News from the EMA

Activities of the COMP

Results from the June meeting 2012 of the COMP

The COMP met on 12-13 June 2012 and adopted the following **nine positive opinions on orphan medicinal product designation**:

- **1-[(2-Chloro-4-methoxyphenoxy)methyl]-4-[(2,6-dichlorophenoxy) methyl]benzene** for prevention of poliomyelitis in patients with immunodeficiencies deemed at risk, ProPhase Development Ltd.
- **Metreleptin** for treatment of Barraquer-Simons syndrome, Aptiv Solutions (UK) Limited.
- **Metreleptin** for treatment of Berardinelli-Seip syndrome, Aptiv Solutions (UK) Limited.
- **Metreleptin** for treatment of familial partial lipodystrophy, Aptiv Solutions (UK) Limited.
- **Metreleptin** for treatment of Lawrence syndrome, Aptiv Solutions (UK) Limited.
- **(2S)-2-{[(2R)-2-[({[3,3-dibutyl-7-(methylthio)-1,1-dioxido-5-phenyl-2,3,4,5-tetrahydro-1,2,5-benzothiadiazepin-8-yl]oxy}acetyl)amino]-2-(4-hydroxyphenyl)acetyl]amino}butanoic acid** for treatment of progressive familial intrahepatic cholestasis, Albireo AB.
- **Hexasodium phytate** for treatment of calciphylaxis, Sanifit Laboratoris, S.L.
- **Human apotransferrin** for treatment of congenital hypotransferrinaemia, Sanquin Blood Supply Foundation.
- **Recombinant human pentraxin-2** for treatment of idiopathic pulmonary fibrosis, Appletree Europe S.à.r.l.

Since the May meeting 2012 the European Commission granted **14 final designations as orphan medicinal product.**

The COMP adopted **eight lists of questions** on initial applications.
Five oral hearings took place and **five applications** were **withdrawn** by their sponsors.

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

- **Kalydeco** (N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide) for treatment of cystic fibrosis, Vertex Pharmaceuticals (U.K.).

Since the April meeting the European Commission has **refused** a **marketing authorisation** after appeal of the applicant for the following orphan medicinal product:

- **Orphacol** (cholic acid) solution for injection from Laboratoires CTRS for treatment of inborn errors in primary bile acid synthesis due to certain deficiencies in infants, children and adolescents aged 1 month to 18 years and adults on 25 May 2012.

The status of orphan designations/authorisations as of 13 June 2012 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
2012	91	69	0	66	23	3
2000-2011	1400	961	18	934	345	60
Total 2000-2012	1491	1030	18	1001	368	63

Next COMP meeting: 10-11 July 2012

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