

News from the EMEA

Activities of the COMP

Results from the September meeting 2010 of the COMP

The COMP met on 6-7 October 2010 and adopted the following **15 positive opinions on orphan medicinal product designation**:

- **Ovine anti-colchicine polyclonal antibody fragments** for treatment of colchicine poisoning; Laboratoires SERB.
- **Para-aminosalicylic acid** for treatment of tuberculosis; Lucane Pharma SAS.
- **Lomitapide** for treatment of familial chylomicronaemia; Dimensione Ricerca S.r.l.
- **1H-Benzimidazole-7-carboxamide, 2-[(2R)-2-methyl-2-pyrrolidinyl]** for treatment of ovarian cancer; Abbott Laboratories.
- **7-beta-hydroxycholesteryl-3-beta-oleate** for treatment of glioma; Intsel Chimos SA.
- **Adeno-associated viral vector containing DNA encoding an RNAi targeting rhodopsin / adeno-associated viral vector containing a rhodopsin gene**; treatment of rhodopsin-linked retinitis pigmentosa, Genable Technologies Ltd.
- **Human heterologous liver cells (for infusion)**, treatment of hyperargininaemia; Cytonet GmbH & Co. KG.
- **Human heterologous liver cells (for infusion)**, treatment of argininosuccinic aciduria; Cytonet GmbH & Co. KG.
- **Human heterologous liver cells (for infusion)**, treatment of citrullinaemia type 1; Cytonet GmbH & Co. KG.
- **Human heterologous liver cells (for infusion)**, treatment of carbamoyl-phosphate synthetase-1 deficiency; Cytonet GmbH & Co. KG.
- **Lentiviral vector carrying the Fanconi anaemia-A (FANCA) gene**, treatment of Fanconi anaemia type A; Center for Biomedical Network Research on Rare Diseases (CIBERER).
- **N-[(2S)-2,3-dihydroxypropyl]-3-[(2-fluoro-4-iodophenyl) amino] isonicotinamide hydrochloride**, treatment of acute myeloid leukaemia; Merck KGaA.
- **Recombinant human lysosomal acid lipase**, treatment of lysosomal acid lipase deficiency; HungaroTrial Ltd.
- **Silibinin-C-2',3-dihydrogensuccinate, disodium salt**, prevention of recurrent hepatitis C in liver transplant recipients; Rottapharm S.p.A.
- **Tesetaxel**, treatment of gastric cancer; Genta Development Ltd.

The European Commission granted 17 final orphan designations.

The COMP adopted nine lists of questions and two protocol assistance letters. Five oral hearings took place. Three applications for orphan medicinal product designation were withdrawn.

The COMP concluded that the following designated orphan medicinal product still fulfils the designation criteria and should be kept in the orphan registry:

- **TOBI Podhaler (tobramycin)** for treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis; Novartis Europharm Limited.

For the following designated orphan product a marketing authorisation application was made:

- **N-methyl D-(2,3,4,5,6-pentahydroxy-hexyl)-ammonium; 2-(3,5-dichlorophenyl)-benzoxazole-6-carboxylate (Tafamidix meglumine)** from FoldRx

Pharmaceuticals Ltd for the treatment of familial amyloid polyneuropathy. (EU designation number: EU/3/06/401).

The status of orphan designations/authorisations as of 8 October 2010 is given in the following table:

Year	Applications submitted	Positive COMP Opinions	Final negative COMP Opinions	Designations granted by Commission	Applications withdrawn	EU marketing authorisations since 2000
2010	136	105	3	86	40	4
2000-2009	1060	727	14	699	249	58
Total 2000-2010	1196	832	17	785	289	62

Next COMP meeting: 9 – 10 November 2010

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Guide to Drug Regulatory Affairs • www.drugregulatoryaffairs.eu

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