

News from the EMA

Activities of the COMP

Results from the July meeting 2011 of the COMP

The COMP met on 6-8 July 2011 and adopted the following **16 positive opinions on orphan medicinal product designation**:

- **Eflornithine** for treatment of neuroblastoma; Cancer Prevention Pharma Ltd.
- **Genetically modified *Lactococcus lactis* bacteria containing the human *trefoil factor 1* gene** for prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy; ActoGeniX N.V.
- **Heterologous human adult liver-derived stem cells** for treatment of ornithine transcarbamylase deficiency, Fresenius Medical Care Deutschland GmbH.
- **Macitentan** for treatment of pulmonary arterial hypertension; Actelion Registration Limited.
- **NH₂-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-CONH₂** for treatment of traumatic spinal cord injury; PHARMAXON.
- **2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl} diacetic acid** for treatment of multiple myeloma; Takeda.
- **20-pentaerythritol poly (oxy-1,2-ethanediyl)-carboxymethyl-glycinate-7-ethyl-10-hydroxycamptothecin 10-[1,4'-bipiperidine]-1'-carboxylate** for treatment of ovarian cancer, Nektar Therapeutics UK Ltd.
- **Dinaciclib** for treatment of chronic lymphocytic leukaemia; MSD.
- **Kifunensine** for treatment of alpha-sarcoglycanopathy; Généthon.
- **Kifunensine** for treatment of beta-sarcoglycanopathy; Généthon.
- **Kifunensine** for treatment of delta-sarcoglycanopathy; Généthon.
- **Kifunensine** for treatment of gamma-sarcoglycanopathy; Généthon.
- **Recombinant human galactocerebrosidase** for treatment of globoid cell leukodystrophy (Krabbe disease); ACE Biosciences A/S.
- **Reparixin** for prevention of graft rejection in pancreatic islet transplantation; Dompé S.p.A.
- **Resminostat** for treatment of hepatocellular carcinoma; 4SC AG.
- **Smilagenin** for treatment of amyotrophic lateral sclerosis; Phytopharm.

Since the June meeting the European Commission granted **ten final designations as orphan medicinal product**.

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

Five oral hearings took place.

Two applications for orphan medicinal product designation were **withdrawn**.

Prior to the granting of a marketing authorisation, the COMP adopted 1 opinion recommending that the following orphan medicinal product should be kept in the EU registry of orphan medicinal products:

- **Everolimus (Votubia)** for treatment of tuberous sclerosis; Novartis Europharm Limited.

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

- **Jakavi** ((R)-3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate) for treatment of myelofibrosis secondary to polycythaemia vera or essential thrombocythaemia and for treatment of chronic idiopathic myelofibrosis; Novartis

- **SAN Idebene** (Idebene) for treatment of Leber's hereditary optic neuropathy; Santhera Pharm.
- **Adcetris** (monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E) for treatment of Hodgkin lymphoma; Takeda.

The European Commission granted the marketing authorisation for the following orphan product:

- **TOBI Podhaler** (tobramycin) for suppressive therapy of chronic pulmonary infection due to *Pseudomonas aeruginosa* in adults and children aged six years and older with cystic fibrosis from Novartis on 20 July 2011.

The COMP agreed to support the initiative to publish the prevalence figures and information on their sources for conditions that have been subject of orphan designations. The initiative aims at increasing transparency on orphan designation and acknowledging the work done for the prevalence calculation in the context of designation, which can be helpful as initial reference for future applications. This initiative will be developed progressively and will focus on those conditions for which the quality and volume of data allows for a stable estimate.

The status of orphan designations/authorisations as of 8 July 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	79	66	1	50	21	2
2000-2010	1234	850	16	827	300	59
Total 2000-2011	1313	916	17	877	321	61

Next COMP meeting: 6-8 September 2011

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