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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

November 2012

The Committee for Orphan Medicinal Products held its 139th plenary meeting on 6-7 November 2012.

Orphan medicinal product designation

The COMP adopted 13 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:

- **Alisertib** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated); Takeda Global Research and Development Centre (Europe) Ltd.
- **Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt** for treatment of acromegaly; Dr Ulrich Granzer.
- **Voclosporin** for treatment of non-infectious uveitis; Granzer Regulatory Consulting & Services.

2. Opinions adopted at the first COMP discussion:

- **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** for treatment of chronic lymphocytic leukaemia; AbbVie Ltd.
- **Allopurinol sodium** for treatment of perinatal asphyxia; Pharmathen S.A.
- **Artesunate** for treatment of malaria; Dafra Pharma International N.V.
- **Erdosteine** for treatment of lead toxicity; Rafifarm SRL.
- **Exon 52 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy; Prosensa Therapeutics B.V.

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- **Exon 55 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy; Prosensa Therapeutics B.V.
- **Humanised single chain monoclonal antibody against CD37** for treatment chronic lymphocytic leukaemia; Emergent Product Development UK Limited.
- **Maytansinoid-conjugated human monoclonal antibody against mesothelin** for treatment of malignant mesothelioma; Bayer Pharma AG.
- **Triheptanoin** for treatment of long-chain 3-hydroxyacyl-CoA dehydrogenase deficiency; B. Braun Melsungen.
- **Triheptanoin** for treatment of very long-chain acyl-CoA dehydrogenase deficiency; B. Braun Melsungen AG.

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 4 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 15 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.

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

Article 5 (12) 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 products:

- **Purified bromelain (NexoBrid)** for treatment of partial deep dermal and full thickness burns; Teva Pharma GmbH (EU/3/02/107).

Upcoming meetings

- The 140th meeting of the COMP will be held on 5-6 December 2012.

Other matters

The main topics addressed during the meeting related to:

- 2 Protocol Assistance letters were adopted.

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
2012	168	177	126 (71%)	50 (28%)	1 (1%)	116	8
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	**1568**	**1497**	**1085 (73%)**	**395 (26%)**	**17 (1%)**	**1051**	**76**

[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 October 2012 COMP monthly report

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
[2-Cyano-3-cyclopropyl-3-hydroxy-N-(3-methyl-4-trifluoromethylphenyl)prop-2-enamide]	Treatment of traumatic spinal cord injury	Algiax Pharmaceuticals GmbH	5 September 2012	10 October 2012
Alpha-1 proteinase inhibitor (for inhalation use)	Treatment of cystic fibrosis	Grifols Deutschland GmbH	12 September 2012[4]	10 October 2012
Asp-Arg-Val-Tyr-Ile-His-Pro	Treatment of acute lung injury	Tarix Pharmaceuticals Limited	5 September 2012	10 October 2012
Belinostat	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)	TopoTarget A/S	5 September 2012	10 October 2012
Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor	Treatment of haemophilia A	Novo Nordisk A/S	12 September 2012[4]	10 October 2012
Liposomal daunorubicin	Treatment of acute myeloid leukaemia	Galen Limited	5 September 2012	10 October 2012
Lurbinectedin	Treatment of ovarian cancer	Pharma Mar SA Sociedad Unipersonal	5 September 2012	10 October 2012
Mavoglurant	Treatment of fragile X syndrome	Novartis Europharm Limited	5 September 2012	10 October 2012
Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine *alpha-(1,3)-galactosyltransferase* gene	Treatment of pancreatic cancer	European Medical Advisory Services Limited	5 September 2012	10 October 2012

[4] Opinion adopted via written procedure following the 4-5 September 2012 COMP meeting

Active substance	Orphan indication	Sponsor	COMP opinion date	EC designation date
Obinutuzumab	Treatment of chronic lymphocytic leukaemia	Roche Registration Limited	12 September 2012[4]	10 October 2012
Recombinant human lecithin cholesterol acyltransferase	Treatment of lecithin cholesterol acyltransferase deficiency	Alphacore Pharma Limited	12 September 2012[4]	10 October 2012
Rucaparib	Treatment of ovarian cancer	Clovis Oncology UK Limited	5 September 2012	10 October 2012

[4] Opinion adopted via written procedure following the 4-5 September 2012 COMP meeting

Annex 3

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

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Tobramycin (inhalation use)	Vantobra	PARI Pharma GmbH	EU/3/09/613	Treatment of *Pseudomonas Aeruginosa* lung infection in cystic fibrosis