4th Workshop
Eurordis Round Table of Companies

"Common Drugs for Common Needs: the EU vs US Approach to Orphan Medicinal Product (OMP) Development"

Barcelona, Spain
Fundació Doctor Robert - UAB Casa Convalescència
June 30th, 2006

Programme

**MORNING 9:00 - 13:00**

Chairmen:
Dr. Marlene E. Haffner, Director, Office of Orphan Products Development, FDA, USA;
Mr. Yann Le Cam, Chief Executive Officer, Eurordis, EU

9:00 - 9:15 Welcome address

9:15 - 9:40 (including 5’ for questions) “OMP Regulations in the USA, the EU, Australia & Japan”
Prof. Josep TORENT-FARNELL, former COMP Chairman, Fundació Doctor Robert, Spain
-Differences in incentives: support for development vs MA approval

9:40 - 10:10 “Orphan-Drug Designations and Marketing Approvals in the New Millennium - FDA and EMEA Experiences”
Dr. Tan NGUYEN, Medical Officer, FDA, USA
- 2000-2005 EU and US experience (designations, marketing authorisation, types of drugs and indications, etc.)
- Determining factors in the designation process

10:10 - 11:00 Open discussion

11.00 - 11.20 – COFFEE BREAK

Dr. Fabrizia BIGNAMI, Therapeutic Development Officer, Eurordis, France
- Size, age and nationality of pharmaceutical companies; number of MAs per company
- Modelling for rate and time until MA
- Possible differences in local motivation of industry for OMPs (incentives and cultural factors)

11:50 – 13:00 Open discussion

13:00-14:00 – LUNCH
AFTERNOON 14:00 -16:30
Chairmen:
Dr Kerstin Westermark, Chair of the COMP, EU;
Mrs Diane Edquist Dorman, Vice President, Public Policy, NORD, USA

14:00 -14:30 “Joint EMEA/FDA Scientific Advice” (including discussion)
Dr Agnès Saint-Raymond, Head of Sector Scientific Advice and Orphan Drugs
Paediatric Medicinal Products, Acting Head of Sector Safety & Efficacy, EMEA, UK

14:30 - 15:30 Debate: “Controversy: Coordinated Development vs Second Market”
Two sponsors defending opposite positions:
Mr Henk Schuring, Genzyme Europe, The Netherlands & Mr Gregg Lapointe, Sigma-Tau, USA
- Incentives and inhibiting factors for an “outside” development
- Which strategy for a universal availability of OMPs?
- Simultaneous development of “US and EU data”
- Early submission of EU or US files on the other side of the Atlantic
- Differed submission of a new file

15:30 – 16:30 Panel discussion
- Is there a place for transatlantic and global cooperation?
- OD designation & protocol assistance tomorrow and beyond?
- MA granted twice in two different markets?
- Is there a medical need for two separate developments?
- Are duplicated studies ethical?
- Sharing economic benefits of larger market: more OMPs for more patients

16:30 End of Workshop