News from the EMEA

Activities of the COMP

Results from the September meeting 2010 of the COMP

The COMP met on 7-9 September 2010 and adopted the following **14 positive opinions on orphan medicinal product designation**:

- **Methylthioninium** for treatment of behavioural variant frontotemporal dementia, Dr Hans Moebius.
- **Methylthioninium** for treatment of progressive non-fluent aphasia, Dr Hans Moebius.
- **Methylthioninium** for treatment of frontotemporal dementia with parkinsonism-17, Dr Hans Moebius.
- **Murine monoclonal antibody against CD26** for treatment of graft-versus-host disease, Adienne S.r.l.
- **Recombinant human von Willebrand factor** for treatment of von Willebrand disease, Baxter Innovations GmbH.
- **Sildenafil citrate** for treatment of postcardiotomy right ventricular failure, Pfizer Limited.
- **2-(2-chlorphenyl)-4-[3-(dimethyamino)phenyl]-5-methyl-1H-pyrazolo[4,3-c]pyridine-3,6(2H,5H)-dione** for treatment of idiopathic pulmonary fibrosis, Fulcrum Pharma (Europe) Ltd.
- **Chimeric monoclonal antibody against claudin-18 splice variant 2** for treatment of gastric cancer, GANYMED Pharmaceuticals AG.
- **Methylthioninium** for treatment of progressive supranuclear palsy, Dr Hans Moebius
- **Nanoparticle albumin-bound paclitaxel** for treatment of pancreatic cancer, Abraxis BioScience Limited.
- **N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate** for treatment of post-polycythaemia vera myelofibrosis, Dr Ulrich Granzer.
- **N-tert-butyl-3-[(5-methyl-2-{[4-(2-pyrrolidin-1-ylethoxy)phenyl]amino}pyrimidin-4-yl)amino] benzenesulfonamide dihydrochloride monohydrate** for treatment of post-essential thrombocythaemia myelofibrosis, Dr Ulrich Granzer.
- **Recombinant fusion protein consisting of the extracellular portion of human activin receptor IIB linked to the human IgG1 Fc domain** for treatment of Duchenne muscular dystrophy, INC Research.
- **Recombinant human arylsulfatase A** for treatment of metachromatic leukodystrophy, Shire Pharmaceuticals Ireland Limited.

The COMP adopted **1 negative opinion** recommending the refusal of the orphan medicinal product designation for the following medicine:

- **Lentiviral vector expressing the truncated form of human tyrosine hydroxylase gene, human aromatic L amino-acid decarboxylase gene, human GTP-cyclohydrolase 1 gene** for treatment of 'OFF'-periods in adult patients with advanced Parkinson's disease who are not responding adequately to L-DOPA treatment, Oxford Biomedica (UK) Ltd.

The European Commission granted 17 final orphan designations.

Five applications for orphan medicinal product designation were withdrawn.
The COMP adopted six lists of questions and three protocol assistance letters. Ten oral hearings took place.
For the following orphan product the EU Commission granted marketing authorisation:

- VPriv (velaglucerase) from Shire for the treatment of patients (2 years and older) with type 1 Gaucher disease on 26 August 2010.

The status of orphan designations/authorisations as of 9 September 2010 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
2010	125	90	3	69	37	4
2000-2009	1060	727	14	699	249	57
Total 2000-2010	1185	817	17	768	286	61

Next COMP meeting: 6-8 October 2010

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