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

London, 12 October 2009
Doc. Ref.: EMEA/COMP/617218/2009

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS
**OCTOBER 2009 PLENARY MEETING**
**MONTHLY REPORT**

The Committee for Orphan Medicinal Products (COMP) held its 105th plenary meeting on 6-7 October 2009.

## ORPHAN MEDICINAL PRODUCT DESIGNATION

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

For the following medicines the EMEA review began on 10 July 2009 with an active review time of 90 days.

- **N-[6-(*cis*-2,6-Dimethylmorpholin-4-yl)pyridine-3-yl]-2-methyl-4'-(trifluoromethoxy)[1,1'-biphenyl]-3-carboxamide** for treatment naevoid basal cell carcinoma syndrome (Gorlin syndrome), Novartis Europharm Limited.
- **Pegylated carboxyhaemoglobin** for treatment of sickle cell disease, Voisin Consulting S.A.R.L.
- **Vaccinia GM-CSF/TK-deactivated virus** for treatment of hepatocellular carcinoma, Sirius Regulatory Consulting Limited.

For the following medicines the EMEA review began on 10 August 2009 with an active review time of 59 days.

- **8-[4-(1-aminocyclobutyl)phenyl]-9-phenyl-1,2,4-triazolo[3,4-f][1,6]naphthyridin-3(2H)-one mono-hydrochloride** for treatment of ovarian cancer, Merck Sharp & Dohme Limited.
- **16-base single-stranded PNA oligonucleotide linked to a 7-aminoacid peptide** for treatment of neuroblastoma, Biogenera srl.
- **1-Cyclopropyl-3-[3-(5-morpholin-4-ylmethyl-1H-benzoimidazol-2-yl)-1H-pyrazol-4-yl]-urea** for treatment of acute myeloid leukaemia, Astex Therapeutics Ltd.
- **6-thioguanine (oral liquid)** for treatment of acute lymphoblastic leukaemia, Only for Children Pharmaceuticals.
- **Human MHC non-restricted cytotoxic T-cell line** for treatment of ovarian cancer, Abiogen Pharma S.p.A.
- **Recombinant chimeric monoclonal antibody against CD20** for treatment of chronic lymphocytic leukaemia, LFB-Biotechnologies.

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.

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## OTHER INFORMATION ON THE ORPHAN MEDICINAL PRODUCT DESIGNATION

**Lists of questions**
The COMP adopted nine 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 hearings**
Five oral hearings took place.

**Appeal**
One sponsor submitted a letter of intention to appeal after the negative opinion adopted in 1-2 September 2009 meeting. Detailed grounds for appeal must be submitted within 90 days of receipt of the opinion.

**Withdrawals of applications for orphan medicinal product designation**
The COMP noted that three 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**.

**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 2**.

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.

**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 or extension of indication, the COMP adopted one opinion recommending to the European Commission that the following orphan medicinal products be kept in the Community registry of orphan medicinal product:

- **Yondelis (trabectedin)** for treatment of ovarian cancer; PharmaMar S.A.

## UPCOMING MEETINGS FOLLOWING THE OCTOBER 2009 COMP PLENARY MEETING

- The 106th meeting of the COMP will be held on 4-5 November 2009.

## ORGANISATIONAL MATTERS

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

- Four 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 | 127 | 88 | 16 | 1 | 64 |
| 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 |

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

| Active substance | Invented name | Sponsor/applicant | EU Designation Number | Designated Orphan Indication |
|---|---|---|---|---|
| Recombinant human C1-inhibitor | Rhucin | Pharming Group N.V. | EU/3/01/036 | Treatment of angioedema caused by C1 inhibitor deficiency |