



European **Rare Diseases**
Research Alliance

Open Academy School on **Medicines Research and Development 2025**

Webinar 1: Introduction to the School and
to the medicines development lifecycle



Co-funded by
the European Union

ERDERA has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement N°101156595.
Views and opinions expressed are those of the author(s) only and do not necessarily reflect those of the European Union or any other granting authority, who cannot be held responsible for them.

Open Academy School on Medicines Research and Development 2025



Meet the team



Virginie Hivert

Therapeutic Development
Director



Judit Baijet

Patient Engagement and
Training Manager
(REMEDI4ALL project)



Claudia Fuchs

Drug Repurposing Projects
Senior Manager



Marta Campabadal

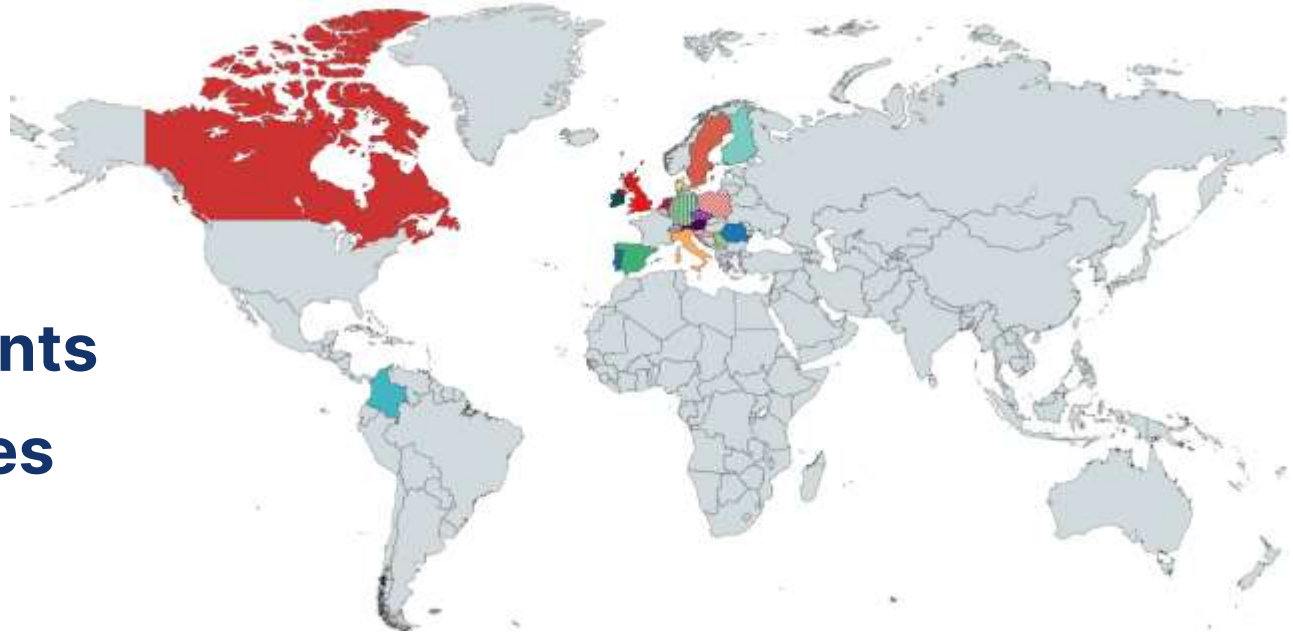
Open Academy Senior
Manager



Gemma Rodríguez

Open Academy Training
Manager

40 participants
22 countries



Epidermolysis_bullosa
 Neurofibromatosis CHMP1_gene_defect Mitochondrial_Diseases
 Haploinsufficiency_MEF2C Batters_Disease
 Scleroderma Ichthyosis Immunoematology
 Myasthenia_Gravis Acquired_hemophilia_A Ehlers_Danlos_Syndrome
 AIM-Myasthenia_Gravis_and_Immunogenenerative_Diseases
 APS Elevated_Lipoprotein
 GRI_gene_disorders Acquired_angioedema MCTD
 All_forms_of_urinary_and_faecal_incontinence
 Chronic_Mieloid_Leukemia Neurometabolic_disorders
 Neuromuscular_diseases Chronic_pelvic_pain Osteogenesis_Imperfecta
 Collagen_VI-related_dystrophies_(COL6-RD)
 Facioscapulohumeral_muscular_dystrophy
 Homozygous_Familial_Hypercholesterolaemia

40 participants
 27 Rare Diseases

Meet the participants

1

**What is your
name?**

2

**Where are you
from?**

3

**What is your
organisation? What
disease are you
representing?**

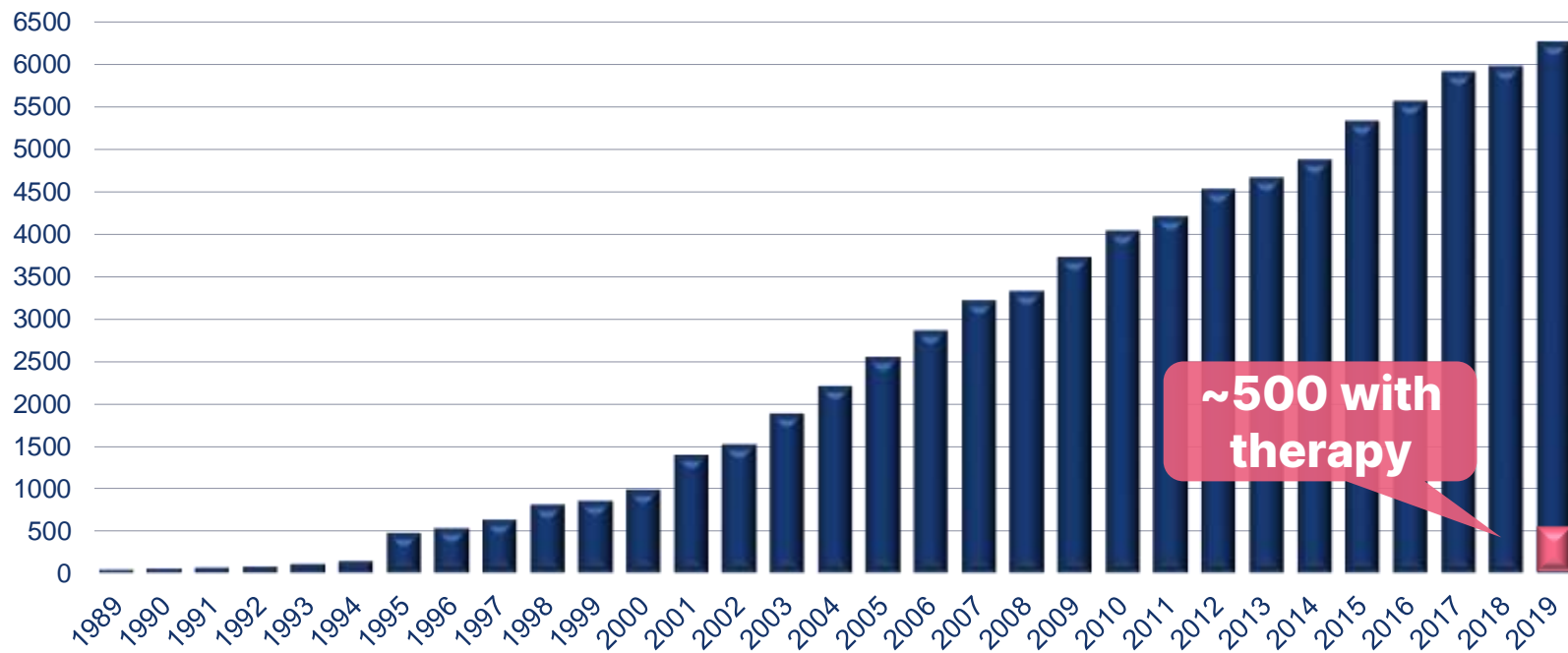
Why are we organising this school?

By 2030, EURORDIS will have made contributions to the goals of
(Based on the Foresight Study Rare 2030)

- Earlier, faster and more accurate **diagnosis** – diagnosis within 6 months
- High-quality national and European **healthcare pathways**, including cross-border healthcare – improving survival by 3 years on average over 10 years and reducing by one third the mortality of children under 5 years of age
- Integrated medical and social care with a **holistic life-long approach** and inclusion in society – reducing the social, psychological and economic burden by one third
- **Research and knowledge development** that is innovative and led by the needs of people living with a rare disease
- Optimised **data and health digital technologies** for the benefit of people living with a rare disease and society at large
- **Development and availability, accessibility, and affordability of treatments, particularly transformative or curative therapies** – a goal of 1000 new therapies within 10 years



Disorders with known molecular bases



Source: *Online Mendelian Inheritance in Man*, *Morbid Anatomy of the Human Genome*

How to address this massive unmet need?

New Chemical Entities (but only 10-20 approved per year)

Gene therapies (e.g. viral based)

Gene-targeted therapies (e.g. antisense oligos)

Cell therapies (e.g. CAR-T cells)

Drug repurposing (on-patent/ off-patent)

For all types of medicines



For patients
of all ages



With a focus on Rare Diseases but applicable to other
disease areas

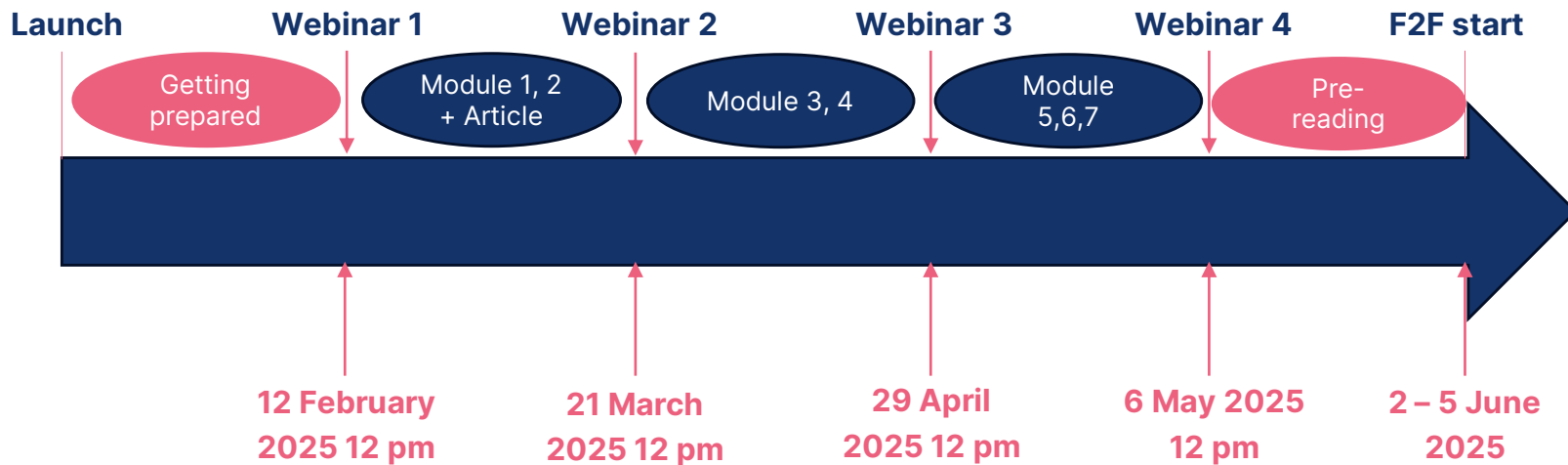
Understanding the Ecosystem & Capacity building

→ **How to be engaged in the process!!**



Pre-training timeline and Q&A

Pre-training timeline



Pre-training: eLearning course



<https://openacademy.eurordis.org/mrd25>



Enrolled

Mandatory pre-training – School on Medicines Research and Development

10h

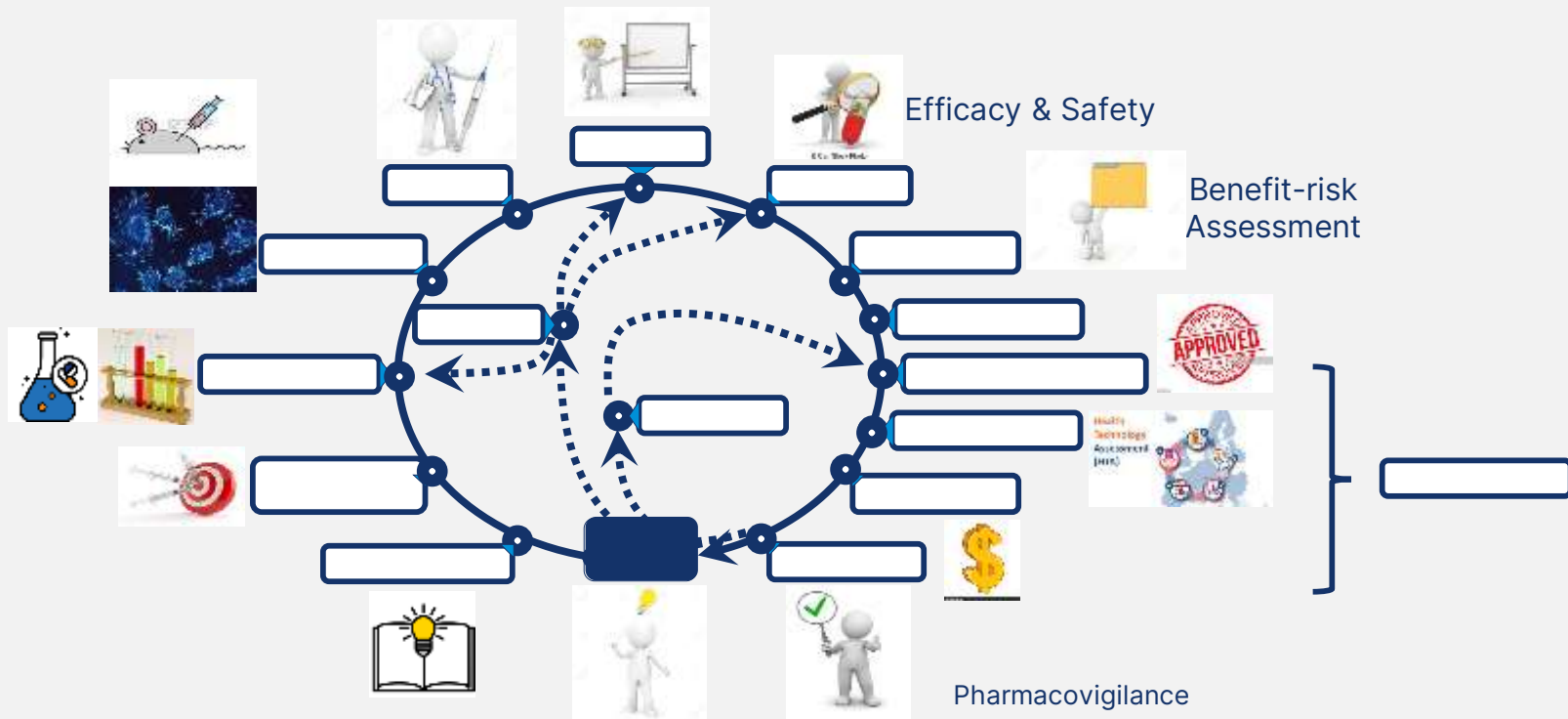
[Continue Study](#)

Information on the Pre-Training & E-learning	
MODULE 1: ETHICS IN CLINICAL TRIALS	
Learning objectives	
Introduction	
Ethics principles in clinical research	
Informed consent	
Ethics Committee	
Case studies on informed consent (optional)	
MODULE 2: MEDICAL RESEARCH AND DEVELOPMENT	
Learning objectives	

A large, stylized logo in the background consisting of a pink 'E', a red 'R', and a pink 'D' in a rounded, modern font. The text "Time for a fun exercise..." is centered over this logo in white.

