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Procedure Management and Committees Support Division

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

February 2016

The Committee for Orphan Medicinal Products held its 175th plenary meeting on 16-18 February 2016.

Orphan medicinal product designation

Positive opinions

The COMP adopted 17 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:

- Acalabrutinib for treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma, Acerta Pharma, BV;
- Allogeneic Epstein-Barr virus specific cytotoxic T lymphocytes for treatment of post-transplant lymphoproliferative disorder, Wainwright Associates Ltd;
- Fenretinide for treatment of cutaneous T-cell lymphoma, Clinipace GmbH;
- Glucopyranosyl lipid A stable emulsion and recombinant New York esophageal squamous cell carcinoma-1 protein for treatment of soft tissue sarcoma, Pharm Research Associates (UK) Limited;
- Florilglutamic acid (^{18}F) for diagnosis of hepatocellular carcinoma, Piramal Imaging GmbH;
- Florilglutamic acid (^{18}F) for diagnosis of glioma, Piramal Imaging GmbH;
- Fosbretabulin tromethamine for treatment of gastro-entero-pancreatic neuroendocrine tumours, Diamond BioPharm Limited;
- Sindbis virus envelope pseudotyped lentiviral vector encoding New York esophageal squamous cell carcinoma-1 protein for treatment of soft tissue sarcoma, Pharm Research Associates (UK) Limited;

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- Synthetic double-stranded siRNA oligonucleotide directed against hydroxyacid oxidase 1 mRNA and covalently linked to a ligand containing three N-acetylgalactosamine residues for treatment of primary hyperoxaluria, Alnylam UK Limited;
- Ubenimex for treatment of pulmonary arterial hypertension, Eiger Biopharmaceuticals Europe Limited.

2. Opinions adopted at the first COMP discussion:

- Acalabrutinib for treatment of lymphoplasmacytic lymphoma, Acerta Pharma, BV;
- Acalabrutinib for treatment of mantle cell lymphoma, Acerta Pharma, BV;
- Adeno-associated viral vector serotype 5 containing a B-domain deleted variant of human coagulation factor VIII gene for treatment of haemophilia A, BioMarin Europe Ltd.;
- Adeno-associated viral vector serotype 8 encoding human ornithine transcarbamylase for treatment of ornithine transcarbamylase deficiency, Pharma Gateway AB;
- Diaspirin cross-linked haemoglobin for treatment of oesophageal cancer, New B Innovation (UK) Limited;
- Exenatide for treatment of idiopathic intracranial hypertension, Alan Boyd Consultants Ltd;
- N-acetyl-D-mannosamine monohydrate for treatment of GNE myopathy, Escala Therapeutics 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

8 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 5 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 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 opinions recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal product:

- Wakix (1-{3-[3-(4-chlorophenyl)propoxy]propyl}piperidine, hydrochloride) for treatment of narcolepsy, Bioprojet (EU/3/07/459);
- Coagadex (human coagulation factor X) for treatment of hereditary factor X deficiency, BIO PRODUCTS LABORATORY, (EU/3/07/471).

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 176th meeting of the COMP will be held on 21-23 March 2016.

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[2]	Final negative COMP opinions	EC designations	Orphan medicinal products[3] authorised	Orphan designations included in authorised therapeutic indication
2016	28	43	31 (72%)	11 (26%)	0	23	0	0
2015	258	272	177 (65%)	94 (35%)	1 (1%)	190	14	21
2014	329	259	196 (76%)	62 (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%)	37 (40%)	1 (1%)	55	5	5
2002	80	75	43 (57%)	32 (42%)	2 (3%)	49	4	4
2001	83	90	62 (70%)	26 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	3 (10%)	0 (0%)	14	0	0
Total	**2413**	**2278**	**1638 (72%)**	**618 (27%)**	**21 (1%)**	**1619**	**114**	**128**

[2] Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000
[3] 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 January 2016 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
2-ethylbutyl (2S)-2-{[(S)-{[(2R,3S,4R,5R)-5-(4-aminopyrrolo[2,1-f][1,2,4]triazin-7-yl)-5-cyano-3,4-dihydroxytetrahydrofuran-2-yl]methoxy}(phenoxy)phosphoryl]amino}propanoate	Treatment of Ebola virus disease	Gilead Sciences International Ltd	21 January 2016	17 February 2016
Allogeneic fetal human retinal progenitor cells expanded ex vivo	Treatment of retinitis pigmentosa	Voisin Consulting S.A.R.L.	21 January 2016	17 February 2016
Arsenic trioxide	Treatment of acute myeloid leukaemia	Orsenix Holdings BV	21 January 2016	17 February 2016
Delta-9-tetrahydrocannabinol and cannabidiol from extracts of the Cannabis sativa L. plant	Treatment of glioma	GW Research Ltd	21 January 2016	17 February 2016
Diclofenamide	Treatment of periodic paralysis	Prof Michael Hanna	21 January 2016	17 February 2016
DNA plasmid encoding a recombinant fusion protein consisting of the extracellular domain of human TNFα p55 receptor linked to the human IgG1 Fc domain	Treatment of non-infectious uveitis	Eyevensys SA	21 January 2016	17 February 2016
Ex-vivo-expanded autologous fibroblasts transduced with lentiviral vector containing the *COL7A1* gene	Treatment of epidermolysis bullosa	Dr Waseem Qasim	21 January 2016	17 February 2016
Humanised IgG4 monoclonal antibody against total complement component 1, subcomponents	Treatment of autoimmune haemolytic anaemia	Assign Group Development UK Ltd	21 January 2016	17 February 2016

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Methyl 3-((2R)-2-hydroxy-4-(((((S)-1-ethoxy-1-oxopropan-2-yl) amino)(phenoxy)phosphoryl)oxy)-3,3-dimethylbutanamido)propanoate	Treatment of pantothenate-kinase-associated neurodegeneration	Retrophin Europe Limited	21 January 2016	17 February 2016
N-(4-Methoxyphenyl)-N,2,6-trimethylfuro[2,3-d]pyrimidin-4-amine	Treatment of glioma	FLAG Therapeutics Ltd	21 January 2016	17 February 2016
S3,S13-cyclo(D-tyrolsyl-L-isoleucyl-L-cysteinyl-L-valyl-1-methyl-L-tryptophyl-L-glutaminyl-L-aspartyl-L-tryptophyl-N-methyl-L-glycyl-L-alanyl-L-histidyl-L-arginyl-L-cysteinyl-N-methyl-L-isoleucinamide)	Treatment of C3 glomerulopathy	Amyndas Pharmaceuticals S.A.	21 January 2016	17 February 2016
Tolfenamic acid	Treatment of progressive supranuclear palsy	RV Developpement	21 January 2016	17 February 2016
Tolfenamic acid	Treatment of behavioural variant frontotemporal dementia	RV Developpement	21 January 2016	17 February 2016
Venetoclax	Treatment of acute myeloid leukaemia	Abbvie Ltd	21 January 2016	17 February 2016

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the January 2016 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Nonacog beta pegol	Treatment of haemophilia B	Novo Nordisk A/S	EU/3/09/640