EURORDIS
SUMMER SCHOOL

EXPERT PATIENTS AND RESEARCHERS

EURORDIS SUMMER SCHOOL

"EXPRESS YOURSELF!"

CASTELLDEFELS, BARCELONA, SPAIN

JUNE 11 -15, 2018

A capacity building programme for patient representatives & researchers on medicines development, ethics in medical research, access to orphan, paediatric, advanced therapies and health technology assessment.

EURORDIS.ORG

#EURORDISSummerSchool

Programme Committee Members:

EUROPEAN MEDICINES AGENCY
Kristina Larsson
Maria Mavris

EURORDIS
Elisa Ferrer
Nancy Hamilton
Virginie Hivert
François Houÿez
Yann Le Cam

PLATAFORMA MALALTIES MINORITÀRIES
Josep Torrent
Iolanda Arbiol

LEIDEN UNIVERSITY MEDICAL CENTER
Annemieke Aarstma-Rus

The Summer School is co-organised with:
European Medicines' Agency
Leiden University Medical Centre
Plataforma malalties minoritàries

Co-funding:
"This Summer School has received funding under an operating grant from the European Union's Health Programme (2014-2020)."

Financial support also provided by:
AFM Telethon
ECRIN
Platforma Malalties Minoritàries
Canadian Institute for Health Research

Venue:
SBN Events Hotel and Conference Centre
Castelldefels, Barcelona, Spain

EURORDIS
Nancy Hamilton
EURORDIS Open Academy Manager
nancy.hamilton@eurordis.org
Platforme Maladies Rares
96 rue Didot 75014 Paris, France
www.eurordis.org

Lucy Michaeloudis
EU Project Assistant
lucy.michaeloudis@eurordis.org

Local Organiser:
Iolanda Arbiol
Plataforma malalties minoritàries

EURORDIS.ORG

EURORDIS RARE DISEASES EUROPE

Introduction

Patients are taking on ever increasing roles in advocating for medicines development, equal access to treatments across Europe, and ensuring that medical information is clear, accurate, and comprehensible. In order to help prepare them for these roles and as part of its commitment to empowering people living with rare diseases, EURORDIS launched its own training programme for expert patients in 2008.

The programme allows patients and researchers to sharpen their advocacy skills and gain an understanding of the regulatory process of therapeutic development so that they are able to advocate at a European level.

The programme has online and face-to-face components. The face-to-face portion trains a group of expert patients annually as part of an intensive 4.5-day course held in Barcelona, Spain.

The Summer School was developed to fill specific needs identified by expert patients, EURORDIS, European Medicines Agency, academic researchers and industry. Expert patients from EURORDIS are involved as representatives on EMA Scientific Committees and working parties. EURORDIS recognises their need for support and training that includes an overview of clinical research and methodology, medicines development and regulatory procedures.

In 2000, the Committee for Orphan Medicinal Products (COMP) was formed at the European Medicines Agency (EMA) and the European authorities had the foresight to include patients' representatives as permanent and full members with equal voting rights. Three patients' representatives sit on this committee and the position of Vice-Chair of the COMP has to date always been held by a patients' representative.

Since this time, a working party and three more scientific committees have been created, all of which include patients' representatives:
Patients' and Consumers' Working Party (PCWP) in 2006
Paediatric Committee (PDCO) in 2008
Committee for Advanced Therapies (CAT) in 2009
Pharmacovigilance and Risk Assessment Committee (PRAC) in 2012
Expert Patients are also frequently solicited for their input on the development of specific products for their disease, e.g.; via the process of Protocol Assistance with the Scientific Advice Working Party (SAWP).

MONDAY JUNE 11, 2018

TIME	ROOM	Session and Trainer(s)
10:00-11:30	Bcn-1	**Involving all stakeholders (patients, academics, and regulators) in therapy development** Annemieke Aarstma-Rus, Elizabeth Vroom
12:15-13:00	**Outside BCN-1**	*Registration*
13:00-14:00	**Rooms BCN-1 and 2**	**Introduction to ExPRESS 2018: English and Spanish participants together** *Virginie Hivert, Josep Torrent*
14:00-15:15	**Terrace**	**Group photo and Light Lunch**
15:15-15:45	**Room BCN-1**	**Review of key concepts in Clinical Trial Methodology:** *Markku Toivonen*
15:45-17:00	**Rooms BCN-1 Calma-1 Calma-2**	**Break-out session on Clinical Trial Methodology:** *Group 1: Markku Toivonen, Virginie Hivert* *Group 2: Eric Koster, Elizabeth Vroom,* *Group 3: Rob Camp, Annemieke Aartsma-Rus*
17:00-17:15	**Room BCN-1**	**Feedback session & wrap-up:** *Markku Toivonen*
17:15-18:15	**Room BCN-1**	**Networking session: trainees introduce themselves** *Annemieke Aartsma-Rus and Elizabeth Vroom*

Welcome Reception and Dinner
Casanova Beach Club
Castelldefels
19:00-22:00

TUESDAY JUNE 12, 2018

TIME	ROOM	Session and Trainer(s)
08:55-09:00	Room BCN-1	**Daily Overview:** *Nancy Hamilton, Virginie Hivert*
09:00-09:45	Room BCN-1	**Review of key concepts on ethical considerations:** *Eric Koster*
09:45-11:15	Rooms BCN-1 Calma-1 Calma-2 Calma-3	**Break-out session on Ethics Committee:** *Group 1: Eric Koster, Annemieke Aartsmaè-Rus* *Group 2: Rob Camp, Nancy Hamilton* *Group 3 Elizabeth Vroom, Markku Toivonen* *Group 4: Elin Haf Davies and Virginie Hivert*
11:15-11:45	Terrace	**Coffee Break**
11:45-12:15	Room BCN-1	**Feedback session:** *Eric Koster, Rob Camp*
12:15-13:00	Room BCN-1	**Affordable orphan drugs: a role for not-for-profit organisations:** *Elin Haf Davies*
13:00-14:00	Restaurant	**Lunch**
14:00-14:45	Room BCN-1	**Big data and rare diseases** *Julian Isla-Gomez*
14:45-15:30	Room BCN-1	**PROs and multistakeholder meetings:** *Elizabeth Vroom*
15:30-16:00	Room BCN-1	**European Clinical Research Infrastructure Network:** *Joaquín Sáez Peñataro*
16:00-16:30	Terrace	**Short break**
16:30-17:30	Room BCN-1	**Laboratory experiment:** *Annemieke Aartsma-Rus*

WEDNESDAY JUNE 13, 2018

TIME	ROOM	Session and Trainer(s)
08:55-09:00	Room BCN-1	**Daily Overview:** *Nancy Hamillton, Virginie Hivert*
09:00-09:30	Room BCN-1	**Engaging with the EMA Methodology and Support:** *Maria Mavris*
09:30-10:30	Room BCN-1	**Mini COMP & SAWP (designation, protocol assistance):** *Maria Mavris, Kristina Larsson*
10:30-11:00	Terrace	**Coffee Break**
11:00-12:00	Room BCN-1	**Mini COMP & SAWP (suite) maintenance of orphan status at the time of MA & how to get prepared:** *Maria Mavris, Kristina Larsson*
12:00-12:30	Room BCN-1	**PRIME Initiative:** *Zahra Hanaizi*
12:30-13:30	Room BCN-1t	**Ensuring the transparency of expert patient contributions in interactions with regulators. EMA policy on the handling of competing interests:** *Maria Mavris*
13:30-14:30	Restaurant	**Lunch**
14:30-15:15	Room BCN-1	**Panorama of actions patient organisations can take in pharmacovigilance:** *Mitul Jadeja*
15:15-15:45	Room BCN-1	**How to organise yourself to keep a close watch on your medicine:** *François Houÿez*
15:45-16:00	Room BCN-1	**Public Hearings: "Go live to the EMA"** *Maria Mavris*
16:00-16:30	Terrace	**Short break**
16:30-17:00	Room BCN-1	**Written consultation with patients by the PRAC:** *Maria Mavris*
17:00-17:30	Room BCN-1	**Navigation of the new EMA website:** *Maria Mavris*

THURSDAY JUNE 14, 2018

TIME	ROOM	Session and Trainer(s)
08:55-09:00	Room BCN-1	**Daily Overview:** *Nancy Hamilton, Virginie Hivert*
09:00-09:30	Room BCN-1	**The new development paradigm as explored through the EMA adaptive pathway initiative and the IMI ADAPT SMART project:** *Solange Rohou*
09:30-10:00	Room BCN-1	**Paradigm project: addressing sustainability of patient engagement:** *Mathieu Boudes, Elisa Ferrer*
10:00-10:30	Room BCN-1	**Introduction to MoCA: Mechanism of Coordinated Access** *Elisa Ferrer*
10:30-11:00	Terrace	**Coffee Break**
11:00-12:00	Room BCN-1	**Workshop on Health Technology Assessment:** *Edmund Jessop*
12:00-12:30	Room BCN-1	**Major changes ahead in HTA – How to engage with EUNetHTA:** *François Houÿez*
12:30-14:00	Restaurant	**Lunch**
14:00-14:50	Room BCN-1	**Marketing authorisation: how patients contribute to benefit/risk assessments:** *Geraldine O'Dea, François Houÿez, Elizabeth Vroom, Dimitrios Athanassiou*
14:50-15:50	Room BCN-1	**Do's and Don'ts when interacting with the EMA Cases studies on potential sources of bias in expert patients' contributions with regulators:** *François Houÿez, Elizabeth Vroom, Dimitrios Athanassiou*
15:50-16:30	Room BCN-1	**Engaging at the national level with regulatory and HTA bodies:** *Violeta Stoyanova, Josep Torrent, Maria José Vicente Edo*

FRIDAY JUNE 15, 2018

TIME	ROOM	Session and Trainer(s)
08:50-09:00	Room BCN-1	**Daily Overview:** *Nancy Hamilton, Virginie Hivert*
09:00-09:30	Room BCN-1	**Megafund for Rare Diseases:** *Dimitris Athanassiou*
09:30-10:00	Room BCN-1	**Introduction to business development (TBC)** *Claudia Hirawat*
10:00-11:00	Room BCN-1	**Real world experience of patients negotiating compassionate use:** *François Houÿez*
11:00-11:30	Restaurant	**Brunch**
11:30-12:00	Room BCN-1	**EURORDIS CABs Programme:** *Rob Camp*
12:00-12:45	Room BCN-1	**Quality of Patient Engagement Plenary Discussion** *François Houÿez*
12:45-13:00	Room BCN-1	**Wrap-up session: How do you see yourself acting based on your participation in the training course:** *Nancy Hamilton, Virginie Hivert*
Close of meeting		

Co-funded by
the Health Programme
of the European Union

Co-organised in 2018 with:

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

PLATAFORMA
MALALTIES MINORITÀRIES

LUMC
LEIDEN UNIVERSITY
MEDICAL CENTER

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

Additional support provided by:

AFMTÉLÉTHON
INNOVER POUR GUERIR

ECRIN
EUROPEAN CLINICAL RESEARCH INFRASTRUCTURES NETWORK

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

EURORDIS.ORG

EURORDIS
RARE DISEASES EUROPE