News from the EMA

Activities of the COMP

Results from the October meeting 2011 of the COMP

The COMP met on 5-7 October 2011 and adopted the following **twelve positive opinions on orphan medicinal product designation**:

- **Plerixafor** for adjunctive treatment to cytotoxic therapy in acute myeloid leukaemia, Genzyme Europe B.V.
- **Alpha-tocotrienol quinone** for treatment of Leigh syndrome, Edison Orphan Pharma BV.
- **Pegylated proline-interferon alpha-2b** for treatment of polycythaemia vera, AOP Orphan Pharmaceuticals AG.
- **4-[[9-[(3S)-tetrahydro-3-furanyl]-8-[(2,4,6-trifluorophenyl)amino]-9H-purin-2-yl]amino]-trans-cyclohexanol** for treatment of idiopathic pulmonary fibrosis, Celgene Europe Limited.
- **Adeno-associated viral vector serotype 8 containing the human *AIPL1* gene** for treatment of Leber's congenital amaurosis type 4**.**
- **Cysteamine** for treatment of cystic fibrosis, NovaBiotics Ltd.
- **Human haptoglobin** for treatment of sickle cell disease, Bio Products Laboratory Ltd.
- **Interferon gamma** for treatment of Friedreich's ataxia, Prof. Roberto Testi.
- **N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-D-gamma-glutamyl-(2S)-2-amino-beta-alanyl-L-alpha-aspartyl-L-cysteine and folic acid** for diagnosis of folate receptor status in ovarian cancer, Endocyte Europe B.V.
- **Nanoliposomal irinotecan** for treatment of pancreatic cancer, Merrimack Pharmaceuticals UK Limited.
- **Resminostat** for treatment of Hodgkin's lymphoma, 4 SC AG.
- **Vincaleukoblastin-23-oic acid, O4-deacetyl-2-[(2-mercaptoethoxy)carbonyl]hydrazide, disulfide with N-[4-[[(2-amino-3,4-dihydro-4-oxo-6-pteridinyl)methyl]amino]benzoyl]-L-gamma-glutamyl-L-alpha-aspartyl-L-arginyl-L-alpha-aspartyl-L-alpha-aspartyl-L-cysteine** for treatment of ovarian cancer, Endocyte Europe B.V.

Since the September meeting the European Commission granted **16 final designations as orphan medicinal product.**
The COMP adopted **five lists of questions** on initial applications.
Five oral hearings took place.
Two applications for orphan medicinal product designation were **withdrawn**.

Prior to the granting of a marketing authorisation, the COMP adopted 1 opinion recommending that the following orphan medicinal product should be kept in the EU registry of orphan medicinal products:

- **Soliris** (Eculizumab) for treatment of atypical haemolytic uremic syndrome; Alexion Europe SAS.

For the following orphan medicinal product an application for centralised approval has been made:

- **Bosulif** (Bosutinib) for treatment of chronic myeloid leukaemia; Pfizer.

The status of orphan designations/authorisations as of 7 October 2011 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
2011	117	91	1	84	36	2
2000-2010	1234	850	16	827	300	57
Total 2000-2011	1351	941	17	911	336	59

Next COMP meeting: 8-9 November 2011

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Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu