7 December 2012
EMA/COMP/731762/2012
Human Medicines Development and Evaluation

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

December 2012

The Committee for Orphan Medicinal Products held its 140th plenary meeting on 5-6 December 2012.

Orphan medicinal product designation

The COMP adopted 13 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:

- **Eflornithine in combination with sulindac** for treatment of familial adenomatous polyposis; Cancer Prevention Pharma Limited.
- **Recombinant modified human growth hormone** for treatment of growth hormone deficiency; Richardson Associates Regulatory Affairs Ltd.

2. Opinions adopted at the first COMP discussion:

- **1,2:5,6-Dianhydrogalactitol** for treatment of glioma; IDIS Ltd.
- **Adeno-associated viral vector serotype 9 containing the human *N-acetylglucosaminidase alpha* gene** for treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome); Laboratorios del Dr. Esteve, S.A.
- **Allogeneic motor neuron progenitor cells derived from human embryonic stem cells** for treatment of 5q spinal muscular atrophy; California Stem Cell (UK) Ltd.
- **Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human *beta$^{A\text{-}T87Q}$-globin* gene** for treatment of beta-thalassaemia intermedia and major; bluebird bio France.
- **Chimeric monoclonal antibody against claudin 6** for treatment of ovarian cancer; GANYMED Pharmaceuticals AG.

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

© European Medicines Agency, 2012. Reproduction is authorised provided the source is acknowledged.

- **Choline tetrathiomolybdate** for treatment of Wilson's disease; Medical Need Europe AB.
- **Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor** for treatment of retinitis pigmentosa; Enpharma Ltd.
- **Lenalidomide** for treatment of follicular lymphoma; Celgene Europe Limited.
- **Modified recombinant human C-type natriuretic peptide** for treatment of achondroplasia; BioMarin Europe Ltd.
- **Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen** for treatment of pancreatic cancer; Astellas Pharma Europe B.V.
- **Terguride** for treatment of systemic sclerosis; Serodapharm UG (haftungsbeschränkt).

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation by the European Commission.

Negative opinion

The COMP adopted 1 negative opinion recommending the refusal of the orphan medicinal product designation for a product for treatment of complex regional pain syndrome. The sponsor was informed about the possibility to appeal.

Other information on the orphan medicinal product designation

Lists of questions

The COMP adopted 7 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

3 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.

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.

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/health/documents/community-register/html/index_en.htm

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 141st meeting of the COMP will be held on 8-9 January 2012.

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 EMA 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	EC designations	Orphan medicinal products authorised
2012	186	191	139 (71%)	51 (28%)	1 (1%)	134	11
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4
2009	164	136	113 (83%)	23 (17%)	0[2] (0%)	106	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55	5
2002	80	75	43 (57%)	30 (40%)	2[3] (3%)	49	4
2001	83	90	62[4] (70%)	27 (29%)	1 (1%)	64	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0
Total	**1586**	**1511**	**1098 (73%)**	**396 (26%)**	**17 (1%)**	**1069**	**79**

[2] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[3] Following a quality assurance exercise it was identified that this figure needed correction

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the November 2012 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5)	Treatment of retinitis pigmentosa	Avena Therapeutics Ltd	11 May 2012	28 November 2012
Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-dimethylaminoethylamino-17-demethocygeldanamycin)	Treatment of retinitis pigmentosa	Avena Therapeutics Ltd	4 October 2012	8 November 2012
Alisertib	Treatment of ovarian cancer	Takeda Global Research and Development Centre (Europe) Ltd	4 October 2012	8 November 2012
Canakinumab	Treatment of tumour necrosis factor receptor-associated periodic syndrome	Novartis Europharm Limited	4 October 2012	8 November 2012
Chimeric monoclonal antibody against GD2	Treatment of neuroblastoma	APEIRON Biologics AG.	4 October 2012	8 November 2012
Doxorubicin (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine)	Treatment of glioma	Avena Therapeutics Ltd	11 May 2012	28 November 2012
Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary	Treatment of macular telangiectasia type 2	Enpharma Ltd	4 October 2012	8 November 2012

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
neurotropic factor				
Erdosteine	Treatment of mercury toxicity	Rafifarm SRL	4 October 2012	8 November 2012
IL-12-secreting dendritic cells, loaded with autologous tumour lysate	Treatment of glioma	Activartis Biotech GmbH	4 October 2012	8 November 2012
Ixazomib	Treatment of systemic light chain amyloidosis	Takeda Global Research and Development Centre (Europe) Ltd	4 October 2012	8 November 2012
Melarsoprol	Treatment of African trypanosomiasis	Pr. Peter Kennedy	4 October 2012	8 November 2012
Milciclib maleate	Treatment of malignant thymoma	Nerviano Medical Science Srl	4 October 2012	8 November 2012
Naloxone hydrochloride dihydrate	Treatment of cutaneous T-cell lymphoma	Winston Laboratories Ltd	4 October 2012	8 November 2012
Panobinostat	Treatment of multiple myeloma	Novartis Europharm Limited	4 October 2012	8 November 2012
Recombinant human dyskerin	Treatment of dyskeratosis congenita	Advanced Medical Projects	4 October 2012	8 November 2012
Synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin)	Treatment of glioma	Avena Therapeutics Ltd	4 October 2012	8 November 2012
Tafamidis	Treatment of senile systemic amyloidosis	Pfizer Limited	4 October 2012	8 November 2012
Tralokinumab	Treatment of idiopathic pulmonary fibrosis	MedImmune Ltd	4 October 2012	8 November 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 November 2012 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
(3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid)	Translarna	PTC Therapeutics Ltd.	EU/3/05/278	Treatment of Duchenne muscular dystrophy
(Folic acid to be used with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine)	Neocepri	Endocyte Europe, B.V.	EU/3/12/1044	Diagnosis of positive folate receptor status in ovarian cancer
(N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine to be used with folic acid)	Folcepri	Endocyte Europe, B.V.	EU/3/12/1043	Diagnosis of positive folate receptor status in ovarian cancer
[Cyclopropane-1,1-dicarboxylic acid [4-(6,7-dimethoxy-quinolin-4-yloxy)-phenyl]-amide (4-fluoro-phenyl)-amide, (L)-malate salt]	Cometriq	TMC Pharma Services Ltd.	EU/3/08/610	Treatment of medullary thyroid carcinoma
Macitentan	Opsumit	Actelion Registration Ltd.	EU/3/11/909	Treatment of pulmonary arterial hypertension
Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine	Vynfinit	Endocyte Europe, B.V.	EU/3/12/959	Treatment of ovarian cancer