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:

Co-funded by the European Commission

ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURES NETWORK

AFMTÉLÉTHON
INNOVER POUR GUÉRIR

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

Organisers:

Dr. Maria Mavris
Therapeutic Development Director
maria.mavris@eurordis.org

Nancy Hamilton
Training Manager
nancy.hamilton@eurordis.org

Fundació Dr. Robert
Iolanda Arbiol
Helena Garrigos

www.eurordis.org

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

EURORDIS
Rare Diseases Europe

June 17- 21, 2013
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…

EURORDIS activities

www.eurordis.org

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

EURORDIS
Rare Diseases Europe

PROGRAMME

Tuesday June 3, 2014 — Day 1

08:45-09:45	**EURORDIS & Fundacio Dr. Robert**	**Welcome Address and Introduction to Summer School (Participants present themselves)**
09:45-10:45	**Small group discussions using session 1 material provided**	
10:15-11:15	**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.
11:15-11:30	**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
13:30-14:30	**Lunch**	
14:30-16:00	**Prof. John Norrie**	**Methodological principles** • Statistical significance • Clinical significance • *p* value • Statistical power • Statistical risks

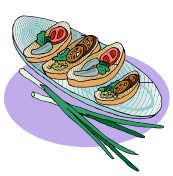
EURORDIS SUMMER SCHOOL FOR PATIENT ADVOCATES IN CLINICAL TRIALS AND DRUG DEVELOPMENT

EURORDIS
Rare Diseases Europe

PROGRAMME

Wednesday June 4, 2014 **Day 2**

09:00-10:00	**Group discussions using session 4 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 5 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



Cocktail evening at Hotel Alimara

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

EURORDIS
Rare Diseases Europe

PROGRAMME

Thursday June 5, 2014 — Day 3

09:00-09:30	**Dr. Juan Garcia Burgos**	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**	
11:30-12:00	**Prof. Josep Torrent i Farnell**	Scientific Advice Working Party (SAWP)
12:00-12:30	**Mini-SAWP session**	
12:30-13:30	**Lunch**	
13:30-14:00	**Dr. Patrick Salmon**	Committee for Medicinal Products for Human Use (CHMP)
14:00-16:00 (Includes 5 minute break)	**Dr. Juan Garcia Burgos**	**Training on Review of Product Information - workshop**

Tour of the Cosmo Caixa Museum

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

EURORDIS
Rare Diseases Europe

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	**Mr. François Houÿez**	Patients' and Consumers' Working Party (PCWP)
12:00-12:30	**To be announced (need a speaker) Albert.**	Pharmaceutical Risk Assessment Committee (PRAC)
12:30-13:30	**Lunch**	
13:30-14:00	**Dr. Christine Kubiak**	Presentation of ECRIN project
14:00-14:45	**Mr. Rob Camp and Mr. François Houÿez**	EURORDIS Clinical Trials Best Practice and demonstration of EUCTR – Clinical Trials Register
14:45-15:30	**Open discussion and Closing of Summer School**	