14 March 2014
EMA/COMP/105712/2014
Committee for Orphan Medicinal Products (COMP)

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

March 2014

The Committee for Orphan Medicinal Products held its 154th plenary meeting on 11-12 March 2014.

Orphan medicinal product designation

Positive opinions

The COMP adopted 6 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission (EC):

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

- **Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta *A-T87Q-globin* gene** for treatment of sickle cell disease; bluebird bio France
- **Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19** for treatment of B-lymphoblastic leukaemia/lymphoma; Novartis Europharm Limited
- **Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor** for treatment of ovarian cancer; Oncos Therapeutics Oy
- **Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues** for treatment of ATTR amyloidosis; Voisin Consulting S.A.R.L.

2. Opinions adopted at the first COMP discussion:

- **Humanised monoclonal antibody against CD38** for treatment of plasma cell myeloma; Sanofi-Aventis Groupe
- **Ibrutinib** for treatment of lymphoplasmacytic lymphoma; Janssen-Cilag International N.V.

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The COMP also reviewed the grounds for their opinions of 6 February 2014 recommending the following medicines for designation as orphan medicinal products to the European Commission:

- **Caffeine citrate** for prevention of bronchopulmonary dysplasia; Viridian Pharma Ltd
- **Recombinant human surfactant protein D** for prevention of bronchopulmonary dysplasia; Dr Ulrich Granzer

Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation[1] by the European Commission.

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 the adoption of an opinion.

Oral hearings

4 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 procedures

An overview of orphan designation procedures since 2000 is provided in Annex 1.

The list of medicinal products for which decisions on orphan designation 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 (EU) marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 3.

Details on the authorised orphan medicinal products can be found on the EMA website.

Article 5(12) (b) 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 product:

- **Vimizim** (Recombinant human N-acetylgalactosamine-6-sulfatase) for treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome); BioMarin Europe Ltd (EU/3/09/657)

[1] Details of all orphan designations granted to date by the European Commission are entered in the EU Register of Orphan Medicinal Products

Appeal opinion

Following the appeal to the COMP opinion of 9 January 2014, the COMP adopted their final opinion recommending to the European Commission that the following orphan medicinal products be kept in the EU registry of orphan medicinal product:

- **Deltyba** ((R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole) for treatment of tuberculosis; Otsuka Novel Products GmbH (EU/3/07/524)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice
- EU Research funding for rare disease research

Upcoming meetings

- The 155th meeting of the COMP will be held on 8-9 April 2014

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

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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	EC designations	Orphan medicinal products[2] authorised	Orphan designations included in authorised therapeutic indication
2014	36	47	34 (72%)	12 (26%)	1 (2%)	28	1	1
2013	201	197	136 (69%)	60 (30%)	1 (1%)	136	7	8
2012	197	192	139 (72%)	52 (27%)	1 (1%)	148	10	12
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4	4
2009	164	136	113 (83%)	23 (17%)	0[3] (0%)	106	9	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6	7
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9	11
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6	6
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55	5	5
2002	80	75	43 (57%)	30 (40%)	2[4] (3%)	49	4	4
2001	83	90	62[4] (70%)	27 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0	0
Total	**1834**	**1756**	**1268 (72%)**	**469 (27%)**	**19 (1%)**	**1247**	**86**	**92**

[2] Number of authorised orphan medicinal products may cover more than one orphan designation
[3] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[4] 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 February 2014 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
11-(4-Dimethylamino-3-hydroxy-6-methyl-tetrahydro-pyran-2-yloxy)-2-ethyl-3,4,10-trihydroxy-3,5,6,8,10,12,14-heptamethyl-1-oxa-6-aza-cyclopentadecane-13,15-dione	Treatment of cystic fibrosis	Synovo GmbH	9 January 2014	19 February 2014
3-Chloro-4-fluorophenyl-[4-fluoro-4-{[(5-methylpyrimidin-2-ylmethyl) amino]methyl}piperidin-1-yl]methanone	Treatment of Rett syndrome	Neurolixis UK Ltd	9 January 2014	19 February 2014
68Ga-2,2'-(7-(4-((S)-1-((4S,7S,10S,13R,16S,19R)-4-((R)-1-amino-3-(4-hydroxyphenyl)-1-oxopropan-2-ylcarbamoyl)-10-(4-aminobutyl)-16-(4-((S)-2,6-dioxohexahydropyrimidine-4-carboxamido)benzyl)-7-((R)-1-hydroxyethyl)-6,9,12,15,18-pentaoxo-13-(4-ureidobenzyl)-1,2-dithia-5,8,11,14,17-pentaazacycloicosan-19-ylamino)-3-(4-chlorophenyl)-1-oxopropan-2-ylamino)-1-carboxy-4-oxobutyl)-1,4,7-triazonane-1,4-diyl)diacetic acid	Diagnosis of gastro-entero-pancreatic neuroendocrine tumours	OctreoPharm Sciences GmbH	9 January 2014	19 February 2014
Asp-Arg-Val-Tyr-Ile-His-Pro	Treatment of Duchenne muscular dystrophy	Gregory Fryer Associates Ltd	9 January 2014	19 February 2014
Autologous dendritic cells pulsed with tumour antigen-derived synthetic peptides (MAGE-1, HER-2, AIM-2, TRP-2, gp-100, and interleukin-13 receptor alpha)	Treatment of glioma	Diamond BioPharm Limited	9 January 2014	19 February 2014

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Cysteamine	Treatment of cystic fibrosis	Istituto Europeo per la Ricerca sulla Fibrosi Cistica – ONLUS	9 January 2014	19 February 2014
Diacerein	Treatment of epidermolysis bullosa	Prof. Johann W. Bauer	9 January 2014	19 February 2014
Eculizumab	Prevention of delayed graft function after solid organ transplantation	Alexion Europe SAS	9 January 2014	19 February 2014
Gallium [Ga-68]-N-[(4,7,10-tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl)acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophanyl-L-lysyl-L-threoninyl-Lcysteinyl-L-threonine-cyclic(2-7)disulfide	Diagnosis of gastro-entero-pancreatic neuroendocrine tumours	Advanced Accelerator Applications SA	9 January 2014	19 February 2014
Mixture of recombinant human IgG1 monoclonal antibodies against human cytomegalovirus envelope glycoproteins	Prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection	Roche Registration Limited	9 January 2014	19 February 2014
N-({Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluoro-phenyl)-ethylamino]-N-isobutyl-acetamide	Treatment of optic neuritis	Bionure Farma SL	15 January 2014	19 February 2014
Phosphorothioate oligonucleotide targeted to apolipoprotein C-III	Treatment of familial chylomicronaemia syndrome	Isis USA Ltd	9 January 2014	19 February 2014
Pioglitazone	Treatment of adrenoleukodystrophy	Minoryx Therapeutics S.L.	9 January 2014	19 February 2014
Recombinant human acid ceramidase	Treatment of Farber disease	QOL Therapeutics EU Ltd	9 January 2014	19 February 2014
Ruxolitinib	Treatment of polycythaemia vera	Novartis Europharm Limited	15 January 2014	19 February 2014

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the February 2014 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Ketoconazole	Treatment of Cushing's syndrome	Laboratoire HRA Pharma	EU/3/12/965