eatris

European infrastructure
for translational medicine

REPURPOSING OF MEDICINES 4ALL
The European Platform for Medicines Repurposing

# Creating a Sustainable Platform for Patient-Centred Repurposing

## Advancement of Treatments for Rare Diseases

LEFKOSIA CYPRUS 16 - 17 June 2026

Anton Ussi, CEO EATRIS

This project has received funding from the European Union's Horizon Europe research and innovation programme under grant agreement No 101057442.
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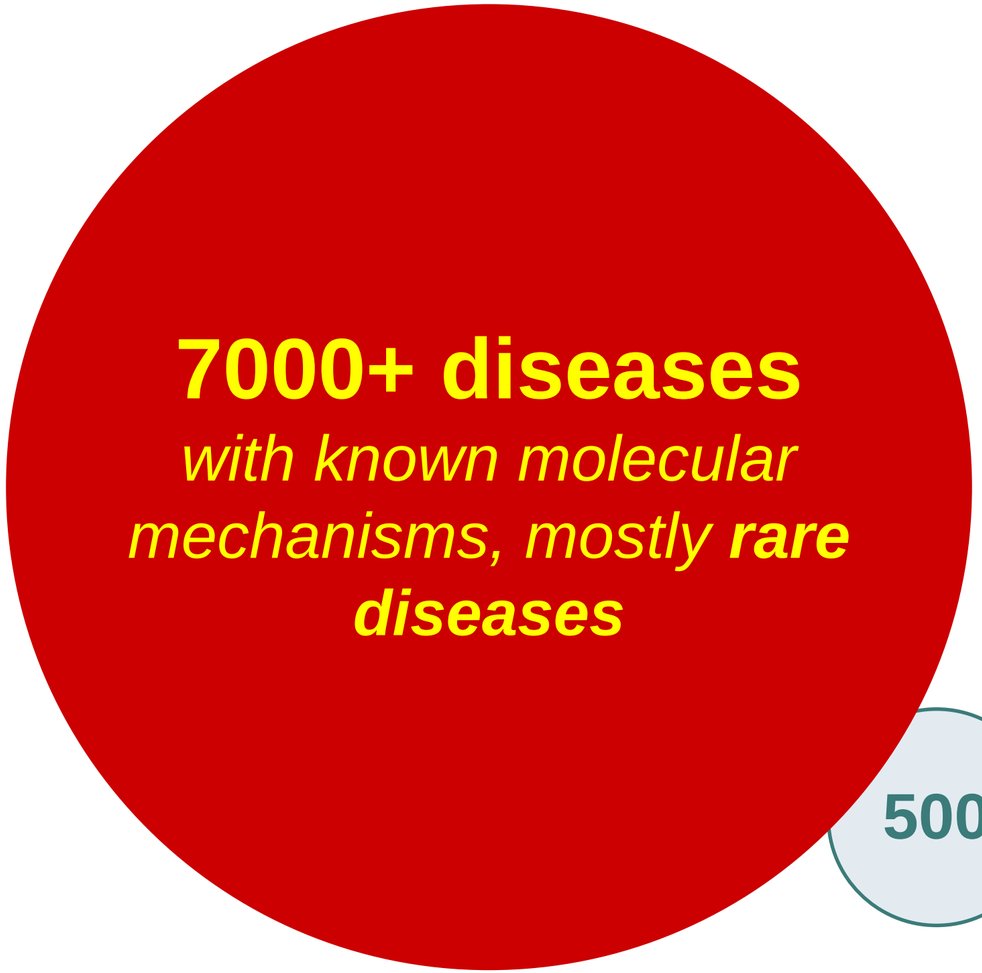
# Can drug repurposing and repositioning help to fill the void?




**The patients are still waiting!**

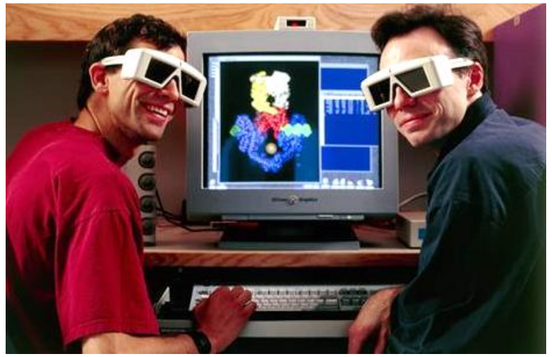
**500** *have approved treatments*

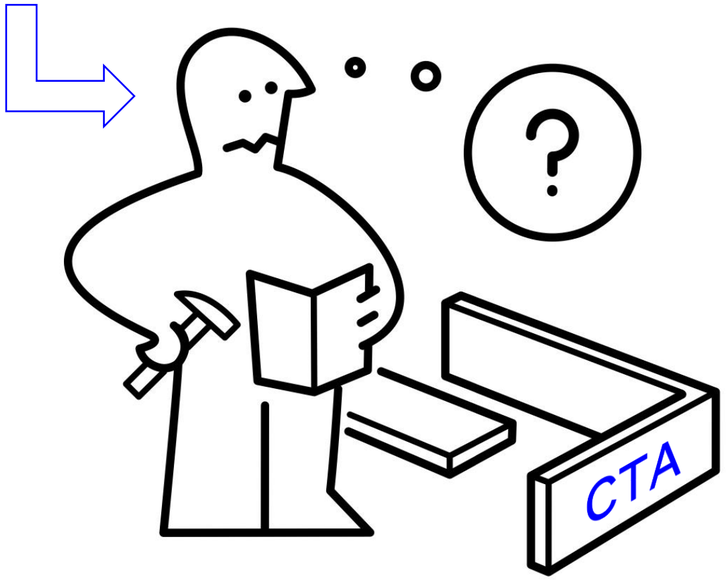
**Problem:**

No shortage of 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***…






How do you know what you don't know?


*Wouldn't it be nice...*

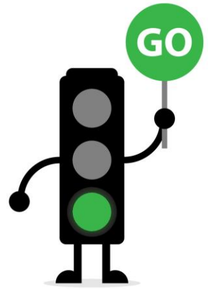
*Therapeutic Hypothesis* for a new or repurposed drug

Regulatory approval and clinical implementation

# The REMEDi4ALL Partnering Platform

*Therapeutic Hypothesis* for repurposing a drug

Regulatory review and approval

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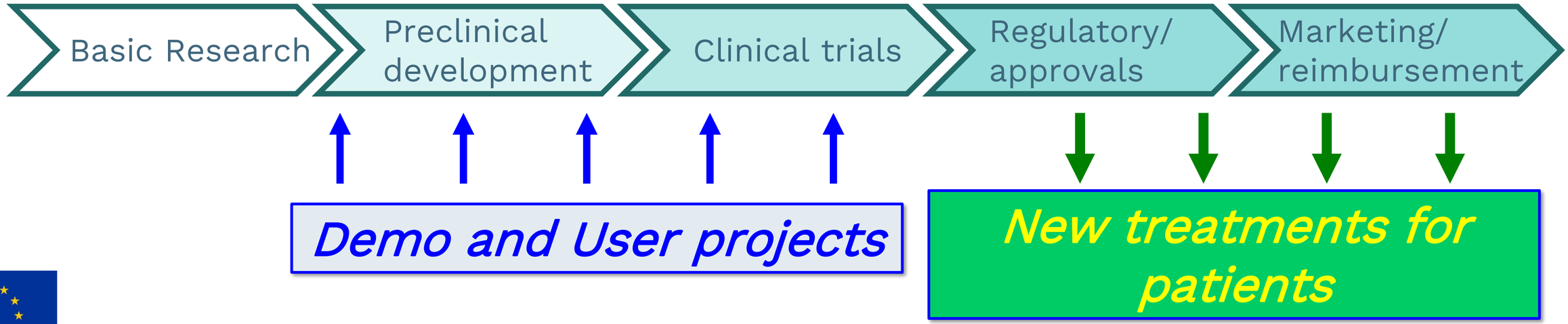
Market entry and clinical practice

# REMEDi4ALL infrastructure is fully up and running

# A big consortium for a big project

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teamit RESEARCH

EURORDIS RARE DISEASES EUROPE

Bundesinstitut für Arzneimittel und Medizinprodukte

Dompé

UNIVERSITY OF HULL

beacon for rare diseases

ZonMw

Chemotargets

FIMM Institute for Molecular Medicine Finland
Nordic EMBL Partnership for Molecular Medicine
HiLIFE UNIT

UPPSALA UNIVERSITET

The University Of Sheffield.

ISTITUTO NAZIONALE TUMORI IRCCS - Fondazione Pascale

UNIVERSITY OF HELSINKI

Fraunhofer

Anticancer Fund

KAROLINSKA INSTITUTET ANNO 1810 Karolinska Institutet

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

ecrin

medicines for europe
better access. better health.

Univerza v Ljubljani

SaludMadrid

ISTITUTO DI RICERCHE FARMACOLOGICHE MARIO NEGRI · IRCCS

syreon Research Institute

UNIVERSITÄTSMEDIZIN GÖTTINGEN : UMG

LifeArc

UCL

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# Support full value-chain – with patients at the heart and goal

Basic Research → Preclinical development → Clinical trials → Regulatory/ approvals → Marketing/ reimbursement

WP4 Research data, tools and *in silico* discovery

WP7 Clinical development & implementation

WP5 *In vitro* discovery

WP11 Business planning and communication

WP8 Market access

WP6 Preclinical development & validation

WP9 Funders network & policy

**WP1 Patient engagement**

WP2 Demo and User Project operations

WP3 Training & capacity building

WP10 Demonstrator projects

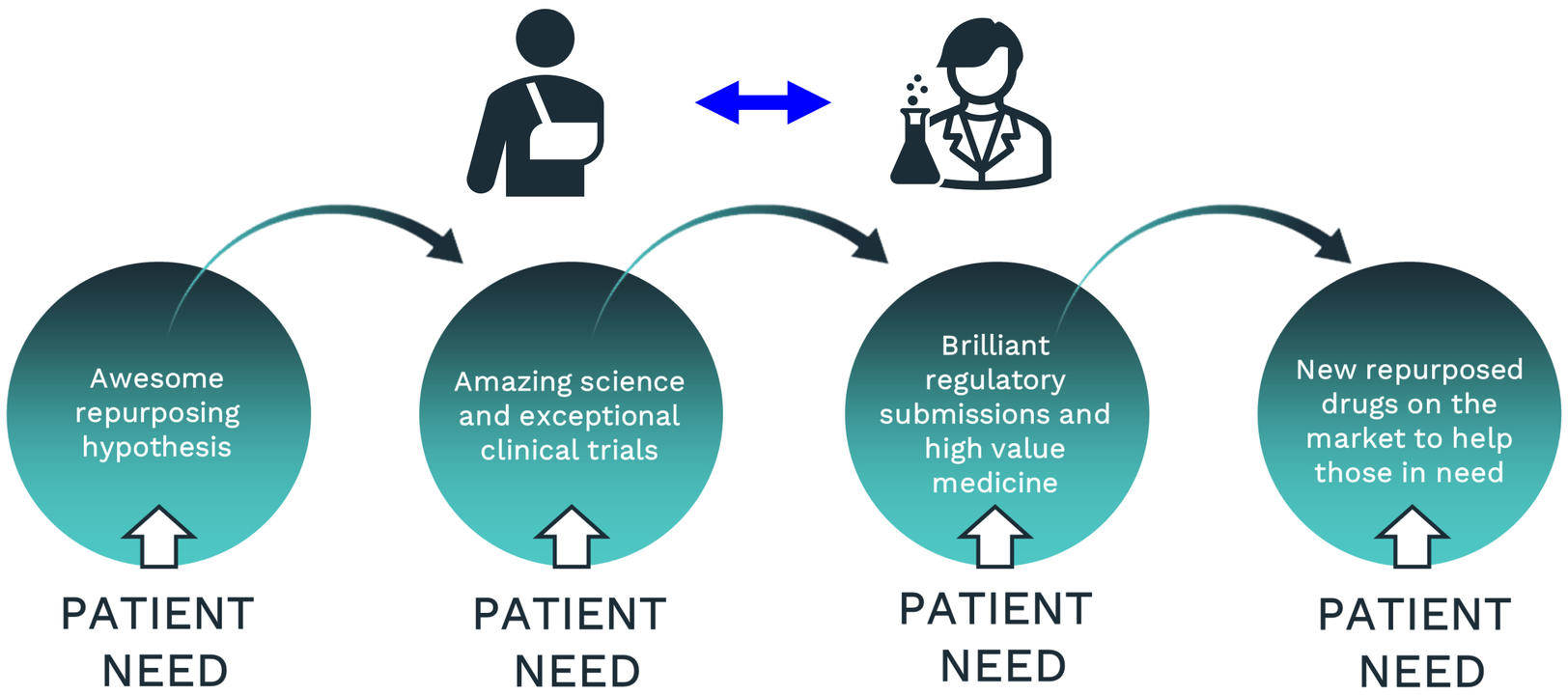
WP12 Internationalisation & Networking

WP13 Project Management

beacon for rare diseases

EURORDIS RARE DISEASES EUROPE

# IMPACT depends on effective patient co-creation




- Patient engagement (PE) across all projects
- **Patient-centric multistakeholder meetings**
- PE in early HTA assessment
- Patient-focused education and training

*slide credit:* Rick Thompson, Beacon for Rare Diseases

# Four embedded "Demo" projects

REPURPOSING OF MEDICINES 4ALL

SciLifeLab

Karolinska Institutet

NIH National Center for Advancing Translational Sciences

DNDi

UNIVERSITÄTSMEDIZIN GÖTTINGEN UMG

Fraunhofer

**Demo1:**
COVID/
Pandemic
Preparedness

**Hit-to-lead stage**

**Demo3:**
Oral tazarotene
For MSD

abbvie

**Preparing CTA filing**

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*

**< half-enrolled**

ISTITUTO NAZIONALE TUMORI
IRCCS - Fondazione Pascale

SaludMadrid

vespa trial

**Demo2:**
Valproate and
simvastatin for
pancr. cancer

***80% enrolled!***

# REMEDi4ALL CONCIERGE:

REPURPOSING OF MEDICINES 4ALL

## > 300 projects mentored

>30%

30%

- Patients
- Patient groups
- Academic Researchers
- Funders
- Charities
- Biotech startups
- Life Sciences Industry
- Service Providers/ RIs

