25 March 2015
EMA/COMP/138154/2015
Committee for Orphan Medicinal Products (COMP)

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

March 2015

The Committee for Orphan Medicinal Products held its 165th plenary meeting on 17-19 March 2015.

Orphan medicinal product designation

Positive opinions

The COMP adopted 15 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-(4-(N-glycylamido)phenyl)-3-trifluoromethyl-5-(phenanthren-2-yl)-pyrazole-hydrochloride for treatment of cryptococcosis, Arno Therapeutics UK, Limited
- 1-(4-(N-glycylamido)phenyl)-3-trifluoromethyl-5-(phenanthren-2-yl)-pyrazole-hydrochloride for treatment of tularaemia, Arno Therapeutics UK, Limited
- Ecothiophate iodide for treatment of Stargardt's disease, JJGConsultancy Ltd
- Fluciclovine (^{18}F) for diagnosis of glioma, Blue Earth Diagnostics Ltd
- Human reovirus type 3 Dearing strain for treatment of pancreatic cancer, Oncolytics Biotech (UK) Limited
- Lenalidomide for treatment of marginal zone lymphoma, Celgene Europe Limited
- Recombinant monoclonal IgG1 antibody against T-cell immune response cDNA 7for prevention of graft rejection following solid organ transplantation, Nekonal S.a.r.l.
- Rimeporide for treatment of Duchenne muscular dystrophy, EUDRAC Limited
- Rintatolimod for treatment of Ebola virus disease, NV Hemipsherx BioPharma Europe

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2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector serotype 5 containing the human *CHM* gene for treatment of choroideremia, HORAMA SAS
- Nitric oxide for treatment of cystic fibrosis, Biological Consulting Europe Ltd
- Phenol, 4-[2-(aminomethyl)-4-thiazolyl]-2,6-bis(1,1-dimethylethyl) monohydrochloride for treatment of Huntington's disease, Ipsen Pharma
- Recombinant human mesencephalic astrocyte-derived neurotrophic factor for treatment of retinitis pigmentosa, Clinipace GmbH
- Sodium 2-hydroxylinoleate for treatment of neuroblastoma, Ability Pharmaceuticals SL
- Xenon for treatment of perinatal asphyxia, Neuroprotexeon 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 11 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

14 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.

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

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 166th meeting of the COMP will be held on 14-16 April 2015

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

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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
2015	49	90	56 (62%)	34 (38%)	0 (0%)	63	3	3
2014	329	259	196 (76%)	61 (24%)	2 (1%)	187	15	16
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 (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 (3%)	49	4	4
2001	83	90	62 (70%)	27 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0	0
Total	**2176**	**2058**	**1486 (72%)**	**552 (27%)**	**20 (1%)**	**1469**	**103**	**110**

[2] Number of authorised orphan medicinal products may cover more than one orphan designation

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the February 2015 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
5'-$A_SC_SA_ST_SC_SA_SG_ST_SC_ST_SG_SA_SU_SA_SA_SG_SC_ST_SA$-3'	Treatment of Alport syndrome	CTI Clinical Trial and Consulting Services Europe GmbH	12 February 2015	19 March 2015
5,10,15,20-tetrakis(2,6-difluoro-3-N-methylsulfamoylphenyl)bacteriochlorin	Treatment of biliary tract cancer	Luzitin S.A.	12 February 2015	19 March 2015
6-ethoxy-7-methoxy-2-(2-methylsulfanylphenyl)-3,1-benzoxazin-4-one	Treatment of Netherton syndrome	Sixera Pharma AB	12 February 2015	19 March 2015
Adeno-associated viral vector serotype 9 containing the human glucocerebrosidase gene	Treatment of Gaucher disease	Gauchers Association	12 February 2015	19 March 2015
Allogeneic CD4+ and CD8+ T lymphocytes ex vivo incubated with synthetic peptides of the viral antigens of cytomegalovirus, adenovirus and Epstein-Barr virus	Treatment of adenovirus infection following haematopoietic stem cell transplantation	Miltenyi Biotec GmbH	12 February 2015	19 March 2015
Allogeneic CD4+ and CD8+ T lymphocytes ex vivo incubated with synthetic peptides of the viral antigens of cytomegalovirus, adenovirus and Epstein-Barr virus	Treatment of Epstein-Barr virus infection following haematopoietic stem cell transplantation	Miltenyi Biotec GmbH	12 February 2015	19 March 2015
Autologous adipose tissue-derived stromal vascular fraction cells	Treatment of systemic sclerosis	Assistance Publique Hôpitaux de Marseille	12 February 2015	19 March 2015
Chimeric 2'-O-(2-methoxyethyl) modified oligonucleotide targeted to huntingtin RNA	Treatment of Huntington's disease	Isis USA Ltd	12 February 2015	19 March 2015
Enoxacin	Treatment of amyotrophic lateral sclerosis	Impasara Ltd	12 February 2015	19 March 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a *COL7A1*-encoding retroviral vector	Treatment of epidermolysis bullosa	Chiesi Farmaceutici S.p.A.	12 February 2015	19 March 2015
Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a *COL17A*-encoding retroviral vector	Treatment of epidermolysis bullosa	Chiesi Farmaceutici S.p.A.	12 February 2015	19 March 2015
Ex-vivo-expanded autologous human keratinocytes containing epidermal stem cells transduced with a *LAMB3*-encoding retroviral vector	Treatment of epidermolysis bullosa	Chiesi Farmaceutici S.p.A.	12 February 2015	19 March 2015
Gallium (^{68}Ga)-edotreotide	Diagnosis of gastro-entero-pancreatic neuroendocrine tumours	Advanced Accelerator Applications SA	12 February 2015	19 March 2015
Humanised anti-folate receptor 1 monoclonal antibody conjugated to maytansinoid DM4	Treatment of ovarian cancer	ImmunoGen Europe Limited	12 February 2015	19 March 2015
Human reovirus type 3 Dearing strain	Treatment of ovarian cancer	Oncolytics Biotech (UK) Limited	12 February 2015	19 March 2015
Human plasma-derived alpha-1 proteinase inhibitor	Treatment of graft-versus-host disease	Richardson Associates Regulatory Affairs Ltd	12 February 2015	19 March 2015
Lenvatinib	Treatment of hepatocellular carcinoma	Eisai Europe Limited	12 February 2015	19 March 2015
Melphalan flufenamide	Treatment of plasma cell myeloma	Oncopeptides AB	12 February 2015	19 March 2015
Recombinant human club cell 10 KDa protein	Prevention of bronchopulmonary dysplasia	RLM Consulting	12 February 2015	19 March 2015
Recombinant human monoclonal antibody binding to vascular adhesion protein-1	Treatment of primary sclerosing cholangitis	Biotie Therapies Corp	12 February 2015	19 March 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one	Treatment of retinitis pigmentosa	Universitätsklinikum Tübingen (UKT)	12 February 2015	19 March 2015
Tideglusib	Treatment of fragile X syndrome	QRC Consultants Ltd.	12 February 2015	19 March 2015
Trientine tetrahydrochloride	Treatment of Wilson's disease	GMP-Orphan SAS	12 February 2015	19 March 2015
[5-(5-chloro-1H-pyrrolo[2,3-b]pyridin-3-ylmethyl)-pyridin-2-yl]-(6-trifluoromethyl-pyridin-3-ylmethyl)-amine hydrochloride	Treatment of tenosynovial giant cell tumour, localised and diffuse type	Daiichi Sankyo Development Ltd	12 February 2015	19 March 2015

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the February 2015 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Amikacin	Treatment of Pseudomonas aeruginosa lung infection in cystic fibrosis	Insmed Limited	EU/3/06/387
Amikacin	Treatment of nontuberculous mycobacterial lung disease	Insmed Limited	EU/3/14/1259
Carfilzomib	Treatment of multiple myeloma	Amgen Europe B.V.	EU/3/08/548
Dexamethasone acetate	Treatment of multiple myeloma	LABORATOIRES CTRS	EU/3/10/745
Lumacaftor / ivacaftor	Treatment of cystic fibrosis	Vertex Pharmaceuticals (U.K.) Ltd.	EU/3/14/1333
Sirolimus	Treatment of chronic non-infectious uveitis	Santen Oy	EU/3/11/898
Recombinant human lysosomal acid lipase	Treatment of lysosomal acid lipase deficiency	Synageva BioPharma Ltd	EU/3/10/827
Selexipag	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension	Actelion Registration Ltd.	EU/3/05/316