22 November 2011
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Human Medicines Development and Evaluation

Monthly report

Committee for Orphan Medicinal Products (COMP)

7-8 December 2010

The Committee for Orphan Medicinal Products held its 118th plenary meeting on 7-8 December 2010.

Orphan medicinal product designation

The COMP adopted 6 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicines the review began on 10 September 2010 with an active review time of 90 days:

- **Axitinib** for treatment of renal cell carcinoma, Pfizer Limited.
- **Sodium thiosulphate** for treatment of calciphylaxis, Promedipharm GmbH.

For the following medicines the review began on 15 October 2010 with an active review time of 55 days:

- **Allogeneic aortic endothelial cells cultured in a porcine gelatin matrix** for prevention of arteriovenous access failure in haemodialysis patients, Gregory Fryer Associates Ltd.
- **Dry extract from Birch bark (DER 0.1-0.2:1), extraction solvent n-heptane 95% (V/V)** for treatment of epidermolysis bullosa, Birken GmbH.
- **Paclitaxel (aqueous gel)** for treatment of oesophagus carcinoma, BTG International Ltd.
- **Pegylated B-domain-deleted sequence-modified recombinant human factor VIII** for treatment of haemophilia A, Bayer Schering Pharma AG.

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.

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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 adoption of the opinion.

Oral hearings

2 oral hearings took place.

Withdrawals of applications for orphan medicinal product designation

The COMP noted that 2 applications for orphan medicinal product designation were withdrawn.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[2]

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 monthly report on the Agency's website.

Upcoming meetings

- The 119th meeting of the COMP will be held on 11-12 January 2011.

Other matters

The main topics addressed during the meeting related to:

- 4 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

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

2 Added information on the recent applications for marketing authorisation for medicinal products (Annex 3)

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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	Designations granted by Commission
2010	167	174	123 (71%)	48 (28%)	2[3] (1%)	113
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	**1227**	**1164**	**850 (73%)**	**297 (26%)**	**16 (1%)**	**812**

[3] One more opinion was re-adopted in 2010 following the appeal to a negative opinion from 2009

Annex 2

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

Active substance	**2-(2-chlorophenyl)-4-[3-(dimethylamino)phenyl]-5-methyl-1H-pyrazolo[4,3-C]pyridine-3,6(2H,5H)-dione**
Sponsor	Fulcrum Pharma (Europe) Ltd
Orphan indication	Treatment of idiopathic pulmonary fibrosis
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	**Chimeric monoclonal antibody against claudin-18 splice variant 2**
Sponsor	GANYMED Pharmaceuticals AG
Orphan indication	Treatment of gastric cancer
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	**Methylthioninium**
Sponsor	Dr Hans Moebius
Orphan Indication	Treatment of behavioural variant frontotemporal dementia
COMP opinion date	9 September 2010
Orphan Designation date	26 November 2010

Active substance	**Methylthioninium**
Sponsor	Dr Hans Moebius
Orphan indication	Treatment of frontotemporal dementia with parkinsonism-17
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	**Methylthioninium**
Sponsor	Dr Hans Moebius
Orphan indication	Treatment of progressive non-fluent aphasia
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	**Methylthioninium**
Sponsor	Dr Hans Moebius
Orphan indication	Treatment of progressive supranuclear palsy
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	Murine monoclonal antibody against CD26
Sponsor	Adienne S.r.l.
Orphan indication	Treatment of graft-versus-host disease
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	Nanoparticle albumin-bound paclitaxel
Sponsor	Abraxis BioScience Limited
Orphan indication	Treatment of pancreatic cancer
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate
Sponsor	Dr Ulrich Granzer
Orphan indication	Treatment of post-essential thrombocythaemia myelofibrosis
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate
Sponsor	Dr Ulrich Granzer
Orphan indication	Treatment of post-polycythaemia vera myelofibrosis
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain
Sponsor	INC Research
Orphan indication	Treatment of Duchenne muscular dystrophy
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	Recombinant human arylsulfatase A
Sponsor	Shire Pharmaceuticals Ireland Limited
Orphan indication	Treatment of metachromatic leukodystrophy
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	Recombinant human von Willebrand factor
Sponsor	Baxter Innovations GmbH
Orphan indication	Treatment of von Willebrand disease
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Active substance	Sildenafil citrate
Sponsor	Pfizer Limited
Orphan indication	Treatment of postcardiotomy right ventricular failure
COMP opinion date	9 September 2010
Orphan designation date	26 November 2010

Annex 3

Designated orphan medicinal products that have been subject of a new European Union marketing authorisation application under the centralised procedure since the November 2010 COMP monthly report

Active substance	Invented name	Sponsor/applicant	EU designation number	Designated orphan indication
Purified bromelain	NexoBrid	Teva Pharma GmbH	EU/3/02/107	Treatment of partial deep dermal and full thickness burns