

News from the EMA

Activities of the COMP

Results from the June meeting 2011 of the COMP

The COMP met on 8-10 June 2011 and adopted the following **seven positive opinions on orphan medicinal product designation**:

- **5-[1-(2,6-dichlorobenzyl)piperidin-4-ylmethoxy]quinazoline-2,4-diamine dihydrochloride** for treatment of 5q spinal muscular atrophy; Repligen Europe Limited.
- **Cardiotrophin-1** for treatment of acute liver failure; Digna Biotech S.L.
- **Everolimus** for treatment of gastric cancer; Novartis Europharm Limited.
- **Hydroxy-propyl-beta-cyclodextrin** for treatment of Niemann-Pick disease, type C; Susan French.
- **Multilamellar microvesicle comprising phosphatidylcholine, sphingomyelin, phosphatidylethanolamine, phosphatidylserine, phosphatidylinositol and cholesterol** for treatment of cystic fibrosis; Lamellar Biomedical Ltd.
- **N-[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide** for treatment of tuberculosis; Pfizer Limited.
- **Sirolimus (Rapamycin)** for treatment of chronic non-infectious uveitis; Santen Oy.

Since the May meeting the European Commission granted **twelve final designations as orphan medicinal product**.

The COMP adopted **eleven lists of questions** on initial applications.

Two oral hearings took place.

Four applications for orphan medicinal product designation were **withdrawn**.

For the following orphan medicinal products applications for centralised approval have been made:

- **Decitabine (Dacogen)** for treatment of acute myeloid leukaemia, Janssen-Cilag
- **Defibrotide (Defitelio)** for prevention of hepatic veno-occlusive disease, Gentium
- **Defibrotide (Defitelio)** for treatment of hepatic veno-occlusive disease, Gentium
- **Recombinant human factor XIII (NovoThirteen)** for treatment of hereditary factor XIII deficiency, Novo Nordisk.

The status of orphan designations/authorisations as of 10 June 2011 is given in the following table:

| Year | Applications submitted | Positive COMP Opinions | Final negative COMP Opinions | Designations granted by Commission | Applications withdrawn | EU marketing authorisations since 2000 |
|-----------------|------------------------|------------------------|------------------------------|------------------------------------|------------------------|--|
| 2011 | 60 | 50 | 1 | 40 | 14 | 1 |
| 2000-2010 | 1234 | 850 | 16 | 827 | 300 | 59 |
| Total 2000-2011 | 1294 | 900 | 17 | 867 | 314 | 60 |

Next COMP meeting: 6-8 July 2011

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