News from the EMA

Activities of the COMP

Results from the September meeting 2011 of the COMP

The COMP met on 6-8 September 2011 and adopted the following **13 positive opinions on orphan medicinal product designation**:

- **2-hydroxyoleic acid** for treatment of glioma, Lipopharma Therapeutics SL
- **Recombinant human minibody against complement component C5** for treatment of primary membranoproliferative glomerulonephritis, ADIENNE S.r.l.
- **1-(4-{4-amino-7-[1-(2-hydroxyethyl)-1*H*-pyrazol-4-yl]thieno[3,2-*c*]pyridin-3-yl}phenyl)-3-(3-fluorophenyl)urea** for treatment of acute myeloid leukaemia; Abbott Laboratories
- **Adeno-associated viral vector containing the human alpha-N-acetylglucosaminidase gene** for treatment of mucopolysaccharidosis type IIIB (Sanfilippo B syndrome); Institut Pasteur
- **Brivanib alaninate** for treatment of hepatocellular carcinoma; BMS Pharma EEIG
- **Clonidine hydrochloride** for prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy; Bioalliance Pharma
- **Gallium (68Ga)-pasireotide tetraxetan** for diagnosis of gastro-entero-pancreatic neuroendocrine tumours; OctreoPharm Sciences GmbH
- **Glycosylation independent lysosomal targeting tagged recombinant human acid alpha glucosidase** for treatment of glycogen storage disease type II (Pompe's disease); BioMarin Europe Ltd
- **Human platelet antigen-1a immunoglobulin** for prevention of fetal and neonatal alloimmune thrombocytopenia due to human platelet antigen-1a incompatibility; Prophylix Pharma AS
- **L-cysteine, L-leucyl-L-alpha-glutamyl-L-alpha-glutamyl-L-lysyl-L-lysylglycyl-L-asparaginyl-L-tyrosyl-L-valyl-L-valyl-L-threonyl-L-alpha-aspartyl-L-histidyl-S-[1-[(4-carboxycyclohexyl)methyl]-2,5-dioxo-3-pyrrolidinyl]-complex with keyhole limpet hemocyanin** for treatment of glioma; Orphix Consulting GmbH
- **Lenalidomide** for treatment of mantle cell lymphoma; Celgene Europe Limited
- **Mifepristone** for treatment of hypercortisolism (Cushing's syndrome) of endogenous origin; Voisin Consulting S.A.R.L
- **Sinapultide, dipalmitoylphosphatidylcholine palmitoyl-oleoyl phosphatidylglycerol, sodium salt and palmitic acid** for treatment of cystic fibrosis; Pharm Research Associates (UK) Limited.

Since the July meeting the European Commission granted **18 final designations as orphan medicinal product.**
The COMP adopted **seven lists of questions** on initial applications.
Nine oral hearings took place.
Eight 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:

- **Mercaptopurine Nova Laboratories (oral suspension))** for treatment of acute lymphoblastic leukaemia, Nova Laboratories Limited – UK
- **Plenadren (hydrocortisone, modified release tablet)** for treatment of adrenal insufficiency; DuoCort Pharma AB – Sweden
- **Vyndaqel (N-methyl D-(2,3,4,5,6-pentahydroxy-hexyl)-ammonium; 2-(3,5-dichloro-phenyl)-benzoxazole-6-carboxylate)** for treatment of familial amyloid polyneuropathy; Pfizer Speciality UK Limited.

For the following orphan medicinal products am application for centralised approval has been made:

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

According to the Regulation (EC) on orphan medicinal products the orphan status is valid for 10 years as a rule. The first two orphan products – Fabrazyme (Agalsidase beta) and Replagal (Agalsidase alfa) for the treatment of Morbus Fabry - had been approved on 3 August 2001. Therefore their orphan status has been expired and these products were removed from the Community register of orphan medicinal products. The figures for marketing authorisations in the following table has been accordingly adapted.

The status of orphan designations/authorisations as of 8 September 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	108	79	1	68	32	2
2000-2010	1234	850	16	827	300	57
Total 2000-2011	1342	929	17	895	332	59

Next COMP meeting: 5-7 October 2011

Exclusively reported by Dr. Siegfried Throm, German Association of Research-Based Pharmaceutical Companies (e-mail: s.throm@vfa.de)
for:
Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu