News from the EMA

Activities of the COMP

Results from the September meeting 2012 of the COMP

The COMP met on 4-5 September 2012 and elected for a 3-year mandate the new Chair, Prof. Bruno Sepodes and the new Vice-Chair, Ms Lesley Greene.
The COMP adopted the following **12 positive opinions on orphan medicinal product designation**:

- **[2-Cyano-3-cyclopropyl-3-hydroxy-N-(3-methyl-4-trifluoromethylphenyl)prop-2-enamide]** for treatment of traumatic spinal cord injury, Algiax Pharmaceuticals GmbH.
- **Asp-Arg-Val-Try-Ile-His-Pro** for treatment of acute lung injury, Tarix Pharmaceuticals Limited.
- **Mavoglurant** for treatment of fragile X syndrome**,** Novartis Europharm Limited.
- **Rucaparib** for treatment of ovarian **cancer,** Clovis Oncology UK Limited.
- **Alpha-1 proteinase inhibitor (for inhalation use)** for treatment of cystic fibrosis**,** Grifols Deutschland GmbH.
- **Belinostat** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), TopoTarget A/S.
- **Humanised monoclonal IgG4 antibody against tissue factor pathway inhibitor** for treatment of haemophilia A, Novo Nordisk A/S2.
- **Lurbinectedin** for treatment of ovarian cancer, Pharma Mar SA Sociedad Unipersonal.
- **Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine *alpha-(1,3)-galactosyltransferase* gene** for treatment of pancreatic cancer, European Medical Advisory Services Limited.
- **Obinutuzumab** for treatment of chronic lymphocytic leukaemia, Roche Registration Limited.
- **Recombinant human lecithin cholesterol acyltransferase** for treatment of lecithin cholesterol acyltransferase deficiency, Alphacore Pharma Limited.
- **Liposomal daunorubicin** for treatment of acute myeloid leukaemia, Galen Limited.

The COMP noted that four applications were **withdrawn** by their sponsors.

Since the July meeting 2012 the European Commission granted **26 final designations as orphan medicinal product.**
The COMP adopted **eight lists of questions** on initial applications and **four Protocol Assistance** letters.
Eight oral hearings took place.

Prior to the granting of an EU marketing authorisation the COMP adopted **3 opinions** recommending that the following **orphan medicinal products be kept in the EU registry** of orphan medicinal products:

- **Adcetris** (monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E) for treatment of anaplastic large cell lymphoma; Takeda (Europe) Ltd.
- **Adcetris** (monoclonal antibody against human CD30 covalently linked to the cytotoxin monomethylauristatin E) for treatment of Hodgkin lymphoma; Takeda (Europe) Ltd.
- **Glybera** (adeno-associated viral vector expressing lipoprotein lipase) for treatment of lipoprotein lipase deficiency; uniQure biopharma B.V.

Since the July meeting for the following orphan medicinal products **marketing authorisation applications** have been made:

- **Winfuran** ((-)-17 cyclopropylmethyl-1,14 ß dihydroxy 4,5 alpha-epoxy 6ß-[N-mnethyl-trans-3-(3-furyl-acrylamido) morphinan hydrochloride) from Toray International UK Ltd. for treatment of uraemic pruritus
- **Masican** (N-(methyl-diazacyclohexyl-methylbenzamide-azaphenyl-aminothiopyrrole) from AB Science for treatment of malignant gastrointestinal stromal tumours.

The status of orphan designations/authorisations as of 5 September 2012 is given in the following table:

Year	Applica-tions submitted	Positive COMP Opinions	Final negative COMP Opinions	Designations granted by Commission	Applications withdrawn	Approved products with active orphan status since 2000
2012	136	95	1	102	30	8
2000-2011	1400	961	16	935	345	57
Total 2000-2012	1536	1056	17	1037	375	65

Next COMP meeting: 4-6 October 2012

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for:
Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu