

News from the EMA

Activities of the COMP

Results from the March meeting 2012 of the COMP

The COMP met on 7-8 March 2012 and adopted the following **13 positive opinions on orphan medicinal product designation**:

- **Exon 45 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy, Prosensa Therapeutics B.V.
- **Exon 53 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy, Prosensa Therapeutics B.V.
- **Pegylated recombinant factor VIII** for treatment of haemophilia A, Novo Nordisk A/S.
- **(E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt** for treatment of myelodysplastic syndromes, JJGConsultancy Ltd.
- **1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H-pyrazolo[3,4 d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one** for treatment of chronic lymphocytic leukaemia, Nexus Oncology Ltd.
- **2-Allyl-1-[6-(1-hydroxy-1-methylethyl)pyridin-2-yl]-6-{[4-(4-methylpiperazin-1-yl)phenyl]amino}-1,2-dihydro-3H-pyrazolo[3,4-d]pyrimidin-3-one** for treatment of ovarian cancer, Merck Sharp & Dohme Limited.
- **Halofuginone hydrobromide** for treatment of Duchenne muscular dystrophy, Biological Consulting Europe Ltd.
- **Heterologous human adult liver-derived stem cells** for treatment of acute liver failure, Fresenius Medical Care Deutschland GmbH.
- **N-hydroxy-4-(3-methyl-2-(S)-phenyl-butrylamino)benzamide** for treatment of neurofibromatosis type 2, Sirius Regulatory Consulting Limited.
- **Pomalidomide** for treatment of systemic sclerosis, Celgene Europe Limited.
- **Recombinant human methionine proinsulin** for treatment of retinitis pigmentosa, ProRetina Therapeutics S.L.
- **Vosaroxin** for treatment of acute myeloid leukaemia, Sunesis Europe Ltd.
- **Yttrium (90Y)-DTPA-radiolabelled chimeric monoclonal antibody against frizzled homologue 10** for treatment of soft tissue sarcoma, Laboratoires OncoTherapy Science France, S.A.R.L.

Since the February meeting 2011 the European Commission granted **18 final designations as orphan medicinal product**.

The COMP adopted **five lists of questions** on initial applications.

Three oral hearings took place.

The status of orphan designations/authorisations as of 8 March 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tions submitted	Positive COMP Opinions	Final negative COMP Opinions	Designations granted by Commission	Applications withdrawn	EU marketing authorisa- tions since 2000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	41	36	0	23	9	0
2000-2011	1400	961	18	934	345	61
Total 2000-2012	1441	997	18	957	354	61

Next COMP meeting: 11-12 April 2012

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for:

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