13 May 2015
EMA/COMP/287101/2015
Committee for Orphan Medicinal Products (COMP)

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

May 2015

The Committee for Orphan Medicinal Products held its 167th plenary meeting on 12-13 May 2015.

Orphan medicinal product designation

Positive opinions

The COMP adopted 11 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-{[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl}thiophene-2-carboxylic acid for treatment of non-infectious uveitis, Panoptes Pharma Ges.m.b.H
- Allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells for prevention of bronchopulmonary dysplasia, PSR Group B.V.
- Antisense oligonucleotide directed against TGF-β2 mRNA for prevention of scarring post glaucoma filtration surgery, Isarna Therapeutics GmbH
- obinutuzumab for treatment of marginal zone lymphoma, Roche Registration Limited
- obinutuzumab for treatment of follicular lymphoma, Roche Registration Limited
- Synthetic 47-amino-acid N-myristoylated lipopeptide, derived from the preS region of hepatitis B virus for treatment of hepatitis delta virus infection, MYR GmbH

2. Opinions adopted at the first COMP discussion:

- Adeno-associated viral vector containing the human factor IX gene for treatment of haemophilia B, Baxter Innovations GmbH

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- Adeno-associated viral vector serotype 9 containing the human *SMN* gene for treatment of spinal muscular atrophy, AveXis EU, Ltd
- Edaravone for treatment of amyotrophic lateral sclerosis, Mitsubishi Tanabe Pharma Europe Ltd
- Trehalose for treatment of spinocerebellar ataxia, Dr Ulrich Granzer
- Triheptanoin for treatment of very long-chain acyl-CoA dehydrogenase deficiency, Ultragenyx UK Limited

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 10 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

3 oral hearings took place.

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 1 opinion recommending to the European Commission that the following orphan medicinal product be kept in the EU registry of orphan medicinal product:

- Hetlioz (tasimelteon) for treatment of non-24-hour sleep-wake disorder; Vanda Pharmaceuticals Limited (EU/3/10/841)

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

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 168th meeting of the COMP will be held on 16-18 June 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	64	123	80 (65%)	43 (35%)	0 (0%)	78	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	**2191**	**2091**	**1510 (72%)**	**561 (27%)**	**20 (1%)**	**1484**	**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 April 2015 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
1-(4-(N-glycylamido)phenyl)-3-trifluoromethyl-5-(phenanthren-2-yl)-pyrazole-hydrochloride	Treatment of cryptococcosis	Arno Therapeutics UK, Limited	19 March 2015	24 April 2015
1-(4-(N-glycylamido)phenyl)-3-trifluoromethyl-5-(phenanthren-2-yl)-pyrazole-hydrochloride	The treatment of tularaemia	Arno Therapeutics UK, Limited	19 March 2015	24 April 2015
Adeno-associated viral vector serotype 5 containing the human *CHM* gene	Treatment of choroideremia	HORAMA SAS	19 March 2015	24 April 2015
Ecothiophate iodide	Treatment of Stargardt's disease	JJGConsultancy Ltd	19 March 2015	24 April 2015
Fluciclovine (^{18}F)	Diagnosis of glioma	Blue Earth Diagnostics Ltd	19 March 2015	24 April 2015
Human reovirus type 3 Dearing strain	Treatment of pancreatic cancer	Oncolytics Biotech (UK) Limited	19 March 2015	24 April 2015
Lenalidomide	Treatment of marginal zone lymphoma	Celgene Europe Limited	19 March 2015	24 April 2015
Nitric oxide	Treatment of cystic fibrosis	Biological Consulting Europe Ltd	19 March 2015	24 April 2015
Phenol, 4-[2-(aminomethyl)-4-thiazolyl]-2,6-bis(1,1-dimethylethyl)monohydrochloride	Treatment of Huntington's disease	Ipsen Pharma	19 March 2015	24 April 2015
Recombinant human mesencephalic astrocyte-derived neurotrophic factor	Treatment of retinitis pigmentosa	Clinipace GmbH	19 March 2015	24 April 2015
Recombinant monoclonal IgG1 antibody against T-cell immune response cDNA 7	Prevention of graft rejection following solid organ transplantation	Nekonal S.a.r.l.	19 March 2015	24 April 2015
Rimeporide	Treatment of Duchenne muscular	EUDRAC Limited	19 March 2015	24 April 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
	dystrophy			
Rintatolimod	Treatment of Ebola virus disease	NV Hemipsherx BioPharma Europe	19 March 2015	24 April 2015
Sodium 2-hydroxylinoleate	Treatment of neuroblastoma	Ability Pharmaceuticals SL	19 March 2015	24 April 2015
Xenon	Treatment of perinatal asphyxia	Neuroprotexeon Ltd	19 March 2015	24 April 2015

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the April 2015 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Recombinant fusion protein linking human coagulation factor IX with human albumin	Treatment of haemophilia B	CSL Behring GmbH	EU/3/09/723