



FONDAZIONE  
PER LA RICERCA FARMACOLOGICA  
**GIANNI BENZI**  
ONLUS



UNIVERSITÀ  
DEGLI STUDI DI BARI  
ALDO MORO

**XVI FORESIGHT TRAINING COURSE**  
**Repurposing to cover unmet needs: the  
current scenario in Europe and the proposed  
changes to the Pharmaceutical Legislation**

# **REMEDi4ALL -**

**A new European consortium to drive patient-  
centric drug repurposing in rare disease and  
**beyond****

**Claudia Fuchs**

EURORDIS: Rare Disease Europe

[claudia.fuchs@eurordis.org](mailto:claudia.fuchs@eurordis.org)





# Drug Repurposing, a promising opportunity for rare diseases?

## Repurposing NITISINONE for ALKAPTONURIA (Black Bone disease)



**NITISINONE**  
Initially developed as weed killer.  
It blocks the production of HGA.



Inherited genetic condition; due to mutations in the *HGD* gene >> **accumulation of homogenistic acid (HGA) and its oxidized form alkapton.**

Progressive disorder.



Long-lasting and fruitful collaboration between patient group and academia >> **Nitisinone is the first licensed drug for Alkaptonuria**



# Drug Repurposing, a promising opportunity for rare diseases?

## Repurposing LONAFARNIB for Hutchinson-Gilford PROGERIA



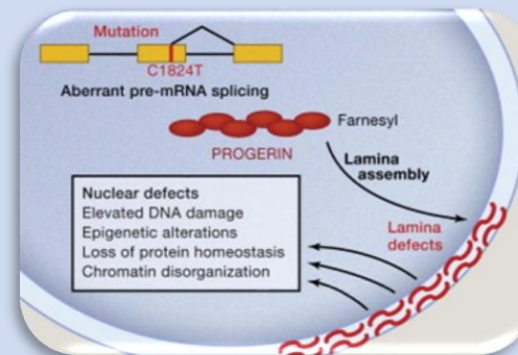
Ultra-rare autosomal dominant genetic condition; due to mutations in the *LMNA* gene >> **altered nuclear protein Lamin A >> cells die prematurely.**

Rapid and drastic aging beginning in early childhood.

### LONAFARNIB

Initially developed to treat cancer.

It acts as farnesyltransferase inhibitor >> reduces the farnesylation of prelamin A (progerin) and inhibits its accumulation



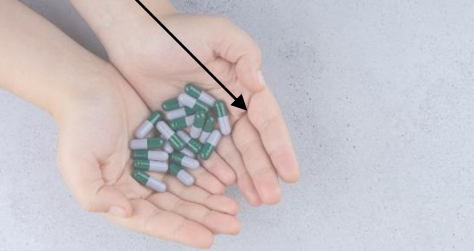
Boston Children's Hospital



Progeria

Research Foundation  
FOR THE CHILDREN ♥ FOR THE CURE

A phase II trial showed an increase in the lifespan of HGPS patients treated with Lonafarnib >> in 2022 Lonafarnib FDA and EMA approval



# Drug Repurposing is promising opportunity for rare diseases, but....

...there are currently several hurdles that hamper the wider adoption of drug repurposing as an alternative solution to increase the throughput of therapeutic solutions for patients...



Lack of a dedicated regulatory pathway in the EU with clarity on role of market authorization holders



Investigators lacking drug development experience in a fragmented ecosystem



Unrecognized benefits of repurposing as a form of innovation in pricing and reimbursement



# Current EU-funded initiatives on Drug Repurposing



Develop a European industry-level online platform for validated **precision** drug repurposing

**Radboudumc**  
**SIMPATHIC**

Accelerate drug repurposing for rare **neurological, neurometabolic and neuromuscular disorders**



Develop a **sustainable** European innovation platform to enhance **patient-centric** repurposing of medicines **for all**



# REMEDi4ALL strategic vision



Develop and implement a European platform that facilitates fast and cost-effective patient-centric development and implementation of repurposed medicines, meeting high unmet medical needs in any disease area.



Build a complete, visible and accessible value chain for patient-centric drug repurposing



Create a think-tank that develops and advances drug repurposing policy



Madrid

ubljani



# How we try to do this...

## THE REMEDI4ALL CONSORTIUM IN NUMBERS

**4**  
DEMONSTRATOR PROJECT

**24**  
PARTNERS

**5**  
YEARS OF WORK  
(start Sep. 2022)

### PILLAR 1

Assembling EU's "Bricks and Brains" to support high impact DR projects at any stage of development



### PILLAR 2

Future-proofing the repurposing process



A PLATFORM THAT

is tested and sustainable

is a complete value chain

is patient-centric

has a vibrant community of practice

improves framework conditions





# The REMEDI4ALL PATIENT-CENTRIC APPROACH

Making medicines not just for patients,  
but with patients!



- ① Patients as partners/ co-owners
- ① Meaningful engagement (clearly planned and defined patient engagement activities)
- ① Continuous engagement
- ① Engagement with the most appropriate patients and at the most appropriate time

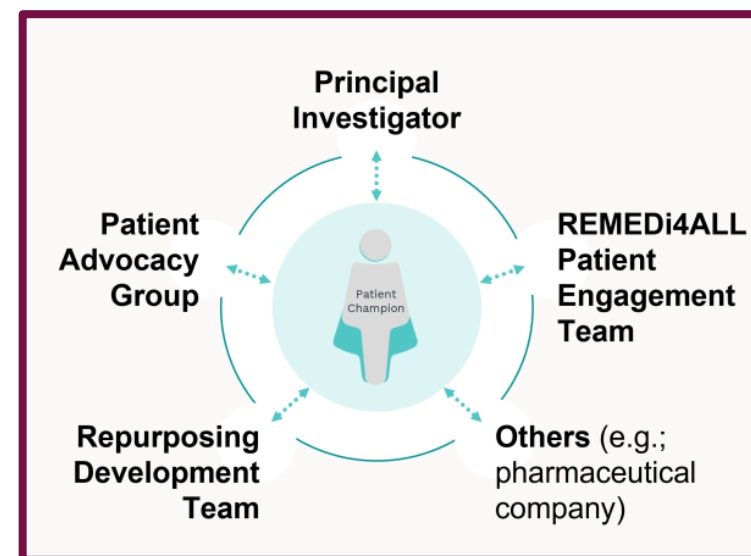
# Making medicines not just for patients, but with patients!

 Patients as partners/ co-owners → **PATIENT CHAMPION**

What is a REMEDI4ALL Patient Champion?

REMEDi4ALL Patient Champions (patient organization representative, patient, carer or patient advocate) are **CORE STAKEHOLDERS**, along with project PIs and technical experts in any repurposing project.

They represent their community and **actively participate in our Repurposing Developmental Team Meetings to provide evidence-based insights in all stages of the project.**

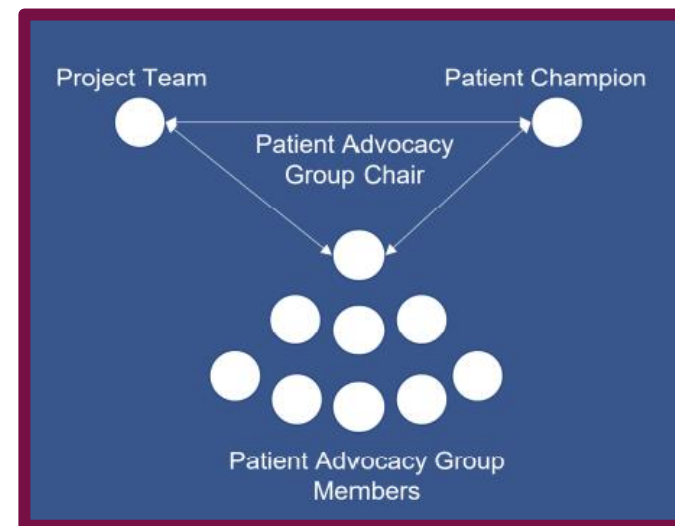


# Making medicines not just for patients, but with patients!

## Engagement with the most appropriate patients → **PATIENT ADVOCACY GROUPS (or alternatives)**

No two experiences with a given condition are the same. By establishing a Patient Advocacy Group (PAG, or alternatives) we guarantee a **more representative, robust and balanced range of insights into the diseases.**

In REMEDI4ALL the PAGs act as an **additional source of support and input** for the Patient Champion, the project team and the wider consortium.



# Making medicines not just for patients, but with patients!

## Meaningful and continuous patient engagement → **REMEDi4ALL PATIENT ENGAGEMENT PLAN**

The REMEDi4ALL Patient Engagement Plan is a living document that **helps the project PIs and teams identify and implement patient engagement activities.**

Drafting the Patient Engagement Plan is a collaborative effort between the Patient Champion, PAG and the project team.

### Aims:

- Define the disease specific patient landscape.
- Provide an overview of how we will engage patients.
- Build a timeline for activities.
- Develop a strategic approach to each engagement activity – what challenge are we looking to solve, and how.
- Assess the impact at every step!

# Making medicines not just for patients, but with patients!



## MULTI-STAKEHOLDER MEETINGS

### OVERALL AIMS OF A MSM :

- Share information and advance learning, in a pre-competitive and collegial setting.
- Dialogue and constructive interaction between **ALL** relevant stakeholder.

### The role of patients in a MSM:

- Share your experience living with the diseases, patient's unmet medical needs and patient's relevant topics in a Multi-Stakeholder environment.
- Learn on the advancement in the field.
- Initiate fruitful collaboration/partnerships with research teams.

# 1<sup>st</sup> REMEDI4ALL Multistakeholder Meeting

## Drug Repurposing, an Attractive Strategy in Pancreatic Cancer Treatment?



14th November 2023



9:00 am - 5:30 pm



Nhow Brussels Bloom.  
Rue Royale 250, 1210. Brussels, Belgium.

Organised by:



With the support of:



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





## GET IN TOUCH!

**Do you have an interesting drug repurposing project?** Get in touch with through our REPURPOSING CONCIERGE and let's talk!

<https://remedi4all.org/repurposing-concierge/>

SAVE THE DATE

for our **1<sup>st</sup> INTERNATIONAL DRUG REPURPOSING CONFERENCE!**

BRIDGING BOUNDARIES

**INNOVATING, CONNECTING & RESHAPING DRUG REPURPOSING**

*6-7 MARCH 2024, Barcelona*

<https://remedi4all.org/international-drug-repurposing-conference/>