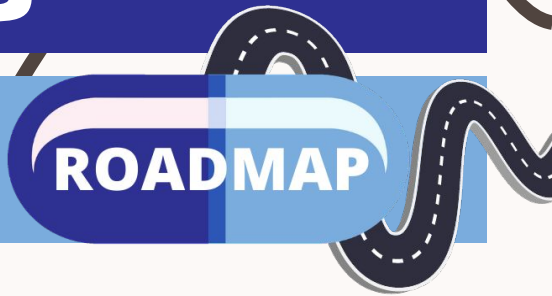
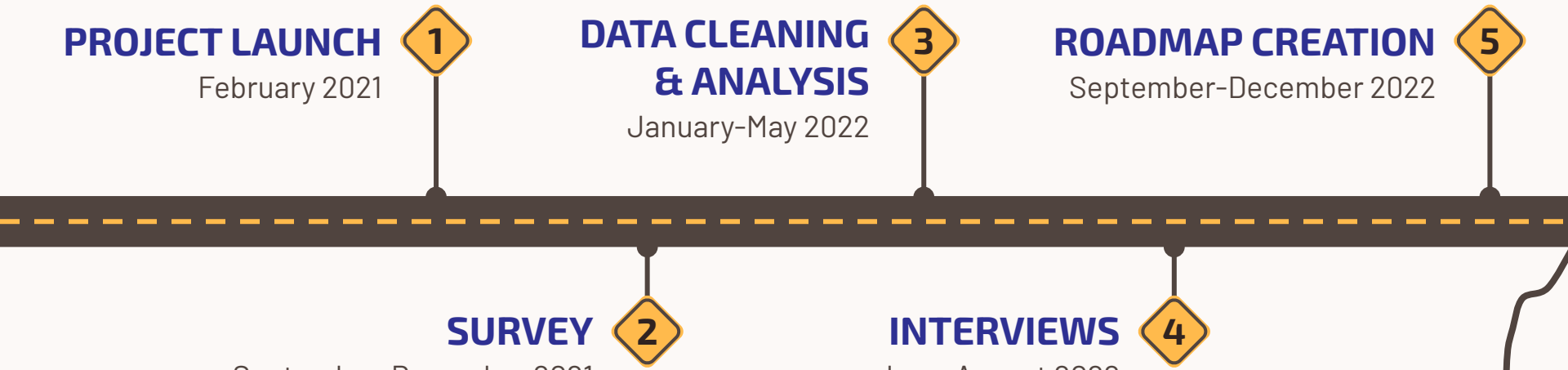
The ‘state of drug repurposing’

Insights from the ROADMAP Project



TIMELINE


PROJECT LAUNCH
1
February 2021
SURVEY
2
September-December 2021
DATA CLEANING & ANALYSIS
3
January-May 2022
INTERVIEWS
4
June-August 2022
ROADMAP CREATION
5
September-December 2022

