EURORDIS
Rare Diseases Europe

"ExPRESS Yourself!"

Expert Patients and Researchers
EURORDIS Summer School

Barcelona, Spain
June 1 - 5, 2015

A capacity building programme for patient representatives and researchers on information and access to orphan, paediatric, advanced therapies and health technology assessment.

cost
EUROPEAN COOPERATION IN SCIENCE AND TECHNOLOGY

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ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURES NETWORK

Fundació Doctor Robert
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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PLATAFORMA
UAB MALALTIES MINORITÀRIES

Inserm
Institut national
de la santé et de la recherche médicale

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Programme Committee
Annemieke Aartsma-Rus
Nathalie Bere
Monica Ensini
Nathalie Goemans
Virginie Hivert
François Houÿez
Yann Le Cam
Josep Torrent
Elizabeth Vroom

Organiser:
Nancy Hamilton
EURORDIS Training Manager
nancy.hamilton@eurordis.org

Local Organiser:
Iolanda Arbiol
Platforma Malaties Minoritàries

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Venue:
To be confirmed

EURORDIS
Platforme Maladies Rare
96 rue Didot
75014 Paris, France
www.eurordis.org

COST Action Exon Skipping (BM1207)
Annemieke Aartsma-Rus
LUMC, Albinusdreef 2
2333 ZA Leiden The Netherlands
A.M.Aartsma-Rus@lumc.nl
www.exonsipping.eu

PROGRAMME AT A GLANCE

Day 1: Monday, June 1st

Introduction
Introduction to Evidence Based Medicine for Expert Patients
Important considerations in Therapy Development for Researchers

WELCOME DINNER

Day 2: Tuesday, June 2nd

Methodology in Clinical Research
Statistics in Clinical Research
Ethical aspects of Clinical Research

Day 3:Wednesday, June 3rd

Handling of patient data
Different marketing authorisations available
Health Technology Assessment

Day 4: Thursday, June 4th

Patient involvement in Regulatory committees
and working parties at the European Medicines Agency

Day 5: Friday, June 5th

Regulatory Committees (continued) …
Hands on Sessions on Communication and Access to Clinical Trials

Monday, June 1st, 2015 Day 1

13:30 14:15	**Yann Le Cam (EURORDIS)** **Josep Torrent i Farnell (Fundacio Dr. Robert)**	**Expressing ourselves in 2015 and beyond: Global approaches and strategies.**
14:15 15:00	**Networking Session**	**Participants meet and introduce themselves** • Name and Association/Organisation • Area of success or an achievement they were proud of with their association/organisation) • Area where they may require advice or could benefit from the experience of another patient group/advocate/organisation)
15:00 16:00	**Small Group Session: Introduction to Evidence Based Medicine** Using documents with health claims from mainstream media: 1) How do you evaluate different sources of information? 2)How do you decide if a source can be trusted? 3)What, if any, evaluation criteria do you use to evaluate the information you receive? 4)What types of information do you need to evaluate information regarding the health benefits of a medicinal product?)	
15:00 16:00	**Annemieke Aartsma-Rus**	Parallel Session for researchers: **Things to consider when developing therapies:** lessons learnt from the DMD example
16:00 17:00	**Markku Tovivonen**	**Introductory overview of medicines development process** • Need for evidence-based medicine • Life cycle of drug development from pre-clinical • Diagram demonstrating stages of drug development
19:00	**WELCOME DINNER**	

Tuesday, June 2nd , 2015 Day 2

08:30 09:30	**Small group discussions** Example of a clinical trial protocol synopsis for discussion of the various aspects and considerations of a clinical trial. Discussion should be focused on terminology, design including endpoints, objectives, inclusion/exclusion criteria etc.	
09:30 10:30	**Markku Toivonen**	**Methodology principle in clinical trials** • The 'Gold Standards' • Controlled • Blind • Randomised • Small populations
10:30 11:00	**Coffee break**	
11:00 12:30	**John Norrie**	**Statistics in Clinical Research** • Statistical significance • Clinical significance • p value • Statistical power • Statistical risks
12:30 13:30	**Lunch**	
13:30 14:30	**Small Group Session** Tuskegee experiment + Analysis of an informed consent form and patient information leaflet – do these documents answer questions that patients or caregivers would have to enrol an individual in a clinical trial. Patient involvement in: 1)design of informed consent form, 2)role in patient recruitment and support, 3)ethics review board, 4)regulatory review..	
14:30 15:30	**Eric Koster**	**Ethical Aspects** • Therapeutic vs Experimental situations • Consent for participants
15:30 16:00	**Rob Camp**	**Ethical Aspects from a US perspective**
16:00 17:00	**Josep Torrent i Farnell**	**SAWP workshop**

