ERDERA
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
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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

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Therapeutic Development Director

Judit Baijet
Patient Engagement and Training Manager (REMEDI4ALL project)

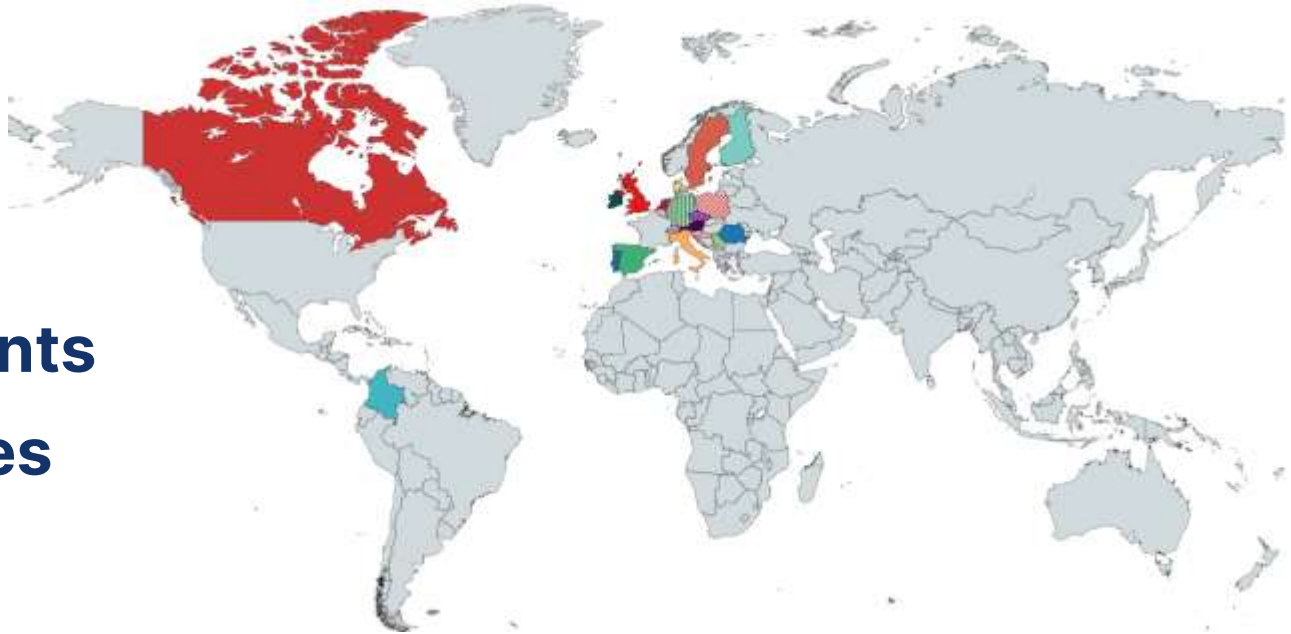
Claudia Fuchs
Drug Repurposing Projects Senior Manager

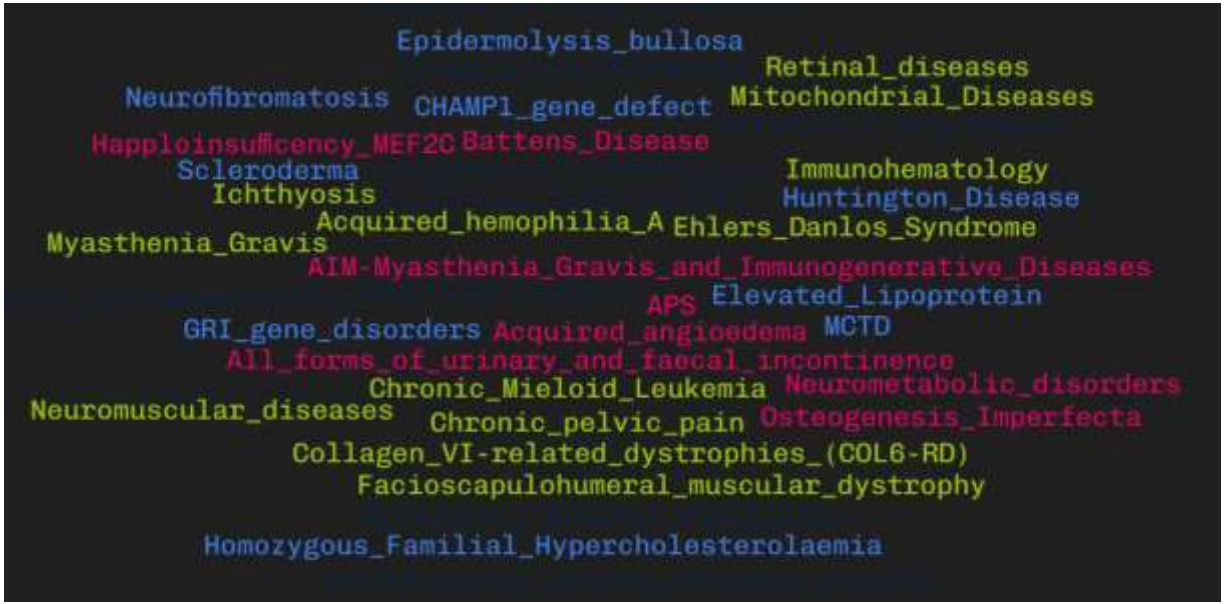
Marta Campabadal
Open Academy Senior Manager

Gemma Rodríguez
Open Academy Training Manager

40 participants

22 countries





40 participants

27 Rare Diseases

Meet the participants

1

What is your name?

2

Where are you from?

3

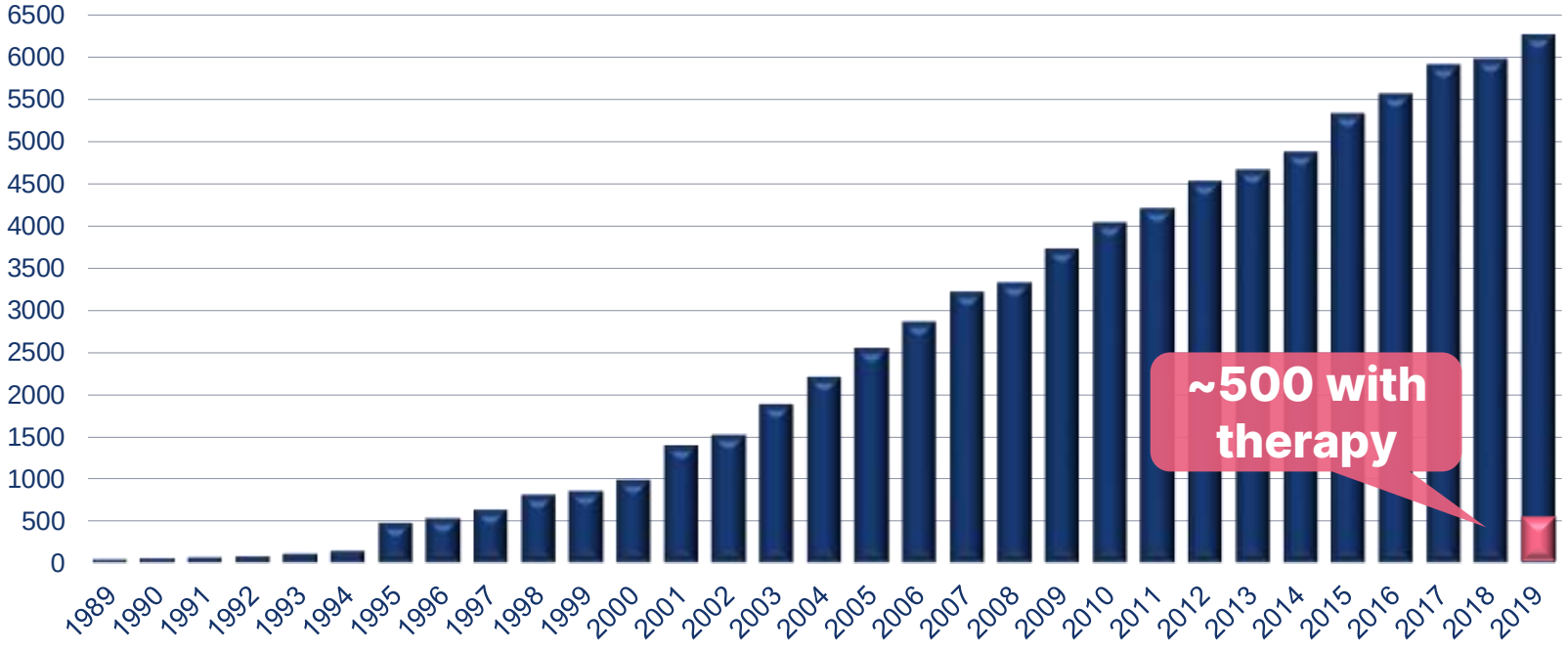
What is your organisation? What disease are you representing?

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

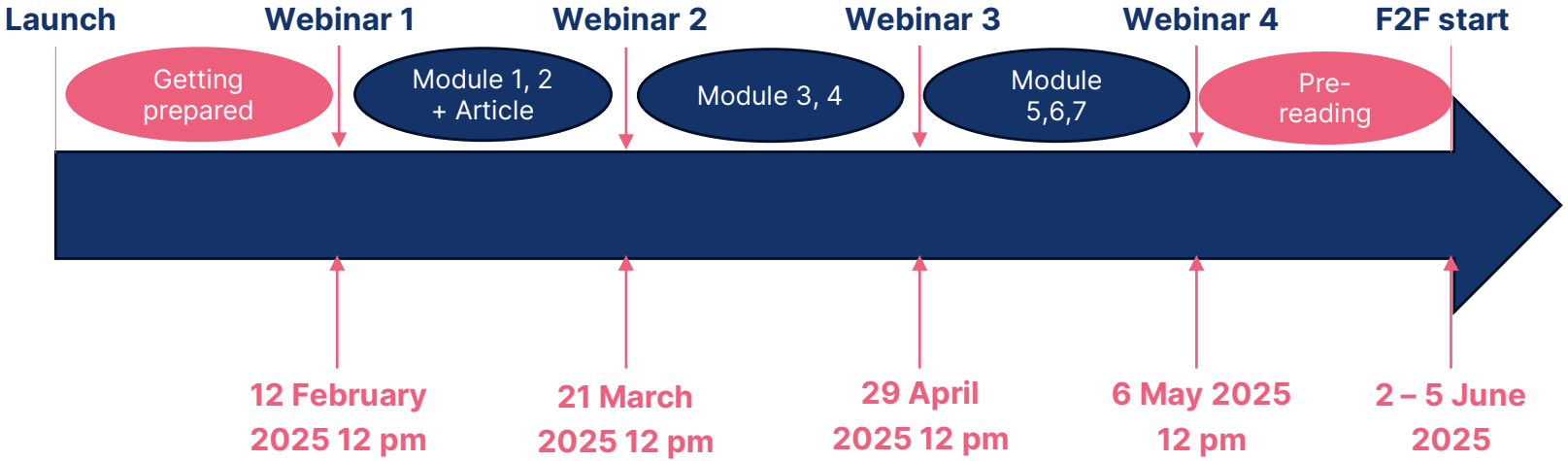
With a focus on Rare Diseases but applicable to other disease areas

Understanding the Ecosystem & Capacity building

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

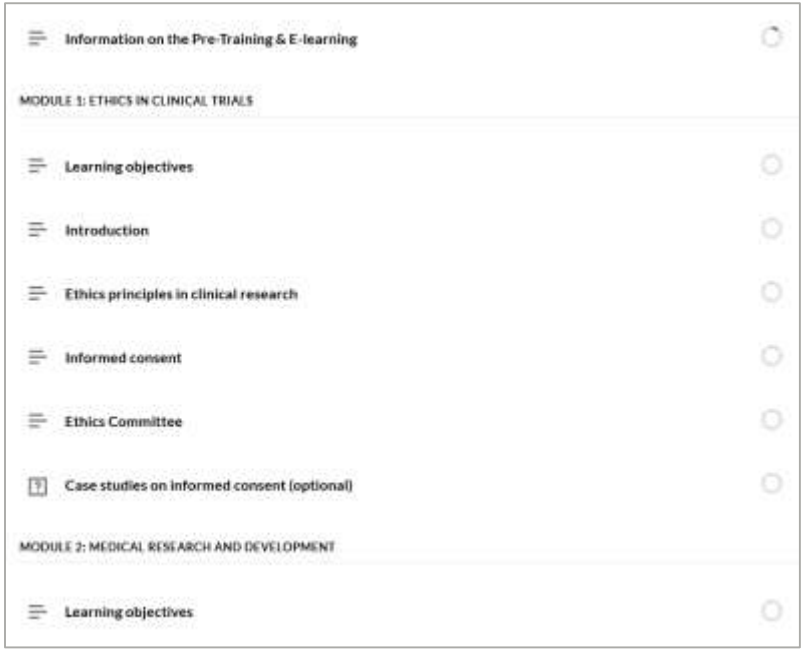
Pre-training timeline and Q&A

Pre-training timeline



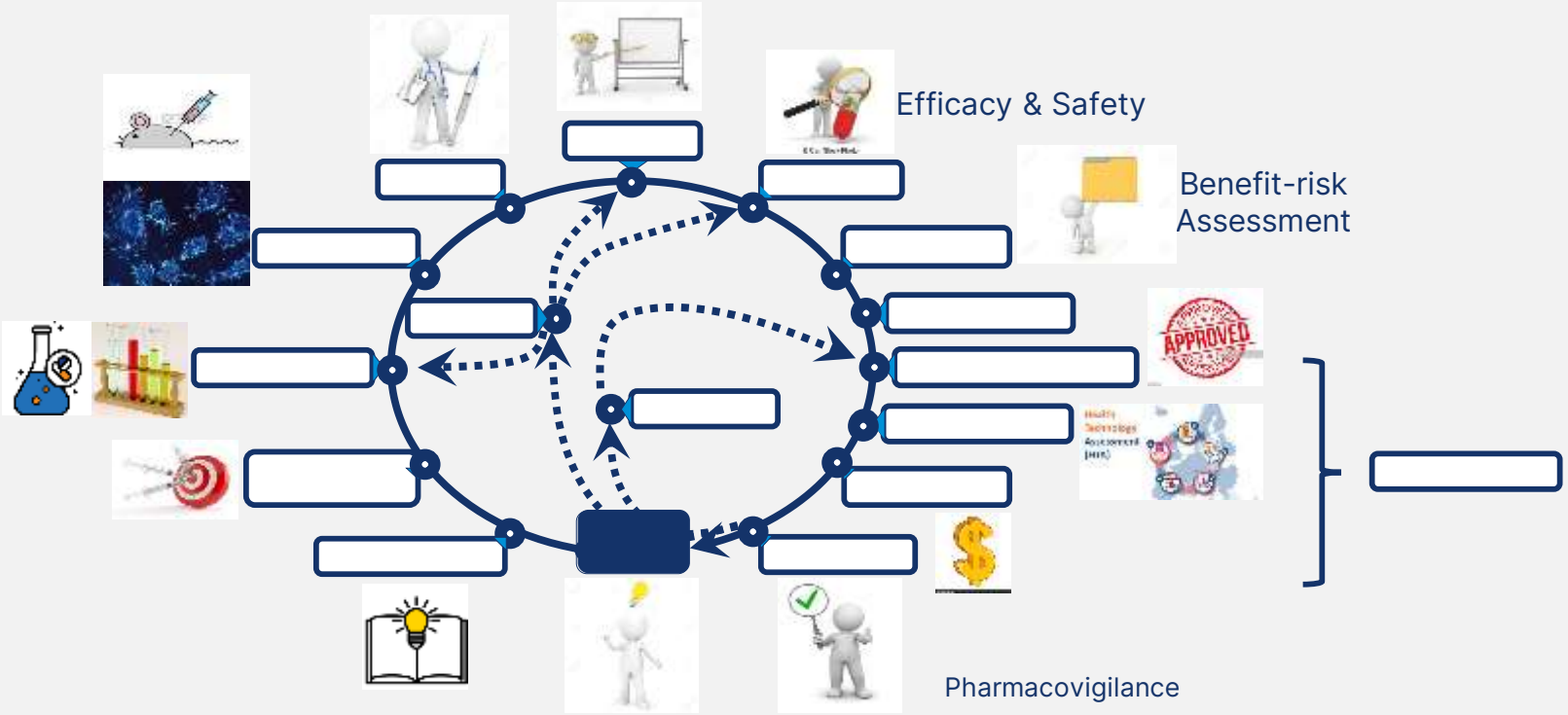
Pre-training: eLearning course

https://openacademy.eurordis.org/mrd25



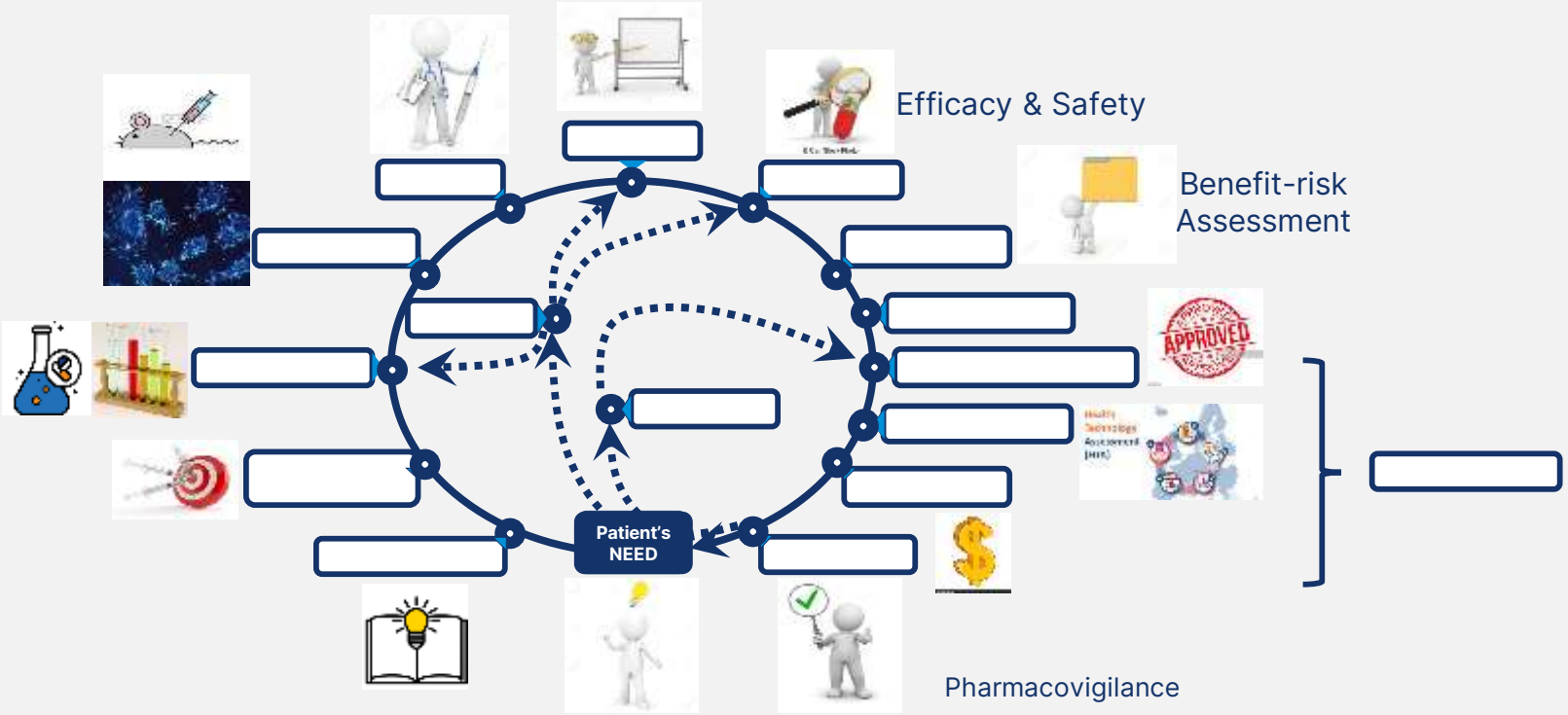
Time for a fun exercise…

Therapeutic development path - general principles



What is missing in the dark blue box?

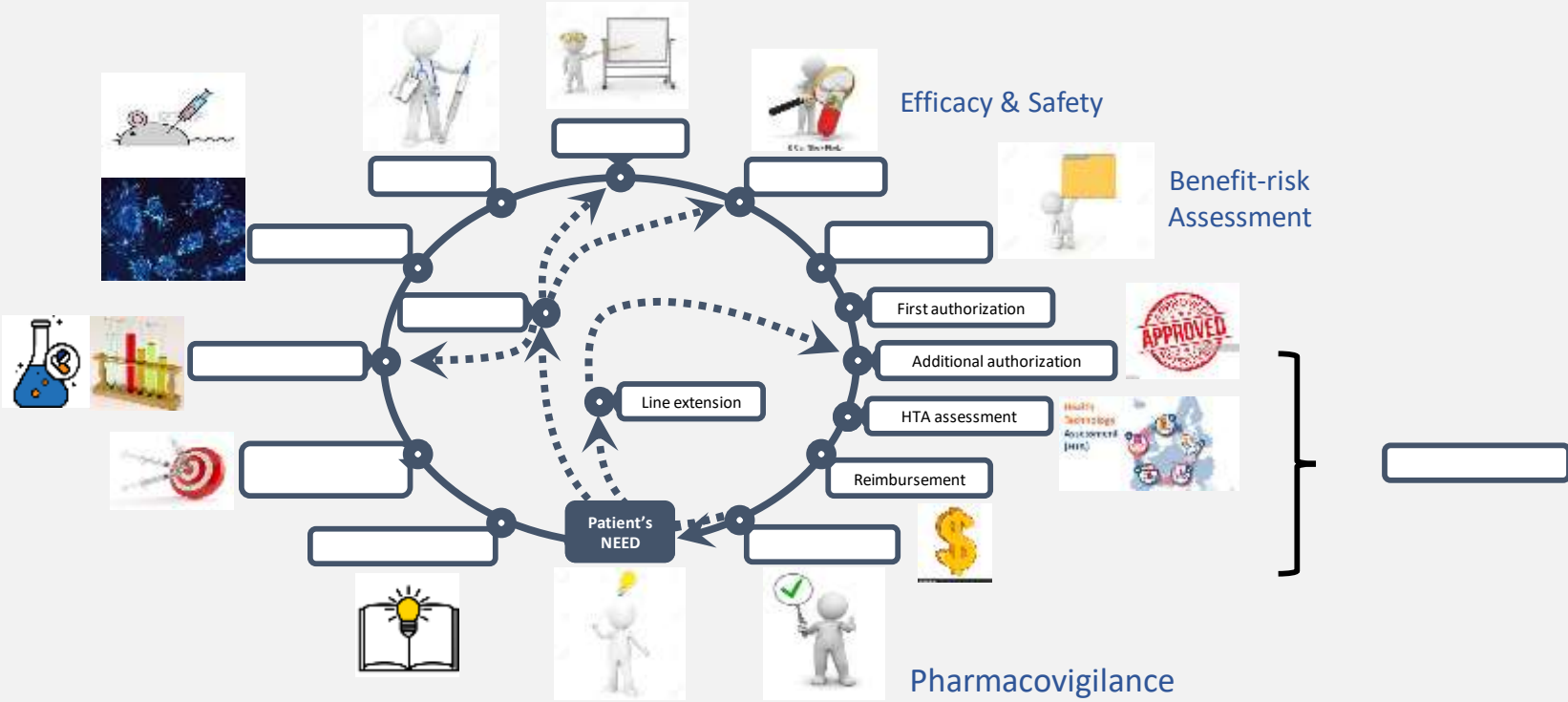
Therapeutic development path - general principles



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

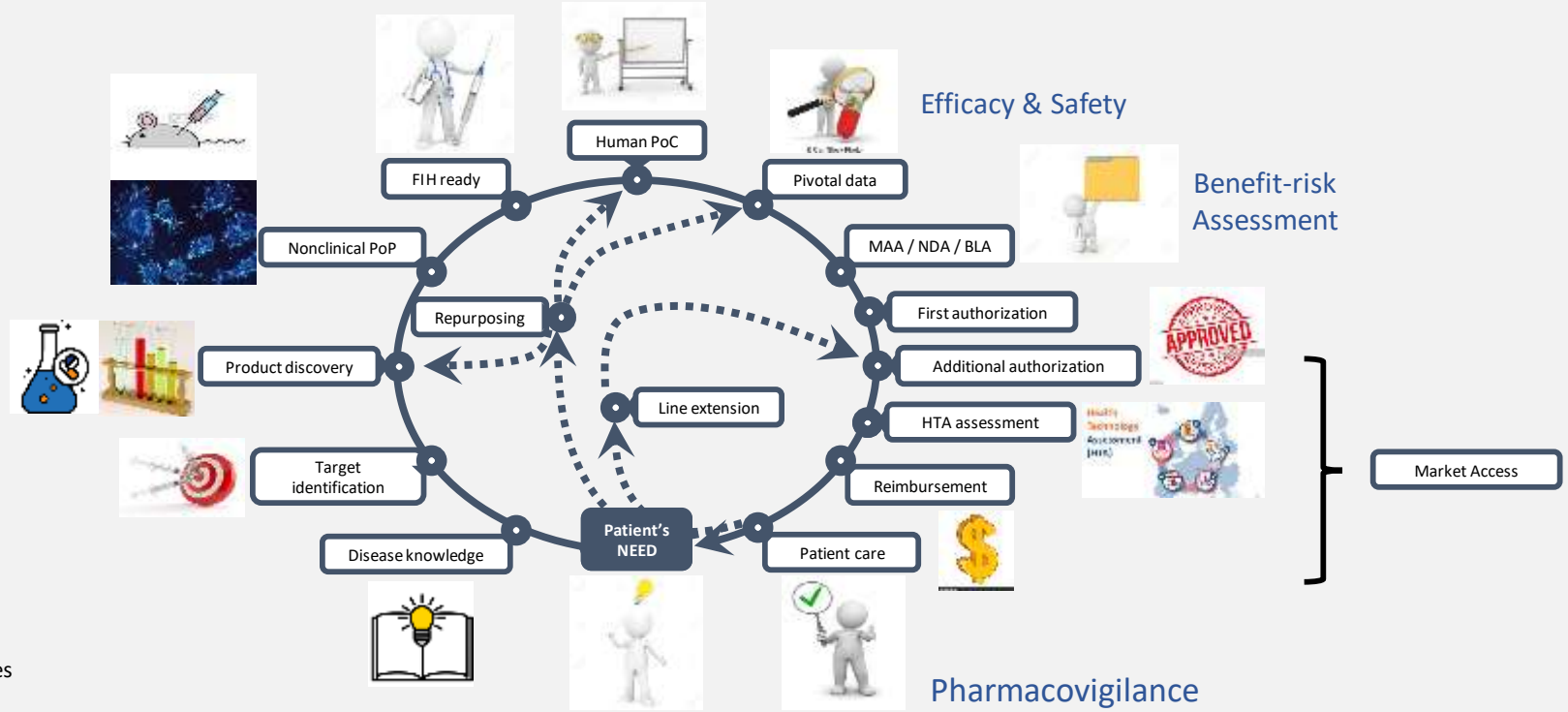
Link in chat – open now!

Breakout room number correspond to slides

15 minutes to complete

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

European Rare Diseases 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/1JmKV5iqg3I2n52MfSa_SBIt0uSlU_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/