



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

9 March 2006  
EMA/COMP/85385/2006

**PRESS RELEASE**  
**Committee for Orphan Medicinal Products**  
**March 2006 Meeting**

The sixty-sixth meeting of the Committee for Orphan Medicinal Products (COMP) took place on 7-8 March 2006.

**COMP Opinions**

The Committee adopted 9 positive opinions on orphan medicinal product designation during this meeting:

- 4-<sup>[131]</sup>I iodo-L-phenylalanine, from Dr Andreas Kluge, for treatment of **glioma** (review time: day 59)
- Adeno associated viral vector containing the human calpain 3 gene, from Généthon, for treatment of **calpainopathy** (review time: day 59)
- Ciclosporin, from Novagali Pharma, for treatment of **vernal keratoconjunctivitis** (active time: day 59)
- Glutathione, from Mukoviszidose e.V., for treatment of **cystic fibrosis** (active time: day 59)
- Human heterologous liver cells (for infusion), from Cytonet GmbH & Co. KG, for treatment of **acute liver failure** (active time: day 87)
- Sorafenib tosylate, from Bayer HealthCare AG, for treatment of **hepatocellular carcinoma** (active time: day 59)
- Temsirolimus, from Wyeth Europa Limited, for treatment of **renal cell carcinoma** (active time: day 59)
- Tobramycin (liposomal), from EUCRO GmbH & Co. KG, for treatment of ***Pseudomonas aeruginosa* lung infection in cystic fibrosis** (active time: day 59)
- **Parathyroid hormone (1-34) transglutaminase fusion protein fibrin matrix complex**, from Kuros Biosurgery International AG, for treatment of **solitary bone cysts** (active time: day 59)

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 The COMP noted that one application for orphan medicinal product designation was withdrawn by the sponsor during the evaluation phase of the procedure.

### **Overview of orphan designation procedures**

The European Commission granted eleven positive decisions on orphan designation<sup>1</sup> since the last COMP meeting on 7 February 2006 (see Annex 1).

The status of orphan designation procedures, to date for 2006, is summarised below:

| <i>Applications submitted</i> | <i>Positive COMP Opinions</i> | <i>Applications withdrawn</i> | <i>Appeals ongoing</i> | <i>Final negative COMP Opinions</i> | <i>Designations granted by Commission</i> |
|-------------------------------|-------------------------------|-------------------------------|------------------------|-------------------------------------|-------------------------------------------|
| 15                            | 19                            | 3                             | -                      | -                                   | 15                                        |

An overview of orphan designation procedures for 2000-2005 is provided in Annex 2.

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

### **Applications for Marketing Authorisation for Orphan Medicinal Products**

Details of those designated orphan medicinal products that have been the subject of a centralised application for marketing authorisation since the last COMP meeting are provided in Annex 3.

### **Date of next COMP meeting**

The next COMP meeting will be held on 4-5 April 2006.

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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/>

For further information, please contact:

Martin Harvey Allchurch, EMEA press officer

Tel. (+44-20) 74 18 84 27, E-mail: [press@emea.eu.int](mailto:press@emea.eu.int)

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<sup>1</sup> 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>)

<sup>2</sup> These documents are available on the EMEA web-site.

**Orphan Medicinal Product Designations received  
since the February 2006 COMP Meeting**

|                                    |                                                            |
|------------------------------------|------------------------------------------------------------|
| <b>Active substance</b>            | 26 base single stranded phosphodiester DNA oligonucleotide |
| <b>Sponsor</b>                     | Antisoma Research Ltd                                      |
| <b>Orphan Indication</b>           | Treatment of pancreatic cancer                             |
| <b>Opinion receipt date</b>        | 25/01/2006                                                 |
| <b>Date of Commission Decision</b> | 16/02/2006                                                 |

|                                    |                                                            |
|------------------------------------|------------------------------------------------------------|
| <b>Active substance</b>            | 26 base single stranded phosphodiester DNA oligonucleotide |
| <b>Sponsor</b>                     | Antisoma Research Ltd                                      |
| <b>Orphan Indication</b>           | Treatment of renal cell carcinoma                          |
| <b>Opinion receipt date</b>        | 25/01/2006                                                 |
| <b>Date of Commission Decision</b> | 16/02/2006                                                 |

|                                    |                                              |
|------------------------------------|----------------------------------------------|
| <b>Active substance</b>            | 2'-O-methyl-phosphorothioate oligonucleotide |
| <b>Sponsor</b>                     | Prosensa B.V.                                |
| <b>Orphan Indication</b>           | Treatment of Duchenne muscular dystrophy     |
| <b>Opinion receipt date</b>        | 25/01/2006                                   |
| <b>Date of Commission Decision</b> | 16/02/2006                                   |

|                                    |                                                 |
|------------------------------------|-------------------------------------------------|
| <b>Active substance</b>            | 4-amino-5-oxo-4 (pyridinium-1-ylmethyl) proline |
| <b>Sponsor</b>                     | Prodimed S.A.                                   |
| <b>Orphan Indication</b>           | Treatment of renal cell carcinoma               |
| <b>Opinion receipt date</b>        | 25/01/2006                                      |
| <b>Date of Commission Decision</b> | 16/02/2006                                      |

|                                    |                                                                               |
|------------------------------------|-------------------------------------------------------------------------------|
| <b>Active substance</b>            | Alpha-1 proteinase inhibitor                                                  |
| <b>Sponsor</b>                     | Octapharma (IP) Limited                                                       |
| <b>Orphan Indication</b>           | Treatment of emphysema secondary to congenital alpha-1 antitrypsin deficiency |
| <b>Opinion receipt date</b>        | 25/01/2006                                                                    |
| <b>Date of Commission Decision</b> | 16/02/2006                                                                    |

|                                    |                                                                                  |
|------------------------------------|----------------------------------------------------------------------------------|
| <b>Active substance</b>            | Apomorphine hydrochloride (inhalation use)                                       |
| <b>Sponsor</b>                     | Vectura Group plc                                                                |
| <b>Orphan Indication</b>           | Treatment of off-periods in Parkinson's disease not responding to oral treatment |
| <b>Opinion receipt date</b>        | 25/01/2006                                                                       |
| <b>Date of Commission Decision</b> | 16/02/2006                                                                       |

|                                    |                                            |
|------------------------------------|--------------------------------------------|
| <b>Active substance</b>            | Human monoclonal antibody against HLA-DR   |
| <b>Sponsor</b>                     | GPC Biotech AG                             |
| <b>Orphan Indication</b>           | Treatment of chronic lymphocytic leukaemia |
| <b>Opinion receipt date</b>        | 19/12/2005                                 |
| <b>Date of Commission Decision</b> | 24/01/2006                                 |

|                                    |                                          |
|------------------------------------|------------------------------------------|
| <b>Active substance</b>            | Human monoclonal antibody against HLA-DR |
| <b>Sponsor</b>                     | GPC Biotech AG                           |
| <b>Orphan Indication</b>           | Treatment of multiple myeloma            |
| <b>Opinion receipt date</b>        | 15/02/2006                               |
| <b>Date of Commission Decision</b> | 28/02/2006                               |

|                                    |                                           |
|------------------------------------|-------------------------------------------|
| <b>Active substance</b>            | Miglustat                                 |
| <b>Sponsor</b>                     | Actelion Registration Ltd                 |
| <b>Orphan Indication</b>           | Treatment of Niemann-Pick Disease, type C |
| <b>Opinion receipt date</b>        | 25/01/2006                                |
| <b>Date of Commission Decision</b> | 16/02/2006                                |

|                                    |                                    |
|------------------------------------|------------------------------------|
| <b>Active substance</b>            | Oxalobacter formigenes strain HC-1 |
| <b>Sponsor</b>                     | OxThera AB                         |
| <b>Orphan Indication</b>           | Treatment of primary hyperoxaluria |
| <b>Opinion receipt date</b>        | 25/01/2006                         |
| <b>Date of Commission Decision</b> | 17/02/2006                         |

|                                    |                                       |
|------------------------------------|---------------------------------------|
| <b>Active substance</b>            | Zosuquidar trihydrochloride           |
| <b>Sponsor</b>                     | Uppsala Medical Information System AB |
| <b>Orphan Indication</b>           | Treatment of acute myeloid leukaemia  |
| <b>Opinion receipt date</b>        | 25/01/2006                            |
| <b>Date of Commission Decision</b> | 17/02/2006                            |

**Overview of Procedures for Orphan Medicinal Product Designation  
for 2000-2005**

| <i>Year</i> | <i>Applications submitted</i> | <i>Positive COMP Opinions</i> | <i>Applications withdrawn</i> | <i>Final negative COMP Opinions</i> | <i>Designations granted by Commission</i> |
|-------------|-------------------------------|-------------------------------|-------------------------------|-------------------------------------|-------------------------------------------|
| 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                                        |

**Overview of Designated Orphan Medicinal Products that have been the subject of a  
Centralised Application for Marketing Authorisation**  
*- update since the last COMP meeting on 7 February 2006 -*

| <i>Active substance</i>       | <i>Sponsor/applicant</i>      | <i>EU<br/>Designation<br/>Number &amp;<br/>Date of Orphan<br/>Designation</i> | <i>Designated Orphan Indication</i> |
|-------------------------------|-------------------------------|-------------------------------------------------------------------------------|-------------------------------------|
| Imatinib mesylate<br>(Glivec) | Novartis Europharm<br>Limited | EU/3/05/306<br>26/08/2005                                                     | Treatment of mastocytosis           |