

News from the EMA

Activities of the COMP

Results from the October meeting 2012 of the COMP

The COMP met from 3-5 October 2012 and adopted the following **16 positive opinions on orphan medicinal product designation**:

- **Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotropic factor** for treatment of macular telangiectasia type 2, Enpharma Ltd.
- **IL-12-secreting dendritic cells, loaded with autologous tumour lysate** for treatment of glioma, Activartis Biotech GmbH.
- **Milciclib maleate** for treatment of malignant thymoma, Nerviano Medical Science Srl.
- **Adeno-associated viral vector encoding an inducible short hairpin RNA targeting claudin-5 (prior to administration of 17-dimethylaminoethylamino-17-demethocycgeldanamycin)** for treatment of retinitis pigmentosa, Avena Therapeutics Ltd.
- **Alisertib** for treatment of ovarian cancer, Takeda Global Research and Development Centre (Europe) Ltd.
- **Canakinumab** for treatment of tumour necrosis factor receptor-associated periodic syndrome, Novartis Europharm Limited.
- **Chimeric monoclonal antibody against GD2** for treatment of neuroblastoma, APEIRON Biologics AG.
- **Erdosteine** for treatment of mercury toxicity, Rafifarm SRL.
- **Ixazomib** for treatment of systemic light chain amyloidosis, Takeda Global Research and Development Centre (Europe) Ltd.
- **Melarsoprol** for treatment of African trypanosomiasis, Pr. Peter Kennedy.
- **Naloxone hydrochloride dihydrate** for treatment of cutaneous T-cell lymphoma, Winston Laboratories Ltd.
- **Panobinostat** for treatment of multiple myeloma, Novartis Europharm Limited.
- **Recombinant human dyskerin** for treatment of dyskeratosis congenita, Advanced Medical Projects.
- **Synthetic double-stranded siRNA oligonucleotide directed against claudin-5 complexed with polyethyleneimine (prior to administration of doxorubicin)** for treatment of glioma, Avena Therapeutics Ltd.
- **Tafamidis** for treatment of senile systemic amyloidosis, Pfizer Limited.
- **Tralokinumab** for treatment of idiopathic pulmonary fibrosis, MedImmune Ltd.

The COMP noted that five applications were **withdrawn** by their sponsors.

Since the September meeting 2012 the European Commission granted **two final designations as orphan medicinal product**.

The COMP adopted **18 lists of questions** on initial applications and **two Protocol Assistance** letters.

Seven oral hearings took place.

Since the September meeting for the following **three orphan medicinal products marketing authorisation applications** have been made:

- **Bedaquiline** from Janssen Cilag for treatment of tuberculosis
- **Iclusig** from ARIAD Pharma for treatment of chronic myeloid leukaemia and for treatment of acute lymphoblastic leukaemia
- **Kinaction** from AB Science for treatment of pancreatic cancer.

The status of orphan designations/authorisations as of 5 October 2012 is given in the following table:

Year	Applications submitted	Positive COMP Opinions	Final negative COMP Opinions	Designations granted by Commission	Applications withdrawn	Approved products with active orphan status since 2000
2012	155	113	1	104	35	7
2000-2011	1400	959	16	935	345	57
Total 2000-2012	1555	1072	17	1039	380	64

Next COMP meeting: 6-7 November 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

© 2012 ECV • Editio Cantor Verlag Germany