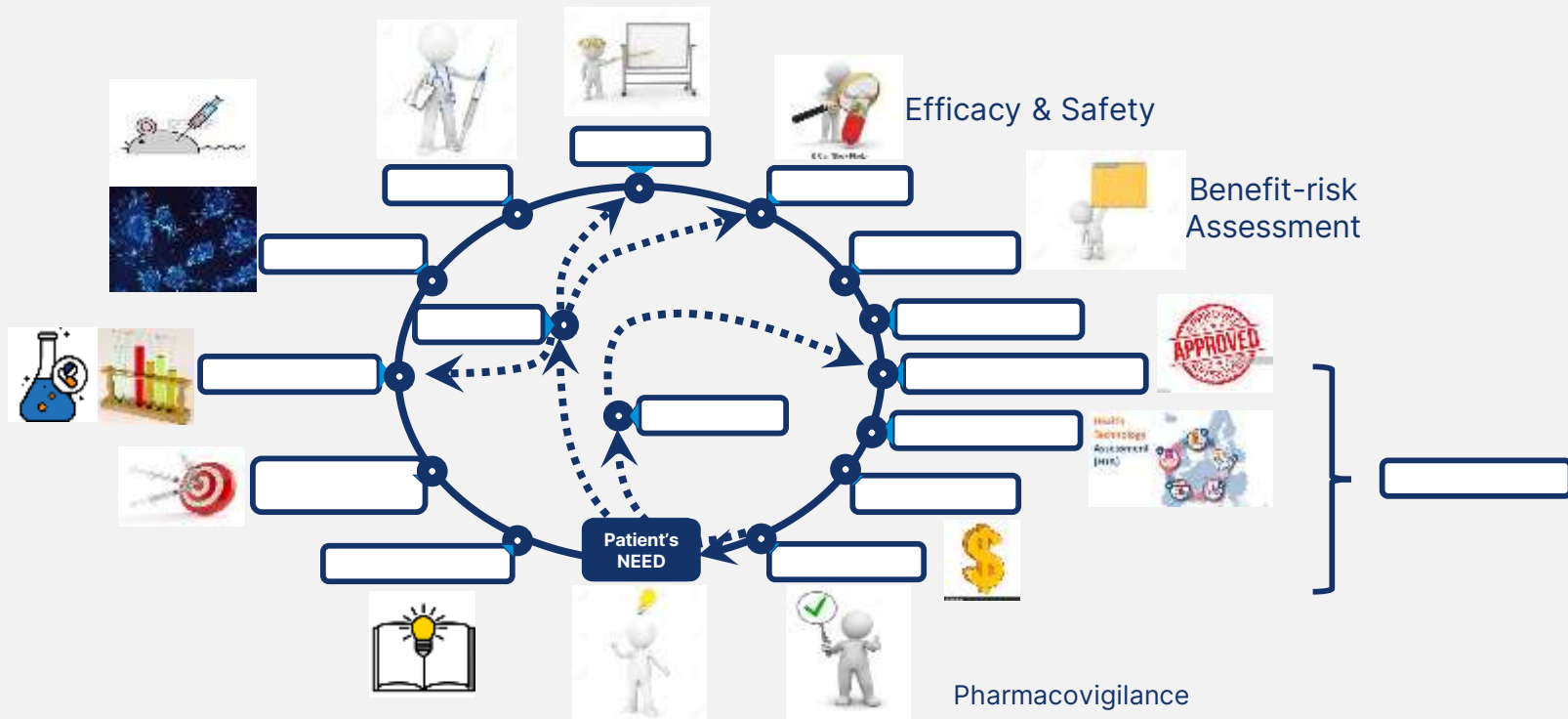
Time for a fun exercise...

Therapeutic development path - general principles



What is missing in the dark blue box?

