



15<sup>th</sup> Workshop of the Eurordis Round Table of Companies (ERTC)

***“Compassionate Access to Rare Disease Therapies”***

***Monday 21 November 2011***  
***Paris, France***

**Les Salons de l’Aéro-Club de France**  
**6, rue Galilée – 75116 Paris**

***Agenda***

***Morning session***

***Chairpersons:***

**Kerstin Westermark (Chairperson, Committee for Orphan Medicinal Products, EMA)**  
**Ségolène Aymé (Chairperson of the EUCERD, Director of Orphanet)**

**9:00 – 9:15:** *Welcome address: Yann Le Cam (CEO, EURORDIS)*

**9:15 – 9:55:** **“EU Legislation on Compassionate Use: Five Years of Experience”** – Segundo Mariz (European Medicines Agency, Orphan Medicines Sector, UK) *(including 10’ for Q&A)*

*(Presentation of the EU legislation on CU and its basis, its guidelines of implementation, the specific experience with CU for OMPs as generator of data useful to support the MA application)*

**9:55 – 10:55:** **“What is the Role of Compassionate Use in Drug Development?”** - Carla Hollak (Academic Medical Centre in Amsterdam, The Netherlands) and Matilde Peñas (PharmaMar, Spain) *(including 10’ for Q&A after each presentation)*

**10:55 – 11:25 - COFFEE BREAK**

**11:25 – 12:00:** **“Overview of Compassionate Use Systems Across Europe”** – Lisette Vromans, MSD-EBE representative, The Netherlands *(EBE survey results on CU)* and Kevin Loth, Celgene & Vice-Chair EBE-EuropaBio Joint Task Force on RD / member of EUCERD, UK *(policy aspects)* *(including 10’ for Q&A)*

**12:00 – 12:45:** **“Overview of Recent Compassionate Use Programmes for OMPs and Issues Raised”** – François Houÿez (EURORDIS) *(including 10’ for Q&A)*

**12:45 – 14:00 - LUNCH**

## *Afternoon session*

### ***Chairpersons:***

**Arielle North (former Institutional Liaison Officer, EMA).  
Yann Le Cam (Chief Executive Officer, EURORDIS)**

### **14:10 – 16:30: Introduction of the Panel and structured discussion with the audience:**

Speakers from the morning and panel members (representatives from NCAs in charge of Compassionate Use):

- Chantal Belorgey (Afssaps, France)
- César Hernández (Spanish Medicines Agency)
- Eda Lopato (State Agency of Medicines, Estonia)
- Antoni Montserrat (European Commission, DG Health and Consumers, Luxembourg)

### **Potential questions for the panel:**

- What is the role of Compassionate Use in solving specific urgent public health issues?
- Does the Directive on Patients Right to Access Cross Border Health Care provide guidance on access to Compassionate Use in a foreign country?
- Could Compassionate Use play a role in the benefit/risk evaluation?
- What are the consequences for the patients accessing a Compassionate Use programme, if the product does not obtain a Marketing Authorisation?
- Should new patients still be included in an ongoing Compassionate Use programme once the product has obtained an MA, but is not yet registered at national level?
- Could the Health Technology Assessment of a product be facilitated by the contribution of the data issued from a Compassionate Use programme?

### **16:30 End of the workshop**