6 September 2013
EMA/COMP/378836/2013
Human Medicines Development and Evaluation

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

September 2013

The Committee for Orphan Medicinal Products held its 148th plenary meeting on 3-4 September 2013.

Orphan medicinal product designation

Positive opinions

The COMP adopted 9 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:

- **Recombinant fusion protein linking coagulation factor VIIa with albumin** for treatment of congenital factor VII deficiency; CSL Behring GmbH
- **Recombinant human monoclonal IgM antibody targeting glucose-regulated protein 78** for treatment of plasma cell myeloma; Patrys GmbH
- **L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser** for treatment of sarcoidosis; Araim Pharma Europe Ltd
- **Mexiletine hydrochloride** for treatment of myotonic disorders; Agenzia Industrie Difesa-Stabilimento Chimico Farmaceutico Militare

2. Opinions adopted at the first COMP discussion:

- **3,5-diiodothyropropionic acid** for treatment of the Allan-Herndon-Dudley syndrome; CATS Consultants GmbH
- **Antisense oligonucleotide targeting the F508delta mutation of CFTR** for treatment of cystic fibrosis; ProQR Therapeutics BV
- **Naproxcinod** for treatment of Duchenne muscular dystrophy; NicOx

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- **Autologous CD34+ cells transduced with a lentiviral vector containing the human Wiskott-Aldrich syndrome gene** for treatment of Wiskott-Aldrich syndrome; Généthon
- **Zoledronic acid** for treatment of complex regional pain syndrome; Axsome Therapeutics 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 10 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

5 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The COMP noted that 3 applications for orphan medicinal product designation were withdrawn.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.

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 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 an opinion recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal products:

- **Defitelio** (defibrotide) for treatment of hepatic veno-occlusive disease; Gentium S.p.A. (EU/3/04/212)

Other matters

The main topics addressed during the meeting related to:

- 10 Protocol Assistance requests were discussed.

Upcoming meetings

- The 149th meeting of the COMP will be held on 8-10 October 2013.

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

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
2013	147	143	98 (69%)	44 (30%)	1 (1%)	101	4	5
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	**1744**	**1655**	**1196 (72%)**	**441 (27%)**	**18 (1%)**	**1184**	**82**	**88**

[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 July 2013 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate	Treatment of pancreatic cancer	Merck KGaA	13 June 2013	17 July 2013
(1R,3R,4R,5S)-3-O-[2-O-benzoyl-3-O-(sodium(2S)-3-cyclohexyl-propanoate-2-yl)-β-D-galactopyranosyl]-4-O-(α-L-fucopyranosyl)-5-orothylamido-cyclohexane-1-carboxylic acid ethyl-2-amidyl-ethyloxy-2-acetyl-(8-amino-1,3,6-naphthalene-tris sodium sulfonate) amide	Treatment of sickle cell disease	Pfizer Limited	11 July 2013	5 August 2013
***(S)*-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one**	Treatment of follicular lymphoma	Voisin Consulting S.A.R.L.	13 June 2013	17 July 2013
Allogeneic motor neuron progenitor cells derived from human embryonic stem cells	Treatment of amyotrophic lateral sclerosis	California Stem Cell (UK) Ltd	13 June 2013	17 July 2013
Apremilast	Treatment of Behçet's disease	Celgene Europe Limited	11 July 2013	5 August 2013
Autologous bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors	Treatment of amyotrophic lateral sclerosis	Brainstorm Cell Therapeutics UK Ltd	13 June 2013	17 July 2013
Belinostat	Treatment of malignant thymomas	TopoTarget A/S	13 June 2013	17 July 2013
Budesonide	Treatment of eosinophilic oesophagitis	Dr Falk Pharma GmbH	11 July 2013	5 August 2013
Chimeric monoclonal antibody against claudin-18 splice variant 2	Treatment of pancreatic cancer	GANYMED Pharmaceuticals AG	11 July 2013	5 August 2013

