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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

April 2015

The Committee for Orphan Medicinal Products held its 166th plenary meeting on 14-16 April 2015.

Orphan medicinal product designation

Positive opinions

The COMP adopted 13 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:

- {2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide for treatment of ovarian cancer, Right Track Regulatory Limited
- AASSGVSTPGSAGHDIITEQPRS (P42) for treatment of Huntington's disease, Centre National de la Recherche Scientifique (CNRS)
- Adeno-associated viral vector serotype 9 containing the human *HGSNAT* gene for treatment of mucopolysaccharidosis IIIC (Sanfilippo C syndrome), Cochamo Systems Ltd
- Fusion proteins composed by a genetically modified cholera toxin subunit A1, peptides from the acetylcholine receptor alpha chain and a dimer of the D fragment from *Staphylococcus aureus* protein A for treatment of myasthenia gravis, Toleranzia AB
- Reduced oxydised N-acetyl heparin for treatment of plasma cell myeloma, Sigma-Tau Pharma Ltd
- Triamcinolone acetonide for treatment of non-infectious uveitis, S-cubed Limited

2. Opinions adopted at the first COMP discussion:

- 2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide for treatment of cystic fibrosis, Clinical Network Services (UK) Ltd

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- 5,7-dichloro-2-dimethylaminomethyl-8-hydroxyquinoline hydrochloride for treatment of Huntington's disease, Prana Biotechnology UK Limited
- Adult human bone-marrow-derived, ex-vivo-expanded, pooled allogeneic mesenchymal stromal cells for treatment of thromboangiitis obliterans (Buerger's disease), Regulatory Resources Group Ltd
- Allopurinol sodium for treatment of perinatal asphyxia, ACE Pharmaceuticals BV
- Humanised anti-CD37 monoclonal antibody conjugated to maytansinoid DM1 for treatment of diffuse large B-cell lymphoma, ImmunoGen Europe Limited
- Trehalose for treatment of oculopharyngeal muscular dystrophy, Dr Ulrich Granzer
- Triheptanoin for treatment of glucose transporter type-1 deficiency syndrome, Pharma Gateway AB

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.

Negative opinion

The COMP adopted 1 negative opinion recommending the refusal of the orphan medicinal product designation for a product for treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma. The sponsor was informed about the possibility to appeal.

Lists of questions

The COMP adopted 9 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 2 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.

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

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:

- Lenvima (lenvatinib); Eisai Ltd

a) treatment of papillary thyroid cancer (EU/3/13/1121)

b) treatment of follicular thyroid cancer (EU/3/13/1119)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 167th meeting of the COMP will be held on 12-13 May 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	59	109	69 (63%)	40 (37%)	0 (0%)	63	3	3
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	**2186**	**2077**	**1499 (72%)**	**558 (27%)**	**20 (1%)**	**1469**	**103**	**110**

[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 March 2015 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 March 2015 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
None			EU/