16 January 2014
EMA/COMP/741655/2013
Committee for Orphan Medicinal Products (COMP)

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

January 2014

The Committee for Orphan Medicinal Products held its 152nd plenary meeting on 7-9 January 2014.

Orphan medicinal product designation

Positive opinions

The COMP adopted 15 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:

- **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** for treatment of cystic fibrosis; Synovo GmbH
- **Cysteamine** for treatment of cystic fibrosis; Istituto Europeo per la Ricerca sulla Fibrosi Cistica – ONLUS
- **Diacerein** for treatment of epidermolysis bullosa; Prof. Johann W. Bauer
- **Eculizumab** for prevention of delayed graft function after solid organ transplantation; Alexion Europe SAS
- **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** for diagnosis of gastro-entero-pancreatic neuroendocrine tumours; Advanced Accelerator Applications SA
- **Mixture of recombinant human IgG1 monoclonal antibodies against human cytomegalovirus envelope glycoproteins** for prevention of congenital cytomegalovirus infection following primary cytomegalovirus infection; Roche Registration Limited

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

2. Opinions adopted at the first COMP discussion:

- **3-Chloro-4-fluorophenyl-[4-fluoro-4-{[(5-methylpyrimidin-2-ylmethyl) amino]methyl}piperidin-1-yl]methanone** for treatment of Rett syndrome; Neurolixis UK Ltd
- **^{68}Ga-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** for diagnosis of gastro-entero-pancreatic neuroendocrine tumours; OctreoPharm Sciences GmbH
- **Asp-Arg-Val-Tyr-Ile-His-Pro** for treatment of Duchenne muscular dystrophy; Gregory Fryer Associates Ltd
- **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)** for treatment of glioma; Diamond BioPharm Limited
- **N-({Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluoro-phenyl)-ethylamino]-N-isobutyl-acetamide** for treatment of optic neuritis; Bionure Farma SL
- **Phosphorothioate oligonucleotide targeted to apolipoprotein C-III** for treatment of familial chylomicronaemia syndrome; Isis USA Ltd
- **Pioglitazone** for treatment of adrenoleukodystrophy; Minoryx Therapeutics S.L.
- **Recombinant human acid ceramidase** for treatment of Farber disease; QOL Therapeutics EU Ltd
- **Ruxolitinib** for treatment of polycythaemia vera; Novartis Europharm Limited.

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 11 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

9 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 3 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.

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

No orphan designation decisions have been given by the European Commission since the last COMP meeting report.

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 2.

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 2 opinions recommending to the European Commission that the following orphan medicinal products be kept in the EU registry of orphan medicinal products:

- **Cometriq** [Cyclopropane-1,1-dicarboxylic acid [4-(6,7-dimethoxy-quinolin-4-yloxy)-phenyl]-amide (4-fluoro-phenyl)-amide, (L)-malate salt] for treatment of medullary thyroid carcinoma; TMC Pharma Services Ltd (EU/3/08/610)
- **Sirturo** ((1R,2S) 6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethano)] for treatment of tuberculosis; Janssen-Cilag International N.V. (EU/3/05/314)

The COMP adopted 2 initial opinions recommending to the European Commission that the following orphan medicinal products be removed from the EU registry of orphan medicinal products:

- **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)
- **Para-aminosalicylic acid Lucane** (Para-aminosalicylic acid) for treatment of tuberculosis; Lucane Pharma SA (EU/3/10/826)

The sponsors were informed about the possibility to appeal.

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance
- Discussion on similarity in orphan medicines

Upcoming meetings

- The 153rd meeting of the COMP will be held on 4-6 February 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

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[2] authorised	Orphan designations included in authorised therapeutic indication
2014	0	18	15 (83%)	3 (17%)	0 (0%)	0	0	0
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	**1798**	**1727**	**1249 (72%)**	**460 (27%)**	**18 (1%)**	**1219**	**85**	**91**

[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

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the December 2013 meeting COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Autologous tumour-derived immunoglobulin idiotype coupled to keyhole limpet haemocyanin	Dasiprotimut-T Biovest	Biovest Europe Ltd	EU/3/06/394	Treatment of follicular lymphoma
Chimeric monoclonal antibody against GD2 Dinutuximab	Unituxin	United Therapeutics Europe Ltd	EU/3/11/879	Treatment of neuroblastoma
Human heterologous liver cells (for infusion)	Heparesc	Cytonet GmbH&Co KG	EU/3/10/821	Treatment of carbamoyl-phosphate synthase-1 deficiency
			EU/3/07/470	Treatment of ornithine-transcarbamylase deficiency
			EU/3/10/818	Treatment of citrullinaemia type 1
			EU/3/10/819	Treatment of hyperargininaemia
			EU/3/10/820	Treatment of argininosuccinic aciduria
L-Asparaginase	Spectrila	medac Gesellschaft fuer klinische Spezialpraeparate mbH	EU/3/04/258	Treatment of acute lymphoblastic leukaemia
Levofloxacin hemihydrate	Levofloxacin – Aptalis	Aptalis Pharma SAS	EU/3/08/566	Treatment of cystic fibrosis
Tolvaptan	Jinarc	Otsuka Pharmaceutical Europe Ltd	EU/3/13/1175	Treatment of autosomal dominant polycystic kidney disease