# REMEDi4ALL CONCIERGE:
## UNDERSTANDING COMMUNITY NEEDS

Over **300** requests to date

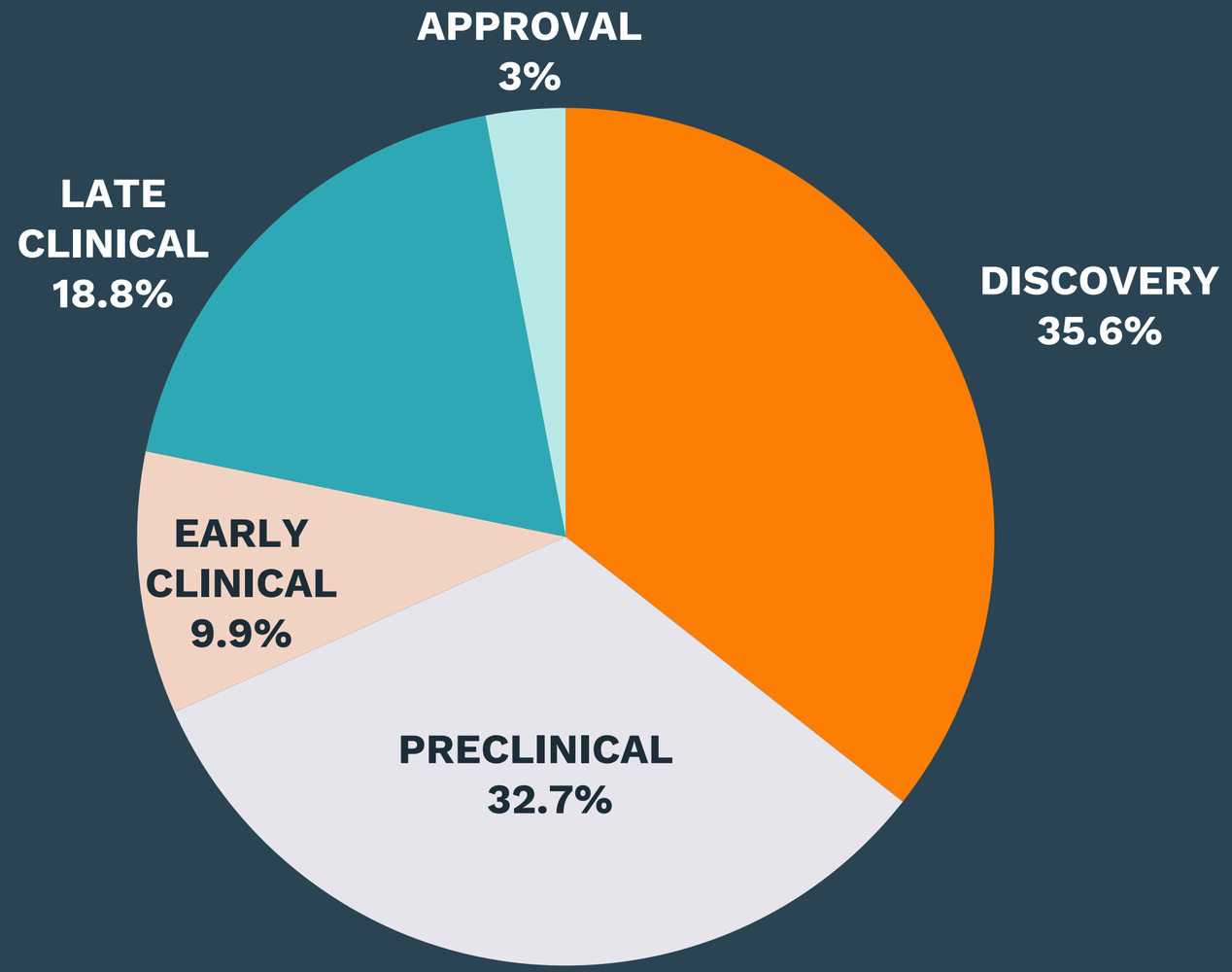
- from diverse stakeholders
- with different needs
- in different phases of development
- from all around the world…


Slide credit: Alicia Soler

# How far has the concierge travelled?

# REMEDi4ALL CONCIERGE:

## DEVELOPMENT STAGE



# Example: the impact of a patient champion


- In a project trying to repurpose a Phase III shelved asset for ultrarare disease MSD, Alan, our **MSD patient champion**, played a key role in connecting us with the decision-makers at Abbvie and a pushing a large data donation.
- Donation allows us to cross-refer to Allergan/Abbvie safety and development data in regulatory submissions, saving development time/costs.

# Starting point for HLH project

Jan-Inge Henter

The Journal of Rheumatology 2021;48:1596–602
doi:10.3899/jrheum.200941
First Release July 15 2021

## Efficacy of Moderately Dosed Etoposide in Macrophage Activation Syndrome–Hemophagocytic Lymphohistiocytosis

AnnaCarin Horne[1], Tatiana von Bahr Greenwood[2], Samuel C.C. Chiang[3], Marie Meeths[1], Caroline Björklund[4], Maria Ekelund[5], Peter Erensjö[6], Stefan Berg[7], Stefan Hagelberg[8], Yenan T. Bryceson[3], Ulf Andersson[8], and Jan-Inge Henter[2]

***ABSTRACT.*** ***Objective.*** Macrophage activation syndrome (MAS) constitutes 1 subtype of the hyperinflammatory syndrome hemophagocytic lymphohistiocytosis (HLH), and the term MAS-HLH was recently proposed for HLH with underlying autoimmune/autoinflammatory conditions. The mortality of MAS-HLH has been estimated at 5–10%. Here we report our experiences with moderately dosed etoposide in severe MAS-HLH; the objective was to effectively reduce severe hyperinflammatory activity with limited side effects.

***Methods.*** In addition to conventional antiinflammatory treatment, moderately dosed etoposide was administered to 7 children affected by rapidly progressing MAS-HLH with central nervous system (n = 5) and/or pulmonary (n = 5) involvement. Three had underlying systemic juvenile idiopathic arthritis (sJIA), 2 had atypical sJIA (no arthritis at diagnosis), and 2 had systemic lupus erythematosus. We performed lymphocyte cytotoxicity analyses in all 7 and genetic analyses in 6.

***Results.*** All children promptly responded to moderately dosed etoposide (50–100 mg/m$^2$ once weekly), added to conventional MAS-HLH treatment that was considered insufficient. The mean accumulated etoposide dose was 671 mg/m$^2$ (range 300–1050 mg/m$^2$) as compared to 1500 mg/m$^2$ recommended in the first 8 weeks of the HLH-94/HLH-2004 protocols. One child developed neutropenic fever and another neutropenic sepsis (neutrophils 0.3 × 10$^9$/L at therapy onset). Five of 7 children had low percentages (< 5%) of circulating natural killer (NK) cells prior to or in association with diagnosis; NK cell activity was pathologically low in 2 of 5 children studied. Disease-causing variants in HLH-associated genes were not found. All children were alive at latest follow-up (2–9 yrs after onset); neurological symptoms had normalized in 4 of 5 affected children.

# ETOPOSIDE FOR HEMOPHAGOCYTIC LYMPHOHISTOCYTOSIS (HLH), LABEL EXTENSION

## HLH

- Rare - Immune disease
- Severe inflammation - life threatening

## Unmet Need

- No approved treatment

## Etoposide

Generic drug:

- Chemotherapy
- Used off label for HLH but multiple patient access/reimbursement issues

## R4ALL support

- Extensive regulatory support to achieve label extension for etoposide in HLH
- Finding, engaging and convincing a generic manufacturer company willing to take responsibility to apply for regulatory approval for etoposide for HLH (become MAH)

## Finding an MAH

- Multiple interactions
- Company analysed data and evaluated the regulatory path to label extension
- **Research agreement established with MAH for label extension and registration!**

# Funders network & future marketplace


Slide credit: Adapted from Don Lo

# REMEDI4ALL **FUNDERS NETWORK**

CONNECTING FUNDERS TO MAXIMISE THE POTENTIAL OF DRUG REPURPOSING RESEARCH

**>35 members**






































# REMEDi4ALL Marketplace


## Project Title

REMEDi4ALL MARKETPLACE

Project essential information

1. Unmet need
2. Therapeutic opportunity
3. Key supporting
4. Team
5. IP/data exclusivity
6. Market opportunity
7. Landscape analysis
8. Critical next steps
9. Path to patient access
10. Current funding needed

### Repurposing glycerol phenylbutyrate (Ravicti) for developmental and epileptic encephalopathies (DEEs)

REMEDi4ALL MARKETPLACE

**Active substance:** glycerol phenylbutyrate (brand name Ravicti)
**Original indication:** urea cycle disorders (≥ 2 years of age)
**Market authorisation status:** branded, on-patent
**New indication:** DEEs including SLC6A1, STXBP1 and SYNGAP1
**Dosage form:** no new formulation needed
**Market:** range of rare DEEs
**Current stage of research:** Phase 2a PoC completed and published
**Previous funding:** grants and support from MAH (Hyperion/Horizon/Amgen)
**Next phase that needs funding:** Phase 2/3 pivotal trial
**Engagement with industry:** ongoing with Amgen (US) and Immedica (EU); drug may go generic in 2026
**Engagement with regulatory agency / Scientific Advice:** planned

**UNMET NEED**

- There are numerous DEEs arising from different genetic mutations
- None has disease-modifying treatments, and seizures are not responsive to standard anticonvulsive therapies
- SLC6A1: ultrarare (100s of known patients)
- STXBP1: (~1000 patients worldwide)
- SYNGAP: (1000s patients worldwide)

**THERAPEUTIC OPPORTUNITY**

- Treatment of seizures associated with DEEs with potential to improve neurodevelopment and slow disease progression

**KEY SUPPORTING DATA**

- Benefit in DEE mouse models
- Positive Phase 2a open-label proof-of-concept (PoC) study in SLC6A1 and STXBP1 patients

**TEAM**

- Academic PIs in UK/EU
- Zach Grinspan MD, Weill Cornell Medicine (US)
- Amber Freed, patient advocate (US)
- Lindsay Randall, patient advocate UK/EU

**IP/DATA EXCLUSIVITY**

- Original Ravicti patents considered weak
- ODD data exclusivity expires 2025/26
- Immedica owns ODD for SYNGAP1 (2023)
- No other ODDs filed for non-urea cycle disorders

**MARKET OPPORTUNITY**

- DEEs are individually rare, but several up to >dozen DEEs could benefit
- Collectively could form viable market

**LANDSCAPE ANALYSIS**

- Ravicit is a prodrug of sodium phenylbutyrate (PBA) which is an inexpensive generic drug
- But PBA dose is >10 g/per day and taste is too noxious for children
- Branded taste-masked PBA formulations are also on the market

**CRITICAL NEXT STEPS**

- Determine whether a generic(s) may enter market in the next 1-2 years
- Organize multinational platform trial for DEEs

**PATH TO PATIENT ACCESS**

- ODD could support conditional approval directly from a Ph2/3 platform trial
- Potential indication of seizure control for DEEs instead of individual approvals

**CURRENT FUNDING NEED TO**

- Develop IP and business plan, including partnering with a branded or generic MAH
- Develop platform trial plan and SA
- Conduct clinical trial

- € xxxK non-clinical work through clinical trial application (CTA)
- € xM for Phase 2/3 pivotal trial

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REPURPOSING OF MEDICINES 4ALL

REMEDi4ALL FOUNDATION

# The REMEDi4ALL Foundation

*Sustaining support for the drug repurposing ecosystem into the future*

REPURPOSING OF MEDICINES 4ALL

The European Platform for Medicines Repurposing

# With thanks to the EATRIS REMEDi4allTeam

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**remedi4all.org** **#R4ALL** **#REMEDi4ALL**

Back up slides






**A global conference that unites the drug repurposing community to advance the field in every critical aspect.**

Sponsors:

LifeArc · Dompé · CZI · ZonMw

# REMEDi4ALL Patient Engagement framework

REMEDi4ALL is positioning **the patient's voice and experience at the heart of every repurposing project** and empowering them as true co-creators.

## CO-CREATING RESEARCH WITH PATIENTS

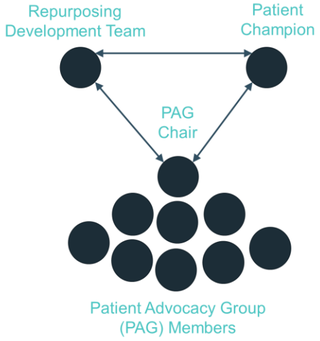
- Establishing Patient Advisory Committees in projects
- Patient Organisations leading patient engagemen t activities in projects
- Involvement of patients for regulatory interactions for clinical trial applications (R4ALL drug repurposing platform)

## Patient Champion

- Offers **unique insight** into a specific disease and the disease community
- **Core stakeholder**
- Represent their community
- Actively participate in our development teams, enabling patient-focused decisions in project


## Patient Advocacy Groups

- Group of **patients, relatives, carers** or **individual experts**
- **Additional source** of patient insight
- Help **access a more representative** selection of patient experience



