

# The 'state of drug repurposing'

Insights from the ROADMAP Project

**ROADMAP**

# TIMELINE

## PROJECT LAUNCH

February 2021

1

## DATA CLEANING & ANALYSIS

January-May 2022

3

## ROADMAP CREATION

September-December 2022

5

## SURVEY

September-December 2021

2

## INTERVIEWS

June-August 2022

4

# GOALS

Gather insights from successful organizations, those who are still in progress or those who have abandoned the repurposing projects

**DATA**

Gather data on the state of drug repurposing projects

**INSIGHTS**

**TOOL**

Produce a "ROADMAP" tool, which can support organizations in future drug repurposing efforts.

# What we wanted to learn

## SUPPORT

In what ways can a rare disease nonprofit organization support a drug repurposing project?

## STEPS

What steps organizations are taking in pursuit of drug repurposing?



## PATHWAYS

What are the different paths to success and what are the different success outcomes?

## ROADBLOCKS

What are the biggest and most common roadblocks encountered by organizations? How do they overcome them?

# SURVEY: 147 US-based Rare Disease Nonprofit Organizations



# INTERVIEWS: 25 Rare Disease Nonprofit Organizations



Pachyonychia Congenita Project



OVERCOMING WEAKNESS WITH STRENGTH



# HIGH LEVEL INSIGHTS

**95**

ongoing drug repurposing projects

**60**

organizations leading drug repurposing projects

**2**

repurposed drugs granted FDA approval

**18**

organizations pursuing multiple drug repurposing projects

**12%**

organizations systematically track off label drug use

**8**

organizations focused on sirolimus (Rapamune)



238

off label drugs are  
being utilized



56

drugs are being repurposed

Sirolimus

Deferiprone

Everolimus

Bevacizumab

Glycerol

Cetuximab OV935/TAK935

Norursodeoxycholic Acid

Alpelisib

Cabozantinib

Earthquake  
Budesonide  
Nivolumab  
Chemotherapy  
Etanercept

Idelalisib  
Lovastatin O  
Ursodeoxycholic Acid

Lamotrigine  
Tacrolimus

Palbociclib  
Abemaciclib

Miglustat

Encalare  
Fingolimod

Bisphosphonates NOS

Desipramine

Trametinib

Nitisinone

Bosutinib

Benralizumab

Afatinib

Brigatinib

Tezepelumab

Omigapil

Metformin

Sunitinib  
Inverse Agonist NOS

Fenofibrate

Cyclodextrin NOS

Fuxolitinib

Cilofexor

Vancomycin

Acetazolamide

Sargramostim

Baclofen

Verapamil

Taselisib

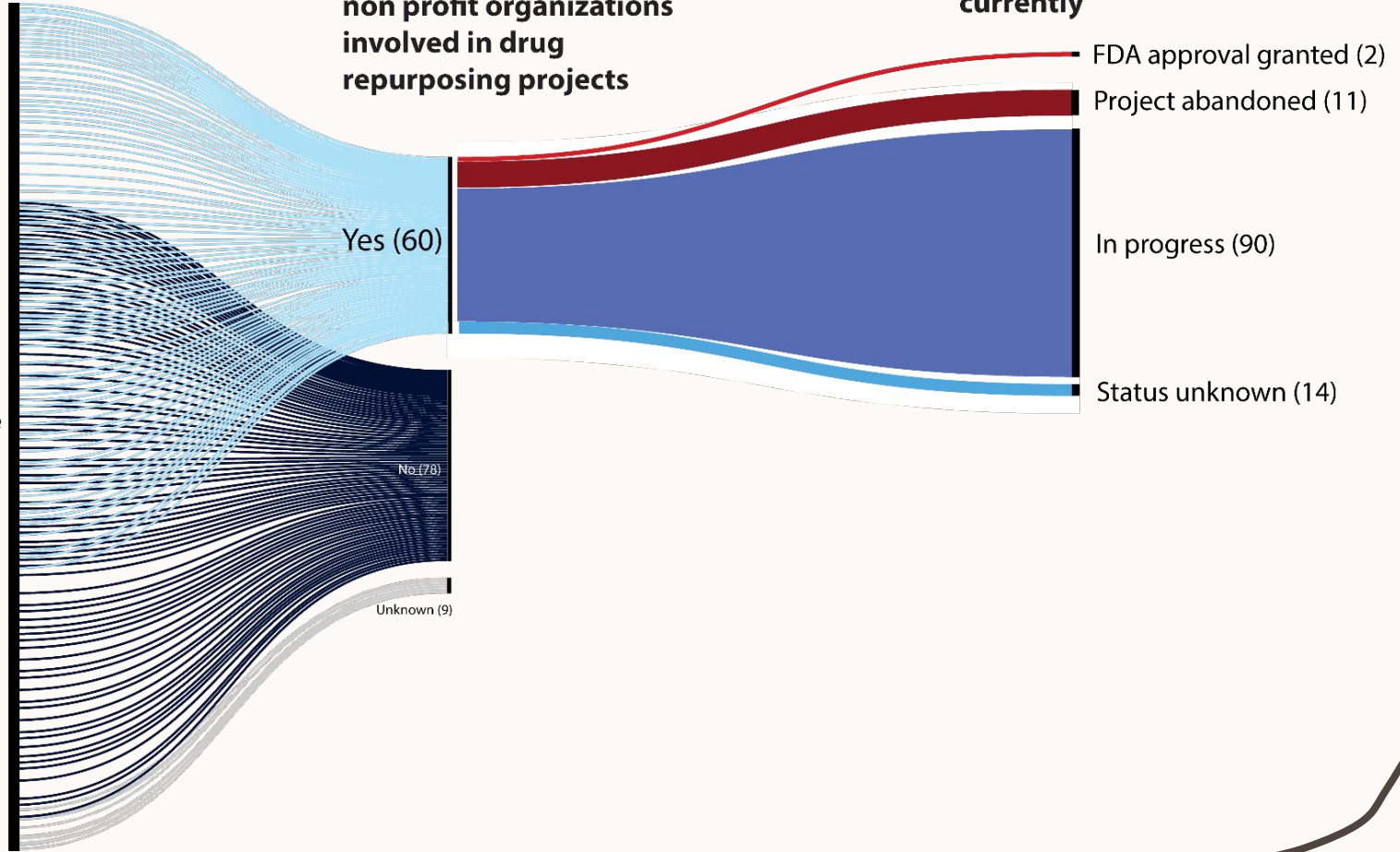
Rituximab

Sulfasalazine

Statins NOS



147 US-based rare disease non-profit organisations, who are participating in the ROADMAP project



Number of rare disease non profit organizations involved in drug repurposing projects

Stage of the repurposing project of each drug currently

Yes (60)

No (78)

Unknown (9)

FDA approval granted (2)

Project abandoned (11)

In progress (90)

Status unknown (14)

# TOP BARRIERS

for organizations to start drug repurposing projects



**Lack of financial resources**



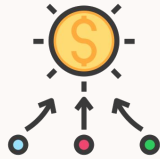
**Lack of understanding of the steps towards successful drug repurposing**



**Lack of staff to support project**

# TOP SUPPORT OPTIONS

- how organizations can push forward drug repurposing projects -



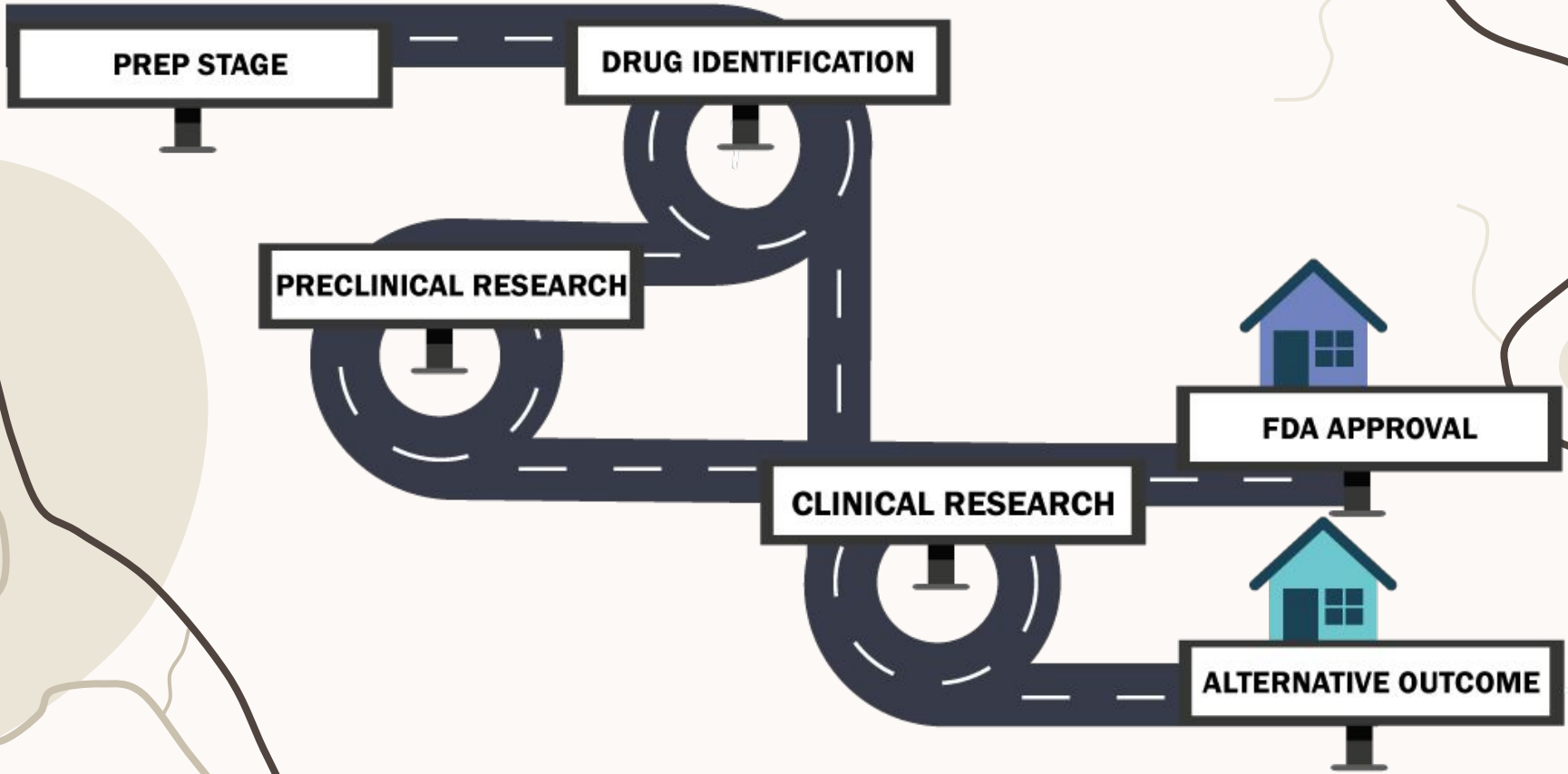
**Funded preclinical research**



**Facilitated national or international researcher collaboration**



**Supported patient recruitment into a clinical trial**



# DRUG IDENTIFICATION



Preclinical & Translational research



High-throughput drug screens



Insights from similar diseases

# SUCCESS OUTCOMES



**Drug to provide significant reduction in symptoms**



**Drug to provide significant improvement in quality of life (QOL)**



**Drug to provide prevention of relapse**

# ROADBLOCKS

Financial  
resources



Pharmaceutical  
company support



Lack of sufficient  
patient population  
to study





# INSIGHT & ADVICE



- Find an internal “champion” at the pharmaceutical company. Invite them to your events.
- Collaborate with researchers who have a collaborative approach and specific experience in translational research. Have a formal contract on data ownership.
- Combine clinical trial recruitment with patients with a similar rare disease.

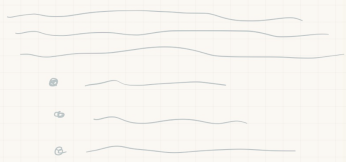
# INSIGHT & ADVICE



- Find ways that your rare disease can be applicable to a more common disease or disease space.
- Find or forge alternative paths and success outcomes that make sense for your patient population.
- Collaborate with other organizations and share data when possible, even if the diseases seem too different.

# ROADMAP

STAGE ONE:

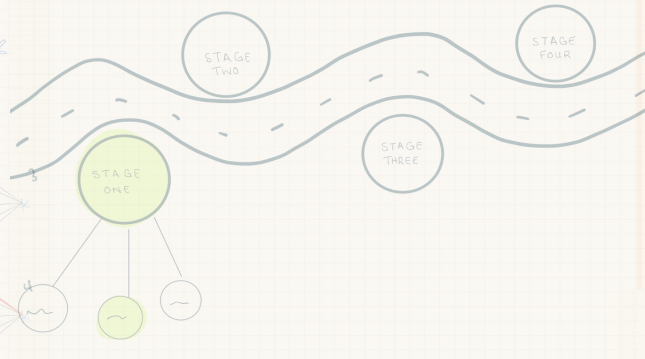
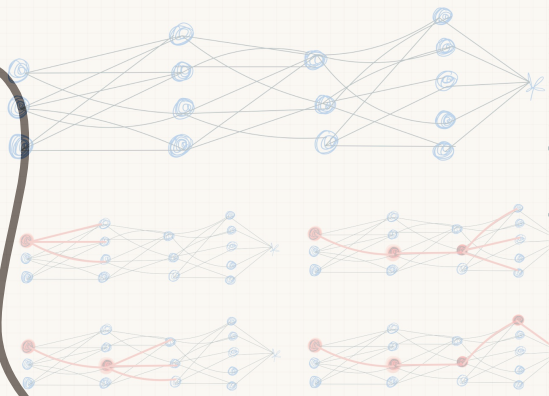
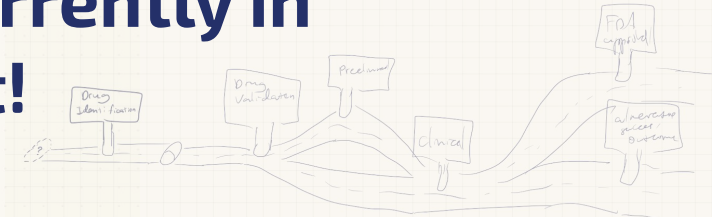
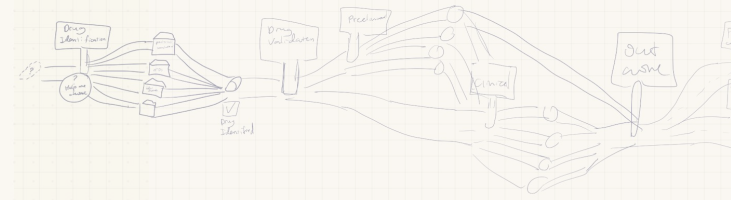


