News from the EMA

Activities of the COMP

Results from the November meeting 2011 of the COMP

The COMP met on 8-9 November 2011 and adopted the following **eleven positive opinions on orphan medicinal product designation**:

- **N,N'-bis(2-mercaptoethyl)isophthalamide** for treatment of mercury toxicity, CTI Life Sciences Ltd.
- **Sodium phenylbutyrate** for treatment of 5q spinal muscular atrophy, GMP-Orphan SAS.
- **Adeno-associated viral vector containing the human factor IX gene** for treatment of haemophilia B, Amsterdam Molecular Therapeutics BV.
- **Brentuximab vedotin** for treatment of cutaneous T-cell lymphoma, Takeda Global Research and Development Centre (Europe) Ltd**.**
- **Chimeric locked nucleic acid-deoxynucleoside phosphorothioate-linked oligonucleotide directed against microRNA-451** for treatment of polycythaemia vera, Miragen Therapeutics Europe Ltd.
- **Recombinant homodimer of the human annexin V** for prevention of the ischaemia/reperfusion injury associated with solid organ transplantation, Astellas Pharma Europe B.V.
- **Lipopolysaccharide of *Ochrobactrum intermedium*** for prevention of sepsis in at-risk premature infants of less than or equal to 32 weeks of gestational age, Diomune, S.L.
- **Liposomal combination of cytarabine and daunorubicin** for treatment of acute myeloid leukaemia, Celator UK (Ltd).
- **Mogamulizumab** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated), Gregory Fryer Associates Ltd.
- **Ornithine phenylacetate** for treatment of acute liver failure, Dr Ulrich Granzer.
- **Recombinant protein consisting of modified human growth hormone releasing hormone and the translocation and endopeptidase domains of botulinum toxin serotype D** for treatment of acromegaly, Syntaxin Limited.

Since the October meeting the European Commission granted **13 final designations as orphan medicinal product.**
The COMP adopted **five lists of questions** on initial applications.
Two 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Two applications for orphan medicinal product designation were **withdrawn**.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:

- **Bronchitol** (Mannitol) for treatment of cystic fibrosis; Pharmaxis Pharm. Ltd.

On 3 November 2011 Plenadren (hydrocortisone), an orphan medicinal product for treatment of adrenal insufficiency in adults was granted EU marketing authorisation.

The status of orphan designations/authorisations as of 9 November 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	139	102	1	97	40	3
2000-2010	1234	850	16	827	300	57
Total 2000-2011	1373	952	17	924	340	60

Next COMP meeting: 6-7 December 2011

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Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu