22 July 2015
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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

July 2015

The Committee for Orphan Medicinal Products held its 169th plenary meeting on 14-16 July 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:

- Glycyl-L-2-methylprolyl-L-glutamic acid for treatment of Rett syndrome, QRC Consultants Ltd.
- Human allogeneic bone-marrow-derived osteoblastic cells for treatment of osteogenesis imperfecta, Bone Therapeutics SA
- Insulin human for treatment of short bowel syndrome, Sirius Regulatory Consulting Limited

2. Opinions adopted at the first COMP discussion:

- (S)-6-hydroxy-2,5,7,8-tetramethyl-N-((R)-piperidin-3-yl)chroman-2-carboxamide hydrochloride for treatment of mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes, Khondrion BV
- 2-(2-phenylvinyl)-4-[4- methylpiperazin-1-yl)]-6-(5-methyl-2H-pyrazol-3-yl-amino)-pyrimidine L(+) tartrate salt for treatment of hepatocellular carcinoma, Dr Ulrich Granzer;
- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)- 2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl -(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidilyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxythymidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-

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phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyguanosylyl-2'-deoxycytidylyl-(3',5'-phosphoryl)-2'-deoxyadenosyl-(3',5'-phosphoryl)-2'-deoxycytidine, sodium salt for treatment of diffuse large B-cell lymphoma, PhaRA bvba;

- Adeno-associated viral vector serotype 8 containing the human *MTM1* gene for treatment of X-linked myotubular myopathy, Audentes Therapeutics UK Limited
- Adeno-associated viral vector serotype 9 containing the human iduronate-2-sulfatase gene for treatment of mucopolysaccharidosis type II (Hunter's syndrome), Laboratorios del Dr. Esteve, S.A.
- Allogeneic umbilical cord blood cells treated ex vivo with 16,16-dimethyl prostaglandin E2 for treatment of acute lymphoblastic leukaemia, Fate Therapeutics, LTD
- CD33-directed antibody-drug conjugate consisting of an antibody conjugated to a DNA cross-linking pyrrolobenzodiazepine dimer drug for treatment of acute myeloid leukaemia, Seattle Genetics UK, Limited
- Fibrinogen-coated albumin spheres for treatment of acute radiation syndrome, Fibreu Limited;
- Fixed-dose combination of fosfomycin disodium and tobramycin for treatment of cystic fibrosis, CURx Pharma (UK) Limited
- Ibrutinib for treatment of marginal zone lymphoma, Janssen-Cilag International N.V.
- Recombinant human acid ceramidase for treatment of cystic fibrosis, Plexcera Therapeutics EU Limited
- Verucerfont for treatment of congenital adrenal hyperplasia, Neurocrine Therapeutics Ltd

Revision of the COMP opinion:

- Lanreotide acetate for treatment of autosomal dominant polycystic kidney disease, Prof. Dr R.T.Gansevoort

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 13 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 3 applications for orphan medicinal product designation were withdrawn.

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

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

- Farydak (panobinostat) for treatment of multiple myeloma, Novartis Europharm Ltd (EU/3/12/1063)
- Kanuma (sebelipase alfa) for treatment of lysosomal acid lipase deficiency, Synageva BioPharma Ltd (EU/3/10/827)
- Raxone (idebenone) for treatment of Leber's hereditary optic neuropathy, Santhera Pharmaceuticals (Deutschland) GmbH (EU/3/07/434)
- Strensiq (asfotase alfa) for treatment of hypophosphatasia, Alexion Europe SAS (EU/3/08/594)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 170th meeting of the COMP will be held on 1-3 September 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	128	172	116 (67%)	55 (32%)	1 (1%)	102	5	6
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	**2255**	**2140**	**1546 (72%)**	**573 (27%)**	**21 (1%)**	**1508**	**105**	**113**

[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 June 2015 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
3-{[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl}thiophene-2-carboxylic acid	Treatment of non-infectious uveitis	Panoptes Pharma Ges.m.b.H	13 May 2015	19 June 2015
Adeno-associated viral vector containing the human factor IX gene	Treatment of haemophilia B	Baxalta Innovations GmbH	13 May 2015	19 June 2015
Adeno-associated viral vector serotype 9 containing the human *SMN* gene	Treatment of spinal muscular atrophy	AveXis EU, Ltd	13 May 2015	19 June 2015
Allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells	Prevention of bronchopulmonary dysplasia	PSR Group B.V.	13 May 2015	19 June 2015
Antisense oligonucleotide directed against TGF-β2 mRNA	Prevention of scarring post glaucoma filtration surgery	Isarna Therapeutics GmbH	13 May 2015	19 June 2015
Edaravone	Treatment of amyotrophic lateral sclerosis	Mitsubishi Tanabe Pharma Europe Ltd	13 May 2015	19 June 2015
Obinutuzumab	Treatment of follicular lymphoma	Roche Registration Limited	13 May 2015	19 June 2015
Obinutuzumab	Treatment of marginal zone lymphoma	Roche Registration Limited	13 May 2015	19 June 2015
Synthetic 47-amino-acid N-myristoylated lipopeptide, derived from the preS region of hepatits B virus	Treatment of hepatitis delta virus infection	MYR GmbH	13 May 2015	19 June 2015
Trehalose	Treatment of spinocerebellar ataxia	Dr Ulrich Granzer	13 May 2015	19 June 2015
Triheptanoin	Treatment of very long-chain acyl-	Ultragenyx UK Limited	13 May 2015	19 June 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
	CoA dehydrogenase deficiency			

Annex 3

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

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Drisapersen	Treatment of Duchenne muscular dystrophy	BioMarin International Limited	EU/3/12/1077
Eftrenonacog alfa	Treatment of haemophilia B (congenital factor IX deficiency)	Biogen Idec Ltd	EU/3/07/453
Migalastat	Treatment of Fabry disease	Amicus Therapeutics UK Ltd	EU/3/06/368
Obeticholic acid	Treatment of primary biliary cirrhosis	Intercept Italia s.r.l	EU/3/10/753