Wednesday, June 3rd, 2015 Day 3

08:30 09:30	**Small Group Discussion on handling of patient data**	
09:30 10:30	**Anna Kole**	**Handling of patient data:** • Current regulatory framework • new legislations and relevance to stakeholders in medicines development
10:30 11:00	**Coffee Break**	
11:00 12:00	**Small Group Session: Different types of Marketing Authorisation** Articles on medicines such as Vioxx and Accomplia: understanding the difference between efficacy and effectiveness and appreciating that the study population in a clinical trial has little variation compared to the 'real-life' situation. The concept of benefit-risk is introduced here.	
12:00 13:00	**Patrick Salmon**	**Description and importance of different marketing authorisations available** • Exceptional circumstances and Conditional approval, • Progressive Patient Access, • Post-Authorisation Safety/Efficacy Studies
13:00 14:00	**Lunch**	
14:00 16:00	**Edmond Jessop**	**Introductory HTA workshop**
16:00 17:00	**Josep Torrent i Farnell**	**SAWP workshop (SEED, parallel Scientific advice/HTA)**

Thursday, June 4th, 2015 Day 4

08:30 09:00	Nathalie Bere	General Introduction to involvement of patients at the European Medicines Agency
09:00 09:45	Lise Murphy	Patients' and Consumers' Working Party (PCWP)
09:45 10:30	Jordi Llinares Garcia	Committee for Orphan Medicinal Products (COMP)
10:30 10:45	Coffee Break	
10:45 11:45	Mini-COMP session	
11:45 12:15	Josep Torrent i Farnell	Scientific Advice Working Party (SAWP)
12:15 12:45	How to prepare if you are invited to take part in Protocol Assistance	
12:45 13:45	Lunch	
13:45 14:15	Patrick Salmon	Committee for Medicinal Products for Human Use (CHMP) • Adaptive Licensing (Pilot phase)
14:15 15:30	Andrea Beyer (tbc)/ François Houÿez	The EMA Committee for Medicinal Products for Human Use (CHMP) (Feedback from pilot phase to involve patients in benefit/risk discussions at CHMP meetings)
15:30 16:45	Nathalie Bere, Annemieke Aartsma-Rus Elizabeth Vroom	Training on Review of Product Information – workshop

Friday, June 5th, 2015 Day 5

08:30 09:00	Michele Lipucci di Paola	Committee for Advanced Therapies (CAT)
09:00 09:30	Fernando de Andres-Trelles	Paediatric Committee (PDCO)
09:30 10:00	Mini-PDCO session/Mini CAT	
10:00 10:30	Coffee break	
10:30 11:00	Albert van der Zeijden	Pharmaceutical Risk Assessment Committee (PRAC)
11:00 11:30	François Houÿez	Pharmacovigilance: the Role of Patient Associations other than the PRAC + EudraVigilance
11:30 12:15	Elizabeth Vroom Annemieke Aartsma-Rus Nathalie Goemans	Outcome measures/Patient reported outcomes measures
12:15 12:30	Annemieke Aartsma-Rus	COST Action Exon Skipping
12:30 12:45	Christine Kubiak	ECRIN- IA
12:15 13:30	Lunch	
13:30 14:00	**Hands-on session: Finding trial information** Finding information Finding information on Clinical Trials: Where can patients find information on clinical trials – EUCTR – EU Clinical Trials Register and clinicaltrials.gov (FDA)	
14:00 15:00	**Hands-on session: Communication and information** Presentations of Rare Connect ,EURORDIS website and Cost lay summary document	
15:00 15:30	Open discussion and Closing of Summer School	