21 June 2016
EMA/COMP/365915/2016
Procedure Management and Committees Support Division

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

June 2016

The Committee for Orphan Medicinal Products held its 179th plenary meeting on 14-16 June 2016.

Orphan medicinal product designation

Positive opinions

The COMP adopted 22 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:

- 3-[4-(1H-imidazol-1-ylmethyl)phenyl]-5-(2-methylpropyl)thiophene-2-[(N-butyloxylcarbamate)-sulphonamide] sodium salt for treatment of idiopathic pulmonary fibrosis, Vicore Pharma AB;
- Adeno-associated viral vector serotype 2.7m8 containing the *ChrimsonR-tdTomato* gene for treatment of retinitis pigmentosa, GenSight Biologics;
- Dimethyl fumarate for treatment of bullous pemphigoid, Immungenetics AG;
- Poly(oxy-1,2-ethanediyl), alpha-(carboxymethyl)-omega-methoxy-, amide with arginase 1 [cobalt cofactor] (synthetic human) (1:10), trimer for treatment of hyperargininaemia, ERA Consulting GmbH;
- Sirolimus for treatment of sporadic lymphangioleiomyomatosis, Best Regulatory Consulting Ltd.

2. Opinions adopted at the first COMP discussion:

- 16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7 amino acid peptide for treatment of soft tissue sarcoma, Biogenera SpA;
- 2-[4-(1-methyl-4-pyridin-4-yl-1H-pyrazol-3-yl)-phenoxymethyl]-quinoline succinic acid for treatment of Huntington's disease, Pfizer Limited;

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

An agency of the European Union

© European Medicines Agency, 2016. Reproduction is authorised provided the source is acknowledged.

- Autologous CD4+ and CD8+ T cells transduced with lentiviral vector containing an affinity-enhanced T-cell receptor targeting the New York esophageal antigen-1 for treatment of soft tissue sarcoma, Adaptimmune Limited;
- Autologous Epstein-Barr virus specific T cells derived from peripheral blood mononuclear cells, expanded ex vivo for treatment of extranodal NK/T-cell lymphoma, nasal type, Cell Medica Ltd.;
- Autologous Epstein-Barr virus specific T cells derived from peripheral blood mononuclear cells, expanded ex vivo for treatment of post-transplant lymphoproliferative disorder, Cell Medica Ltd.;
- Brincidofovir for treatment of adenovirus infection in immunocompromised patients, Chimerix UK Ltd;
- Mifamurtide for treatment of echinococcosis, Delta Proteomics SAS;
- Mifamurtide for treatment of hepatocellular carcinoma, Delta Proteomics SAS;
- Recombinant human monoclonal antibody to insulin receptor for treatment of congenital hyperinsulinism, XOMA UK Limited;
- Setmelanotide for treatment of pro-opiomelanocortin deficiency, TMC Pharma Services Ltd;
- Sodium benzoate for treatment of carbamoyl-phosphate synthetase-1 deficiency, Lucane Pharma SA;
- Sodium benzoate for treatment of citrullinaemia type 1, Lucane Pharma SA;
- Sodium benzoate for treatment of hyperargininaemia, Lucane Pharma SA;
- Sodium benzoate for treatment of ornithine transcarbamylase deficiency, Lucane Pharma SA;
- Sodium hypochlorite for treatment of partial deep dermal and full thickness burns, Hypo-Stream Ltd;
- Triheptanoin for treatment of McArdle's disease, Vall d'Hebron Institute of Research;
- Volanesorsen sodium for treatment of familial partial lipodystrophy, Ionis USA 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 14 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 5 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.

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 180th meeting of the COMP will be held on 11-13 July 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

Contact our press officer

Monika Benstetter
Tel. +44 (0)20 3660 8427
E-mail: press@ema.europa.eu

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	128	133	104 (78%)	29 (22%)	0	72	7	7
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	**2513**	**2368**	**1711 (72%)**	**636 (27%)**	**21 (1%)**	**1668**	**121**	**135**

[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 May 2016 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
(R)-6-(2-fluorophenyl)-N-(3-(2-((2-methoxyethyl)amino)ethyl)phenyl)-5,6-dihydrobenzo[h]quinazolin-2-amine dihydrochloride	Treatment of biliary tract cancer	Coté Orphan Consulting UK Limited	21 April 2016	30 May 2016
4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide	Treatment of retinitis pigmentosa	Shire Pharmaceuticals Ireland Limited	21 April 2016	30 May 2016
Arimoclomol citrate	Treatment of inclusion body myositis	Orphazyme ApS	21 April 2016	30 May 2016
Autologous CD34+ cells transduced with lentiviral vector encoding the human beta globin gene	Treatment of beta thalassaemia intermedia and major	Fondazione Telethon	21 April 2016	30 May 2016
Fc- and CDR-modified humanised monoclonal antibody against C5	Treatment of paroxysmal nocturnal haemoglobinuria	Alexion Europe SAS	21 April 2016	30 May 2016
H-Phe-Ser-Arg-Tyr-Ala-Arg-OH-acetate	Treatment of amyotrophic lateral sclerosis	QRC Consultants Ltd	21 April 2016	30 May 2016
Pentosan polysulfate sodium	Treatment of interstitial cystitis	Nextrasearch di Gasparetto Adolfo & C., Sas	21 April 2016	30 May 2016
Polyethylene glycol-modified human recombinant truncated cystathionine beta-synthase	Treatment of homocystinuria	Alan Boyd Consultants Ltd	21 April 2016	30 May 2016
Recombinant adeno-associated viral vector containing the human *RPGR* gene	Treatment of retinitis pigmentosa caused by mutations in the *RPGR*	TMC Pharma Services Ltd	21 April 2016	30 May 2016

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
	gene			
Rimiducid	Treatment of graft-versus-host disease	QRC Consultants Ltd	21 April 2016	30 May 2016
Rovalpituzumab tesirine	Treatment of small cell lung cancer	Aceso Biologics Consulting Ltd	21 April 2016	30 May 2016
Sodium nitrite and ethylenediaminetetraacetic acid	Treatment of cystic fibrosis	Arch Bio Ireland Ltd	21 April 2016	30 May 2016
Temsirolimus	Treatment of adrenoleukodystrophy	Centro de Investigación Biomédica en Red (CIBER)	21 April 2016	30 May 2016
Vemurafenib	Treatment of Langerhans' cell histiocytosis	Groupe d'étude des histiocytoses	21 April 2016	30 May 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 May 2016 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Inotuzumab ozogamicin	Treatment of B-cell acute lymphoblastic leukaemia	Pfizer Limited	EU/3/13/1127
Lutetium 177Lu dotatate	Treatment of gastro-entero-pancreatic neuroendocrine tumours	Advanced Accelerator Applications	EU/3/07/523
Masitinib	Treatment of Mastocytosis	AB Science	EU/3/04/242

Annex 4

COMP opinions on amendment of existing orphan drug designations since May 2016 COMP monthly report

Active substance	Initial orphan indication	Amended orphan indication	Sponsor/applicant	COMP opinion date	EC designation date
(S)-ethyl 2-amino-3-(4-(2-amino-6((R)-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate	Treatment of carcinoid tumours	Treatment of carcinoid syndrome	Ipsen Pharma	21 April 2016	30 May 2016