14 September 2010
EMA/COMP/383921/2010
Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

7-9 September 2010

The Committee for Orphan Medicinal Products held its 115th plenary meeting on 7-9 September 2010.

Orphan medicinal product designation

The COMP adopted 14 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 11 June 2010 with an active review time of 91 days:

- **Methylthioninium** for treatment of behavioural variant frontotemporal dementia, Dr Hans Moebius.
- **Methylthioninium** for treatment of progressive non-fluent aphasia, Dr Hans Moebius.
- **Methylthioninium** for treatment of frontotemporal dementia with parkinsonism-17, Dr Hans Moebius.
- **Murine monoclonal antibody against CD26** for treatment of graft-versus-host disease, Adienne S.r.l.
- **Recombinant human von Willebrand factor** for treatment of von Willebrand disease, Baxter Innovations GmbH.
- **Sildenafil citrate** for treatment of postcardiotomy right ventricular failure, Pfizer Limited.

For the following medicines the review began on 12 July 2010 with an active review time of 60 days:

- **2-(2-chlorphenyl)-4-[3-(dimethyamino)phenyl]-5-methyl-1H-pyrazolo[4,3-c]pyridine-3,6(2H,5H)-dione** for treatment of idiopathic pulmonary fibrosis, Fulcrum Pharma (Europe) Ltd.
- **Chimeric monoclonal antibody against claudin-18 splice variant 2** for treatment of gastric cancer, GANYMED Pharmaceuticals AG.

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- **Methylthioninium** for treatment of progressive supranuclear palsy, Dr Hans Moebius.
- **Nanoparticle albumin-bound paclitaxel** for treatment of pancreatic cancer, Abraxis BioScience Limited.
- **N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate** for treatment of post-polycythaemia vera myelofibrosis, Dr Ulrich Granzer.
- **N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate** for treatment of post-essential thrombocythaemia myelofibrosis, Dr Ulrich Granzer.
- **Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain** for treatment of Duchenne muscular dystrophy, INC Research.
- **Recombinant human arylsulfatase A** for treatment of metachromatic leukodystrophy, Shire Pharmaceuticals Ireland Limited.

Negative opinion

The COMP adopted 1 negative opinion recommending the refusal of the orphan medicinal product designation for the following medicine:

- **Lentiviral vector expressing the truncated form of human tyrosine hydroxylase gene, human aromatic L amino-acid decarboxylase gene, human GTP-cyclohydrolase 1 gene** for treatment of 'OFF'-periods in adult patients with advanced Parkinson's disease who are not responding adequately to L-DOPA treatment, Oxford Biomedica (UK) Ltd. The review began on 11 June 2010 with an active review time of 91 days.

Public summaries of opinions will be available on the Agency's 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 6 lists of questions on initial applications. These applications will be discussed again at the next COMP meeting prior to adoption of the opinion.

Oral hearings

10 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 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 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 monthly report on the Agency's website.

Article 5 (12) of Regulation (EC) No 141/2000 of the European Parliament and of the Council

No opinions were adopted under this procedure.

Upcoming meetings

- The COMP Informal meeting will be held on 30 September-1 October 2010 in Antwerp (Belgium).
- The 116th meeting of the COMP will be held on 6-8 October 2010.

Other matters

The main topics addressed during the meeting related to:

- 3 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 Agency's 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 Community Register of Orphan Medicinal Products http://ec.europa.eu/enterprise/sectors/pharmaceuticals/documents/community-register/html/index_en.htm

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	Designations granted by Commission
2010	125	130	90 (69%)	37 (28%)	3 (2%)	69
2009	164	137	113 (82%)	23 (17%)	1 (1%)	106
2008	119	118	86 (73%)	31 (26%)	1 (1%)	73
2007	125	117	97 (83%)	19 (16%)	1 (1%)	98
2006	104	103	81 (79%)	20 (19%)	2 (2%)	80
2005	118	118	88 (75%)	30 (25%)	0 (0%)	88
2004	108	101	75 (74%)	22 (22%)	4 (4%)	72
2003	87	96	54 (56%)	41 (43%)	1 (1%)	55
2002	80	76	43 (57%)	30 (39%)	3 (4%)	49
2001	83	92	64 (70%)	27 (29%)	1 (1%)	64
2000	72	32	26 (81%)	6 (19%)	0 (0%)	14
Total	**1185**	**1120**	**817 (73%)**	**286 (26%)**	**17 (2%)**	**768**

Annex 2

Medicinal products granted a European Union designation as orphan medicinal product by the European Commission since the July 2010 COMP monthly report

