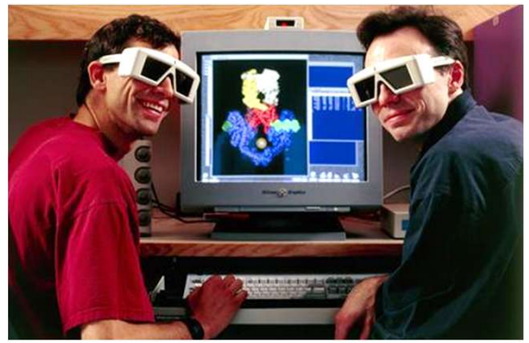
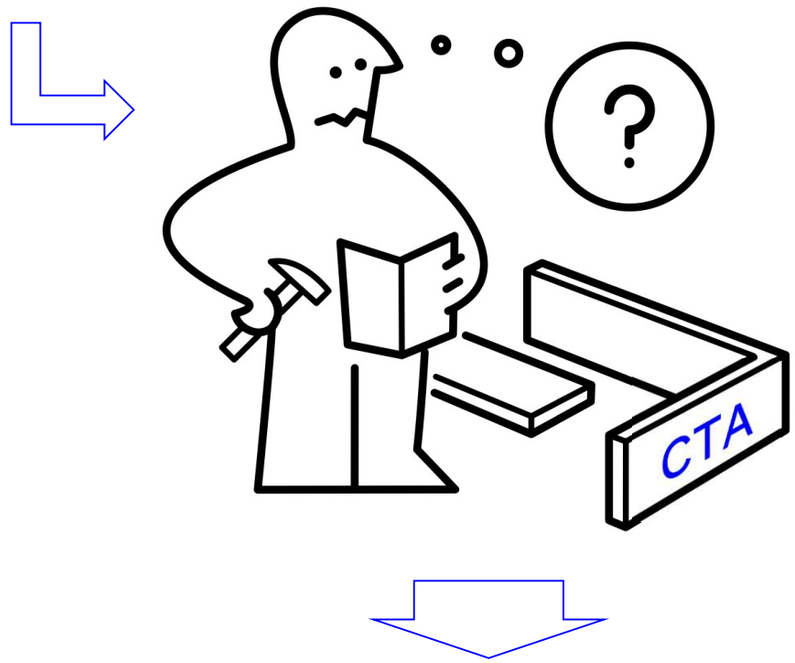
Problem:

No shortage of drug repurposing hypotheses, but ***most projects never reach patients!***

Why?

Most DR hypotheses generated by people/groups who are undertaking drug development ***for the very first time***…





REPURPOSING OF MEDICINES 4ALL

EUROPEAN MEDICINES AGENCY

FDA U.S. FOOD & DRUG ADMINISTRATION

Two "sibling" drug repurposing initiatives launched in 2022

RePo4EU

Prof Dr med Harald HHW Schmidt

To reimagine drug repurposing in the age of AI

REPURPOSING OF MEDICINES 4ALL
The European Platform for Medicines Repurposing

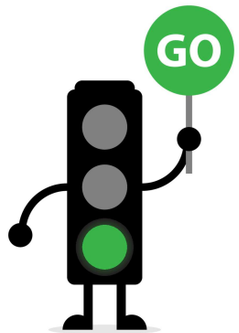
To ensure that good drug repurposing ideas can gain regulatory approval and reach patients

This project has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement No 101057442.

