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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

December 2017

The Committee for Orphan Medicinal Products held its 195th plenary meeting on 05-07 December 2017.

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:

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

- Adeno-associated viral vector serotype 2/6 encoding zinc-finger nucleases and the human alpha L-iduronidase gene for treatment of mucopolysaccharidosis type I, Quintiles Ireland Limited;
- Adeno-associated viral vector serotype 2/6 encoding zinc-finger nucleases and the human iduronate 2-sulfatase gene for treatment of mucopolysaccharidosis type II (Hunter's syndrome), Quintiles Ireland Limited;
- Adeno-associated viral vector serotype 5 encoding a microRNA targeted to human huntingtin gene for treatment of Huntington's disease, uniQure biopharma B.V.;
- Humanised Fc-engineered monoclonal antibody against CD19 for treatment of IgG4-related disease, MWB Consulting Ltd;
- N-(bromoacetyl)-3,3-dinitroazetidine for treatment of small cell lung cancer, Sirius Regulatory Consulting Limited;
- Pyrazolo[1,5-a]pyrimidine, 3-[4-chloro-2-(4-morpholinyl)-5-thiazolyl]-7-(1-ethylpropyl)-2,5-dimethyl-pyrazolo[1,3-a]pyrimidine for treatment of congenital adrenal hyperplasia, RegIntel Limited.



2. Opinions adopted at the first COMP discussion:

- Allogeneic umbilical cord blood CD34+ cells cultured ex vivo with Notch ligand Delta1 for treatment in haematopoietic stem cell transplantation, Voisin Consulting S.A.R.L.;
- Cannabidiol for treatment of tuberous sclerosis, GW Research Ltd;
- Ciclopirox for treatment of congenital erythropoietic porphyria, Atlas Molecular Pharma S.L.;
- Gilteritinib for treatment of acute myeloid leukaemia, Astellas Pharma Europe B.V.;
- Hydroxychloroquine sulphate for treatment of LIPIN1 disease, Professor Pascale De Lonlay;
- Itacitinib for treatment of graft-versus-host disease, Incyte Biosciences UK Ltd;
- Metformin and L-citrulline for treatment of Duchenne muscular dystrophy, Duchenne UK;
- N-[2,6-bis(1-methylethyl)phenyl]-N'-[[1-[4-(dimethylamino)phenyl]cyclopentyl]methyl]urea, hydrochloride salt for treatment of congenital adrenal hyperplasia, Millendo Therapeutics Ltd;
- Recombinant adeno-associated viral vector serotype 2/1 encoding human beta-hexosaminidase alpha and beta subunits for treatment of GM2 gangliosidosis, University of Cambridge;
- Sirolimus for treatment of sickle cell disease, Rare Partners srl Impresa Sociale;
- Vatiquinone for treatment of RARS2 syndrome, Edison Orphan Pharma BV.

3. Opinion(s) following appeal procedures:

None

Public summaries of opinions will be available on the [EMA website](#) following adoption of the respective decisions on orphan designation¹ by the European Commission. Please also refer to the Community Register of orphan medicinal products for human use.

Negative opinion(s)

1. Opinion(s) adopted following the sponsor's response to the COMP list of questions:

- Autologous skeletal myoblasts expanded ex vivo for treatment of oculopharyngeal muscular dystrophy, Assistance Publique - Hopitaux de Paris (APHP).

2. Opinion(s) following appeal procedures:

None

Lists of questions

The COMP adopted 17 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

4 oral hearings took place.

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

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 7 applications for orphan medicinal product designation were withdrawn by the sponsor before adoption of the COMP opinion.

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 granted by the European Commission since the last COMP meeting is provided in Annex 2.

Re-assessment of orphan designation at time of marketing authorisation

(Article 5(12) (b) of Regulation (EC) No 141/2000 of the European Parliament and of the Council)

1. Opinion adopted at time of CHMP opinion:

- Adcetris (Brentuximab vedotin) – Type II variation, for treatment of cutaneous T-cell lymphoma, Takeda Pharma A/S – Denmark (EU/3/11/939);
- Jorveza (budesonide) for treatment of eosinophilic esophagitis, Dr. Falk Pharma GmbH (EU/3/13/1181);
- Prevymis (letermovir - S)-{8-fluoro-2-[2-[4-(3-methoxyphenyl)-1-piperazinyl]-3-[2-methoxy-5-(trifluoromethyl)-phenyl]-3,4-dihydro-4-quinazoliny] acetic acid) for prevention of cytomegalovirus disease in patients with impaired cell-mediated immunity deemed at risk, Merck Sharp & Dohme Limited (EU/3/11/849). The opinion was adopted by written procedure after the 30-31 October 2017 meeting.

2. Opinion(s) following appeal procedures:

None

Details of the designated orphan medicinal products that have been subject of a new European Union (EU) marketing authorisation application since the last COMP monthly report are provided in Annex 3.

Details on the authorised orphan medicinal products can be found on the [EMA website](#).

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 196th meeting of the COMP will be held on 16-18 January 2018.

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

Please also refer to the Community Register of orphan medicinal products for human use.

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 ⁴
2017	260	241	144 (60%)	96 (40%)	1 (1%)	138	14	15
2016	330	304	220 (72%)	82 (27%)	2 (1%)	209	14	14
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

² Revision of the figures for 2015, 2014, 2003, 2002, 2001 and 2000

³ The number of orphan medicinal products authorised includes the products for which the market exclusivity has expired.

⁴ The market authorisation of an orphan medicinal product may cover more than one orphan designation.

Year	Applications submitted	Applications discussed in reporting year	Positive COMP opinions	Applications withdrawn	Final negative COMP opinions	EC designations	Orphan medicinal products authorised	Orphan designations included in authorised therapeutic indication
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	2975	2780	1971 (71%)	785 (28%)	24 (1%)	1943	142	157

Annex 2

Designations granted by the European Commission following COMP opinion on the fulfilment of the orphan designation criteria since last COMP plenary meeting

No new designations were granted by the European Commission since last COMP plenary meeting.

Annex 3

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

Please also refer to the Community Register of orphan medicinal products for human use.

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Daunorubicin/Cytarabine	Treatment of acute myeloid leukaemia	Jazz Pharmaceuticals Ireland Limited	EU/3/11/942
Tisagenlecleucel	Treatment of diffuse large B-cell lymphoma	Novartis Europharm Limited	EU/3/16/1745
Inotersen	Treatment of ATTR amyloidosis	Ionis USA Limited	EU/3/14/1250