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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

January 2013

The Committee for Orphan Medicinal Products held its 141st plenary meeting on 8-9 January 2013.

Orphan medicinal product designation

The COMP adopted 8 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

1. Opinions adopted at the second COMP discussion, following the sponsor's response to the COMP list of questions:

- **Cyclo-Cys-Gly-Gln-Arg-Glu-Thr-Pro-Glu-Gly-Ala-Glu-Ala-Lys-Pro-Trp-Tyr-Cys** for treatment of high altitude pulmonary oedema; Apeptico Forschung und Entwicklung GmbH.
- **Progesterone** for treatment of moderate and severe traumatic brain injury; BHR Pharma Belgium.
- **Treprostinil sodium** for treatment of chronic thromboembolic pulmonary hypertension; SciPharm S.a.r.L.

2. Opinions adopted at the first COMP discussion:

- **Humanised monoclonal antibody against myostatin** for treatment of Duchenne muscular dystrophy; Pfizer Limited.
- **Humanised IgG1 kappa antibody against serum amyloid A and AL amyloid** for treatment of amyloid light-chain amyloidosis; Onclave Therapeutics Limited.
- **L-asparaginase encapsulated in erythrocytes** for treatment of acute myeloid leukaemia; ERYtech Pharma S.A.
- **Recombinant adeno-associated viral vector containing the human *CNGB3* gene** for treatment of achromatopsia caused by mutations in the *CNGB3* gene; TMC Pharma.
- **Terguride** for treatment of systemic sclerosis; High Tech Participations GmbH.

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Public summaries of opinions will be available on the EMA website following adoption of the respective decisions on orphan designation by the European Commission.

Other information on the orphan medicinal product designation

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

5 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The COMP noted that 4 applications for orphan medicinal product designation were withdrawn.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 procedure

An overview of orphan designation procedures since 2000 is provided in Annex 1.

The list of medicinal products for which decisions on orphan designation[1] 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 marketing authorisation application through the centralised procedure since the last COMP plenary meeting are provided in Annex 3.

Details on the opinions for marketing authorisation for orphan medicinal products adopted by the Committee for Medicinal Products for Human Use (CHMP) can be found in the CHMP meeting reports on the EMA website: http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000508.jsp&mid=WC0b01ac0580028d2a.

Upcoming meetings

- The 142nd meeting of the COMP will be held on 5-6 February 2013.

Other matters

The main topics addressed during the meeting related to:

- 2 Protocol Assistance letters were adopted.

1 Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products http://ec.europa.eu/health/documents/community-register/html/index_en.htm

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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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 authorised
2013	0	12	8 (67%)	4 (33%)	0 (0%)	0	0
2012	197	192	139 (72%)	52 (27%)	1 (1%)	148	12
2011	166	158	111 (70%)	45 (29%)	2 (1%)	107	5
2010	174	176	123 (70%)	51 (29%)	2 (1%)	128	4
2009	164	136	113 (83%)	23 (17%)	0[2] (0%)	106	9
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73	6
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98	13
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80	9
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88	4
2004	108	101	75 (74%)	22 (22%)	4 (4%)	73	6
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55	5
2002	80	75	43 (57%)	30 (40%)	2[3] (3%)	49	4
2001	83	90	62[4] (70%)	27 (29%)	1 (1%)	64	3
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14	0
Total	**1597**	**1524**	**1106 (73%)**	**401 (26%)**	**17 (1%)**	**1083**	**80**

[2] Negative opinion adopted in 2009 was revised and finalised in 2010, therefore it is not included in 2009 listing
[3] 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 December 2012 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
4-(4-{[2-(4-chlorophenyl)-4,4-dimethylcyclohex-1-en-1-yl]methyl}piperazin-1-yl)-N-({3-nitro-4-[(tetrahydro-2H-pyran-4-ylmethyl)amino]phenyl}sulfonyl)-2-(1H-pyrrolo[2,3-b]pyridin-5-yloxy)benzamide	Treatment of chronic lymphocytic leukaemia	AbbVie Ltd	7 November 2012	6 December 2012
Alisertib	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)	Takeda Global Research and Development Centre (Europe) Ltd	7 November 2012	6 December 2012
Allopurinol sodium	Treatment of perinatal asphyxia	Pharmathen S.A.	7 November 2012	6 December 2012
Artesunate	Treatment of malaria	Dafra Pharma International N.V.	7 November 2012	6 December 2012
Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt	Treatment of acromegaly	Dr Ulrich Granzer	7 November 2012	6 December 2012
Erdosteine	Treatment of lead toxicity	Rafifarm SRL	7 November 2012	6 December 2012
Exon 52 specific phosphorothioate oligonucleotide	Treatment of Duchenne muscular dystrophy	Prosensa Therapeutics B.V.	7 November 2012	6 December 2012
Exon 55 specific phosphorothioate oligonucleotide	Treatment of Duchenne muscular dystrophy	Prosensa Therapeutics B.V.	7 November 2012	6 December 2012
Humanised single chain monoclonal antibody against CD37	Treatment chronic lymphocytic leukaemia	Emergent Product Development UK Limited	7 November 2012	6 December 2012

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Maytansinoid-conjugated human monoclonal antibody against mesothelin	Treatment of malignant mesothelioma	Bayer Pharma AG	7 November 2012	6 December 2012
Triheptanoin	Treatment of long-chain 3-hydroxyacyl-CoA dehydrogenase deficiency	B. Braun Melsungen	7 November 2012	6 December 2012
Triheptanoin	Treatment of very long-chain acyl-CoA dehydrogenase deficiency	B. Braun Melsungen	7 November 2012	6 December 2012
Voclosporin	Treatment of non-infectious uveitis	Granzer Regulatory Consulting & Services	7 November 2012	6 December 2012

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the December 2012 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Dexamethasone (40 mg tablet)	Neofordex	Laboratoires CTRS (Cell Therapies Research & Services)	EU/3/10/745	Treatment of multiple myeloma