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Inspections, Human Medicines Pharmacovigilance and Committees Division

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

October 2016

The Committee for Orphan Medicinal Products held its 182th plenary meeting on 4-6 October 2016.

Orphan medicinal product designation

Positive opinions

The COMP adopted 25 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:

- 5-[4-[2-(5-(1-hydroxyethyl)-2-pyridinyl)ethoxy]benzyl]-2,4-thiazolidinedione hydrochloride for treatment of adrenoleukodystrophy, Minoryx Therapeutics S.L.;
- Adeno-associated viral vector serotype 8 containing the human *UGT1A1* gene for treatment of Crigler-Najjar syndrome, Audentes Therapeutics UK Limited;
- Allogeneic cytomegalovirus-specific cytotoxic T lymphocytes for treatment of cytomegalovirus infection in patients with impaired cell-mediated immunity, Wainwright Associates Ltd;
- Allogeneic peripheral blood mononuclear cells incubated ex-vivo with 16, 16-dimethyl prostaglandin E2 and dexamethasone for treatment in haematopoietic stem cell transplantation, Fate Therapeutics Ltd;
- Budesonide for treatment of primary IgA nephropathy, Pharmalink AB;
- Live-attenuated non-replicative *Pseudomonas aeruginosa* strain expressing large T antigen of Merkel cell polyomavirus for treatment of Merkel cell carcinoma, APCure SAS;
- N-(5-(6-chloro-2,2-difluorobenzo[d][1,3]dioxol-5-yl)pyrazin-2-yl)-2-fluoro-6-methylbenzamide for treatment of acute pancreatitis, EMAS Pharma Limited;
- R-azasetron besylate for treatment of sudden sensorineural hearing loss, Sensorion;

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- Synthetic human hepcidin for treatment of sickle cell disease, EMAS Pharma Limited;
- Vaccine consisting of 5 survivin peptides with different human leukocyte antigen restrictions for treatment of ovarian cancer, Dr Ulrich Granzer;
- Valproic acid for treatment of diffuse large B-cell lymphoma, Valcuria AB.

2. Opinions adopted at the first COMP discussion:

- 2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl)methoxy)benzaldehyde for treatment of sickle cell disease, SynteractHCR Deutschland GmbH;
- Adeno-associated viral vector serotype 8 containing the human glucose-6-phosphatase gene for treatment of glycogen storage disease type Ia, Pharma Gateway AB;
- Alpha-tocopherol for treatment of facioscapulohumeral muscular dystrophy, Université de Montpellier;
- Ascorbic acid for treatment of facioscapulohumeral muscular dystrophy, Université de Montpellier;
- Brincidofovir for treatment of smallpox, Chimerix UK Ltd;
- Human monoclonal antibody against activin A for treatment of fibrodysplasia ossificans progressiva, Regeneron Ireland;
- Ibrutinib for treatment of graft-versus-host disease, Janssen-Cilag International N.V.;
- L-selenomethionine for treatment of facioscapulohumeral muscular dystrophy, Université de Montpellier;
- Particles comprised of methacrylic acid based co-polymer, cross-linked with a bi-functional cross-linker, purified to bind L-phenylalanine and L-phenylalanine containing peptides for treatment of hyperphenylalaninaemia, MipSalus ApS – Denmark;
- Recombinant adeno-associated viral vector serotype 2 carrying the gene for the human aromatic L-amino acid decarboxylase protein for treatment of aromatic L-amino acid decarboxylase deficiency, Voisin Consulting S.A.R.L.;
- Sodium benzoate for treatment of argininosuccinic aciduria, Lucane Pharma SA;
- Sodium benzoate for treatment of N-acetylglutamate synthase deficiency, Lucane Pharma SA;
- Tyr-Gly-Arg-Lys-Lys-Arg-Arg-Gln-Arg-Arg-Gly-Gly-Asp-Leu-Leu-Pro-Arg-Gly-Ser for treatment of Huntington's disease, Dr Ulrich Granzer;
- Zinc gluconate for treatment of facioscapulohumeral muscular dystrophy, Université de Montpellier.

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.

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

Negative opinion

Appeal opinion

Following the appeal to the COMP opinion adopted on 13 July 2016, the COMP adopted its final opinion recommending the refusal of the orphan medicinal product designation for the following product:

- Naltrexone for treatment of fibromyalgia, Able AB.

Lists of questions

The COMP adopted 12 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

9 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 10 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 2 opinions recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal product:

- Ninlaro (ixazomib) for treatment of multiple myeloma, Takeda Pharma A/S (EU/3/11/899);
- Lartruvo (olaratumab) for treatment of soft tissue sarcoma, Eli Lilly Nederland B.V. (EU/3/15/1447).

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 183th meeting of the COMP will be held on 3-4 November 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	252	242	186(77%)	55 (23%)	1	137	8	8
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	**2637**	**2477**	**1793 (72%)**	**662 (27%)**	**22 (1%)**	**1733**	**122**	**136**

[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 September 2016 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
None				

Annex 3

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

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Cerliponase alfa	Treatment of neuronal ceroid lipofuscinosis type 2	BioMarin International Limited	EU/3/13/1118
Idebenone	Treatment of Lebrer's hereditary optic neuropathy	Santhera Pharmaceuticals (Deutschland) GmbH	EU/3/07/434
Telotristat ethyl	Treatment of carcinoid tumours	Ipsen Pharma	EU/3/09/661