News from the EMA

Activities of the COMP

Results from the November meeting 2012 of the COMP

The COMP met from 6-7 November 2012 and adopted the following **13 positive opinions on orphan medicinal product designation**:

- **Alisertib** for treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated); Takeda
- **Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt** for treatment of acromegaly; Dr Ulrich Granzer
- **Voclosporin** for treatment of non-infectious uveitis; Granzer Regulatory Consulting & Services
- **4-(4-{[2-(4-chlorophenyl)-4,4-dimethylcyclohex-1-en-1-yl]methyl}piperazin-1-yl)-N-({3-nitro-4-[(tetrahydro-2H-pyran-4-ylmethyl)amino] phenyl}sulfonyl)-2-(1H-pyrrolo[2,3-b]pyridin-5-yloxy)benzamide** for treatment of chronic lymphocytic leukaemia; AbbVie Ltd.
- **Allopurinol sodium** for treatment of perinatal asphyxia; Pharmathen S.A.
- **Artesunate** for treatment of malaria; Dafra Pharma International N.V.
- **Erdosteine** for treatment of lead toxicity; Rafifarm SRL.
- **Exon 52 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy; Prosensa Therapeutics B.V.
- **Exon 55 specific phosphorothioate oligonucleotide** for treatment of Duchenne muscular dystrophy; Prosensa Therapeutics B.V.
- **Humanised single chain monoclonal antibody against CD37** for treatment chronic lymphocytic leukaemia; Emergent Product Development UK Ltd.
- **Maytansinoid-conjugated human monoclonal antibody against mesothelin** for treatment of malignant mesothelioma; Bayer Pharma.
- **Triheptanoin** for treatment of long-chain 3-hydroxyacyl-CoA dehydrogenase deficiency; B. Braun Melsungen.
- **Triheptanoin** for treatment of very long-chain acyl-CoA dehydrogenase deficiency; B. Braun Melsungen.

The COMP noted that 15 applications were **withdrawn** by their sponsors.

Since the October meeting 2012 the European Commission granted **12 final designations as orphan medicinal product.**
The COMP adopted **four lists of questions** on initial applications and **two Protocol Assistance** letters.
Six oral hearings took place.

Since the October meeting for the following **orphan medicinal product** a **marketing authorisation application** has been made:

- Tobramycin (inhalation use; Vantobra) from PARI Pharma GmbH for treatment of *Pseudomonas Aeruginosa* lung infection in cystic fibrosis

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

- **Purified bromelaine (NexoBrid)** from Teva Pharma for treatment of partial deep dermal and full thickness burns.

Since the October meeting the European Commission has granted **marketing authorization** for the following **orphan medicinal product:**

- **Glybera (Alipogene tiparvovec)** from Amsterdam Molecular Therapeutics, the first gene therapy product having got approval in the western world for treatment of adults with familial lipoprotein lipase deficiency on 29. October 2012.

The status of orphan designations/authorisations as of 7 November 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	168	126	1	116	50	9
2000-2011	1400	959	16	935	345	57
Total 2000-2012	1568	1085	17	1051	395	66

Next COMP meeting: 5-6 December 2012

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Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu