2011 with an active review time of 89 days:

- **(1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate** for treatment of soft tissue sarcoma, Nexus Oncology Ltd.
- **Allogeneic human dendritic cells derived from a CD34+ progenitor cell line** for treatment of acute myeloid leukaemia, DCPrime BV.
- **Carbetocin** for treatment of Prader-Willi syndrome, Ferring Pharmaceuticals A/S.
- **Chlormethine** for treatment of cutaneous T-cell lymphoma, TMC Pharma Services Ltd.
- **Doxycycline hyclate** for treatment of systemic amyloidosis caused by beta-2 microglobulin, Giampaolo Merlini.
- **Sodium nitrite** for treatment of pulmonary arterial hypertension, FGK Representative Service GmbH.

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

- **6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one** for treatment of amyotrophic lateral sclerosis, ICON Clinical Research UK Limited.

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

- **Adeno-associated viral vector of serotype 5 containing the human alanine-glyoxylate aminotransferase gene** for treatment of primary hyperoxaluria type 1, Amsterdam Molecular Therapeutics BV.
- **Chimeric monoclonal antibody against kappa myeloma antigen** for treatment of multiple myeloma, Gregory Fryer Associates Ltd.
- **Glucagon** for treatment of congenital hyperinsulinism, Biodel UK Limited.
- **Heterologous human adult liver-derived stem cells** for treatment of carbamoyl-phosphate synthase-1 deficiency, Fresenius Medical Care Deutschland GmbH.
- **Human monoclonal antibody targeting *Staphylococcus aureus* alpha-toxin** for treatment of pneumonia caused by *Staphylococcus aureus*, Envestia Limited.
- **Ketoconazole** for treatment of Cushing's syndrome, Laboratoire HRA Pharma.
- **Oleylphosphocholine** for treatment of leishmaniasis, Dafra Pharma International NV.
- **Recombinant human beta-glucuronidase** for treatment of mucopolysaccharidosis type VII (Sly syndrome), NDA Regulatory Science Ltd.
- **Sialic acid** for treatment of hereditary inclusion body myopathy, NDA Regulatory Science Ltd.

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 8 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

6 oral hearings took place.

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[1] have been given by the European Commission since the last COMP meeting is provided in Annex 2.

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

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 131st meeting of the COMP will be held on 7-8 February 2012.

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 or Sabine Haubenreisser

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
2012	1	17	16 (94%)	1 (6%)	0 (0%)	11
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107
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	**1401**	**1341**	**977 (73%)**	**346 (26%)**	**18 (1%)**	**945**

[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 December 2011 COMP monthly report

Active substance	**4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol**
Orphan indication	Treatment of idiopathic pulmonary fibrosis
Sponsor	Celgene Europe Limited
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	**Adeno-associated viral vector containing the human factor IX gene**
Orphan indication	Treatment of haemophilia B
Sponsor	Amsterdam Molecular Therapeutics BV
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	**Adeno-associated viral vector serotype 8 containing the human *AIPL1* gene**
Orphan indication	Treatment of Leber's congenital amaurosis type 4
Sponsor	Fondazione Telethon
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	**Alpha-tocotrienol quinone**
Orphan Indication	Treatment of Leigh syndrome
Sponsor	Edison Orphan Pharma BV
COMP opinion date	7 October 2011
Orphan Designation date	9 December 2011

Active substance	**Brentuximab vedotin**
Orphan indication	Treatment of cutaneous T-cell lymphoma
Sponsor	Takeda Global Research and Development Centre (Europe) Ltd
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Chimeric locked nucleic acid-deoxynucleoside phosphorothioate-linked oligonucleotide directed against microRNA-451
Orphan indication	Treatment of polycythaemia vera
Sponsor	Miragen Therapeutics Europe Ltd
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Cysteamine
Orphan indication	Treatment of cystic fibrosis
Sponsor	NovaBiotics Ltd
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	Human haptoglobin
Orphan indication	Treatment of sickle cell disease
Sponsor	Bio Products Laboratory Ltd
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	Interferon gamma
Orphan indication	Treatment of Friedreich's ataxia
Sponsor	Prof. Roberto Testi
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	Lipopolysaccharide of *Ochrobactrum intermedium*
Orphan indication	Prevention of sepsis in at-risk premature infants of less than or equal to 32 weeks of gestational age
Sponsor	Diomune, S.L.
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Liposomal combination of cytarabine and daunorubicin
Orphan indication	Treatment of acute myeloid leukaemia
Sponsor	Celator UK (Ltd)
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Mogamulizumab
Orphan indication	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)
Sponsor	Gregory Fryer Associates Ltd
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	N,N'-bis(2-mercaptoethyl)isophthalamide
Orphan indication	Treatment of mercury toxicity
Sponsor	CTI Science Ltd
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Nanoliposomal irinotecan
Orphan indication	Treatment of pancreatic cancer
Sponsor	Merrimack Pharmaceuticals UK Limited
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	Ornithine phenylacetate
Orphan indication	Treatment of acute liver failure
Sponsor	Dr Ulrich Granzer
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Pegylated proline-interferon alpha-2b
Orphan indication	Treatment of polycythaemia vera
Sponsor	AOP Orphan Pharmaceuticals AG
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	Plerixafor
Orphan indication	Adjunctive treatment to cytotoxic therapy in acute myeloid leukaemia
Sponsor	Genzyme Europe B.V.
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	Recombinant homodimer of the human annexin V
Orphan indication	Prevention of the ischaemia/reperfusion injury associated with solid organ transplantation
Sponsor	Astellas Pharma Europe B.V.
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Recombinant protein consisting of modified human growth hormone releasing hormone and the translocation and endopeptidase domains of botulinum toxin serotype D
Orphan indication	Treatment of acromegaly
Sponsor	Syntaxin Limited
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Active substance	Resminostat
Orphan indication	Treatment of Hodgkin's lymphoma
Sponsor	4 SC AG
COMP opinion date	7 October 2011
Orphan designation date	9 December 2011

Active substance	Sodium phenylbutyrate
Orphan indication	Treatment of 5q spinal muscular atrophy
Sponsor	GMP-Orphan SAS
COMP opinion date	9 November 2011
Orphan designation date	11 January 2012

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the December 2011 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
4-(3,5-bis(hydroxy-phenyl)-1,2,4) triazol-1-yl) benzoic acid	Exjade	Novartis Europharm Limited	EU/3/02/092	Treatment of chronic iron overload requiring chelation therapy
Mercaptopurine	Loulla	Only For Children Pharmaceuticals	EU/3/07/496	Treatment of acute lymphatic leukaemia