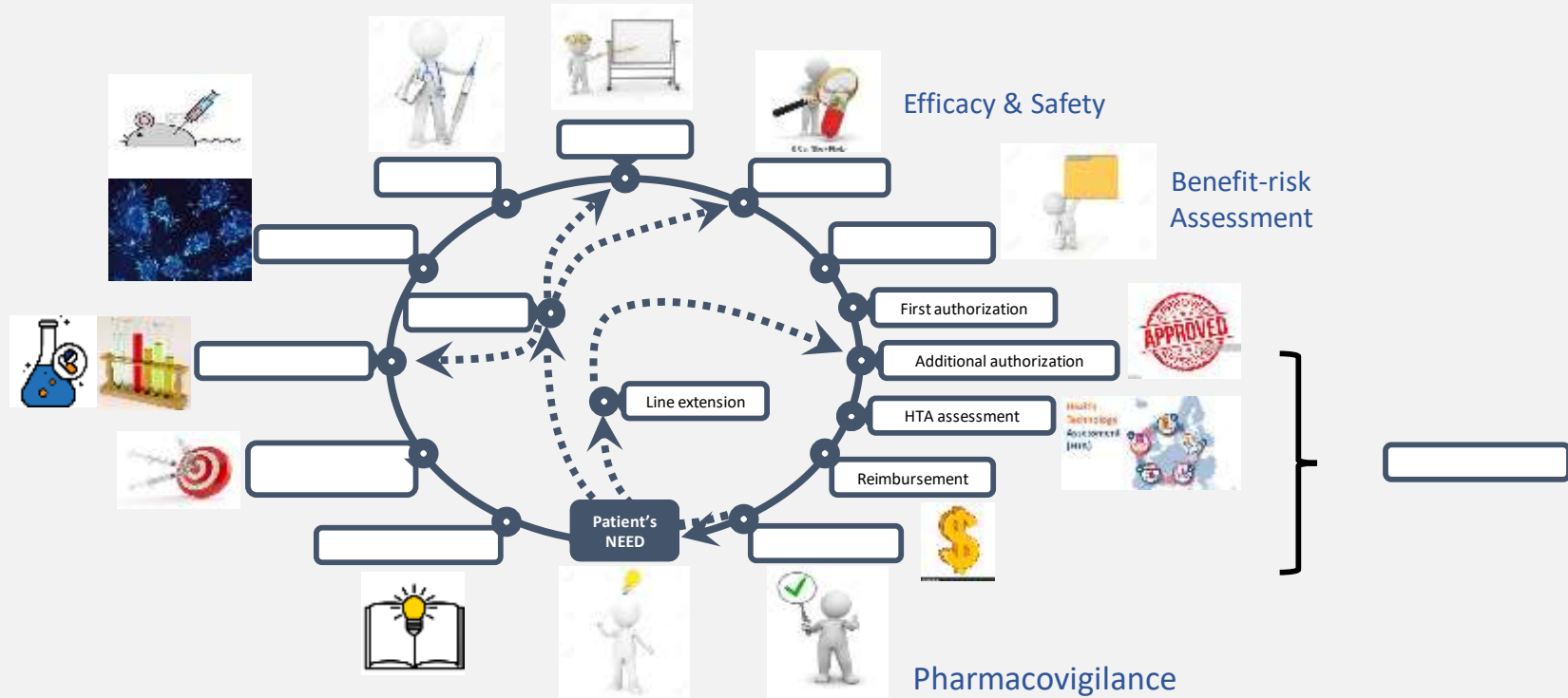
Therapeutic development path - general principles



Each therapeutic development must be triggered by the patient's need!

Now more fun in small groups!

Therapeutic development path - general principles



Place
these:

FIH ready

Pivotal
data

Patient
care

Product discovery

Disease knowledge

MAA
NDA/BLA

Target identification

Market
Access

Nonclinical
PoP

Human
PoC

Repurposing

Link in chat – open now!

