EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3 May 2012
EMA/COMP/175202/2012 Rev.
Human Medicines Development and Evaluation

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

April 2012

The Committee for Orphan Medicinal Products held its 133rd plenary meeting on 11-12 April 2012.

Orphan medicinal product designation

The COMP adopted 8 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- **Adenovirus-associated vector containing human *Fas-c* gene** for treatment of glioma, Gregory Fryer Associates Ltd.
- **N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide** for treatment of meningioma, Sirius Regulatory Consulting Limited.
- **N-hydroxy-4-(3-methyl-2-(S)-phenyl-butyrylamino) benzamide** for treatment of schwannoma, Sirius Regulatory Consulting Limited.

2. Opinions adopted at the first COMP discussion:

- **1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1H-pyrazol-4-yl] thieno [3,2-c]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea** for treatment of ovarian cancer, Abbott Laboratories.
- **Autologous CD34+ cells transfected with lentiviral vector containing the Wiskott-Aldrich syndrome protein gene** for treatment of Wiskott-Aldrich syndrome, Fondazione Telethon.
- **Autologous haematopoietic stem cells transduced with lentiviral vector Lenti-D encoding the human *ABCD1* cDNA** for treatment of adrenoleukodystrophy, bluebird bio France.
- **Letermovir** for treatment of cytomegalovirus disease in patients with impaired cell mediated immunity, AiCuris GmbH & Co. KG.[1]

[1] Corrected product details

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

- **Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine** for treatment of pancreatic cancer, Bioncotech Therapeutics S.L.

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 the adoption of an opinion.

Oral hearings

4 oral hearings took place.

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 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 meeting reports on the EMA website: http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000508.jsp&mid=WC0b01ac0580028d2a.

Upcoming meetings

- The 134th meeting of the COMP will be held on 10-11 May 2012.

Other matters

The main topics addressed during the meeting related to:

- 3 Protocol Assistance letters were discussed.

Note

This monthly report, together with other information on the work of the European Medicines Agency, can be found on the EMA website: www.ema.europa.eu

2 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

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	EC designations
2012	49	55	44 (80%)	11 (20%)	0 (0%)	40
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107
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%)	73
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	**1449**	**1379**	**1005 (73%)**	**356 (26%)**	**18 (1%)**	**975**

[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 March 2012 COMP monthly report

Active substance	Adenovirus-associated viral vector serotype 2 containing the human *RPE65* gene
Orphan indication	Treatment of Leber's congenital amaurosis
Sponsor	Alan Boyd Consultants Ltd
COMP opinion date	8 February 2012
Orphan designation date	2 April 2012

Active substance	Adeno-associated viral vector of serotype 5 containing the human alanine-glyoxylate aminotransferase gene
Orphan indication	Treatment of primary hyperoxaluria type 1
Sponsor	Amsterdam Molecular Therapeutics BV
COMP opinion date	11 January 2012
Orphan designation date	21 March 2012

Active substance	Antisense oligonucleotide targeted to the *SMN2* gene
Orphan indication	Treatment of 5q spinal muscular atrophy
Sponsor	Isis USA Ltd
COMP opinion date	8 February 2012
Orphan designation date	2 April 2012

Active substance	Carbetocin
Orphan indication	Treatment of Prader-Willi syndrome
Sponsor	Ferring Pharmaceuticals A/S
COMP opinion date	11 January 2012
Orphan designation date	21 March 2012

Active substance	Dipalmitoylphosphatidylcholine, 1-palmitoyl-2-oleoyl-sn-glycero-3-phosphoglycerol, sodium salt, synthetic surfactant protein C analogue and synthetic surfactant protein B analogue
Orphan indication	Treatment of respiratory distress syndrome in premature neonates of less than 37 weeks of gestational age
Sponsor	Chiesi Farmaceutici S.P.A.
COMP opinion date	8 February 2012
Orphan designation date	2 April 2012

Active substance	Doxycycline hyclate
Orphan indication	Treatment of familial amyloid polyneuropathy
Sponsor	Giampaolo Merlini
COMP opinion date	7 December 2011
Orphan designation date	2 April 2012

Active substance	Genistein sodium salt dihydrate
Orphan indication	Treatment of mucopolysaccharidosis type III (Sanfilippo syndrome)
Sponsor	Axcentua Pharmaceuticals AB
COMP opinion date	8 February 2012
Orphan designation date	2 April 2012

Active substance	Linsitinib
Orphan indication	Treatment of adrenal cortical carcinoma
Sponsor	Astellas Pharma Europe B.V.
COMP opinion date	8 February 2012
Orphan designation date	2 April 2012

Active substance	Melatonin
Orphan indication	Treatment of perinatal asphyxia
Sponsor	Dr Nicola J. Robertson
COMP opinion date	8 February 2012
Orphan designation date	2 April 2012

Active substance	Recombinant human beta-glucuronidase
Orphan indication	Treatment of mucopolysaccharidosis type VII (Sly syndrome)
Sponsor	NDA Regulatory Science Ltd
COMP opinion date	11 January 2012
Orphan designation date	21 March 2012

Active substance	Sodium thiosulfate
Orphan indication	Treatment of calciphylaxis
Sponsor	Aptiv Solutions (UK) Limited
COMP opinion date	8 February 2012
Orphan designation date	2 April 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 March 2012 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
(R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole	Delamanid	Otsuka Novel Products GmbH	EU/3/07/524	Treatment of tuberculosis
(3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione)	Revlimid	Celgene Europe Limited	EU/3/04/192	Treatment of myelodysplastic syndromes
Afamelanotide	Scenesse	Clinuvel (UK) Limited	EU/3/09/648	Treatment of solar urticaria
Cholic Acid	Cholic Acid FGK	FGK Representative Service GmbH	EU/3/09/683	Treatment of inborn errors of primary bile acid synthesis responsive to treatment with cholic acid
Cysteamine bitartrate (gastroresistant)	Cysteamine bitartrate	Raptor Pharmaceuticals Europe B.V.	EU/3/10/778	Treatment of cystinosis
Para-aminosalicylic acid	PAS-GR	Lucane Pharma SA	EU/3/10/826	Treatment of tuberculosis
Sodium phenylbutyrate	Pheburane	Lucane Pharma SA	EU/3/12/951	Treatment of carbamoyl-phosphate synthase-1 deficiency