19 June 2015
EMA/COMP/334381/2015
Committee for Orphan Medicinal Products (COMP)

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

June 2015

The Committee for Orphan Medicinal Products held its 168th plenary meeting on 16-18 June 2015.

Orphan medicinal product designation

Positive opinions

The COMP adopted 21 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:

- Anti-H5N1 equine immunoglobulin F(ab')$_2$ fragments for treatment of avian influenza, Fab'entech
- Doxorubicin for treatment of hepatoblastoma, Double Bond Pharmaceutical AB
- Human plasminogen for treatment plasminogen deficiency, ProMetic BioTherapeutics Ltd
- Lanreotide acetate for treatment of autosomal dominant polycystic kidney disease, Prof. Dr R.T.Gansevoort
- Synthetic hypericin for treatment of cutaneous T-cell lymphoma, Kinesys Consulting Ltd

2. Opinions adopted at the first COMP discussion:

- 2-((3-((4-((3-aminopropyl)amino)butyl)amino)propyl)amino)-N-((5S,5aS,8aR,9R)-9-(4-hydroxy-3,5-dimethoxyphenyl)-8-oxo-5,5a,6,8,8a,9-hexahydrofuro[3',4':6,7]naphtho[2,3-d][1,3]dioxol-5-yl)acetamide, tetrahydrochloride for treatment of acute myeloid leukaemia, Pierre Fabre Médicament
- Adenovirus-associated viral vector serotype 2 containing the human *RPE65* gene for treatment of retinitis pigmentosa, Alan Boyd Consultants Ltd

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- Allogeneic human adult stem cells, isolated from skeletal muscle and expanded ex vivo for treatment of Duchenne muscular dystrophy, Karl Rouger
- Artesunate for treatment of malaria, Dr Ulrich Granzer
- Beloranib for treatment of craniopharyngioma, Dr Ulrich Granzer
- Cannabidiol for treatment of perinatal asphyxia, GW Pharma Ltd
- Glycyl-L-2-methylprolyl-L-glutamic acid for treatment of fragile X syndrome, QRC Consultants Ltd
- Humanised IgG4 monoclonal antibody against extracellular tau for treatment of progressive supranuclear palsy, Bristol-Myers Squibb Pharma EEIG
- Hydrocinnamate-[Orn-Pro-dCha-Trp-Arg]acetate for treatment of amyotrophic lateral sclerosis, PBS Regulatory Consulting Group Limited
- Inecalcitol for treatment of acute myeloid leukaemia, Hybrigenics SA
- Modified adenovirus serotype 5/35 containing a CMV promoter-driven transgene cassette with the human transgenes for a membrane-bound CD40 ligand and full length 4-1BBL for treatment of pancreatic cancer, Lokon Pharma AB
- Sarizotan hydrochloride for treatment of Rett syndrome, Newron Pharmaceuticals SpA
- Synthetic double-stranded RNA oligonucleotide specific to hydroxyacid oxidase 1 gene for treatment of primary hyperoxaluria, Dicerna EU Limited
- Triheptanoin for treatment of carnitine palmitoyltransferase II deficiency, Ultragenyx UK Limited
- Triheptanoin for treatment of long-chain 3-hydroxyacyl-coA dehydrogenase deficiency, Ultragenyx UK Limited
- Triheptanoin for treatment of mitochondrial trifunctional protein 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 6 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

9 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.

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

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:

- Unituxin (chimeric monoclonal antibody against GD2) for treatment of neuroblastoma; United Therapeutics Europe Ltd (EU/3/11/879)
- Imbruvica (ibrutinib) for treatment of lymphoplasmacytic lymphoma - type II variation; Janssen-Cilag International NV (EU/3/14/1264)

Other matters

The main topics addressed during the meeting related to:

- Protocol assistance advice

Upcoming meetings

- The 169th meeting of the COMP will be held on 14-16 July 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	92	153	101 (66%)	51 (33%)	1 (1%)	91	4	5
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	**2219**	**2121**	**1531 (72%)**	**569 (27%)**	**21 (1%)**	**1497**	**104**	**112**

[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 May 2015 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
{2-amino-8-[4-(pyrrolidinylcarbonyl)phenyl]-(3H-benzo[f]azepin-4-yl)}-N,N-dipropylcarboxamide	Treatment of ovarian cancer	Right Track Regulatory Limited	16 April 2015	21 May 2015
2-(7-ethoxy-4-(3-fluorophenyl)-1-oxophthalazin-2(1H)-yl)-N-methyl-N-(2-methylbenzo[d]oxazol-6-yl)acetamide	Treatment of cystic fibrosis	Clinical Network Services (UK) Ltd	16 April 2015	21 May 2015
5,7-dichloro-2-dimethylaminomethyl-8-hydroxyquinoline hydrochloride	Treatment of Huntington's disease	Prana Biotechnology UK Limited	16 April 2015	21 May 2015
AASSGVSTPGSAGHDIITEQPRS	Treatment of Huntington's disease	Centre National de la Recherche Scientifique (CNRS)	16 April 2015	21 May 2015
Adeno-associated viral vector serotype 9 containing the human *HGSNAT* gene	Treatment of mucopolysaccharidosis IIIC (Sanfilippo C syndrome)	Cochamo Systems Ltd	16 April 2015	21 May 2015
Adult human bone-marrow-derived, ex-vivo-expanded, pooled allogeneic mesenchymal stromal cells	Treatment of thromboangiitis obliterans (Buerger's disease)	Regulatory Resources Group Ltd	16 April 2015	21 May 2015
Allopurinol sodium	Treatment of perinatal asphyxia	ACE Pharmaceuticals BV	16 April 2015	21 May 2015
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*	Treatment of myasthenia gravis	Toleranzia AB	16 April 2015	21 May 2015

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
aureus protein A				
Humanised anti-CD37 monoclonal antibody conjugated to maytansinoid DM1	Treatment of diffuse large B-cell lymphoma	ImmunoGen Europe Limited	16 April 2015	21 May 2015
Reduced oxidised N-acetyl heparin	Treatment of plasma cell myeloma	Sigma-Tau Pharma Ltd	16 April 2015	21 May 2015
Trehalose	Treatment of oculopharyngeal muscular dystrophy	Dr Ulrich Granzer	16 April 2015	21 May 2015
Triamcinolone acetonide	Treatment of non-infectious uveitis	S-cubed Limited	16 April 2015	21 May 2015
Triheptanoin	Treatment of glucose transporter type-1 deficiency syndrome	Pharma Gateway AB	16 April 2015	21 May 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 May 2015 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Dinutuximab beta	Treatment of neuroblastoma	APEIRON Biologics AG	EU/3/12/1062
Chlormethine	Treatment of cutaneous T-cell lymphoma	Actelion Registration Ltd.	EU/3/12/963
Irinotecan hydrochloride trihydrate	Treatment of pancreatic cancer	Baxter Innovations GmbH	EU/3/11/933
Autologous cd34+ enriched cell fraction that contains cd34+ cells transduced with retroviral vector that encodes for the human ada cdna sequence	Treatment of severe combined immunodeficiency (SCID) due to adenosine deaminase (ADA) deficiency	GlaxoSmithKline Trading Services	EU/3/05/313