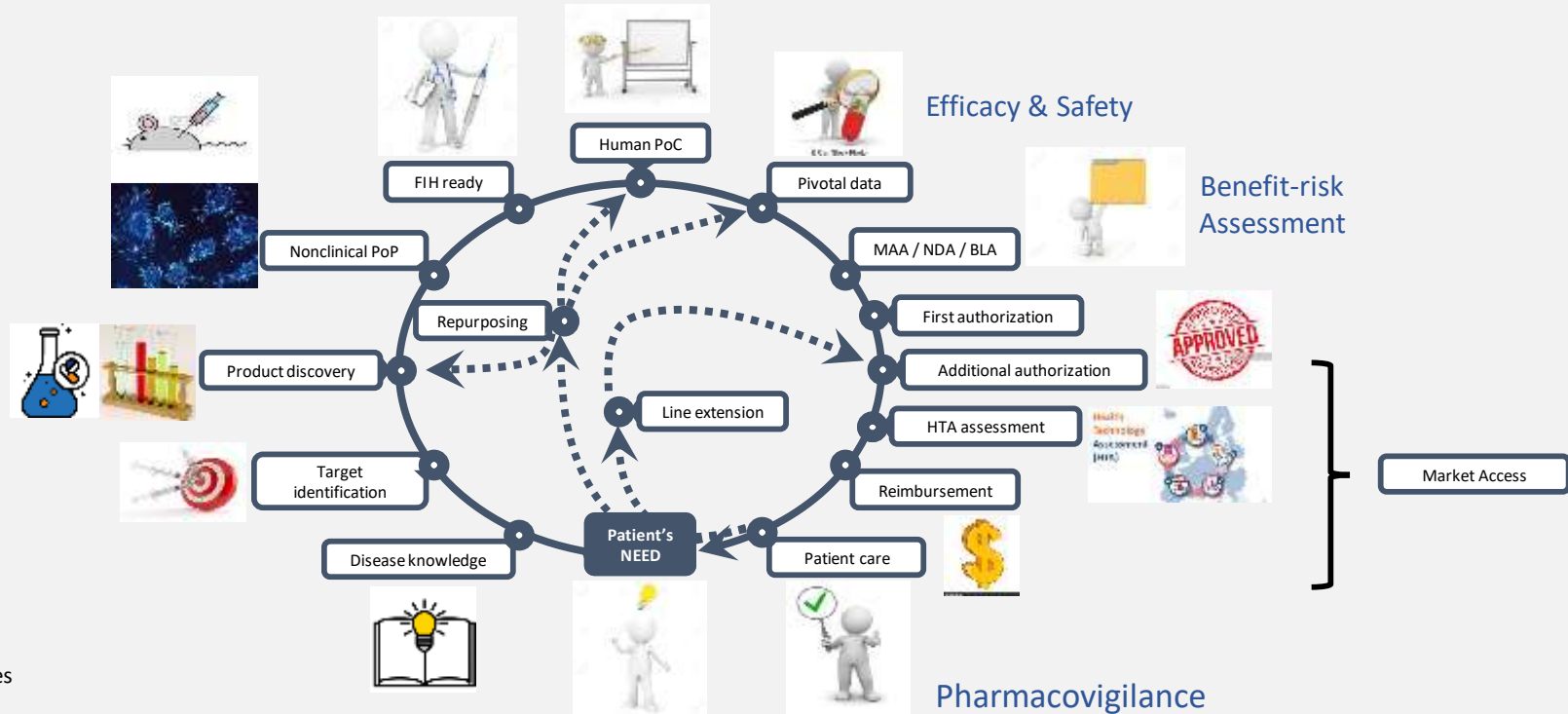
**Breakout room number correspond to
slides**

15 minutes to complete

A large, stylized graphic of the letters 'ERD' in a light pink color, serving as a background for the main text. The letters are thick and rounded, with the 'E' and 'D' having a slight shadow effect.

And the results are...

Therapeutic development path - general principles



PoP: Proof-of-Principles

FIH: First-In-Human

PoC: Proof-of-Concept

MAA: Marketing Authorisation Application

NDA: New Drug Application (USA)

BLA: Biologics License Application (USA)

HTA: Health Technology Assessment

Pre-training timeline





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Research Alliance



Thank you!



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Links to add to chat (updated 29/01/2027)

Slides to annotate:

https://docs.google.com/presentation/d/1JmKV5iqg3l2n52MfSa_SBI0uSIU_eEznG_qpNA6QbQ/edit?usp=sharing

Feedback form : <https://www.surveymonkey.com/r/VDCSFNY>

The link to recording will be on the MRD25 webpage:

<https://openacademy.eurordis.org/mrd25/>