27th Workshop of the EURORDIS Round Table of Companies (ERTC)

Patient engagement in the product life-cycle and community advisory boards (CABs)

Tuesday, 16 October 2018 (09:00 to 17:00)
Recinte Modernista – Barcelona - Spain

PROGRAMME

Morning Session Chaired by: **Co-chairs:** **Fabrizia Bignami, EU Medical Lead, Bluebird Bio** **Jonathan Pearce, Regional Director Europe, Lymphoma Coalition**	
Engaging with patients: from ticking a box to real value for business and patients	
09:00 – 09:15	**Welcome introduction, setting the scene & goals for the day** • **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe
09:15 – 10:15	**Introducing the concept of patient engagement** • **Merlin Williams,** Consultant, Executive Insight • **Luciana Ballini,** Senior Researcher, Health Technology Assessment and Health Services Research - Regione Emilia-Romagna • **Nathalie Morgensztejn,** Virology and gene therapy, Division of vaccines, anti-infectives, hepato-gastroenterology, dermatology, gene therapy and rare metabolic diseases, Agence Nationale de Sécurité des Médicaments (ANSM)

10:15 – 11:00	**Patient engagement with healthcare industry: needs and expectations**
	• **The PARADIGM project: objectives and outcomes**
	  ○ **Magda Chlebus,** Director, Science Policy, EFPIA
	• **Benefits for healthcare industry**
	  ○ **Joëlle Rebetez,** Associate Director, Global Patient Advocacy, Global Medical Affairs, Actelion
	• **Joint development between a pharmaceutical company and a patient organisation**
	  ○ **Carla Fladrowski,** CAB E-TSC, European Tuberous Sclerosis Complex Association
	  ○ **Serge Smeets,** Reg. Medical Director Rare Diseases, Region Europe, Novartis Pharma B.V.

11 :00 – 11 :20	***Coffee break***
11 :20 – 12 :45	**Community Advisory Boards (CABs)** **Introduction & historical perspective – The Patient Investigator** • **Rob Camp,** Patient Engagement Senior Manager - CABs, EURORDIS **The EUROCAB programme** *How to join a CAB, practical aspects, financial arrangements*

	• **François Houÿez,** Treatment Information and Access Director / Health Policy Advisor, EURORDIS **Interactive session with voting and Q&A**
12:45– 13:45	***Lunch***
Afternoon Session Chaired by: **François Houÿez,** Treatment Information and Access Director / Health Policy Advisor, EURORDIS **Wiebke Sauter,** Senior Clinical Research Scientist, Boehringer Ingelheim	
From Concept to Reality	

13 :45 – 14 :30	**Case studies: recent CABs (active or in creation)**
	• **The Cystic Fibrosis Europe CAB**
	  ○ **Flaminia Macchia,** Director EU GAPP, Vertex
	  ○ **Hilde de Keyser,** Cystic Fibrosis Europe
	• **The Duchenne CAB**
	  ○ **Sally Hofmeister**, World Duchenne Organization / UPPMD
	  ○ **Elena Zhuravleva,** Patient Partnership Director, Roche
	• **Rare eye diseases CAB**
	  ○ **Russell Wheeler,** LHON Society UK

14:30 – 14:40	**Introduction to the breakout sessions** Afternoon co-chairs
14:45 – 15:55	**<u>Breakout session 1</u>: Enterprises, non-profits and/or patients' organisations as sponsors of clinical trials** In this session we will address: ✓ How can a CAB function? ✓ What special arrangements are needed? • Moderator: **François Houÿez,** Treatment Information and Access Director / Health Policy Advisor, EURORDIS • Rapporteur: **Carol Pitcher Towner**, Vice President, International Regulatory Affairs, Alnylam
	<u>Breakout session 2:</u> Research projects that require cooperation between competitors In this session we will address: ✓ multi-investigational products trials ✓ treatment strategy trials ✓ creation / management of disease registries ✓ pre-competitive research and selection/development/adaptation of PROs ✓ evaluation guidelines (EMA, HTA) • Moderator: **Alexandre Mejat,** EURORDIS Board Member and Scientific International Affairs Manager for AFM-Téléthon • Rapporteur: **Jennifer Wilson,** Head of Patient Advocacy – International, Amicus
	<u>Breakout session 3</u>: Outstanding issues In this session, we will discuss: ✓ The EURORDIS Charter for Clinical Trials - legal issues ✓ Standardisation of documents such as confidentiality undertaking, insider trading agreements. Can we have a working group of companies involved in CABs developing a template? ✓ Transparency: making names of CAB members and sponsor's delegation public ✓ Sponsors' behaviour vis-à-vis CABs and their members. Points for discussion. • Moderator: **Rob Camp,** Patient Engagement Senior Manager - CABs, EURORDIS • Rapporteur: **Fatima Scipione,** Senior Director, Patient Advocacy, Takeda Oncology, USA

16:00 – 16:20	**Feedback from breakout sessions** Moderated by afternoon co-chairs
16:20 – 16:45	**Questions to key actors** • **Luciana Ballini,** Senior Researcher, Health Technology Assessment and Health Services Research - Regione Emilia-Romagna • **Magda Chlebus,** Director, Science Policy, EFPIA • **Nathalie Morgensztejn,** Virology and gene therapy, Division of vaccines, anti-infectives, hepato-gastroenterology, dermatology, gene therapy and rare metabolic diseases, Agence Nationale de Sécurité des Médicaments (ANSM) • **Alexandra Moutet,** Global Head of Patient Affairs, UCB • **Alexander Natz,** Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)
16:45 – 17:00	**Concluding remarks** • **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe
17:00	***Meeting ends***