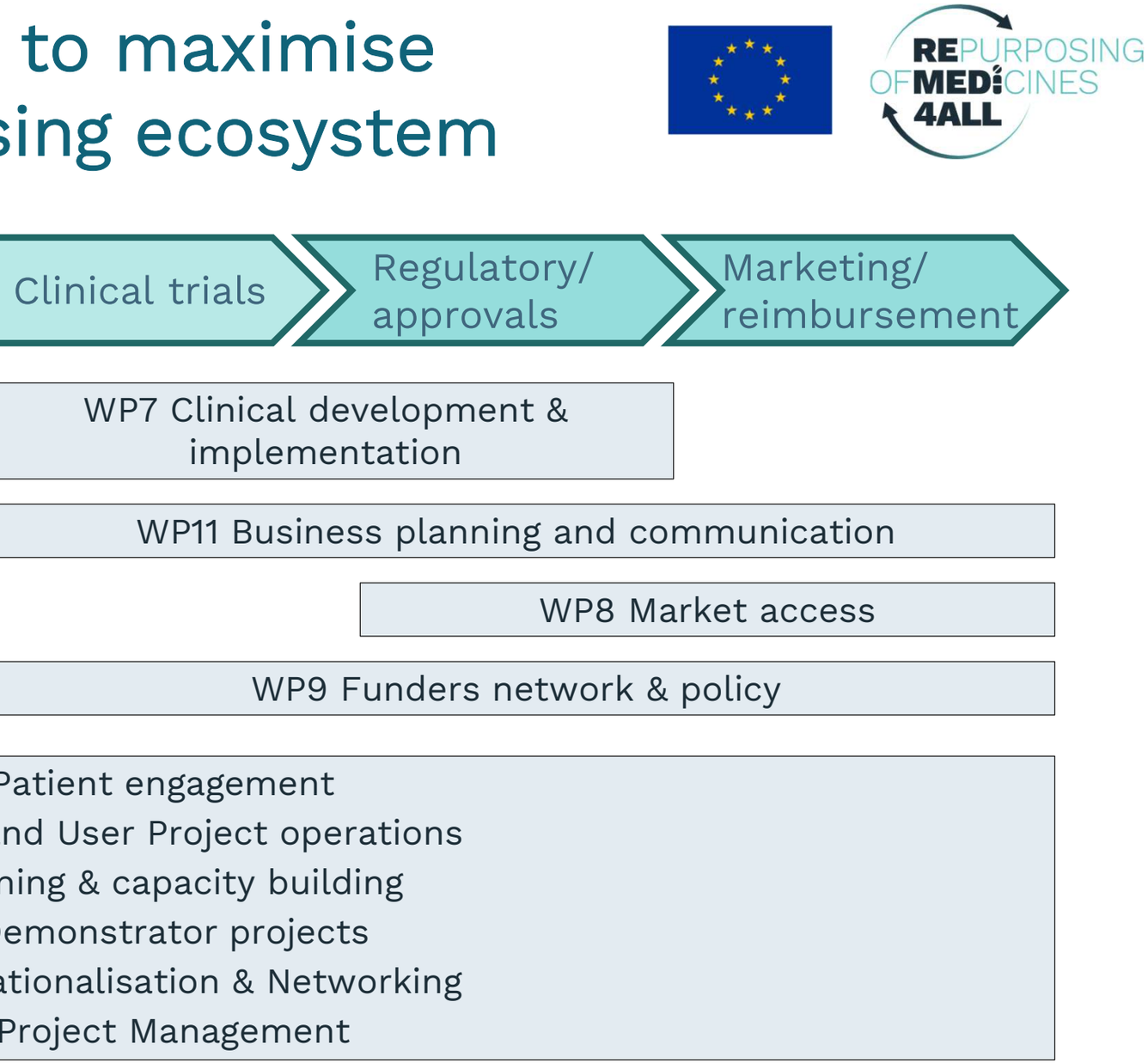
Wouldn't it be nice...

Therapeutic Hypothesis for a new or repurposed drug

Regulatory approval and clinical implementation



Support full value-chain to maximise impact on drug repurposing ecosystem



A big consortium for a big project

This project has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement No 101057442.

Flying the plane while building it…

SciLifeLab

Karolinska Institutet

NIH National Center for Advancing Translational Sciences

DNDi

UNIVERSITÄTSMEDIZIN GÖTTINGEN UMG

Fraunhofer

Demo1: COVID/ Pandemic Preparedness

Completed

Demo3: Oral tazarotene For MSD

abbvie

Filing CTA

The University Of Sheffield.

UNIVERSITY OF HULL

SERVIZIO SANITARIO REGIONALE EMILIA - ROMAGNA
Istituto Ortopedico Rizzoli di Bologna
Istituto di Ricovero e Cura a Carattere Scientifico

Demo4: Losartan for *osteogenesis imperfecta*

>70% enrolled

ISTITUTO NAZIONALE TUMORI IRCCS - Fondazione Pascale

SaludMadrid

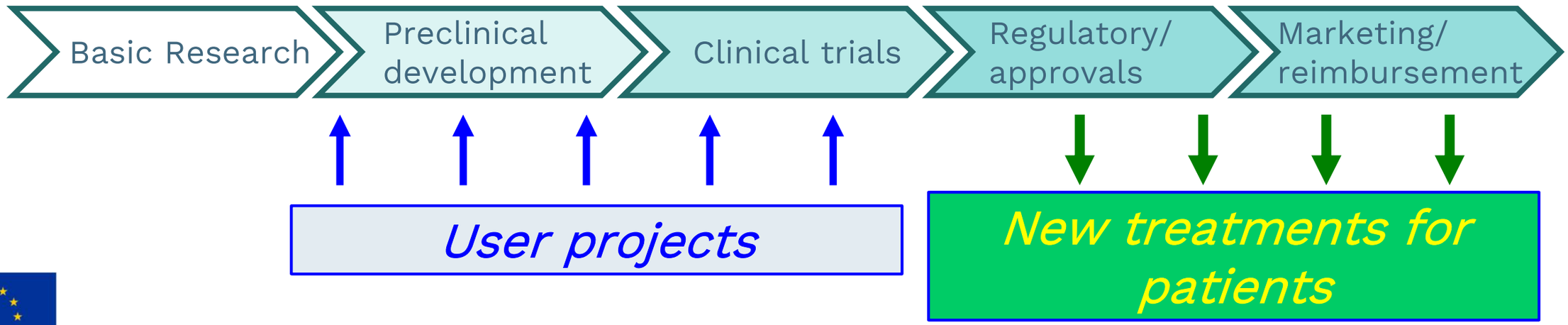
vespa trial

Demo2: Valproate and simvastatin for pancr. cancer

>80% enrolled!

Opening up the shop

Therapeutic Hypothesis for repurposing a drug



REMEDi4ALL CONCIERGE:
UNDERSTANDING COMMUNITY NEEDS

Over **300** requests to date

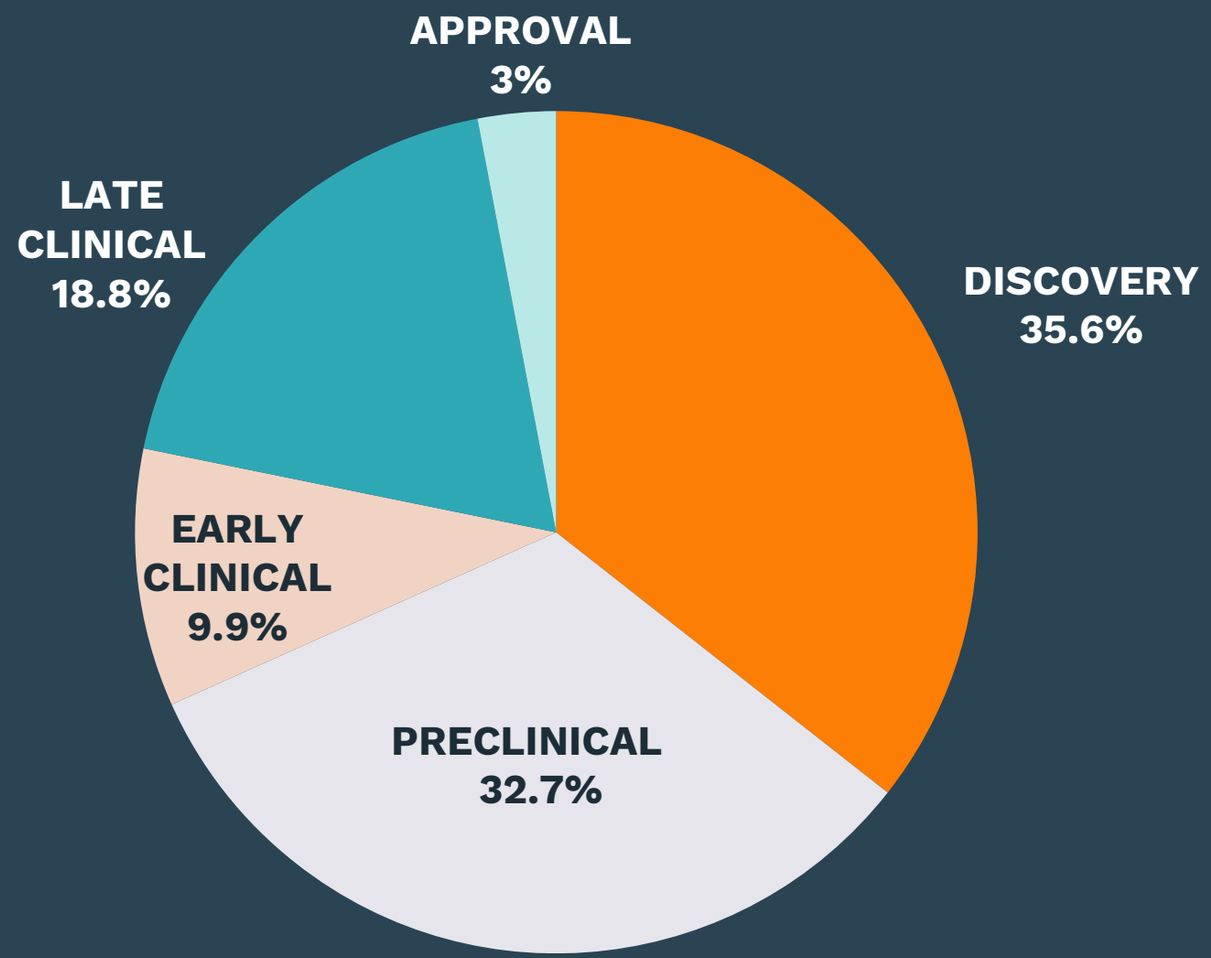
- from diverse stakeholders
- with different needs
- in different phases of development
- from all around the world…

Slide credit: Alicia Soler

How far has the concierge travelled?

REMEDi4ALL CONCIERGE:

DEVELOPMENT STAGE



Typical regulatory stumbling blocks for first-time academic drug developers

Preclinical

- Dose rationale
- Toxicology and safety pharmacology
- Obtaining GMP-grade test article: *CMC, formulation and route*
- Scientific Advice

Clinical

- Proof-of-concept vs. registrational evidence generation
- Registerable/validated endpoints

Partnering with industry (*for new drug approval or drug label extension*)

Managing the Scientific Advice process

Questions in lay terms		**Questions in SA format**
I'm just going to use the MTD (maximal tolerated dose), that's OK, right?	⇨	Does the agency agree with the rationale for starting dose and scheduling for pediatric patients?
What kinds of extra safety studies should I do for my new patient population?	⇨	Does the agency agree that no additional nonclinical safety and toxicology studies beyond those referenced from (*the previous trial*) are required to support the initiation of the proposed clinical study?

The target product profile (TPP) interfaces with the regulator

Indications: Which diseases?

Population: Which patients and where?

Clinical Efficacy: Does it kill the parasite effectively?

Safety and Tolerability: What kind and how many adverse events?

Stability: How long can it be stored in the field?

Route of Administration: How is it given to patients?

Dosing Frequency: How often and how long must it be given?

Cost: Will it be affordable to target population?

Time to Availability: How long will it take to develop?

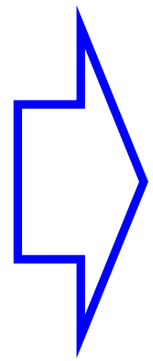
Example for neglected diseases

DNDi

https://dndi.org/research-and-development/target-product-profiles/

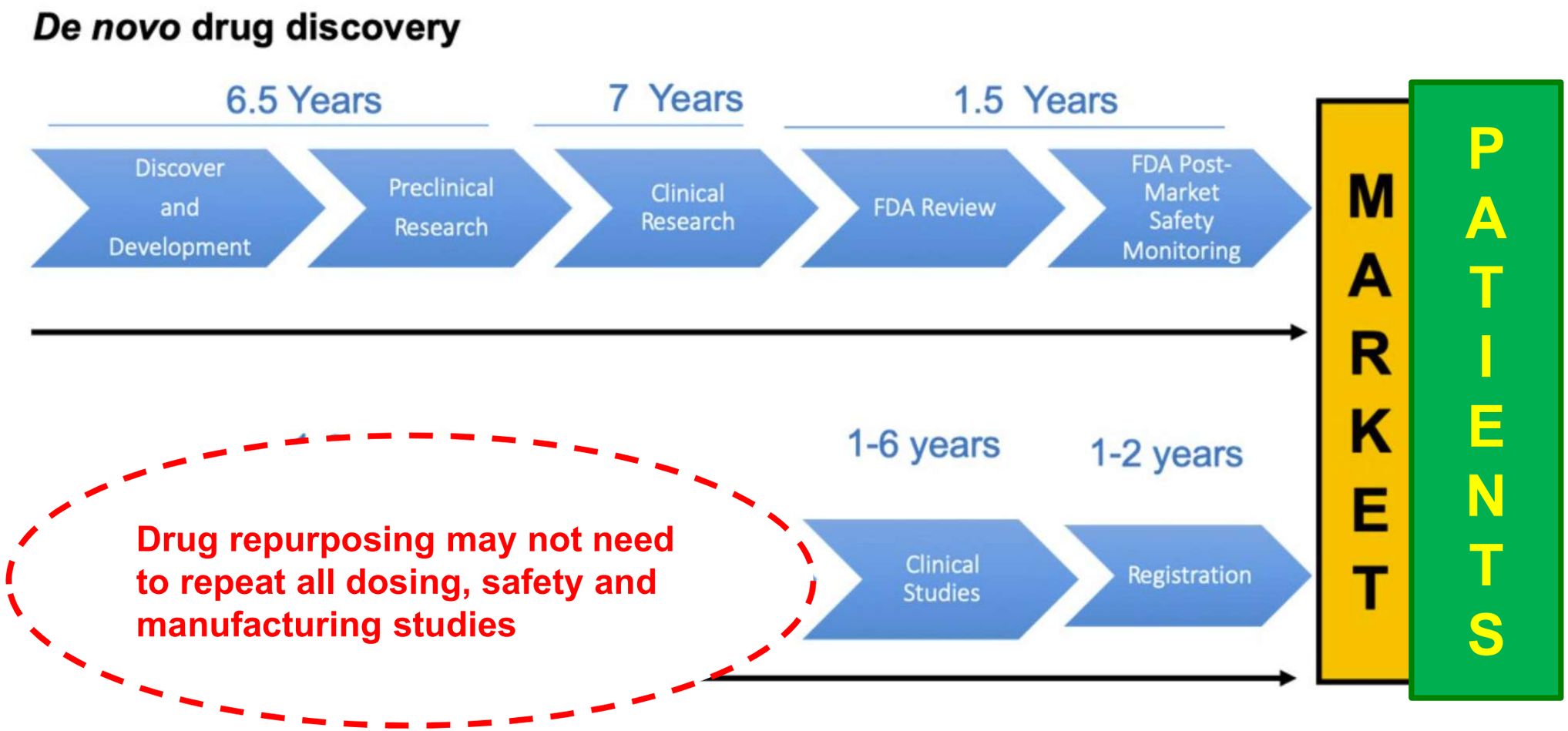
A universal template Repurposing Development Plan (RDP) template for project design

1. Repurposing Hypothesis
2. Patient Engagement
3. Scientific Approach
4. Clinical and regulatory approach
5. Project planning and timelines
6. Risk mitigation plan
7. Intellectual property (IP) assessment
8. Early Health Technology Assessment (eHTA)
9. Market uptake and clinical practice



Construct *Repurposing Development Plans* for each repurposing project

Realizing the promise of drug repurposing: *Faster, cheaper, safer?*



slide credit: Annika Jenmalm Jensen, Karolinska Institutet

INTERNATIONAL DRUG REPURPOSING CONFERENCE

#iDR26

Navigating the future

12-13 May 2026

Maison de la Poste, Brussels

CO-ORGANISERS

REPURPOSING OF MEDICINES 4ALL

beacon for rare diseases

Funded by the European Union

REMEDi4ALL is funded by the European Union's Horizon Europe Research & Innovation programme under grant agreement No 101057442

#iDR26

Brussels, Belgium
12-13 May 2026
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Questions?
Comments?