News from the EMA

Activities of the COMP

Results from the December meeting 2012 of the COMP

The COMP met from 5-6 December 2012 and adopted the following **13 positive opinions on orphan medicinal product designation**:

- **Eflornithine in combination with sulindac** for treatment of familial adenomatous polyposis; Cancer Prevention Pharma Limited.
- **Recombinant modified human growth hormone** for treatment of growth hormone deficiency; Richardson Associates Regulatory Affairs Ltd.
- **1,2:5,6-Dianhydrogalactitol** for treatment of glioma; IDIS Ltd.
- **Adeno-associated viral vector serotype 9 containing the human *N-acetylglucosaminidase alpha* gene** for treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome); Laboratorios del Dr. Esteve, S.A.
- **Allogeneic motor neuron progenitor cells derived from human embryonic stem cells** for treatment of 5q spinal muscular atrophy; California Stem Cell (UK) Ltd.
- **Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human *betaA-T87Q-globin* gene** for treatment of beta-thalassaemia intermedia and major; bluebird bio France.
- **Chimeric monoclonal antibody against claudin 6** for treatment of ovarian cancer; GANYMED Pharmaceuticals AG.
- **Choline tetrathiomolybdate** for treatment of Wilson's disease; Medical Need Europe AB.
- **Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor** for treatment of retinitis pigmentosa; Enpharma Ltd.
- **Lenalidomide** for treatment of follicular lymphoma; Celgene Europe Limited.
- **Modified recombinant human C-type natriuretic peptide** for treatment of achondroplasia; BioMarin Europe Ltd.
- **Recombinant human monoclonal antibody of the IgG1 kappa class against prostate stem cell antigen** for treatment of pancreatic cancer; Astellas Pharma Europe B.V.
- **Terguride** for treatment of systemic sclerosis; Serodapharm UG.

The COMP adopted **1 negative opinion** recommending the refusal of the orphan medicinal product designation for a product for treatment of complex regional pain syndrome.

The COMP noted that 1 application was **withdrawn** by its sponsor.

Since the November meeting 2012 the European Commission granted **19 final designations as orphan medicinal product.**

The COMP adopted **seven lists of questions** on initial applications and **two Protocol Assistance** letters. **Three oral hearings** took place.

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

- Translarna from PTC Therapeutics for treatment of Duchenne muscular dystrophy
- Neocepri from Endocyte Europe for diagnosis of positive folate receptor status in ovarian cancer
- Folcepri from Endocyte Europe for diagnosis of positive folate receptor status in ovarian cancer
- Cometriq from TMC Pharma Services for treatment of medullar thyreoid carcinoma
- Opsumit (macitentan) from Actelion for treatment of pulmonary arterial hypertension
- Vynfinit from Endocyte for treatment of ovarian cancer.

Since the November meeting the European Commission has published the **marketing authorization** for the following **orphan medicinal product:**

- **Adcetris (brentuximab vedotin)** from Takeda for treatment of certain adult patients with relapsed or refractory CD30+Hodgkin lymphoma (HL) and for the treatment of adult patients with relapsed or refractory systemic anaplastic large cell lymphoma (sALCL) on 25 October 2012.

The status of orphan designations/authorisations as of 6 December 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 products with active orphan status since 2000
2012	186	139	1	134	51	10
2000-2011	1400	959	16	935	345	57
Total 2000-2012	1586	1098	17	1069	396	67

Next COMP meeting: 8-9 January 2013

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Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu