News from the EMA

Activities of the COMP

Results from the July meeting 2012 of the COMP

The COMP met on 10-11 July 2012 and adopted the following **14 positive opinions on orphan medicinal product designation**:

- **(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro- 1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid** for treatment of Alagille syndrome, Albireo AB
- **(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro- 1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid** for treatment of primary biliary cirrhosis, Albireo AB.
- **Covalently closed DNA plasmids coding for cytomegalovirus *phosphoprotein 65* and *glycoprotein B* genes** for prevention of cytomegalovirus disease in patients with impaired cell mediated immunity deemed at risk, Astellas Pharma Europe B.V.
- **Humanised monoclonal antibody against epidermal growth factor receptor** for treatment of glioma, Abbott Laboratories.
- **Humanised monoclonal antibody against P-selectin** for treatment of sickle cell disease, Quintiles Ireland Ltd.
- **N-Butyldeoxygalactonojirimycin** for treatment of Fabry disease, Actelion Registration Limited.
- **Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), AOP Orphan Pharmaceuticals AG.
- **Recombinant human monoclonal antibody against activin receptor type IIB** for treatment of inclusion body myositis, Novartis Europharm Limited.
- **Elotuzumab** for treatment of multiple myeloma, Bristol-Myers Squibb Pharma EEIG.
- **Ketoconazole** for treatment of Cushing's syndrome, Agenzia Industrie Difesa-Stabilimento Chimico Farmaceutico Militare.
- **Recombinant anti-CD3-bi-single-chain-Fv-diphtheria toxin fusion protein** for treatment of cutaneous T-cell lymphoma, AOP Orphan Pharmaceuticals AG.
- **Trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexane acetic acid sodium salt** for treatment of familial chylomicronaemia, Novartis Europharm Limited.
- **Vatreptacog alfa (activated)** for treatment of haemophilia A, Novo Nordisk A/S.
- **Vatreptacog alfa (activated)** for treatment of haemophilia B, Novo Nordisk A/S.

The COMP noted the **withdrawal** of the sponsor's **intent** to **appeal** to the **negative opinion** adopted by the COMP on 3 March 2012, recommending the refusal of the orphan medicinal product designation for the following medicine:

• **Tariquidar** for treatment of P-gp positive breast cancer, Avaant Holdings Ltd.

Since the June meeting 2012 the European Commission granted **14 final designations as orphan medicinal product.**
The COMP adopted **eleven lists of questions** on initial applications.
Three oral hearings took place.

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

- **Revestive ([gly2]-recombinant human glucagon-like peptide)** for treatment of short bowel syndrome, NYCOMED DANMARK APS.

Since the June meeting for the following orphan medicinal product **a marketing authorisation application** has been made:

- **Pomalidomide Celgene** from Celgene Europe for treatment of multiple myeloma.

The status of orphan designations/authorisations as of 11 July 2012 is given in the following table:

Year	Applica-tions submitted	Positive COMP Opinions	Final negative COMP Opinions	Designations granted by Commission	Applications withdrawn	Approved orphan products since 2000
2012	103	83	1	80	24	3
2000-2011	1400	961	16	935	345	55
Total 2000-2012	1503	1044	17	1015	369	58

Next COMP meeting: 4-5 September 2012

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Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu