ROADMAP (**R**epurposing **O**f **A**ll **D**rugs, **M**apping **A**ll **P**aths)

Project Overview

BACKGROUND

There are about 7,000 known rare diseases and many of them are deadly. 95% of these rare diseases do not have an effective treatment. Since incentives for new drug development for rare diseases are limited, **drug repurposing** provides a promising avenue to identify effective treatments for rare diseases faster and cheaper than novel drug development.

However, there are challenges in the drug repurposing space, such as a lack of consensus of the roles that various stakeholders play in drug repurposing, a lack of a "roadmap" of how rare disease organizations should go about pursuing drug repurposing for their rare disease population, and a lack of understanding of the role inter-organizational collaboration, information, and knowledge sharing both within and across rare diseases plays in supporting drug repurposing.

The ROADMAP Project proposes to fill some of these knowledge gaps.

GOALS

In our study, we seek to:

- Answer some fundamental questions about the experience of drug repurposing for various stakeholders (rare disease non profit organizations, physicians, researchers, patients and loved ones)
- Produce a "roadmap" for rare disease non profit organizations to follow if they would like to pursue drug repurposing
- Understand the collaboration and knowledge sharing practices of rare disease non profit organizations within the rare disease space in regards to drug repurposing
- Build a network of rare disease non profit organizations and their collaborators in order to get a better understanding of the rare disease space in general.

RESEARCH

We will utilize a combination of methods to pursue our research questions as follows: document analysis, surveys, network analysis, interviews, and participatory design sessions.

1. **Document Analysis:** We compiled a comprehensive list of US-based rare disease non profit organizations through combining lists from multiple sources. So far in our sample, we have 889 organizations. We are utilizing volunteers to crowdsource data collection from the organization's websites.

2. Survey: To the best of our knowledge, there is no prior work that systematically captures information about all rare disease non profit organizations in the US and their levels of engagement with drug repurposing. This lack of data may hinder collaboration between rare disease non profits and may slow down the pace of drug repurposing. Thus, we are pursuing a survey as a way of creating a foundational dataset. *(Detailed information about the survey on next page)*

3. Network Mapping: Through the surveys and document analysis we will be capturing data that allows us to visually map networks on two levels: 1) the existence of rare disease non profit organizations for rare diseases, capturing instances of multiple organizations focusing on the same, similar or overlapping diseases and 2) the collaborations between them (or lack there of).

4. Interviews & Participatory design sessions: We will select 10-20 organizations and do a deep dive on their drug repurposing journey, engage in live sketching of their personal "roadmap" of the steps they have taken in both preparing for and in the pursuit of a drug repurposing agenda, and gather detailed data on their information-based challenges and how they were able to overcome them.

LEADERSHIP & PARTNERS

This project is an effort by the Castleman Disease Collaborative Network (CDCN). Principal Investigator is Dr. David Fajgenbaum, Assistant Professor at the Perelman School of Medicine (University of Pennsylvania), Board President and Co-Founder of the CDCN. Co-Investigator is Ania Korsunska, Ph.D. Candidate at Syracuse University and Biomedical Leadership Fellow at the CDCN. Other members of the CDCN team include Mileva Repasky (Chief Patient and Development Officer) and Johnson Khor (Healthcare consultant). The CDCN was awarded a grant from the Chan Zuckerberg Initiative to pursue this research.

For more information, visit https://cdcn.org/roadmap or email ania@castlemannetwork.org

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Survey Overview

General

The survey has multiple goals:

- To capture the experiences of various stakeholders (rare disease organization representatives, patients, loved ones, physicians, and researchers) with drug repurposing
- To gather data to basic organizational and disease characteristics of rare disease non profit organizations, levels of knowledge sharing/collaboration and interest/success in drug repurposing
- To explore associations between certain characteristics and interest/success in drug repurposing
- To gather preliminary data on the "roadmaps" of rare disease organizations currently pursuing drug repurposing

Benefits of participating:

- **Your experiences will help inform future drug repurposing efforts for all rare diseases**
- **Your data will help researchers understand the challenges involved in drug repurposing for your rare disease**

For each of our stakeholder populations, we are interested in questions focusing on various variables of interest. Below is a brief overview of what we're looking for in each population.

Rare Disease Non Profit Organizations - Variables of Interest

- **Rare disease organization's characteristics:** mission, age, resources (biobank, patient registry, natural history study, scientific advisory board, etc), level of funding, existence of other organizations that focus on the same disease, level of collaboration with those organizations and others.
- **Rare disease characteristics:** state of research, treatment guidelines, diagnostic criteria, ICD code, biomarkers.
- **Drug repurposing:** existence of FDA approved drugs and their efficacy and availability, existence of off-label drugs and their efficacy and availability, organization's level of interest and involvement in drug repurposing, definition of drug repurposing “success”, drug repurposing roadblocks.

Patient & Loved Ones - Variables of Interest

- Access to and benefit/lack of benefit from existing FDA approved drugs for rare disease
- Access to and benefit/lack of benefit from identified off-label drugs for rare disease
- Factors impacting decision-making in taking off-label drugs
- Awareness and attitude towards drug repurposing

Researchers - Variables of Interest

- Awareness and interest in doing drug repurposing research
- Previous and current level of engagement in drug repurposing research
- Motivations for and challenges in pursuing drug repurposing research
- Availability of research tools, techniques and levels of funding to support drug repurposing research

Physicians - Variables of Interest

- Physician specialty, frequency of treating patients with rare diseases, and which rare diseases
- Sources of information about rare diseases and their treatments
- Frequency of off-label drug prescription
- Factors taken into consideration when deciding to prescribe a drug off-label
- Avenues of sharing information about off-label drug prescription and usage
- Levels of engagement in clinical trials

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