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Human Medicines Development and Evaluation

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

June 2013

The Committee for Orphan Medicinal Products held its 146th plenary meeting on 11-13 June 2013.

Orphan medicinal product designation

Positive opinions

The COMP adopted 22 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 bone marrow-derived mesenchymal stromal cells secreting neurotrophic factors** for treatment of amyotrophic lateral sclerosis; Brainstorm Cell Therapeutics UK Ltd
- **Granulocyte-macrophage colony-stimulating factor** for treatment of pulmonary alveolar proteinosis; Serendex ApS
- **Moxetumomab pasudotox** for treatment of B-lymphoblastic leukaemia/lymphoma; MedImmune Ltd.

2. Opinions adopted at the first COMP discussion:

- **(1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl)diamidophosphate** for treatment of pancreatic cancer; Merck KGaA
- ***(S)*-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one** for treatment of follicular lymphoma; Voisin Consulting S.A.R.L.
- **Allogeneic motor neuron progenitor cells derived from human embryonic stem cells** for treatment of amyotrophic lateral sclerosis; California Stem Cell (UK) Ltd
- **Belinostat** for treatment of malignant thymomas; TopoTarget A/S

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- **Daratumumab** for treatment of plasma cell myeloma; Janssen-Cilag International N.V.
- **Dexamethasone sodium phosphate for encapsulation in human autologous erythrocytes** for treatment of ataxia telangiectasia; Erydel S.p.A.
- **Ex-vivo expanded autologous human corneal epithelium containing stem cells** for treatment of limbal stem cell deficiency; University Newcastle upon Tyne
- **Fosbretabulin tromethamine** for treatment of ovarian cancer; Diamond BioPharm Limited
- **Heterologous human adult liver-derived progenitor cells** for treatment of hyperargininaemia; Promethera Biosciences
- **Heterologous human adult liver-derived progenitor cells** for treatment of carbamoyl-phosphate synthase-1 deficiency; Promethera Biosciences
- **Heterologous human adult liver-derived progenitor cells** for treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome); Promethera Biosciences
- **Heterologous human adult liver-derived progenitor cells** for treatment of citrullinaemia type 2; Promethera Biosciences
- **Heterologous human adult liver-derived progenitor cells** for treatment of argininosuccinic aciduria; Promethera Biosciences
- **Heterologous human adult liver-derived progenitor cells** for treatment of citrullinaemia type 1; Promethera Biosciences
- **Heterologous human adult liver-derived progenitor cells** for treatment of N-acetylglutamate synthetase (NAGS) deficiency; Promethera Biosciences
- **Human hemin** for prevention of ischaemia/reperfusion injury associated with solid organ transplantation; Borders Technology Management Ltd
- **Idelalisib** for treatment of follicular lymphoma; Gilead Sciences International Ltd
- **Idelalisib** for treatment of lymphoplasmacytic lymphoma; Gilead Sciences International Ltd
- **Recombinant human monoclonal antibody against hepatitis B virus** for prevention of hepatitis B re-infection following liver transplantation; CRO-PharmaNet Services GmbH

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 13 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

6 oral hearings took place.

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

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 6 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:

- **Pomalidomide Celgene** (Pomalidomide) for treatment of multiple myeloma; Celgene Europe Ltd. (EU/3/09/672)

Other matters

The main topics addressed during the meeting related to:

- 1 Protocol Assistance request was discussed.

Upcoming meetings

- The 147th meeting of the COMP will be held on 9-11 July 2013.

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	87	106	70 (66%)	35 (33%)	1 (1%)	51	1	1
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	**1684**	**1618**	**1168 (72%)**	**432 (27%)**	**18 (1%)**	**1134**	**79**	**84**

[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 May 2013 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
5-[1-(2,6-dichlorobenzyl)piperidin-4-ylmethoxy]quinazoline-2,4-diamine dihydrochloride	Treatment of 5q spinal muscular atrophy	Repligen Sweden AB	17 April 2013	7 June 2013
Allogeneic bone marrow derived mesenchymal cells expanded *ex vivo* in synthetic media	Treatment of graft-versus-host disease	Cell2B Advanced Therapeutics SA	17 April 2013	7 June 2013
Autologous CD34+ cells transduced with a lentiviral vector containing the human *ADA* gene	Treatment of adenosine deaminase-deficient severe combined immunodeficiency	Prof. Bobby Gaspar	17 April 2013	7 June 2013
Inotuzumab ozogamicin	Treatment of B-cell acute lymphoblastic leukaemia	Pfizer Limited	17 April 2013	7 June 2013
Maribavir	Treatment of cytomegalovirus disease in patients with impaired cell mediated immunity	ViroPharma SPRL	23 April 2013	7 June 2013
Mexiletine hydrochloride	Treatment of non-dystrophic myotonia	Prof. Michael Hanna	17 April 2013	7 June 2013
N-[2,6-bis(1-methylethyl)phenyl]-N'-[[1-[4-(dimethylamino) phenyl]cyclopentyl]methyl]urea, hydrochloride salt	Treatment of adrenocortical carcinoma	Atterocor Ltd	17 April 2013	7 June 2013
N-methyl-4-({4-[({3-methyl(methylsulfonyl)aminopyrazin-2-yl}methyl)amino]-5-(trifluoromethyl)pyrimidin-2-yl}amino)benzamide hydrochloride	Treatment of malignant mesothelioma	TMC Pharma Services Ltd	17 April 2013	7 June 2013
Recombinant human CXCL8 mutant	Treatment of cystic fibrosis	ProtAffin Biotechnologie AG	17 April 2013	7 June 2013
Recombinant human nerve growth factor	Treatment of retinitis pigmentosa	Dompé S.p.A.	17 April 2013	7 June 2013

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Recombinant human transglutaminase 1 encapsulated into liposomes	Treatment of transglutaminase-1-deficient autosomal recessive congenital ichthyosis	Westfälische Wilhelms-Universität Münster	17 April 2013	7 June 2013