EURORDIS SUMMER SCHOOL
FOR PATIENT ADVOCATES
IN CLINICAL TRIALS AND DRUG DEVELOPMENT

EURORDIS
Rare Diseases Europe

Programme
June 2-6, 2014

A capacity building programme for patient representatives involved in the development, information and access to orphan, paediatric, advanced therapies, and health technology assessment

Co-funded by:

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ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURES NETWORK

AFMTÉLÉTHON
INNOVER POUR GUERIR

Developed with the support of:

Fundació Doctor Robert
UAB

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Inserm
Institut national
de la santé et de la recherche médicale

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June 2-6, 2014
BARCELONA, SPAIN

Welcome Dinner
2 June

Day 1
June 3rd

Life-cycle of Drug Development

Principles of clinical trials

Day 2
June 4th

Ethics

Post-marketing phases

Health Technology Assessment

Day 3
June 5th

Regulatory committees and working parties at the European Medicines Agency

Day 4
June 6th

Regulatory committees cont…

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PROGRAMME

Tuesday June 3, 2014 — Day 1

08:45-09:00	**EURORDIS** **Fundacio Dr. Robert**	**Welcome Address and Introduction to Summer School**
09:00-10:00	**Small group discussions using session 1 material provided**	
10:00-11:00	**Dr. Markku Toivonen**	**Clinical Research** • Need for evidence-based medicine • Life cycle of drug development from pre-clinical (specificity of orphan medicinal products) • Diagram demonstrating stages of drug development.
11:00-11:30	**Coffee break**	
11:30-12:30	**Small group discussions using session 2 material provided**	
12:30-13:30	**Dr. Markku Toivonen**	**Methodology principle in clinical trials** • The 'Gold Standards' ➢ Controlled ➢ Blind ➢ Randomised ➢ Small populations
13:30-15:00	**Lunch** **(Participants present themselves)**	
15:00-16:30	**Prof. John Norrie**	**Methodological principles** • Statistical significance • Clinical significance • *p* value • Statistical power • Statistical risks
19:00	***1st Focus Group on ethical and social issues on data sharing:*** *Led by Pauline McCormack and Anna Kole* *(Interested participants will have to register separately if they would like to attend. This session will be held at the Hotel Alimara)*	

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PROGRAMME

Wednesday June 4, 2014 **Day 2**

09:00-10:00	**Group discussions using session 3 material provided**	
10:00-11:00	**ds Eric Koster MA**	**Ethical aspects** • Therapeutic v Experimental situation • Consent for participation
11:00-11:30	**Mr. Rob Camp**	**Ethical Aspects from a US perspective**
11:30-11:45	**Coffee Break**	
11:45-12:45	**Group discussions using session 4 material provided**	
12:45-13:45	**Dr. Patrick Salmon**	**Regulatory procedures** • Importance of Post-Marketing phases • Compassionate use • Accelerated review • Conditional Approval • Marketing Authorisation under exceptional circumstances • Risk management plans
13:45-15:00	**Lunch**	
15:00-16:30	**Dr. Edmond Jessop**	• Introductory HTA workshop
19:00	***2nd Focus Group on ethical and social issues on data sharing:*** *Led by Pauline McCormack and Anna Kole* *(Interested participants will have to register separately if they would like to attend. This session will be held at the Hotel Alimara)*	

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PROGRAMME

Thursday June 5, 2014 — Day 3

09:00-09:30	**Ms. Nathalie Bere**	General Introduction to the European Medicines Agency
09:30-10:15	**Dr. Jordi Llinares Garcia**	Committee for Orphan Medicinal Products (COMP)
10:15-11:15	**Mini-COMP session**	
11:15-11:30	**Coffee Break**	
11:30-12:30	**Prof. Josep Torrent i Farnell**	Scientific Advice Working Party (SAWP)
12:30-14:00	**Lunch**	
14:00-14:45	**Dr. Patrick Salmon**	Committee for Medicinal Products for Human Use (CHMP)
14:45-16:30 (Includes 5 minute break)	**Ms. Nathalie Bere**	**Training on Review of Product Information - workshop**

Tour of the Cosmo Caixa Museum

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PROGRAMME

Friday June 6, 2014 — Day 4

09:00-09:30	**Dr. Fernando de Andres-Trelles**	Paediatric Committee (PDCO)
09:30-10:30	**Mini-PDCO session**	
10:30-11:00	**Dr. Michele Lipucci di Paola**	Committee for Advanced Therapies (CAT)
11:00-11:15	**Coffee break**	
11:15-12:00	**Ms. Lise Murphy**	Patients' and Consumers' Working Party (PCWP)
12:00-12:30	**Mr. Albert van der Zeijden**	Pharmaceutical Risk Assessment Committee (PRAC)
12:30-13:30	**Lunch**	
13:30-14:00	**Dr. Christine Kubiak**	Presentation of ECRIN project
14:00-14:25	**Mr. François Houÿez**	Pharmacovigilence: the Role of Patient Associations other than the PRAC
14:25-14:45	**Mr. Rob Camp**	EUPATI: European Patients Academy for Therapeutic Innovation
14:45-15:30	**Open discussion and Closing of Summer School**	

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