5th Workshop
Eurordis Round Table of Companies

"Rare Disease Patient Registries: an Essential Tool in the Development of Therapies? "

November 20th, 2006
Hôtel Lutetia
45, boulevard Raspail - 75006 Paris- France

Programme

8:30 Registration & coffee

MORNING 9:00 - 13:00
Chairpersons:
Dr. Marlene E. Haffner, Director, Office of Orphan Products Development, FDA, USA;
Dr. Domenica Taruscio, National Centre Rare Diseases, Italian Public Health Institute, COMP member, Italy

9:00 - 9:15 *Welcome address*

9:15 - 9:50 ***"Discovering registries"*** *(35' including discussion)*
(Dr. Maurizio Clementi, University of Padova, North East Italy Congenital Malformation Registry, member of EUROCAT)

9:50 -10:25 ***"Role of registries as major tools for medicinal product development in the pre and post-marketing phases"****(35')*
(Dr. Per Nilsson, Actelion)

10:25 -11:00 Discussion *(35')*

11.00 - 11.20 – COFFEE BREAK

11:20 – 11:55 ***"Legal and ethical issues related to registries in Europe"***
(Dr. Stella Blackburn, EMEA, UK) *(35')*

11:55 – 13:00 ***Discussion*** *(1h05')*

13:00-14:15 – LUNCH

AFTERNOON 14:15 -16:30

Chairpersons:
Mrs Maria Hardin, Vice President for Patient Services, NORD, USA
Mr Yann Le Cam, Chief Executive Officer, Eurordis, EU

14:15 -15:30 ***"Rare Disease registries: real-life experiences"*** *(1h 15')*

- **EuroWilson project database**

(Prof. Stuart Tanner, University of Sheffield, UK)

- **European Paediatric Cancer Registry**

(Dr Jacqueline Clavel, National Registry of Childhood Blood Malignancies, INSERM U754, France)

- **PTC Therapeutics and the Cystic Fibrosis Foundation's registry**

(Mrs Cláudia Hirawat, PTC Therapeutics, USA and Dr Hanne Vebert Olesen, CF Centre Aarhus, Denmark)

15:30 -16:30 ***Panel discussion*** *(1h)*

Registries for Rare Diseases: legitimacy of all interested parties in the creation, management access and ownership of patient registries. Respective responsibilities, best practices, etc.

16:30 **End of Workshop**