Active substance	(3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate salt
Sponsor	Merck Sharp & Dohme Limited
Orphan indication	Treatment of ovarian cancer
COMP opinion date	6 May 2010
Orphan designation date	4 August 2010

Active substance	3-(6-(1-(2,2-difluorobenzo [d] [1,3] dioxol-5-yl)cyclopropanecarboxamido)-3-methylpyridin-2-yl)benzoic acid
Sponsor	Voisin Consulting S.A.R.L.
Orphan indication	Treatment of cystic fibrosis
COMP opinion date	6 May 2010
Orphan designation date	4 August 2010

Active substance	6alpha-ethyl-chenodeoxycholic acid
Sponsor	Intercept Pharma
Orphan indication	Treatment of primary biliary cirrhosis
COMP opinion date	8 April 2010
Orphan designation date	27 July 2010

Active substance	11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene
Sponsor	Voisin Consulting S.A.R.L.
Orphan indication	Treatment of primary myelofibrosis
COMP opinion date	2 June 2010
Orphan designation date	25 August 2010

Active substance	11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene
Sponsor	Voisin Consulting S.A.R.L.
Orphan indication	Treatment of post-polycythaemia vera myelofibrosis
COMP opinion date	2 June 2010
Orphan designation date	25 August 2010

Active substance	11-(2-pyrrolidin-1-yl-ethoxy)-14,19-dioxa-5,7,26-triaza-tetracyclo[19.3.1.1(2,6).1(8,12)] heptacosa-1(25),2(26),3,5,8,10,12(27),16,21,23-decaene
Sponsor	Voisin Consulting S.A.R.L.
Orphan indication	Treatment of post-essential thrombocythaemia myelofibrosis
COMP opinion date	2 June 2010
Orphan designation date	25 August 2010

Active substance	Bosutinib
Sponsor	Wyeth Europa Limited
Orphan indication	Treatment of chronic myeloid leukaemia
COMP opinion date	6 May 2010
Orphan designation date	4 August 2010

Active substance	Dexamethasone (intravitreal implant)
Sponsor	Allergan Pharmaceuticals Ireland
Orphan indication	Treatment of non-infectious uveitis affecting the posterior segment of the eye
COMP opinion date	6 May 2010
Orphan designation date	4 August 2010

Active substance	Everolimus
Sponsor	Novartis Europharm Limited
Orphan indication	Treatment of tuberous sclerosis
COMP opinion date	6 May 2010
Orphan designation date	4 August 2010

Active substance	Heparin-activated recombinant human fibroblast growth factor 1 (on a biodegradable device made from alpha-calcium sulphate hemihydrate)
Sponsor	BioArctic Neuroscience AB
Orphan indication	Treatment of traumatic spinal cord injury
COMP opinion date	8 April 2010
Orphan designation date	27 July 2010

Active substance	Midostaurin
Sponsor	Novartis Europharm Limited
Orphan indication	Treatment of mastocytosis
COMP opinion date	6 May 2010
Orphan designation date	4 August 2010

Active substance	Octenidine dihydrochloride
Sponsor	Schülke & Mayr GmbH
Orphan Indication	Prevention of late-onset sepsis in premature infants of less than or equal to 32 weeks of gestational age
COMP opinion date	8 April 2010
Orphan Designation date	27 July 2010

Active substance	Pomalidomide
Sponsor	Celgene Europe Limited
Orphan indication	Treatment of primary myelofibrosis
COMP opinion date	8 April 2010
Orphan designation date	27 July 2010

Active substance	Pomalidomide
Sponsor	Celgene Europe Limited
Orphan indication	Treatment of post-essential thrombocythaemia myelofibrosis
COMP opinion date	8 April 2010
Orphan designation date	27 July 2010

Active substance	Pomalidomide
Sponsor	Celgene Europe Limited
Orphan indication	Treatment of post-polycythaemia vera myelofibrosis
COMP opinion date	8 April 2010
Orphan designation date	27 July 2010

Active substance	Pyr-His-Trp-Ser-Tyr-D-Lys(doxorubicinylglutarate)-Leu-Arg-Pro-Gly-NH2, acetate salt
Sponsor	Æterna Zentaris GmbH
Orphan indication	Treatment of ovarian cancer
COMP opinion date	6 May 2010
Orphan designation date	4 August 2010

Active substance	Tranilast
Sponsor	Altacor Ltd
Orphan indication	Prevention of scarring post glaucoma filtration surgery
COMP opinion date	8 April 2010
Orphan designation date	27 July 2010