GOALS

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

Gather data on the state of drug repurposing projects

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

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

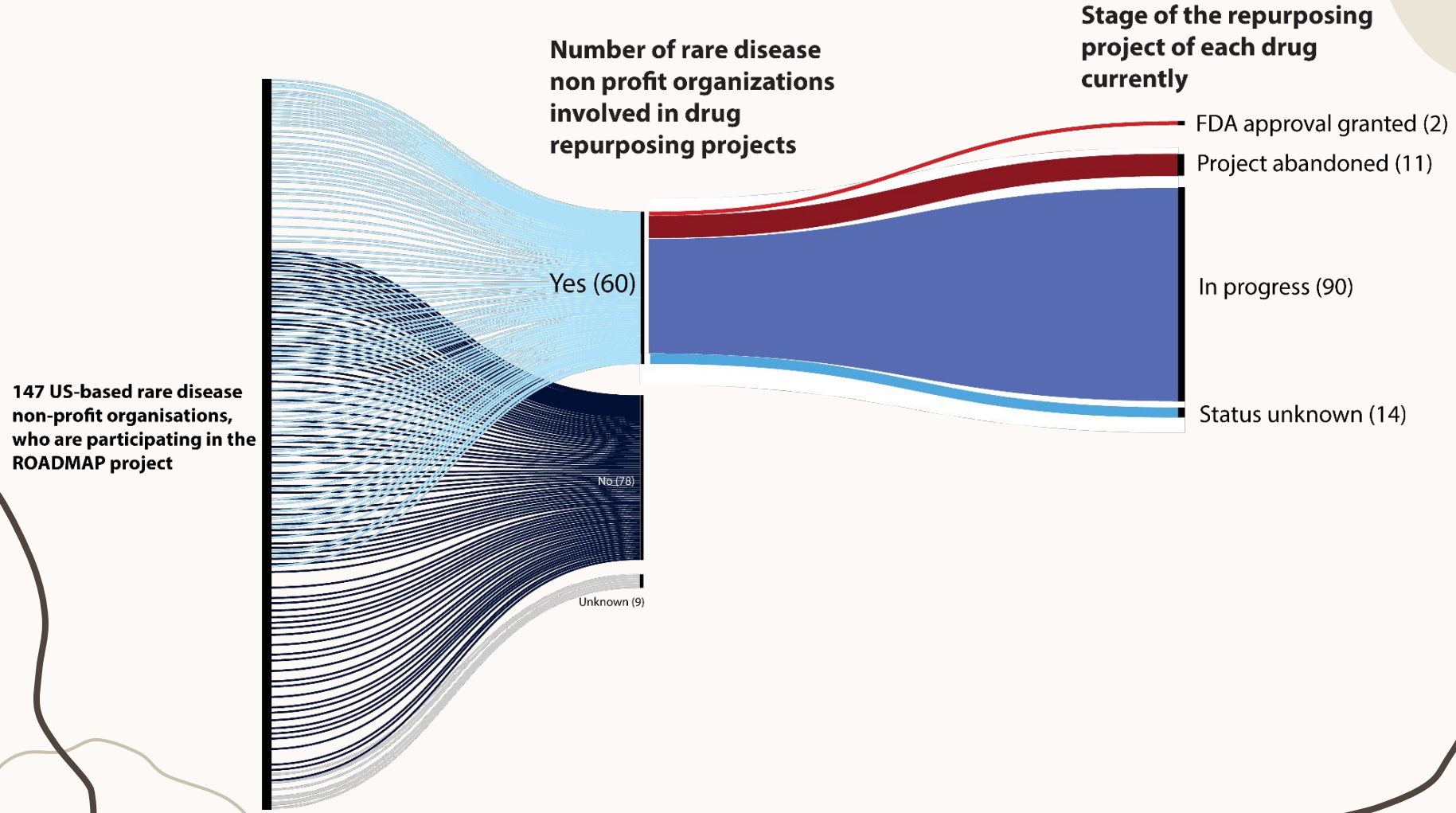
organizations focused on sirolimus (Rapamune)

238

off label drugs are being utilized

95

drugs are being repurposed


Number of rare disease non profit organizations involved in drug repurposing projects
Stage of the repurposing project of each drug currently
147 US-based rare disease non-profit organisations, who are participating in the ROADMAP project
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

