

THEME 4

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

THEME LEADERS:

Diego Ardigo, Global Rare Diseases R&D Head, Chiesi Farmaceutici, Italy & Chair, Therapies Scientific Committee of IRDiRC

Virginie Hivert, Therapeutic Development Director, EURORDIS and vice-Chair, Therapies Scientific Committee of IRDiRC

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.

PRE-RECORDED SESSION: (Available on demand)

Galaxy Guide for Rare Disease therapies development

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

The Orphan Drug Development Guidebook Task Force was set up within the International Rare Disease Research Consortium (IRDiRC) with the aim to create a guidebook describing the available tools and initiatives specific to rare disease development and how to best use them, in order to address the multiple challenges inherent to drug development for rare diseases for which the traditional model of development is becoming less and less suitable.

The aim of the Guidebook is to benefit the various stakeholders working in the orphan drug development environment. It includes fact-sheets describing each 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 of the development. 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.

SESSION 0401: Thursday 14th May 2020, 14:45 – 16:15

What do patients expect from therapy development?

This session aims to look at the present and future of medicine's 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 medicine's 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 patients' lives.

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

Speakers:

Loris Brunetta, Thalassaemia International Federation, Italy

Alain Cornet, Lupus Europe, Belgium

Veronica Popa, MCT8-AHDS Foundation, Greece

Eva Stumpe, SMA Europe, Germany

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

SESSION 0402: Thursday 14th May 2020, 16:45 – 18:15

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

Dr. Nigel Hughes, Janssen Research and Development, Belgium

Prof. Armando Magrelli, Istituto Superiore di Sanità, Italy

Elizabeth Vroom, World Duchenne Organization, Netherlands

Pooja Merchant, Bayer, USA

Prof. Veronica Miller, University of California, USA

SESSION 0403: Friday 15th May 2020, 11:30 – 13:00

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: **Dr. 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

Patrick Célis, European Medicines Agency, The Netherlands

SESSION 0404: Friday 15th May 2020, 14:00 – 15:30

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:

Dr. Anja Schiel, Norwegian Medicines Agency

Prof. Faisal Ahmed, Endo-ERN EuRRECa, UK

Dr. Madeline Pe, EORTC, Belgium

Dr. Nick Sireau, AKU Society, UK