

THEME 4

WHEN THERAPIES MEET THE NEEDS: ENABLING A PATIENT-CENTRIC APPROACH TO THERAPEUTIC DEVELOPMENT

THEME LEADERS:

Dr. Diego Ardigo, Chair, Therapies Scientific Committee of IRDiRC and Project Manager, Chiesi Farmaceutici, Italy

Virginie Hivert, Vice-Chair, Therapies Scientific Committee of IRDiRD and Therapeutic Development Director, EURORDIS

THEME SUPPORT:

Maria Cavaller, Patient Engagement Junior Manager, EURORDIS

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?

This session aims to look at the present and future of medicines development and to reflect what patients are expecting for the next decade.

Building on results from the Rare Barometer Surveys (e.g. RD patients' experience with accessibility to treatments), the current work around patient engagement and a few figures illustrating the current state-of-play of therapies development for rare diseases, will also help to set the scene.

A panel of patients representing different rare disease areas, with expertise in several aspects of the medicines life-cycle and engaging with the ecosystem in a variety of capacities (EMA, EURO-CAB, IMI PARADIGM on sustainable patient engagement, ERNs, HTA, etc) will discuss the actual challenges, the needs and main expectations vis-à-vis the development of medicines, and the way for each stakeholder to contribute to improving RD patient lives.

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, Thalassaemia International Federation, Italy

Alain Cornet, Lupus Europe, Belgium

Veronica Popa, MCT8-AHDS Foundation, Greece

Eva Stumpe, SMA Europe, Germany

SESSION 0402: Friday 15th May 2020, 16:30 – 18:00

Disruptive Innovations in clinical research

This session will focus on innovative trends in clinical research, both in study design and execution, as well as innovative approaches to data collection. We will discuss the opportunities and challenges posed by these developments, together with the challenges foreseen in terms of regulatory and HTA assessment and the impact for the rare disease patients.

Attendees will leave this session with a broader view and understanding of the opportunities and challenges generated by current changes to how clinical research is conceived and executed, and the impact these changes will have on evidence generation in the future.

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 Rare Diseases (IRDiRC)

There are multiple challenges inherent to drug development for rare diseases and the traditional model of development is becoming less and less suitable.

The Orphan Drug Development Guidebook Task Force was set up within the International Rare Disease Research Consortium (IRDiRC) with the aim to create a simple guidebook describing the available tools and initiatives specific to rare disease development and how to best use them. The aim of the Guidebook is to benefit the various stakeholders working in orphan drug development.

The Guidebook includes fact-sheet descriptions of each drug development tool or resource (covering a large number of initiatives that are available worldwide), a series of standard use cases defining how and when to use them, and a series of practical checklists of items to consider at each step. Integration of such elements within a defined drug development framework is set out to generate better data quality, shorter development timelines, and better R&D efficiency.

This session will introduce the Guidebook in itself, the materials developed and the way to navigate this framework and aims to engage the audience in an interactive manner.

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

The patient should be central to all aspects of drug discovery, development, regulatory approval and future evidence generation of medicines, ensuring a complete life cycle approach to patient engagement. This session showcases where patients can bring their real-life experiences into different areas of the drug development pathway. We will explore how patient engagement in clinical trial design is increasingly valued, ensuring that studies capture what is important to measure from the end user perspective.

Patient reported outcomes (PRO) measure how a patient feels and functions whilst on a therapy. Developing PRO standards ensures robust data collection and interpretation, adding value to the information available about the patient experience whilst on a therapy.

The European Reference Networks facilitate discussions on rare diseases, concentrating knowledge and allowing for the collection of real world data which can be used to learn more about rare conditions and available therapies. Drug repurposing is a hot topic and an area where rare disease groups are now often leading the way, directing the development pathway for the benefit of their patient group.

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

Dr. Madeline Pe, EORTC, Belgium

Dr. Anja Schiel, Norwegian Medicines Agency