11 April 2014
EMA/COMP/189040/2014
Committee for Orphan Medicinal Products (COMP)

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

April 2014

The Committee for Orphan Medicinal Products held its 155th plenary meeting on 8-9 April 2014.

Orphan medicinal product designation

Positive opinions

The COMP adopted 12 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:

- **(5R,5aR,8aR,9S)-9-[[4,6-O-[(R)-Ethylidene]-β-D-glucopyranosyl]-oxy]-5-(4-({[(2,2-dimethyl-1,3-dioxolan-4-yl)methoxy]carbonyl}oxy)-3,5-dimethoxyphenyl)-5,8,8a,9-tetrahydroisobenzofuro[5,6-f][1,3]benzodioxol-6(5aH)-one** for treatment of biliary tract cancer; CellAct Pharma GmbH
- **^{177}Lu-tetraxetan-tetulomab** for treatment of follicular lymphoma; Nordic Nanovector AS
- **Lutetium (^{177}Lu) edotreotide** for treatment of gastro-entero-pancreatic neuroendocrine tumours; ITG Isotope Technologies Garching GmbH
- **Recombinant human alpha 1 chain homotrimer of type VII collagen** for treatment of epidermolysis bullosa; Shire Pharmaceuticals (Ireland) Limited

2. Opinions adopted at the first COMP discussion:

- **4-(4-Methoxy-phenylamino)-6-methylcarbamyl-quinoline-3-carboxylic acid** for prevention of scarring in post glaucoma filtration surgery; Clanotech AB
- **Adeno-associated viral vector serotype 2 containing the human *CHM* gene encoding human Rab escort protein 1** for treatment of choroideraemia; Alan Boyd Consultants Ltd

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

- **Aganirsen** for treatment of central retinal vein occlusion; Gene Signal SAS
- **Autologous CD34+ cells transduced with a lentiviral vector containing the human *SGSH* gene** for treatment of mucopolysaccharidosis IIIA (Sanfilippo A syndrome); Cochamo Systems Ltd
- **Autologous dendritic cells pulsed with RNA from glioma stem cells** for treatment of glioma; Epitarget AS
- **Isavuconazonium sulfate** for treatment of mucormycosis; Astellas Pharma Europe B.V.
- **Paclitaxel-succinate-Arg-Arg-Leu-Ser-Tyr-Ser-Arg-Arg-Arg-Phe** for treatment of glioma; CLL Pharma
- **Plasmid DNA encoding the human cystic fibrosis transmembrane conductance regulator gene complexed with a non-viral, cationic lipid based gene transfer agent** for treatment of cystic fibrosis; Imperial Innovations 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 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.

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 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.

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

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

- **Folcepri** (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 for diagnosis of positive folate receptor status in ovarian cancer; Endocyte Europe, B.V. (EU/3/12/1043)
- **Neocepri** (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) for diagnosis of positive folate receptor status in ovarian cancer; Endocyte Europe, B.V. (EU/3/12/1044)
- **Sylvant** (Chimeric-anti-interleukin-6 monoclonal antibody) for treatment of Castleman's disease; Janssen-Cilag International N.V. (EU/3/07/508)
- **Vynfinit** (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) for treatment of ovarian cancer; Endocyte Europe, B.V. (EU/3/12/959)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 156th meeting of the COMP will be held on 13-14 May 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	73	60	46 (77%)	13 (22%)	1 (2%)	39	4	4
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	**1871**	**1769**	**1280 (72%)**	**470 (27%)**	**19 (1%)**	**1258**	**89**	**95**

[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 March 2014 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Adeno-associated viral vector serotype 8 containing the human *GUCY2D* gene	Treatment of Leber's congenital amaurosis	Fondazione Telethon	6 February 2014	26 March 2014
Amikacin sulfate	Treatment of nontuberculous mycobacterial lung disease	Insmed Limited	6 February 2014	8 April 2014
Autologous CD34+ cells transduced with a lentiviral vector containing the human *RAG1* gene	Treatment of recombination-activating gene 1 deficient severe combined immunodeficiency	Prof. F.J.T. Staal	6 February 2014	26 March 2014
Cysteamine bitartrate	Treatment of pancreatic cancer	Raptor Pharmaceuticals Europe BV	6 February 2014	26 March 2014
Doxorubicin (6-maleimidocaproyl) hydrazone	Treatment of soft tissue sarcoma	Eudax Srl	6 February 2014	26 March 2014
Eculizumab	Prevention of graft rejection following solid organ transplantation	Alexion Europe SAS	6 February 2014	26 March 2014
Ex-vivo-cultured human mesenchymal stromal cells	Prevention of graft rejection following solid organ transplantation	iCell Science AB	6 February 2014	26 March 2014
Fixed-dose combination of (R-S) baclofen, naltrexone hydrochloride and D-sorbitol	Treatment of Charcot-Marie-Tooth disease type 1A	Pharnext SAS	6 February 2014	26 March 2014
Phosphorothioate oligonucleotide targeted to transthyretin	Treatment of ATTR- amyloidosis	Isis USA Ltd	6 February 2014	26 March 2014
Recombinant human alpha-glucosidase conjugated with multiple copies of synthetic bismannose-6-phosphate-tetra-mannose glycan	Treatment of glycogen storage disease type II (Pompe's disease)	Genzyme Europe BV	6 February 2014	26 March 2014

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Volasertib	Treatment of acute myeloid leukaemia	Boehringer Ingelheim International GmbH	6 February 2014	26 March 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 March 2014 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number