

## News from the EMA

### Activities of the COMP

#### Results from the January meeting 2012 of the COMP

The COMP met on 11-12 January 2012 and adopted the following **16 positive opinions on orphan medicinal product designation**:

- **(1-methyl-2-nitro-1H-imidazole-5-yl)methyl N,N'-bis(2-bromoethyl) diamidophosphate** for treatment of soft tissue sarcoma, Nexus Oncology Ltd.
- **Allogeneic human dendritic cells derived from a CD34+ progenitor cell line** for treatment of acute myeloid leukaemia, DCPrime BV.
- **Carbetocin** for treatment of Prader-Willi syndrome, Ferring Pharmaceuticals A/S.
- **Chlormethine** for treatment of cutaneous T-cell lymphoma, TMC Pharma Services Ltd.
- **Doxycycline hyclate** for treatment of systemic amyloidosis caused by beta-2 microglobulin, Giampaolo Merlini.
- **Sodium nitrite** for treatment of pulmonary arterial hypertension, FGK Representative Service GmbH.
- **6-ethynyl-1-(pentan-3-yl)-1H-imidazo[4,5-b]pyrazin-2(3H)-one** for treatment of amyotrophic lateral sclerosis, ICON Clinical Research UK Limited.
- **Adeno-associated viral vector of serotype 5 containing the human alanine-glyoxylate aminotransferase gene** for treatment of primary hyperoxaluria type 1, Amsterdam Molecular Therapeutics BV.
- **Chimeric monoclonal antibody against kappa myeloma antigen** for treatment of multiple myeloma, Gregory Fryer Associates Ltd.
- **Glucagon** for treatment of congenital hyperinsulinism, Biodel UK Limited.
- **Heterologous human adult liver-derived stem cells** for treatment of carbamoyl-phosphate synthase-1 deficiency, Fresenius Medical Care Deutschland GmbH.
- **Human monoclonal antibody targeting *Staphylococcus aureus* alpha-toxin** for treatment of pneumonia caused by *Staphylococcus aureus*, Envestia Limited.
- **Ketoconazole** for treatment of Cushing's syndrome, Laboratoire HRA Pharma.
- **Oleylphosphocholine** for treatment of leishmaniasis, Dafra Pharma International NV.
- **Recombinant human beta-glucuronidase** for treatment of mucopolysaccharidosis type VII (Sly syndrome), NDA Regulatory Science Ltd.
- **Sialic acid** for treatment of hereditary inclusion body myopathy, NDA Regulatory Science Ltd.

Since the December meeting 2011 the European Commission granted **21 final designations as orphan medicinal product**.

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

**Six oral hearings** took place.

**One application** for orphan medicinal product designation was **withdrawn**.

For the following two orphan products a centralised marketing application was made:

- **Exjade** (4-(3,5-bis(hydroxy-phenyl)-1,2,4) triazol-1-yl) benzoic acid for treatment of chronic iron overload requiring chelation therapy; Novartis Europharm Ltd.
- **Loulla** (mercaptapurine) for treatment of acute lymphatic leukaemia; Only for Children pharmaceuticals.

The status of orphan designations/authorisations as of 11 January 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	1	16	0	11	1	0
2011	166	111	2	107	45	4
2000-2010	1234	850	16	827	300	57
Total 2000-2012	1401	977	18	945	346	61

Next COMP meeting: 7-8 February 2012

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for:

**Guide to Drug Regulatory Affairs** [www.drugregulatoryaffairs.eu](http://www.drugregulatoryaffairs.eu)

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