EURORDIS
European Organisation for Rare Diseases

# 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**

**11:20 - 11:50** ***"Orphan Drug Development: US and EU Experiences"***

**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**

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**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**