THEME DESCRIPTION:

The objectives of this theme are to take stock of the experience gained so far in the development of medicines for people living with rare diseases, and to examine the evolution of the field. We will look at recent scientific innovations and clinical research, regulatory solutions, roadblocks and challenges in developing therapies that match the needs of the patients, as well as ways of embedding real life evidence into the therapeutic development processes.

SESSION 0401: Friday 15th May 2020, 14:00 – 15:30

What do patients expect from therapy development?

Chair: Virginie Hivert, Therapeutic Development Director, EURORDIS & Vice-Chair, Therapies Scientific Committee of IRDiRC

Speakers:

Russell Wheeler, Leber’s Hereditary Optic Neuropathy Society, UK

Loris Brunetta, Thalassaelia International Federation, Italy

Alain Cornet, Lupus Europe, Belgium

Veronica Popa, MCT8-AHDS Foundation, Greece
SESSION 0402: Friday 15th May 2020, 16:30 – 18:00

Disruptive Innovations in clinical research

Chairs: Dr. Diego Ardigo, Global Rare Diseases R&D Head, Chiesi Farmaceutici, Italy and Chair, Therapies Scientific Committee of IRDiRC & Dr. Violeta Stoyanova-Beninska, Chair, Committee for Orphan Medicinal Products, EMA

Speakers:
Dr. Simon Day, Clinical Trials Consulting & Training, UK
Prof. Armando Magrelli, Istituto Superiore di Sanità, Italy

SESSION 0403: Saturday 16th May 2020, 09:00 – 10:30

Galaxy Guide for RD (IRDiRC)

Chairs: Dr. Diego Ardigo, Global Rare Diseases R&D Head, Chiesi Farmaceutici, Italy and Chair, Therapies Scientific Committee of IRDiRC & Virginie Hivert, Therapeutic Development Director, EURORDIS & Vice-Chair, Therapies Scientific Committee of IRDiRC

SESSION 0404: Saturday 16th May 2020, 11:00 – 12:30

Innovation in Advanced Therapy

In this session we will dive into the development and use of an ATMP from idea to approval and beyond. Using a particular product as an example, we will look at it from every angle: developer, patient, physician and regulator. Participants should leave this session with a greater understanding of the challenges relating to developing an ATMP and the subsequent use of it in clinical practice, which can be rather different from a standard product.

Chair: Kristina Larsson, Head of Orphan Medicines, European Medicines Agency

Speakers:
Tomasz Grybek, Fundacja Bohatera Borysa (Boris the Hero Foundation), Poland
Michela Gabaldo, Head of Alliance Management & Regulatory Affairs, Fondazione Telethon, Italy
SESSION 0405: Saturday 16th May 2020, 14:30 – 16:00

Bringing Real Life Into Therapeutic Development

Chairs: Dr. Daria Julkowska, Assistant Director, Institute GGB; Coordinator, European Joint Programme on Rare Diseases & Dr. Daniel O’Connor, Medical Assessor, Medicines and Healthcare Products Regulatory Agency (MHRA)

Speakers:

Prof. Faisal Ahmed, Endo-ERN EuRRECa, UK

Nick Sireau, AKU Society, UK