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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

July 2013

The Committee for Orphan Medicinal Products held its 147th plenary meeting on 9-11 July 2013.

Orphan medicinal product designation

Positive opinions

The COMP adopted 19 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 regulatory T cells with an immunophenotype of CD4+CD25hiFoxP3+** for prevention of graft rejection following solid organ transplantation; iReg Medical AB
- **Idelalisib** for treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma; Gilead Sciences International Ltd
- **Idelalisib** for treatment of extranodal marginal zone lymphoma of mucosa-associated lymphoid tissue (MALT lymphoma); Gilead Sciences International Ltd
- **Idelalisib** for treatment of nodal marginal zone lymphoma; Gilead Sciences International Ltd
- **Idelalisib** for treatment of splenic marginal zone lymphoma; Gilead Sciences International Ltd
- **Lipid-complexed cisplatin** for treatment of osteosarcoma; Richardson Associates Regulatory Affairs Ltd
- **Octreotide acetate (oral use)** for treatment of acromegaly; Larode Ltd
- **Tolvaptan** for treatment of autosomal dominant polycystic kidney disease; Otsuka Pharmaceutical Europe Ltd

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- **Trans-N1-((1R,2S)-2-phenylcyclopropyl)cyclohexane-1,4-diamine bis-hydrochloride** for treatment of acute myeloid leukaemia; Oryzon Genomics SA

2. Opinions adopted at the first COMP discussion:

- **(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** for treatment of sickle cell disease; Pfizer Limited
- **Apremilast** for treatment of Behçet's disease; Celgene Europe Limited
- **Budesonide** for treatment of eosinophilic oesophagitis; Dr Falk Pharma GmbH
- **Chimeric monoclonal antibody against claudin-18 splice variant 2** for treatment of pancreatic cancer; GANYMED Pharmaceuticals AG
- **Cladribine** for treatment of mastocytosis; Lipomed GmbH
- **Eculizumab** for treatment of neuromyelitis optica; Alexion Europe SAS
- **Human allogeneic bone marrow derived osteoblastic-like cells** for treatment of non-traumatic osteonecrosis; Bone Therapeutics SA
- **Pegylated recombinant anti-*Pseudomonas aeruginosa* PcrV Fab' antibody** for treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis; KaloBios Ltd
- **Recombinant human growth hormone modified by fusion with two hydrophilic polypeptide chains** for treatment of growth hormone deficiency; Larode Ltd
- **Sacrosidase** for treatment of congenital sucrase-isomaltase deficiency; QOL Therapeutics EU Ltd.

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 6 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

7 oral hearings took place.

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.

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

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:

- **Procysbi**, cysteamine bitartrate (gastroresistant) for treatment of cystinosis; Raptor Pharmaceuticals Europe B.V. (EU/3/10/778)

Other matters

The main topics addressed during the meeting related to:

- 5 Protocol Assistance requests were discussed.

Upcoming meetings

- The 148th meeting of the COMP will be held on 3-4 September 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	107	131	89 (68%)	41 (31%)	1 (1%)	61	3	4
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	**1704**	**1643**	**1187 (72%)**	**438 (27%)**	**18 (1%)**	**1144**	**81**	**87**

[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 June 2013 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
4,6,4'-trymethylangelicin	Treatment of cystic fibrosis	Rare Partners srl Impresa Sociale	15 May 2013	19 June 2013
Adenovirus associated viral vector serotype 5 containing the human *pde6β* gene	Treatment of retinitis pigmentosa	Centre Hospitalier Universitaire de Nantes	15 May 2013	19 June 2013
Copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin	Treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy	MorEx Development Partners LLP	21 May 2013[5]	27 June 2013
Expanded human allogeneic neural retinal progenitor cells extracted from neural retina	Treatment of retinitis pigmentosa	ReNeuron Ltd	15 May 2013	19 June 2013
Genetically modified serotype 5/3 adenovirus coding for granulocyte-macrophage colony-stimulating factor	Treatment of soft tissue sarcoma	Oncos Therapeutics Ltd	15 May 2013[5]	19 June 2013
Immortalised human C3A hepatoblastoma cells	Treatment of acute liver failure	Vital Therapies Limited	21 May 2013[5]	19 June 2013
Recombinant human alpha-N-acetylglucosaminidase	Treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome)	Synageva BioPharma Ltd	15 May 2013	19 June 2013
Sodium chlorite	Treatment of amyotrophic lateral sclerosis	Shore Limited	15 May 2013	19 June 2013
Synthetic double-stranded siRNA oligonucleotide directed against the keratin 6a N171K mutation	Treatment of pachyonychia congenita	Alan Irvine	21 May 2013[5]	19 June 2013
Unoprostone isopropyl	Treatment of retinitis pigmentosa	Sucampo Pharma Europe Ltd	15 May 2013	19 June 2013

[5] Opinions adopted via written procedure