EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

11 October 2013
EMA/COMP/557300/2013
Human Medicines Research & Development Support

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

October 2013

The Committee for Orphan Medicinal Products held its 149th plenary meeting on 8-10 October 2013.

Orphan medicinal product designation

Positive opinions

The COMP adopted 12 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:

- **Autologous ex-vivo-expanded leucocytes treated with 5-aza-2'-deoxycytidine** for treatment of glioma; CytoVac A/S
- **Defibrotide** for prevention of graft-versus-host disease; Gentium S.p.A.
- **Recombinant human insulin receptor monoclonal antibody-fused iduronate 2-sulfatase** for treatment of mucopolysaccharidosis type II (Hunter's syndrome); Voisin Consulting S.A.R.L.
- **Sorafenib tosylate** for treatment of follicular thyroid cancer; Bayer HealthCare AG (Leverkusen)
- **Sorafenib tosylate** for treatment of papillary thyroid cancer; Bayer HealthCare AG (Leverkusen)

2. Opinions adopted at the first COMP discussion:

- **Human monoclonal antibody against human interleukin 13** for treatment of eosinophilic oesophagitis; Novartis Europharm Limited
- **Ibrutinib** for treatment of diffuse large B-cell lymphoma; Janssen-Cilag International N.V.
- **Sirolimus** for prevention of arteriovenous access dysfunction in patients undergoing surgical creation of an arteriovenous access for haemodialysis; S-Cubed Limited

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

- **Soraprazan** for treatment of Stargardt's disease; Katairo GmbH
- **Synthetic 12 amino acids peptide designed after subcommissural organ spondin** for treatment of spinal cord injury; Neuronax SAS
- **Tivantinib** for treatment of hepatocellular carcinoma; Daiichi Sankyo Development Ltd
- **Trebananib** for treatment of ovarian cancer; Amgen Europe BV.

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 11 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.

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:

- 5 Protocol Assistance letters were adopted.

Upcoming meetings

- The 150th meeting of the COMP will be held on 5-6 November 2013.

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

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 7418 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	Final negative COMP opinions	EC designations	Orphan medicinal products[2] authorised	Orphan designations included in authorised therapeutic indication
2013	162	160	110 (69%)	49 (30%)	1 (1%)	111	6	7
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[3] (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[4] (3%)	49	4	4
2001	83	90	62[4] (70%)	27 (29%)	1 (1%)	64	3	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0	0
Total	**1759**	**1672**	**1208 (72%)**	**446 (27%)**	**18 (1%)**	**1194**	**84**	**90**

[2] Number of authorised orphan medicinal products may cover more than one orphan designation
[3] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[4] Following a quality assurance exercise it was identified that this figure needed correction

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the September 2013 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
3,5-diiodothyropropionic acid	Treatment of the Allan-Herndon-Dudley syndrome	CATS Consultants GmbH	4 September 2013	7 October 2013
Antisense oligonucleotide targeting the F508delta mutation of CFTR	Treatment of cystic fibrosis	ProQR Therapeutics BV	4 September 2013	7 October 2013
Autologous CD34+ cells transduced with a lentiviral vector containing the human Wiskott-Aldrich syndrome gene	Treatment of Wiskott-Aldrich syndrome	Généthon	4 September 2013	7 October 2013
Autologous regulatory T cells with an immunophenotype of CD4+CD25hiFoxP3+	Prevention of graft rejection following solid organ transplantation	iReg Medical AB	11 July 2013 Revision adopted on 4 September 2013	7 October 2013
L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Treatment of sarcoidosis	Araim Pharma Europe Ltd	4 September 2013	7 October 2013
Mexiletine hydrochloride	Treatment of myotonic disorders	Agenzia Industrie Difesa-Stabilimento Chimico Farmaceutico Militare	4 September 2013	7 October 2013
Naproxcinod	Treatment of Duchenne muscular dystrophy	NicOx	4 September 2013	7 October 2013
Recombinant fusion protein linking coagulation factor VIIa with albumin	Treatment of congenital factor VII deficiency	CSL Behring GmbH	4 September 2013	7 October 2013

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Recombinant human monoclonal IgM antibody targeting glucose-regulated protein 78	Treatment of plasma cell myeloma	Patrys GmbH	4 September 2013	7 October 2013
Zoledronic acid	Treatment of complex regional pain syndrome	Axsome Therapeutics Limited	4 September 2013	7 October 2013

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 2013 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
(1R,2R)-octanoic acid[2-(2',3'-dihydro-benzo[1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt	Cerdelga	Genzyme Europe BV	EU/3/07/514	Treatment of Gaucher Disease
Chimeric-anti-interleukin-6 monoclonal antibody	Sylvant	Janssen-Cilag International N.V.	EU/3/07/508	Treatment of Castleman's disease
Olaparib	Olaparib AstraZeneca AB	AstraZeneca AB	EU/3/07/501	Treatment of ovarian cancer
Ramucirumab	Cyramza	Eli Lilly Nederland B.V.	EU/3/12/1004	Treatment of gastric cancer