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Committee for Orphan Medicinal Products (COMP)

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

May 2014

The Committee for Orphan Medicinal Products held its 156th plenary meeting on 13-14 May 2014.

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):

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- 1-(2,2-difluoro-1,3-benzodioxol-5-yl)-N-{1-[(2R)-2,3-dihydroxypropyl]-6-fluoro-2-(1-hydroxy-2-methylpropan-2-yl)-1H-indol-5-yl}cyclopropanecarboxamide for treatment of cystic fibrosis; Vertex Pharmaceuticals (U.K.) Limited
- Isavuconazonium sulfate for treatment of invasive aspergillosis; Basilea Medical Ltd.
- Mixture of two adeno-associated viral vectors of serotype 8 containing the 5'-half sequence of human *ABCA4* gene and the 3'-half sequence of human *ABCA4* gene for treatment of Stargardt's disease; Fondazione Telethon
- Mixture of two adeno-associated viral vectors serotype 8 containing the 5'-half sequence of human *MYO7A* gene and the 3'-half sequence of human *MYO7A* gene for treatment of Usher syndrome; Fondazione Telethon

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 2 containing the human *REP1* gene for treatment of choroideraemia; NightstaRx 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

- Afamelanotide for treatment of familial benign chronic pemphigus (Hailey-Hailey disease); Clinuvel UK Limited
- Beloranib for treatment of Prader-Willi syndrome; Dr Ulrich Granzer
- Humanised Fc engineered monoclonal antibody against CD19 for treatment of chronic lymphocytic leukaemia /small lymphocytic lymphoma; MorphoSys AG
- Norursodeoxycholic acid for treatment of primary sclerosing cholangitis; Dr Falk Pharma GmbH
- Recombinant human alpha-1-microglobulin for treatment of pre-eclampsia; A1M Pharma AB

The COMP also reviewed the grounds for their opinion of 9 April 2014 commending the following medicine for designation as orphan medicinal product to the European Commission:

- Autologous CD34+ cells transduced with a lentiviral vector containing the human *SGSH* gene for treatment of mucopolysaccharidosis IIIA (Sanfilippo A syndrome); Cochamo Systems 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 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

6 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 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.

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

- **Nexavar** (Sorafenib tosylate); Bayer HealthCare AG:

a) treatment of follicular thyroid cancer (EU/3/13/1199)

b) treatment of papillary thyroid cancer (EU/3/13/1200)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 157th meeting of the COMP will be held on 10-12 June 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	79	77	56 (73%)	20 (26%)	1 (1%)	47	7	7
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	**1877**	**1786**	**1290 (72%)**	**477 (27%)**	**19 (1%)**	**1266**	**92**	**98**

[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 2014 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human beta *A-T87Q-globin* gene	Treatment of sickle cell disease	bluebird bio France	12 March 2014	29 April 2014
Autologous T cells transduced with lentiviral vector containing a chimeric antigen receptor directed against CD19	Treatment of B-lymphoblastic leukaemia/lymphoma	Novartis Europharm Limited	12 March 2014	29 April 2014
Caffeine citrate	Prevention of bronchopulmonary dysplasia	Viridian Pharma Ltd	6 February 2014	11 April 2014
Genetically modified serotype 5/3 adenovirus coding for granulocyte macrophage colony-stimulating factor	Treatment of ovarian cancer	Oncos Therapeutics Oy	12 March 2014	29 April 2014
Humanised monoclonal antibody against CD38	Treatment of plasma cell myeloma	Sanofi-Aventis Groupe	12 March 2014	29 April 2014
Ibrutinib	Treatment of lymphoplasmacytic lymphoma	Janssen-Cilag International N.V.	12 March 2014	29 April 2014
Recombinant human surfactant protein D	Prevention of bronchopulmonary dysplasia	Dr Ulrich Granzer	6 February 2014	11 April 2014
Synthetic double-stranded siRNA oligonucleotide directed against transthyretin mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues	Treatment of ATTR amyloidosis	Voisin Consulting S.A.R.L.	12 March 2014	29 April 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 April 2014 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Herpes simplex 1 virus-thymidine kinase and truncated low affinity nerve growth factor receptor transfected donor lymphocytes	Adjunctive treatment in haematopoietic cell transplantation	MolMed S.p.A.	EU/3/03/168