News from the EMA

Activities of the COMP

Results from the May meeting 2012 of the COMP

The COMP met on 10-11 May 2012 and adopted the following **16 positive opinions on orphan medicinal product designation**:

- **Eculizumab** for treatment of infection-associated haemolytic uraemic syndrome, Alexion Europe SAS.
- **Levoglutamide** for treatment of sickle cell disease, Emmaus Medical Europe Limited.
- **Recombinant human interleukin-7** for treatment of progressive multifocal leukoencephalopathy, CYTHERIS SA.
- **Talarozole** for treatment of autosomal recessive congenital ichthyosis, Stiefel Laboratories (Maidenhead) Limited.
- **Talarozole** for treatment of keratinopathic ichthyosis, Stiefel Laboratories (Maidenhead) Limited.
- **Talarozole** for treatment of recessive X-linked ichthyosis, Stiefel Laboratories (Maidenhead) Limited.
- **16-base single-stranded peptide nucleic acid oligonucleotide linked to a 7-amino acid peptide** for treatment of neuroblastoma, Biogenera srl.
- **17-(Dimethylaminoethylamino)-17-demethoxygeldanamycin** (after administration of adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5) for treatment of retinitis pigmentosa, Avena Therapeutics Ltd.
- **2S, 4R ketoconazole** for treatment of Cushing's syndrome, Cortendo AB.
- **Ataluren** for treatment of Becker muscular dystrophy, PTC Therapeutics Limited.
- **Doxorubicin** (administered after synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine) for treatment of glioma, Avena Therapeutics Ltd.
- **Givinostat** for treatment of Duchenne muscular dystrophy, Italfarmaco S.p.A.
- **Human erythrocytes encapsulating inositol hexaphosphate** for treatment of sickle cell disease, ERYtech Pharma S.A.
- **Ramucirumab** for treatment of gastric cancer, Eli Lilly Nederland B.V.
- **Ramucirumab** for treatment of hepatocellular carcinoma, Eli Lilly Nederland B.V.
- **Recombinant adeno-associated viral vector containing human acid alfa-glucosidase-gene** for treatment of glycogen storage disease type II (Pompe's disease), TMC Pharma Services Ltd.

Since the April meeting 2012 the European Commission granted **14 final designations as orphan medicinal product.**
The COMP adopted **ten lists of questions** on initial applications.
Six oral hearings took place and **three applications** were **withdrawn** by their sponsors. **1 sponsor** submitted a letter of intention to **appeal** after **a negative opinion** was adopted during the April meeting.

Prior to the granting of EU marketing authorisations the COMP adopted **2 opinions** recommending that the following **orphan medicinal products be kept in the EU registry** of orphan medicinal products:

- **Jakavi** ((R)-3-(4-(7H-pyrrolo[2,3-d]pyrimidin-4-yl)-1H-pyrazol-1-yl)-3-cyclopentylpropanenitrile phosphate) for treatment of chronic idiopathic myelofibrosis, Novartis Europharm Ltd.
- **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, Novartis Europharm Ltd.

Since the April meeting the European Commission has **granted** a **marketing authorisation** for the following orphan medicinal product:

- **Signifor** (Pasireotide) solution for injection from Novartis for treatment of certain adult patients with Cushing's disease on 24. April 2012.

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

Year	Applica-tions submitted	Positive COMP Opinions	Final negative COMP Opinions	Designations granted by Commission	Applications withdrawn	EU marketing authorisa-tions since 2000
2012	54	60	0	54	15	3
2000-2011	1400	961	18	934	345	55[1]
Total 2000-2012	1454	1021	18	989	360	58[1]

[1]In April 2012 six orphan medicinal products containing imatinib from Novartis were removed from the EU registry of orphan medicinal products due to expiry of their orphan status after ten years validity or due to requests of Novartis.

Next COMP meeting: 12-13 June 2012

Exclusively reported by Dr. Siegfried Throm, German Association of Research-Based Pharmaceutical Companies (e-mail: s.throm@vfa.de)
for:
Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu