



# THIRD MULTI-STAKEHOLDER SYMPOSIUM

## Introduction to Breakout Session 1

13<sup>th</sup> February 2019, Brussels  
Victoria Hedley, Newcastle University

**EURODIS.ORG**



# A new blueprint to spend efficiently and fast-track R&D

- **Room: Clarity & Vision, 8<sup>th</sup> Floor**
- “Access issues have roots long before time when a product nears market entry”
- “We need to seek and then implement ways to cut costs and fast-track R&D much further upstream”
- *Rare disease R&D in Europe can be improved to overcome fragmentation, **leading to more effective use of data and resources**, faster scientific progress to decrease unnecessary hardship and improve the lives of people living with a rare disease.*

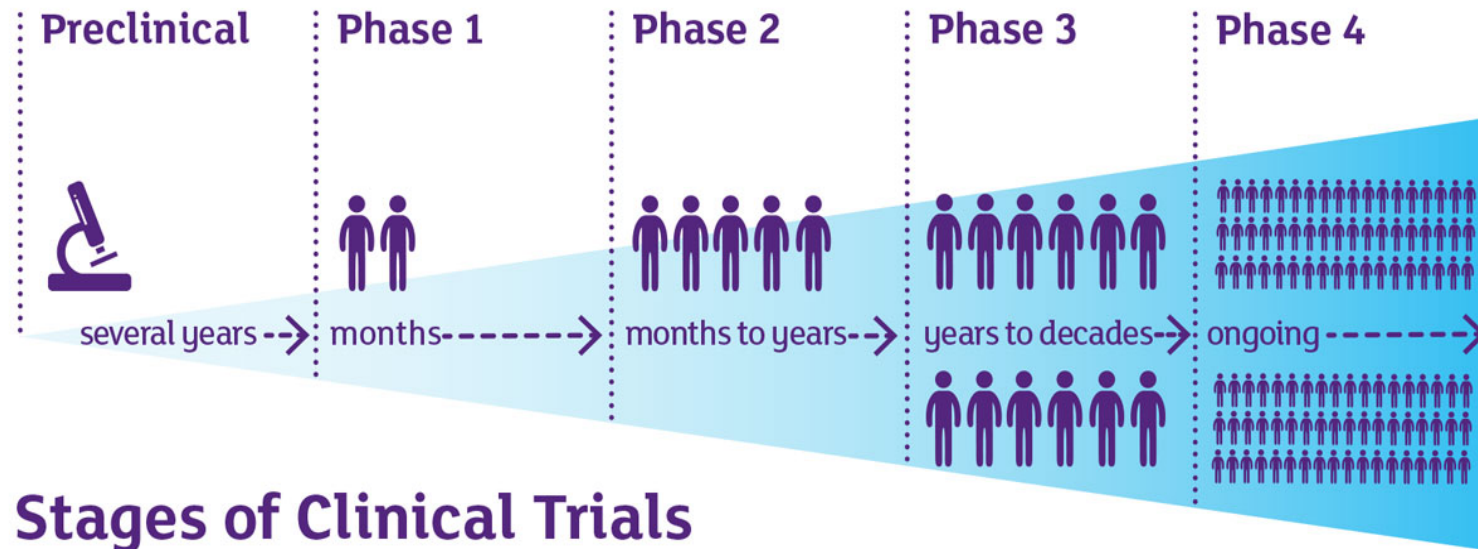


# The good news

- We have a better idea than ever before of some of the ways to cut costs and fast-track R&D in rare diseases
- We have hands-on knowledge from specific disease fields: successes and not so successful
- We have many years of funded projects, initiatives, infrastructures exploring different 'pieces of the puzzle'
- **"It is timely to maximize the potential of already funded tools, networks, projects and programmes by supporting them further, scaling them up, linking them together, and most importantly, adapting them to the needs of end-users through implementation tests in real settings.**

# Many of the possible 'solutions' concern data

- Often see the potential for better collection and use of data (of varying sorts) to streamline therapy development pipeline



Several actors now addressing the fragmentation and lack of interoperability of open science



European Reference Networks



# Speakers and format of the Breakout Session

- This session will discuss how a concerted effort is necessary to develop a sustainable ecosystem, allowing a virtuous circle between RD care, research and medical innovation.
- **Moderator:** Virginie Bros-Facer, Scientific Director, EURORDIS-Rare Diseases Europe
- **Rapporteur:** Victoria Hedley, Rare Disease Policy Manager, Newcastle University; John Walton Muscular Dystrophy Research Centre



<b>Andreas Jung</b>	Senior Physician Pulmonology, Children's Hospital Zurich	Share experience of a pan-European registry on Cystic Fibrosis
<b>Victoria Hedley</b>	Rare Disease Policy Manager, UNEW; John Walton Muscular Dystrophy Research Centre	Will share latest insights on the added value of ERNs for research – how they can foster clinical research: current opportunities and barriers
<b>Mark Turner/Virginie Hivert</b>	Professor/Consultant in Neonatology, University of Liverpool, Co-Coordinator, c4c	Introduction to new IMI project, conect4children, c4c

*Discussion – 20mins + audience participation*

<b>Hanneke van der Lee</b>	Clinical Epidemiologist, Academic Medical Center (AMC)	How to speed up clinical trials: greater acceptance of new models and methods
<b>Ralf-Dieter Hilgers/Diego Ardigò</b>	Institute Director, University Hospital Aachen R&D Rare Diseases Unit Head, Chiesi Group	What can the EJP do to help implement our virtuous circle

*Discussion – 25' + audience participation*

# Can we structure our debates to reach some practical solutions? For example:

- How well do we understand what sort of data can support each stage of therapy development? How much can we get out of our different sorts of registries?
  - Is the CF registry platform experience what we should each be aiming for? How can we act strategically in setting-up new registries or linking existing ones?
  - We often hear that ERNs can advance RD patient registration significantly – how can we get here?
- What do people feel is the single most important thing ERNs can do/that can be done for ERNs, to allow them to fulfil their research ambitions?
- How do we move to faster use of the methodologies for clinical trials in small populations?
- 2018 and 2019 are the years when c4c and the EJP (respectively) began – how do we make these work for their target populations (and how do we bring all the necessary stakeholders in to the discussions?)