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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

May 2013

The Committee for Orphan Medicinal Products held its 145th plenary meeting on 14-15 May 2013.

Orphan medicinal product designation

Positive opinions

The COMP adopted 10 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission (EC):

- **4,6,4'-trymethylangelicin** for treatment of cystic fibrosis; Rare Partners srl Impresa Sociale
- **Adenovirus associated viral vector serotype 5 containing the human *pde6β* gene** for treatment of retinitis pigmentosa; Centre Hospitalier Universitaire de Nantes
- **Copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-12,20-dinitroporphyrin** for treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy; MorEx Development Partners LLP
- **Expanded human allogeneic neural retinal progenitor cells extracted from neural retina** for treatment of retinitis pigmentosa; ReNeuron Ltd
- **Genetically modified serotype 5/3 adenovirus coding for granulocyte-macrophage colony-stimulating factor** for treatment of soft tissue sarcoma; Oncos Therapeutics Ltd
- **Immortalised human C3A hepatoblastoma cells** for treatment of acute liver failure; Vital Therapies Limited
- **Recombinant human alpha-N-acetylglucosaminidase** for treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome), Synageva BioPharma Ltd
- **Sodium chlorite** for treatment of amyotrophic lateral sclerosis; Shore Limited

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- **Synthetic double-stranded siRNA oligonucleotide directed against the keratin 6a N171K mutation** for treatment of pachyonychia congenita; Alan Irvine
- **Unoprostone isopropyl** for treatment of retinitis pigmentosa; Sucampo Pharma Europe 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 9 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

3 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 8 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 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.

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

- **Revlimid** (3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione) for treatment of myelodysplastic syndromes; Celgene Europe Limited (EU/3/04/192)

Other matters

The main topics addressed during the meeting related to:

- 2 Protocol Assistance letters were adopted.

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

Upcoming meetings

- The 146th meeting of the COMP will be held on 11-13 June 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	71	78	48 (62%)	29 (37%)	1 (1%)	40	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	**1668**	**1590**	**1146 (72%)**	**426 (27%)**	**18 (1%)**	**1123**	**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 April 2013 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(S)-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one	Treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma	Voisin Consulting S.A.R.L.	13 March 2013	26 April 2013
2-hydroxypropyl-β-cyclodextrin	Treatment of Niemann-Pick disease, type C	International Niemann-Pick Disease Alliance (INPDA)	13 March 2013	26 April 2013
4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate	Treatment of glioma	Eli Lilly Nederland B.V.	13 March 2013	26 April 2013
Lenvatinib	Treatment of follicular thyroid cancer	Eisai Europe Limited	13 March 2013	26 April 2013
Lenvatinib	Treatment of papillary thyroid cancer	Eisai Europe Limited	13 March 2013	26 April 2013
Nintedanib	Treatment of idiopathic pulmonary fibrosis	Boehringer Ingelheim International GmbH	13 March 2013	26 April 2013
R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride	Treatment of Duchenne muscular dystrophy	N-GENE Kutatási és Fejlesztési Kft	13 March 2013	26 April 2013

Annex 3

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

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Obinutuzumab	Gazyva	Roche Registration Limited	EU/3/12/1054	Treatment of chronic lymphocytic leukaemia
Recombinant human N-acetylgalactosamine-6-sulfatase	Vimizim	BioMarin Europe Ltd.	EU/3/09/657	Treatment of mucopolysaccharidosis, type IVA (Morquio A syndrome)