News from the EMA

Activities of the COMP

Results from the December meeting 2011 of the COMP

The COMP met on 6-7 December 2011 and adopted the following **nine positive opinions on orphan medicinal product designation**:

- **(1S,3S)-3-amino-4-(difluoromethylene) cyclopentanecarboxylic acid hydrochloride** for treatment of West Syndrome, Catalent Pharma Solutions Limited.
- **Nimorazole maleate** for treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy, Conventia Medical LLP.
- **S[+] apomorphine** for treatment of amyotrophic lateral sclerosis, University of Sheffield.
- **Sodium phenylbutyrate** for treatment of carbamoyl-phosphate synthase-1 deficiency, Lucane Pharma SAS.
- **Sodium phenylbutyrate** for treatment of citrullinaemia type 1, Lucane Pharma SAS.
- **Sodium phenylbutyrate** for treatment of ornithine transcarbamylase deficiency, Lucane Pharma SAS.
- **Autologous haematopoietic cells genetically modified with a lentiviral vector containing the human *gp91(phox)* gene** for treatment of X-linked chronic granulomatous disease, Généthon.
- **Doxycycline hyclate** for treatment of familial amyloid polyneuropathy, Giampaolo Merlini.
- **Human monoclonal antibody against Fas ligand** for treatment of pemphigus, PinCell s.r.l.

Since the November meeting the European Commission granted **no more final designations as orphan medicinal product.**
The COMP adopted **nine lists of questions** on initial applications and one protocol assistance letter.
Two oral hearings took place.
Three applications for orphan medicinal product designation were **withdrawn**.

For the following orphan product a centralised marketing application was made:

- **Kalydeco** (N-(2,4-Di-tert-butyl-5-hydroxyphenyl)-1,4-dihydro-4-oxoquinoline-3-carboxamide) for treatment of cystic fibrosis; Vertex Pharm. (UK) Ltd.

The status of orphan designations/authorisations as of 7 December 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submitted	Positive COMP Opinions	Final negative COMP Opinions	Designations granted by Commission	Applications withdrawn	EU marketing authorisa-tions since 2000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	150	111	2	97	43	3
2000-2010	1234	850	16	827	300	57
Total 2000-2011	1384	961	18	924	343	60

Next COMP meeting: 10-11 January 2012

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Guide to Drug Regulatory Affairs www.drugregulatoryaffairs.eu