



EURODIS
RARE DISEASES EUROPE

EXPERT PATIENTS AND RESEARCHERS
EURODIS SUMMER SCHOOL

“ExPRESS YOURSELF!”

**CASTELLDEFELS,
BARCELONA, SPAIN
JUNE 5 - 9, 2017**

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



Co-funded by
the Health Programme
of the European Union

Co-organised in 2017 with:



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH



PLATAFORMA
UMB MALALTIES MINORITÀRIES



LEIDEN UNIVERSITY
MEDICAL CENTER



HOSPITAL DE LA
SANTA CREU I
SANT PAU
UNIVERSITAT AUTÒNOMA DE BARCELONA

Additional support provided by:



Inserm

Institut national
de la santé et de la recherche médicale



CIHR IRSC



Canadian Institutes of Health Research
Instituts de recherche en santé du Canada



RARE
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A EURORDIS INITIATIVE

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The Summer School was co-organised with:

European Medicines' Agency
Leiden University Medical Centre
Plataforma malalties minoritàries

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Plataforma Malalties Minoritàries

Venue:

SBN Events Hotel and Conference Centre
Castelldefels, Barcelona, Spain

EURORDIS

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MONDAY JUNE 5, 2017

10:00 -11:30	<i>Session for Researchers: the EMA framework dedicated to academics: Annemieke Aartsma-Rus and Nathalie Goemans</i>
12:00	Registration opens
12:30 -13:15	Welcome and finger food lunch: <i>Nancy Hamilton and Virginie Hivert</i>
13:15 -13:45	Introduction to ExPRESS 2017: <i>Yann Le Cam / Josep Torrent</i>
13:45 - 15:15	Networking Session, trainees introduce themselves: <i>Annemieke Aartsma-Rus and Elizabeth Vroom</i>
15:15 – 15:30	Short coffee break.
15:30 – 15:45	Review of Key Concepts in Clinical Trial Methodology: <i>Markku Toivonen</i>
15:45 - 17:15	Break-out Session on Clinical Trial Methodology: <i>Markku Toivonen, Rob Camp, Eric Koster, Elizabeth Vroom, Elisa Ferrer, Annemieke Aartsma-Rus</i>
17:15 - 18:00	Feedback Session & wrap-up: <i>Markku Toivonen</i>
19:00 - 22:00	Welcome Dinner

TUESDAY JUNE 6, 2017

08:55 - 09:00	Daily Overview: <i>Nancy Hamilton / Virginie Hivert</i>
09:00 - 09:15	Review of Key Concepts on Ethical Considerations: <i>Eric Koster</i>
09:15 - 10:45	Break-out Session on Ethics Committee <i>Eric Koster, Rob Camp, Markku Toivonen, Elizabeth Vroom, Kim Wever</i>
10:45 - 11:15	COFFEE BREAK
11:15 -12:00	Feedback Session: <i>Eric Koster / Rob Camp</i>
12:00 - 13:00	Affordable orphan drugs: A role for not-for-profit organisations <i>Elin Haf Davies (tbc)</i>
13:00 – 14:00	LUNCH
14:00 - 14:45	When patients take the lead <i>Julian Isla-Gomez</i>
14:45 - 15:30	PROs and multistakeholder meetings <i>Elisabeth Vroom</i>
15:30 – 15:45	Short Coffee break
15:45 -16:30	Making your voice heard as a patient advocate. <i>Kim Wever</i>
16:30 - 17:30	Laboratory Experiment: <i>Annemieke Aartsma-Rus</i>
Optional - tbc	Workshop on EU Funding opportunities for Researchers and Patient Expert: eRare, Infrastructures, ... <i>Mathieu Boudes</i>

WEDNESDAY JUNE 7, 2017

08:55 - 09:00	Daily Overview: <i>Nancy Hamilton / Virginie Hivert</i>
09:00 - 09:30	Introduction to the European Medicines' Agency & Public Engagement framework <i>Nathalie Bere</i>
09:30 – 10:30	Mini COMP & SAWP (designation, Protocol Assistance): <i>Maria Mavris, Kristina Larsson, Elisa Ferrer</i>
10:30 - 11:00	COFFEE BREAK
11:00 - 12:00	Mini COMP & SAWP (suite) (maintenance of orphan status at the time of MA) + How to get prepared: <i>Maria Mavris, Kristina Larsson, Elisa Ferrer</i>
12:00 - 13:00	Marketing authorization and patients input in B/R assessment: <i>Patrick Salmon /François Houÿez</i>
13:00 - 14:00	LUNCH
14:00 – 15:30	Mock written PRAC Consultation with Patients <i>Nathalie Bere, Maria Mavris</i>
15:30 - 16:00	Short Coffee Break
16:00 - 16:45	PRACTical Cases <i>François Houÿez</i>
16:45 - 17:30	Reporting side effects on SCOPE <i>François Houÿez</i>
17:30 – 18:00	Help lines workshop <i>François Houÿez</i>

THURSDAY JUNE 8, 2017

08:55 - 09:00	Daily Overview: <i>Nancy Hamilton / Virginie Hivert</i>
09:00 - 09:45	Cases studies on potential sources of bias in expert patients' contributions with regulators <i>François Houÿez</i>
09:45 – 10:30	Ensuring the credibility and transparency of expert patient contributions in interactions with regulators. <i>Nathalie Bere, Maria Mavris, François Houÿez</i>
10:30– 11:00	COFFEE BREAK
11:00 - 12:30	Workshop on Health Technology Assessment: <i>Edmund Jessop (NHS)</i>
12:30 - 13:30	LUNCH
13:30- 14:30	Break-out Session on HTA and Market Access: <i>Two mock appraisal committees.</i> <i>Committee A: Edmund Jessop / Violeta Stoyanova</i> <i>Committee B: Solange Rohou / Virginie Hivert</i>
14:30 – 15:00	Adaptive pathways and ADAPT-SMART <i>Solange Rohou (tbc)</i>
15:00 – 15:30	Short Coffee break
15:30-16:30	Round table: Engaging at the national level with regulatory and HTA bodies <i>Lena Ring, Laura Oliveira, Violeta Stoyanova, François Houÿez</i>
16:30 - 17:00	Introduction to MoCA: Mechanism of Coordinated Access <i>Yann Le Cam, Elisa Ferrer</i>

FRIDAY JUNE 9, 2017

08:55 - 09:00	Daily Overview: <i>Nancy Hamilton / Virginie Hivert</i>
09:00 - 10:00	Real World Experience of Patients Negotiating Compassionate Use <i>François Houyez</i>
10:00 -10:30	COFFEE BREAK
10:30 - 11:30	Social Media for Patients and Researchers <i>Rob Camp, Denis Costello, Tom Koukoulis</i>
11:00 - 12:30	Wrap-Up Session: How do you see yourself acting based on your participation in the training course <i>Josep Torrent and Fernando de Andres-Trelles</i>
12:30	Close of meeting