17 November 2014
EMA/COMP/559651/2014 Rev. 1
Committee for Orphan Medicinal Products (COMP)

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

October 2014

The Committee for Orphan Medicinal Products held its 160th plenary meeting on 7-9 October 2014.

Orphan medicinal product designation

Positive opinions

The COMP adopted 29 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:

- 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid for treatment of systemic sclerosis; Inventiva
- 1-(6-benzothiazolylsulfonyl)-5-chloro-1H-indole-2-butanoic acid for treatment of idiopathic pulmonary fibrosis; Inventiva
- 4-[[(1S,4S)-5-[[4-[4-(oxazol-2-yl)phenoxy]phenyl]methyl]-2,5-diazabicyclo[2.2.1]hept-2-yl]methyl]benzoic acid for treatment of cystic fibrosis; Coté Orphan Consulting UK Limited
- A combination of H-Lys-Lys-Gly-Pro-Arg-Cys(SH)-Leu-Thr-Arg-Tyr-Tyr-Ser-Ser-Phe-Val-Asn-Met-Glu-Gly-Lys-Lys-OH and H-Lys-Lys-Gly-Asp-Asn-Ile-Met-Val-Thr-Phe-Arg-Asn-Gln-Ala-Ser-Arg-Pro-Tyr-Gly-Lys-Lys-OH for treatment of haemophilia A; Apitope International NV
- Donor T lymphocytes depleted ex vivo of host alloreactive T cells using photodynamic treatment for treatment of acute myeloid leukaemia; Kiadis Pharma Netherlands B.V
- Imatinib for treatment of acute respiratory distress syndrome; Numedicus Limited
- Mexiletine hydrochloride for treatment of myotonic disorders; Temmler Pharma GmbH & Co. KG
- Recombinant human pentraxin-2 for treatment of post-essential thrombocythaemia myelofibrosis; FGK Representative Service GmbH

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

- Recombinant human pentraxin-2 for treatment of post-polycythaemia vera myelofibrosis; FGK Representative Service GmbH
- Recombinant human pentraxin-2 for treatment of primary myelofibrosis; FGK Representative Service GmbH
- Selinexor for treatment of plasma cell myeloma; Clinipace GmbH
- Selinexor for treatment of treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma ; Clinipace GmbH

2. Opinions adopted at the first COMP discussion:

- (2R,3S)-2-(4-cyclopentylaminophenyl)-1-(2-fluoro-6-methylbenzoyl)piperidine-3-carboxylic acid(4-methyl-3-trifluoromethylphenyl)amide for treatment of microscopic polyangiitis; ChemoCentryx Limited
- (2R,3S)-2-(4-cyclopentylaminophenyl)-1-(2-fluoro-6-methylbenzoyl)piperidine-3-carboxylic acid(4-methyl-3-trifluoromethylphenyl)amide for treatment of granulomatosis with polyangiitis; ChemoCentryx Limited
- (3S)-1-azabicyclo[2.2.2]oct-3-yl{2-[2-(4-fluorophenyl)-1,3-thiazol-4-yl]propan-2-yl}carbamate for treatment of Gaucher disease; Genzyme Europe BV
- Adeno-associated viral vector serotype 8 containing the human *MD1* gene for treatment of Duchenne muscular dystrophy; Généthon
- Arimoclomol citrate for treatment of Niemann-Pick disease, type C; Orphazyme ApS
- Ataluren for treatment of mucopolysaccharidosis type I; PTC Therapeutics, Limited
- Bazedoxifene acetate for treatment of hereditary haemorrhagic telangiectasia; Consejo Superior de Investigaciones Cientificas (CSIC)
- Chloroquine for treatment of glioma; DualTpharma B.V.
- Dantrolene sodium for treatment of malignant hyperthermia; Eagle Laboratories Ltd
- Diaspirin cross-linked haemoglobin for treatment of hepatocellular carcinoma; New B Innovation (UK) Limited
- Humanised IgG1 monoclonal antibody against human eotaxin-2 for treatment of systemic sclerosis; CBR Biotech Strategies GmbH
- Olaptesed pegol for treatment of glioma; Noxxon Pharma AG
- Palovarotene for treatment of fibrodysplasia ossificans progressiva; Medpace Germany GmbH
- Pentosan polysulfate sodium for treatment of mucopolysaccharidosis type I; Plexcera Therapeutics EU Limited
- Pro-Pro-Thr-Val-Pro-Thr-Arg for treatment of xeroderma pigmentosum; Alain TAIEB
- Siponimod for treatment of dermatomyositis; Novartis Europharm Limited
- Siponimod for treatment of polymyositis; Novartis Europharm 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 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

14 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 4 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.

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:

- Ketoconazole HRA (ketoconazole) for treatment of Cushing's syndrome; Laboratoire HRA (EU/3/12/965)
- Signifor (pasireotide) for treatment of agromegaly – extension of MA indication; Novartis Europharm Limited (EU/3/09/670)

Other matters

The main topics addressed during the meeting related to:

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

- Protocol assistance advice

Upcoming meetings

- The 161st meeting of the COMP will be held on 11-13 November 2014

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	Final negative COMP opinions	EC designations	Orphan medicinal products[2] authorised	Orphan designations included in authorised therapeutic indication
2014	236	193	147 (76%)	45 (23%)	1 (1%)	112	10[5]	10[5]
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[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	**2034**	**1902**	**1381 (73%)**	**502 (26%)**	**19 (1%)**	**1331**	**95**	**101**

[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
[5] One product with two indications removed as it was not a new MA application

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the September 2014 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 September 2014 COMP monthly report

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Cysteamine hydrochloride	Treatment of cystinosis	Orphan Europe S.A.R.L.	EU/3/08/578
Lenvatinib	Treatment of papillary thyroid cancer	Eisai Ltd	EU/3/13/1121
Lenvatinib	Treatment of follicular thyroid cancer	Eisai Ltd	EU/3/13/1119
Glyceryl tri-(4-phenylbutyrate)	treatment of carbamoyl-phosphate synthase-1 deficiency	Hyperion Therapeutics Limited	EU/3/10/733
Glyceryl tri-(4-phenylbutyrate)	Treatment of ornithine carbamoyltransferase deficiency	Hyperion Therapeutics Limited	EU/3/10/734
Glyceryl tri-(4-phenylbutyrate)	Treatment of citrullinaemia type 1	Hyperion Therapeutics Limited	EU/3/10/735

Active substance	Designated orphan indication	Sponsor/applicant	EU designation number
Glyceryl tri-(4-phenylbutyrate)	Treatment of argininosuccinic aciduria	Hyperion Therapeutics Limited	EU/3/10/736
Glyceryl tri-(4-phenylbutyrate)	Treatment of hyperargininaemia	Hyperion Therapeutics Limited	EU/3/10/737
Glyceryl tri-(4-phenylbutyrate)	Treatment of ornithine translocase deficiency (hyperornithinaemia-hyperammonaemia homocitrullinuria (HHH) syndrome)	Hyperion Therapeutics Limited	EU/3/10/738
Glyceryl tri-(4-phenylbutyrate)	Treatment of citrullinaemia type 2	Hyperion Therapeutics Limited	EU/3/10/739