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Human Medicines Development and Evaluation

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

March 2013

The Committee for Orphan Medicinal Products held its 143th plenary meeting on 12-13 March 2013.

Orphan medicinal product designation

Positive opinions

The COMP adopted 7 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:

- **Lenvatinib** for treatment of follicular thyroid cancer; Eisai Europe Limited.
- **Lenvatinib** for treatment of papillary thyroid cancer; Eisai Europe Limited.
- **4-[2-(6-methylpyridin-2-yl)-5,6-dihydro-4H-pyrrolo[1,2-b]pyrazol-3-yl]-quinoline-6-carboxamide monohydrate** for treatment of glioma; Eli Lilly Nederland B.V.

2. Opinions adopted at the first COMP discussion:

- **(S)-3-(1-(9H-purin-6-ylamino)ethyl)-8-chloro-2-phenylisoquinolin-1(2H)-one** for treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma; Voisin Consulting S.A.R.L.
- **2-hydroxypropyl-β-cyclodextrin** for treatment of Niemann-Pick disease, type C; International Niemann-Pick Disease Alliance (INPDA).
- **Nintedanib** for treatment of idiopathic pulmonary fibrosis; Boehringer Ingelheim International GmbH.
- **R,S-O-(3-piperidino-2-hydroxy-1-propyl)-nicotinic acid amidoxime dihydrochloride** for treatment of Duchenne muscular dystrophy; N-GENE Kutatási és Fejlesztési Kf.

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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

6 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 8 applications for orphan medicinal product designation were withdrawn.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:

- 4 Protocol Assistance letters were adopted.

Upcoming meetings

- The 144th meeting of the COMP will be held on 16-17 April 2013.

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

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

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	33	43	27 (63%)	15 (35%)	1 (2%)	21	0	0
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	**1630**	**1555**	**1125 (72%)**	**412 (26%)**	**18 (1%)**	**1104**	**78**	**83**

[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 February 2013 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Cyclo-Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys	Treatment of high altitude pulmonary oedema	Apeptico Forschung und Entwicklung GmbH	9 January 2013	8 February 2013
Humanised IgG1 kappa antibody against serum amyloid A and AL amyloid	Treatment of amyloid light-chain amyloidosis	Onclave Therapeutics Limited	9 January 2013	8 February 2013
Humanised monoclonal antibody against myostatin	Treatment of Duchenne muscular dystrophy	Pfizer Limited	9 January 2013	8 February 2013
L-asparaginase encapsulated in erythrocytes	Treatment of acute myeloid leukaemia	ERYtech Pharma S.A.	9 January 2013	8 February 2013
Progesterone	Treatment of moderate and severe traumatic brain injury	BHR Pharma Belgium	9 January 2013	8 February 2013
Recombinant adeno-associated viral vector containing the human *CNGB3* gene	Treatment of achromatopsia caused by mutations in the *CNGB3* gene	TMC Pharma	9 January 2013	8 February 2013
Terguride	Treatment of systemic sclerosis	High Tech Participations GmbH	9 January 2013	8 February 2013
Treprostinil sodium	Treatment of chronic thromboembolic pulmonary hypertension	SciPharm S.a.r.L.	9 January 2013	8 February 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 February 2013 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Methyl 4,6-diamino-2-[1-(2-fluorobenzyl)-1H-pyrazolo[3,4-b]pyridine-3-yl]-5-pyrimidinyl(methyl)carbamate	Adempas	Bayer Pharma AG	EU/3/07/518	Treatment of pulmonary arterial hypertension including treatment of chronic thromboembolic pulmonary hypertension