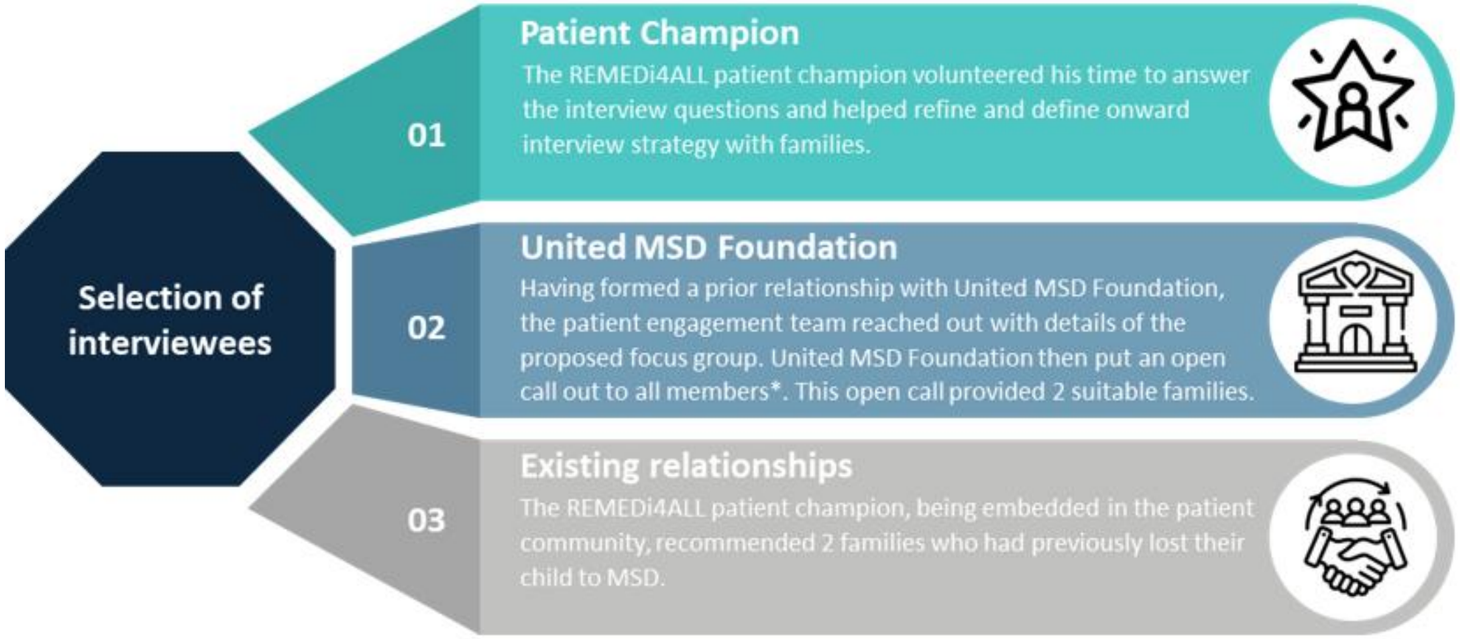

## Multi-stakeholder meetings

- **Sharing information** and advance learning, promoting dialogue and constructive interaction
- **Facilitating collaborations** between all stakeholders
- **Patient centricity** running as the core narrative

# Example: the impact of patient champions + advocacy groups

Support second round of scientific advice, responding to patient-relevant queries with real evidence from patients and the community

Structured focus groups, 1:1 interviews with patient representatives including families currently caring for a child with MSD and ‘angel’ families. All interviewees were independent of REMEDi4ALL




**Due to our ‘open call’ approach we had multiple interested volunteers.*

*The short turnaround time for this piece of work meant at this time, we could not engage families who required an English language interpreter.*

*We endeavour to re-engage with these families later on in the project and capture wider community thoughts and feelings.*

# R4ALL PE framework

R4ALL is educating both patients & researchers.

## SUPPORTING PATIENT EDUCATION

- Providing material to EUPATI Toolbox & co-developing *Patient Expert Training Program* modules
- Offering free-of-charge seats to patient advocates to EATRIS training courses
- R4ALL Academy – developed online learning materials about drug repurposing for patients

## TRAINING RESEARCHERS

- Patient Engagement Resource Centre (PERC)
- Inclusion of PE modules to our flagship training courses
- Support to EATRIS national nodes for organising PE events
- Organisation of webinars & roundtables
- Drug repurposing development teams

## Patient bootcamp

- In-person training for patient representatives to increase knowledge and skills in drug repurposing

## Academic bootcamp

- In-person training for academic researchers to increase knowledge and skill in drug repurposing

## R4ALL Hackathon

- In-person training for early career researchers up to 2-years post PhD
- Learning to choose drug candidates and build drug development plans putting patient needs at the center
- **Patients interviewed by students**

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## Digital Academy

REPURPOSING OF MEDICINES 4ALL ACADEMY

- https://academy.remedi4all.org/
- Digital education covering the key steps of the scientific pathway associated with drug repurposing