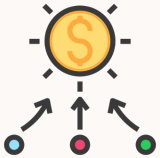
Lack of financial resources

Lack of understanding of the steps towards successful drug repurposing

Lack of staff to support project

TOP SUPPORT OPTIONS

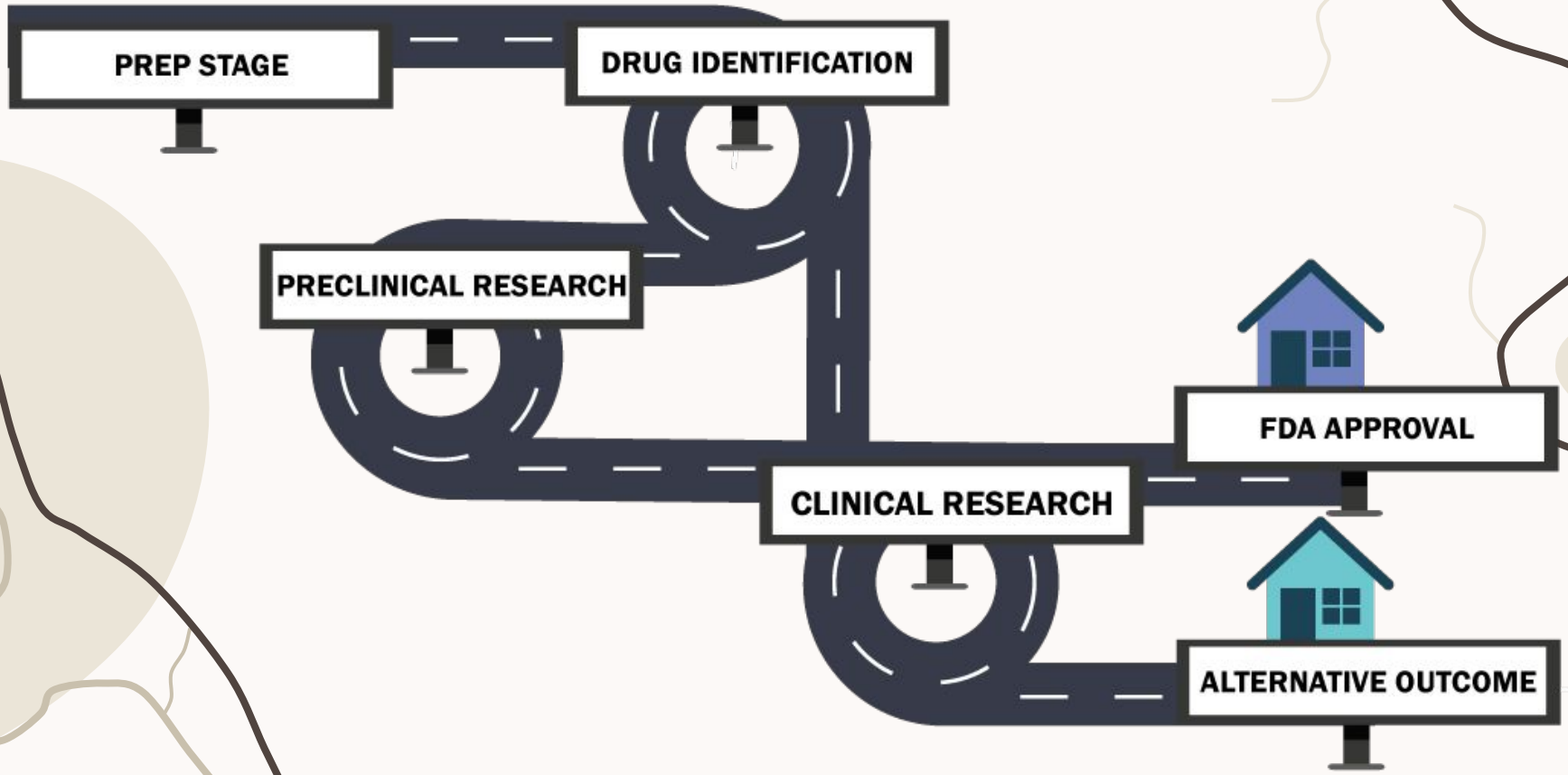
- how organizations can push forward drug repurposing projects -



