The ‘state of drug repurposing’

Insights from the ROADMAP Project
TIMELINE

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

TOOL

Gather data on the state of drug repurposing projects

INSIGHTS

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

DATA

PRODUCE A "ROADMAP" TOOL, WHICH CAN SUPPORT ORGANIZATIONS IN FUTURE DRUG REPURPOSING EFFORTS.
What we wanted to learn

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

**STEPS**
What steps organizations are taking in pursuit of drug repurposing?

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

**PATHWAYS**
What are the different paths to success and what are the different success outcomes?
SURVEY: 147 US-based 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)
off label drugs are being utilized
95 drugs are being repurposed.
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:
- Yes (60)
- Unknown (9)

Stage of the repurposing project of each drug currently:
- 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” tool is currently in development!
All ROADMAP survey data will be available to explore in an interactive dashboard format!