European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
DECEMBER 2009 PLENARY MEETING
MONTHLY REPORT

The Committee for Orphan Medicinal Products (COMP) held its 107th plenary meeting on 2-3 December 2009. The European Medicines Agency has received to date for the first time 150 applications for orphan medicinal product designation in one calendar year, this corresponds with an increase of 25% more applications to 2008.

In the context of the last months' supply shortage of products for rare diseases the Committee for Orphan Medicinal Products has considered the impact of the discontinuation of supply on the orphan designation process. In line with the Communication from the Commission on Regulation (EC) No 141/2000 of the European Parliament and of the Council on orphan medicinal products (2003/C 178/02), the Committee considers that when recurring problems in supply or a long term interruption have been identified, orphan designation can be based on a justification of significant benefit based on a potential increased supply.

ORPHAN MEDICINAL PRODUCT DESIGNATION

The COMP adopted 11 positive opinions recommending the following medicines for designation as orphan medicinal products to the European Commission:

For the following medicine the EMEA review began on 15 September 2009 with an active review time of 80 days.

- **Recombinant human monoclonal antibody to human Interleukin (IL)-17A of the IgG1/k class** for treatment of chronic non-infectious uveitis, from Novartis Europharm Limited.

For the following medicines the EMEA review began on 9 October 2009 with an active review time of 55 days.

- **Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]** for treatment of acute lymphoblastic leukaemia, from ARIAD Pharma Ltd.
- **Benzamide, 3-(2-imidazo[1,2-b]pyridazin-3-ylethynyl)-4-methyl-N-[4-[(4-methyl-1-piperazinyl)methyl]-3-(trifluoromethyl)phenyl]** for treatment of chronic myeloid leukaemia, from ARIAD Pharma Ltd.
- **Ecopipam** for treatment of Lesch-Nyhan disease, from Dr Alain Munoz.
- **Fingolimod** for treatment of chronic inflammatory demyelinating polyneuropathy, from Novartis Europharm Limited.
- **Givinostat** for treatment of polycythemia vera, from Italfarmaco S.p.A.
- **Lentiviral vector containing the human *ABCA4* gene** for treatment of Stargardt's disease, from Oxford Biomedica (UK) Ltd.
- **Panobinostat** for treatment of Hodgkin's lymphoma, from Novartis Europharm Limited.

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- **Pixantrone dimaleate** for treatment of diffuse large B-cell lymphoma, from CTI Life Sciences Ltd.
- **Recombinant fusion protein linking human coagulation factor IX with human albumin** for treatment of haemophilia B, from CSL Behring GmbH.
- **RNA, [P-deoxy-P-(dimethylamino)] (2',3'-dideoxy-2',3'-imino-2',3'-seco) (2'a→5') (C-m5U-m5U-A-C-A-G-G-C-m5U-C-C-A-A-m5U-A-G-m5U-G-G-m5U-C-A-G-m5U), 5' [P-[4-[[2-[2-(2-hydroxyethoxy)ethoxy]ethoxy]carbonyl]-1-piperazinyl]-N,N-dimethylaminophosphonamidate], 3'-[2'a-[N2-acetyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-L-arginyl-6-aminohexanoyl-L-arginyl-L-arginyl-β-alanyl-L-arginyl-6-aminohexanoyl-β-alanyl], octahydrochloride** for treatment of Duchenne muscular dystrophy, from AVI BioPharma International Ltd.

Public summaries of opinion will be available on the EMEA website which the Agency updates 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 five lists of questions on initial applications. These applications will be discussed again at the next COMP plenary meeting prior to adoption of the opinion.

Oral hearing

One oral hearing took place.

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 plenary 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 community 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 EMEA website.

UPCOMING MEETINGS FOLLOWING THE DECEMBER 2009 COMP PLENARY MEETING

- The 108th meeting of the COMP will be held on 5-6 January 2010.

[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)

OTHER MATTERS

The main topics addressed during the December 2009 COMP meeting related to:

- Discussion on the comments received from stakeholders for *Recommendation on elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation (EMEA/COMP/15893/2009).*
- The Committee received a presentation on the internal reorganisation of the European Medicines Agency http://www.emea.europa.eu/pdfs/general/direct/pr/52017309en.pdf
- The Committee was informed that the Pharmaceutical Products and Cosmetics Units ENTR F.2 and F.3 of the European Commission move to DG SANCO from DG Enterprise and Industry (ENTR), consequently the European Medicines Agency comes under the Health and Consumer portfolio http://europa.eu/rapid/pressReleasesAction.do?reference=IP/09/1837&format=HTML&aged=0&language=EN&guiLanguage=en
- Three Protocol Assistance letters were adopted.

NOTE: This Monthly Report and other documents may be found on the internet at the following location: http://www.emea.europa.eu

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OVERVIEW FOR ORPHAN MEDICINAL PRODUCT DESIGNATION PROCEDURE SINCE 2000

Year	Applications submitted	Positive COMP Opinions	Applications withdrawn	Final negative COMP Opinions	Designations granted by Commission
2009	150	112	21	1	97
2008	119	86	31	1	73
2007	125	97	19	1	98
2006	104	81	20	2	80
2005	118	88	30	0	88
2004	108	75	22	4	72
2003	87	54	41	1	55
2002	80	43	30	3	49
2001	83	64	27	1	64
2000	72	26	6	0	14
Total	1046	726	247	14	690

MEDICINAL PRODUCTS GRANTED A COMMUNITY DESIGNATION AS ORPHAN MEDICINAL PRODUCT SINCE THE NOVEMBER 2009 COMP PLENARY REPORT BY THE EUROPEAN COMMISSION

Active substance	1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea
Sponsor	Astex Therapeutics Limited
Orphan Indication	Treatment of acute myeloid leukaemia
COMP Opinion date	07/10/2009
Orphan Designation date	26/11/2009

Active substance	16-base single-stranded PNA oligonucleotide linked to a 7-aminoacid peptide
Sponsor	Biogenera srl
Orphan Indication	Treatment of neuroblastoma
COMP Opinion date	07/10/2009
Orphan Designation date	25/11/2009

Active substance	N-[6-(cis-2,6-dimethylmorpholin-4-yl)pyridine-3-yl]-2-methyl-4'-(trifluoromethoxy)[1,1'-biphenyl]-3-carboxamide
Sponsor	Novartis Europharm Limited
Orphan Indication	Treatment of naevoid basal cell carcinoma syndrome (Gorlin syndrome)
COMP Opinion date	07/10/2009
Orphan Designation date	25/11/2009

DESIGNATED ORPHAN MEDICINAL PRODUCTS THAT HAVE BEEN SUBJECT OF A NEW COMMUNITY MARKETING AUTHORISATION APPLICATION UNDER THE CENTRALISED PROCEDURE SINCE THE NOVEMBER 2009 COMP MONTHLY REPORT

Active substance	Invented name	Sponsor/applicant	EU Designation Number	Designated Orphan Indication
Mannitolum	Bronchitol	Pharmaxis Pharmaceuticals Ltd	EU/3/05/325	Treatment of cystic fibrosis
Cholic acid	Orphacol	Laboratoires CTRS	EU/3/02/127	Treatment of inborn errors of primary bile acid synthesis
Tegafur/gimeracil/oteracil potassium	S-1	Taiho Pharma Europe ltd.	EU/3/07/515	Treatment of gastric cancer
Homoharringtonine (Omacetaxine Mepesuccinate)	Tekinex	ChemGenex Europe S.A.S	EU/3/04/224	Treatment of chronic myeloid leukaemia