Funded preclinical research

Facilitated national or international researcher collaboration

Supported patient recruitment into a clinical trial


PREP STAGE
DRUG IDENTIFICATION
PRECLINICAL RESEARCH
FDA APPROVAL
CLINICAL RESEARCH
ALTERNATIVE OUTCOME

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

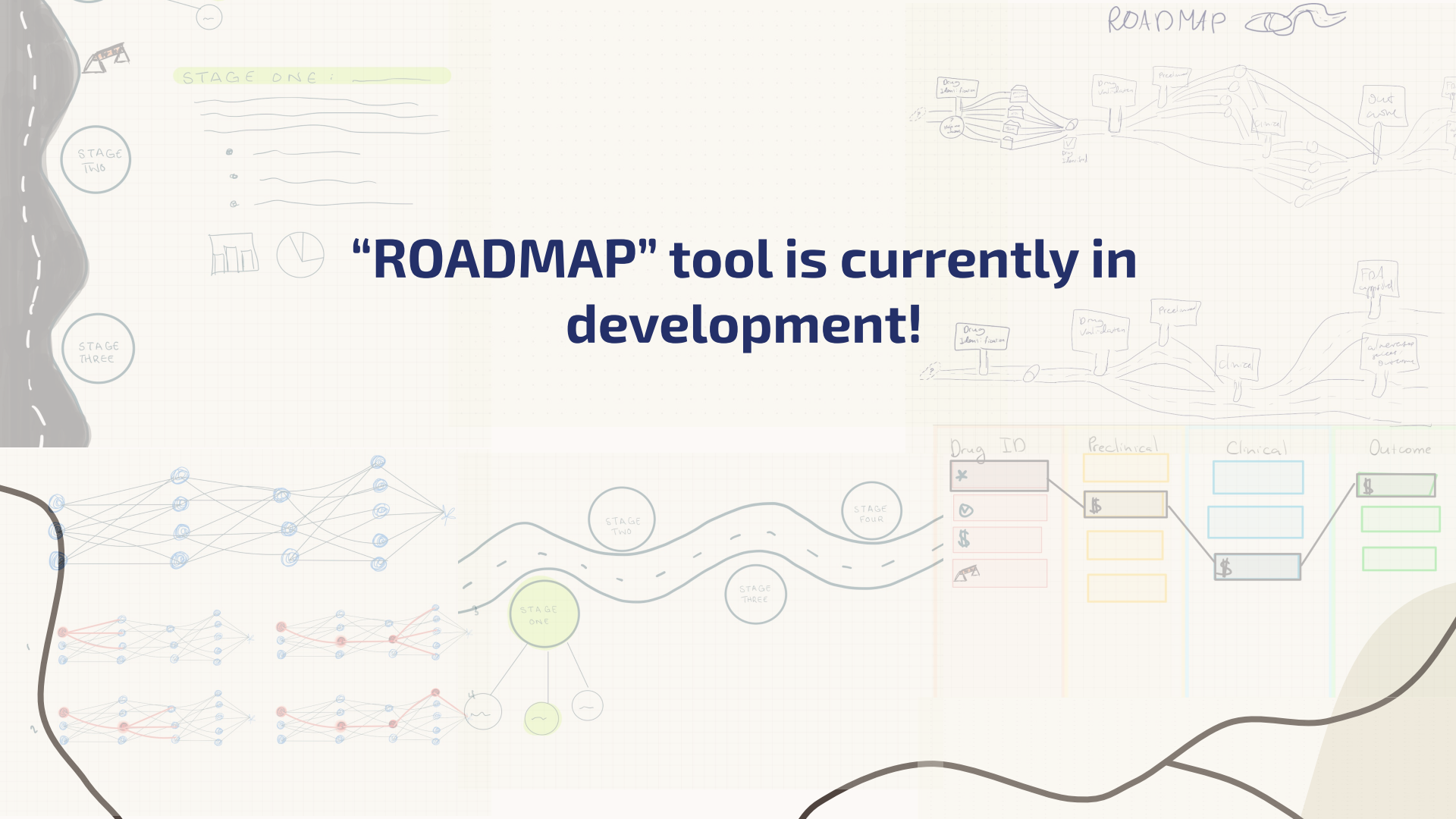
INSIGHT & ADVICE

- 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” tool is currently in development!



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 1,900 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.

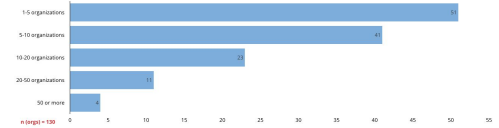
1,923 responses

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

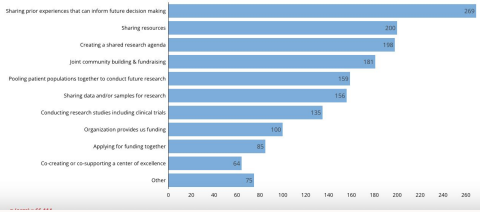
All ROADMAP survey data will be available to explore in an interactive dashboard format!

Collaboration

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



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



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.

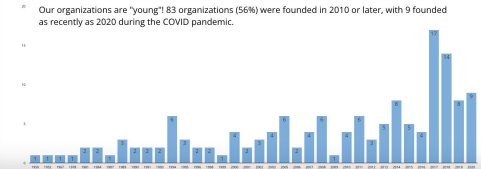
Leadership Titles

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

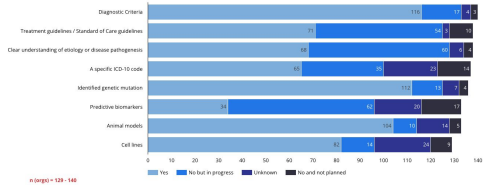
n = 146

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



Rare Disease State of Research

Does your rare disease have the following?



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