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

20 June 2005
EMEA/COMP/203574/2005

**PRESS RELEASE**
**Committee for Orphan Medicinal Products**
**June 2005 Meeting**

The fifty-eighth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 14-15 June 2005.

The Committee welcomed Dr Ana Corrêa Nunes who will replace Prof. José Manuel Toscano Rico as the Portuguese COMP member. The Committee thanked Prof. Toscano Rico for his contribution and collaboration.

Dr F. Donnelly, from the European Commission DG Research, attended the meeting to present the European Technology Platform for Innovative Medicines to the Committee.

## COMP Opinions

The Committee adopted 5 positive opinions on orphan medicinal product designation during this meeting:

- Adeno-associated viral vector containing modified U7 snRNA gene, from Généthon, for treatment of **Duchenne muscular dystrophy** (review time: 60 days).
- 4-imino-1, 3-diazobicyclo-[3.1.0]-hexan-2-one, from ICON Clinical Research Limited, for treatment of **pancreatic cancer** (review time: 60 days).
- Hydrocortisone (modified release tablet), from Professor Richard JM Ross, for treatment of treatment of **congenital adrenal hyperplasia** (review time: 60 days).
- Mifepristone, from Laboratoire HRA Pharma, for treatment of **Cushing's syndrome secondary to ectopic ACTH secretion** (review time: 60 days).
- Nemorubicin hydrochloride, from Nerviano Medical Sciences Srl, for **treatment of hepatocellular carcinoma** (review time: 60 days).

One oral explanation took place during the meeting. The COMP noted that one application for orphan medicinal product designation was withdrawn by a sponsor.

## Overview of orphan designation procedures

The European Commission granted nine positive decisions on orphan designation[1] since the last COMP meeting on 11-12 May 2005 (see Annex 1).

The status of orphan designation procedures, to date for 2005, is summarised below:

| *Applications submitted* | *Positive COMP Opinions* | *Applications withdrawn* | *Appeals ongoing* | *Final negative COMP Opinions* | *Designations granted by Commission* |
|---|---|---|---|---|---|
| 53 | 40 | 11 | - | - | 30 |

An overview of orphan designation procedures for 2000-2004 is provided in Annex 2.

[1] Details of all orphan designations granted to date by the European Commission are entered in the Community Register of Orphan Medicinal Products (http://pharmacos.eudra.org/F2/register/index.htm )

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Further information on designated orphan medicinal products is publicly available in the form of summarised COMP Opinions[2], which the Agency routinely publishes following adoption of the respective decisions on orphan designation by the European Commission.

**Date of next COMP meeting**

The next COMP meeting will be held on 12-14 July 2005.

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NOTE: This Press Release, together with other information about the work of the EMEA, may be found on the internet at the following location: http://www.emea.eu.int/

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[2] These documents are available on the EMEA web-site.

**Medicinal products Designated as Orphan Medicinal Products since the May 2005 COMP Meeting**

| | |
|---|---|
| **Active substance** | 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid |
| **Sponsor** | The Matthews Consultancy Ltd |
| **Orphan Indication** | Treatment of cystic fibrosis |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | 3-[5-(2-fluoro-phenyl)-[1,2,4]oxadiazole-3-yl]-benzoic acid |
| **Sponsor** | The Matthews Consultancy Ltd |
| **Orphan Indication** | Treatment of Duchenne muscular dystrophy |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | (E)-(1S,4S,10S,21R)-7-[(Z)-ethylidene]-4,21-diisopropyl-2-oxa-12,13-dithia5,8,20,23tetraazabicyclo[8.7.6]tricos-16-ene-3,6,9,19,22-pentone |
| **Sponsor** | The Matthews Consultancy Ltd |
| **Orphan Indication** | Treatment of cutaneous Tcell lymphoma |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | Acadesine |
| **Sponsor** | Advanced In Vitro Cell Technologies (Advancell) |
| **Orphan Indication** | Treatment of B-cell chronic lymphocytic leukemia (B-CLL) |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | Bimosiamose disodium |
| **Sponsor** | Revotar Biopharmaceuticals AG |
| **Orphan Indication** | Treatment of acute lung injury |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | Interferon gamma |
| **Sponsor** | mondoBIOTECH Laboratories |
| **Orphan Indication** | Treatment of idiopathic pulmonary fibrosis |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | Miltefosine |
| **Sponsor** | Obwaller Forschungs- und Entwicklungs GmbH |
| **Orphan Indication** | Treatment of Acanthamoeba keratitis |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | Recombinant megakaryopoeisis-stimulating protein |
| **Sponsor** | Amgen Europe BV |
| **Orphan Indication** | Treatment of idiopathic thrombocytopenic purpura |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

| | |
|---|---|
| **Active substance** | Sodium butyrate (rectal use) |
| **Sponsor** | Promefarm srl |
| **Orphan Indication** | Prevention of radiation proctitis |
| **Opinion receipt date** | 20/04/2005 |
| **Date of Commission Decision** | 27/05/2005 |

## Overview of Procedures for Orphan Medicinal Product Designation for 2000-2004

| *Year* | *Applications submitted* | *Positive COMP Opinions* | *Applications withdrawn* | *Final negative COMP Opinions* | *Designations granted by Commission* |
|---|---|---|---|---|---|
| 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 |