STAGE TWO



# “ROADMAP” tool is currently in development!

STAGE THREE



Drug ID	Preclinical	Clinical	Outcome
*			\$
✓	\$		
\$			
⚠		\$	

# Drug Repurposing ROADMAP

Throughout 2021-2022, the Castleman Disease Collaborative Network (CDCN) has conducted a survey of US-based rare disease non-profit organizations (RDNPs) and their stakeholder populations (patients, loved ones, physicians and researchers).

We had 147 organizations participate, and along with their stakeholders we received over 1,923 survey responses.

In the following report, we would like to present a high-level overview of the findings from our data to help provide answers to some fundamental questions regarding the state of rare disease drug repurposing in the US.

## Introduction

The CDCN reached out to 755 US-based rare disease non-profit organizations on 9/29/2021, and throughout the next two months 147 (19.4%) organizations participated in the survey. \_\_\_\_\_% of these also forwarded the survey on to their patient, loved one, physician and researcher communities.

**1,923** responses

- 147 Organizations
- 340 Patients
- 170 Loved ones
- 23 Physicians
- 43 Researchers

# ALL ROADMAP survey data will be available to explore in an interactive dashboard format!

## Rare Disease Non-profit Characteristics

In this section, we will provide information about the general characteristics of the organizations in our sample (size, funding, activities, etc). These characteristics are important to understand the types of organizations who participated in the project, as well as identify characteristics that are potentially precursors for success in both general research support and successful pursuit of drug repurposing.

### Leadership Titles

We invited only members of the leadership team to provide data for their organization. Most of our responses were from the Executive Director, President or Founder of the organization.

### Organization year of founding

Our organizations are "young"! 83 organizations (56%) were founded in 2010 or later, with 9 founded as recently as 2020 during the COVID pandemic.

## Collaboration

### How many organizations do you collaborate with on a regular basis for either research or community-centered projects?

1-5 organizations	47
5-10 organizations	41
10-20 organizations	25
20-50 organizations	11
50 or more	5

n (total) = 130

### What kinds of activities do you engage in with these organizations?

Sharing prior experiences that can inform future decision making	267
Sharing resources	206
Creating a shared research agenda	186
Joint community building & fundraising	181
Pooling patient populations together to conduct future research	159
Sharing data and/or samples for research	156
Conducting research studies including clinical trials	136
Organization provides us funding	120
Applying for funding together	82
Co-creating or co-supporting a center of excellence	54
Other	48

## Rare Disease State of Research

### Does your rare disease have the following?

Diagnostic Criteria	Yes: 176	No but in progress: 11	Unknown: 11	No and not planned: 0
Treatment guidelines / Standard of Care guidelines	Yes: 78	No but in progress: 16	Unknown: 16	No and not planned: 0
Clear understanding of etiology or disease pathogenesis	Yes: 68	No but in progress: 16	Unknown: 16	No and not planned: 0
A specific CD-10 code	Yes: 61	No but in progress: 26	Unknown: 23	No and not planned: 14
Identified genetic mutation	Yes: 112	No but in progress: 13	Unknown: 13	No and not planned: 0
Predictive Biomarkers	Yes: 57	No but in progress: 27	Unknown: 27	No and not planned: 0
Animal models	Yes: 82	No but in progress: 14	Unknown: 14	No and not planned: 0
Cell lines	Yes: 14	No but in progress: 24	Unknown: 24	No and not planned: 0

n (total) = 130-148

### Are there any FDA-approved drugs for your rare disease or diseases of focus?