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Cladribine	Treatment of mastocytosis	Lipomed GmbH	11 July 2013	5 August 2013
Daratumumab	Treatment of plasma cell myeloma	Janssen-Cilag International N.V.	13 June 2013	17 July 2013
Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes	Treatment of ataxia telangiectasia	Erydel S.p.A.	13 June 2013	17 July 2013
Eculizumab	Treatment of neuromyelitis optica	Alexion Europe SAS	11 July 2013	5 August 2013
Ex-vivo expanded autologous human corneal epithelium containing stem cells	Treatment of limbal stem cell deficienc	University Newcastle upon Tyne	13 June 2013	17 July 2013
Fosbretabulin tromethamine	Treatment of ovarian cancer	Diamond BioPharm Limited	13 June 2013	17 July 2013
Granulocyte-macrophage colony-stimulating factor	Treatment of pulmonary alveolar proteinosis	Serendex ApS	13 June 2013	17 July 2013
Heterologous human adult liver-derived progenitor cells	Treatment of argininosuccinic aciduria	Promethera Biosciences	13 June 2013	17 July 2013
Heterologous human adult liver-derived progenitor cells	Treatment of carbamoyl-phosphate synthase-1 deficiency	Promethera Biosciences	13 June 2013	17 July 2013
Heterologous human adult liver-derived progenitor cells	Treatment of citrullinaemia type 1	Promethera Biosciences	13 June 2013	17 July 2013
Heterologous human adult liver-derived progenitor cells	Treatment of citrullinaemia type 2	Promethera Biosciences	13 June 2013	17 July 2013
Heterologous human adult liver-derived progenitor cells	Treatment of hyperargininaemia	Promethera Biosciences	13 June 2013	17 July 2013
Heterologous human adult liver-derived progenitor cells	Treatment of N-acetylglutamate synthetase (NAGS) deficiency	Promethera Biosciences	13 June 2013	17 July 2013
Heterologous human adult liver-derived progenitor cells	Treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome)	Promethera Biosciences	13 June 2013	17 July 2013

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Human allogeneic bone marrow derived osteoblastic-like cells	Treatment of non-traumatic osteonecrosis	Bone Therapeutics SA	11 July 2013	5 August 2013
Human hemin	Prevention of ischaemia/reperfusion injury associated with solid organ transplantation	Borders Technology Management Ltd	13 June 2013	17 July 2013
Idelalisib	Treatment of follicular lymphoma	Gilead Sciences International Ltd	13 June 2013	17 July 2013
Idelalisib	Treatment of lymphoplasmacytic lymphoma	Gilead Sciences International Ltd	13 June 2013	17 July 2013
Idelalisib	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma	Gilead Sciences International Ltd	11 July 2013	5 August 2013
Idelalisib	Treatment of extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma)	Gilead Sciences International Ltd	11 July 2013	5 August 2013
Idelalisib	Treatment of nodal marginal zone lymphoma	Gilead Sciences International Ltd	11 July 2013	5 August 2013
Idelalisib	Treatment of splenic marginal zone lymphoma	Gilead Sciences International Ltd	11 July 2013	5 August 2013
Lipid-complexed cisplatin	Treatment of osteosarcoma	Richardson Associates Regulatory Affairs Ltd	11 July 2013	5 August 2013
Moxetumomab pasudotox	Treatment of B-lymphoblastic leukaemia/lymphoma	MedImmune Ltd	13 June 2013	17 July 2013
Octreotide acetate (oral use)	Treatment of acromegaly	Larode Ltd	11 July 2013	6 August 2013
Pegylated recombinant anti-*Pseudomonas aeruginosa* PcrV Fab' antibody	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis	KaloBios Ltd	11 July 2013	5 August 2013

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains	Treatment of growth hormone deficiency	Larode Ltd	11 July 2013	6 August 2013
Recombinant human monoclonal antibody against hepatitis B virus	Prevention of hepatitis B re-infection following liver transplantation	CRO-PharmaNet Services GmbH	13 June 2013	17 July 2013
Sacrosidase	Treatment of congenital sucrase-isomaltase deficiency	QOL Therapeutics EU Ltd	11 July 2013	5 August 2013
Tolvaptan	Treatment of autosomal dominant polycystic kidney disease	Otsuka Pharmaceutical Europe Ltd	11 July 2013	5 August 2013
Trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride	Treatment of acute myeloid leukaemia	Oryzon Genomics SA	11 July 2013	5 August 2013