

# RARE RESEARCH ROADMAP

Let's  
Collaborate!

## The Road from Bench to Bedside

You're in the  
driver's seat.

Load Your Bus

Understanding  
your disease leads  
to treatment, but  
sometimes you  
have to circle back.

## Basic Research

Fuel up with  
Scientific Tools

### RESEARCHER'S TOOLBOX

Decide which tools  
you'll need:

- Animal models
- Biobanks
- Biomarkers
- Cell lines
- IPS cells
- Patient registries
- Natural history studies

## Preclinical Research

Follow this route to  
treatments that are  
safe and effective

## Lead Optimization

- Efficacy
- Toxicity
- Pharmacokinetics

Improve  
molecule profile

## Hit to Lead

- Computer Modeling
- Cell/Animal Models
- High Throughput Screening

Found some!  
Now narrow  
the possibilities.

N of 1

Drug  
Repurposing  
This Way

## Drug Discovery

Look for the  
needle in a  
haystack.

- High Throughput Screening
- Engineering  
New Therapies

## Drug Development

Ok to test  
this on  
humans?

- Evaluate Toxicity Risk
- Estimate Dose
- Regulatory Activities
- Manufacture the Drug

## Candidate Selected

We need more studies!

## Submit IND

Investigational  
New Drug  
Application

## Clinical Trials

Advocacy  
groups drive  
clinical trial  
recruitment

## Phase I

Alternate Routes  
Available →

Basket Trials, Umbrella  
Trials, Remote Trials,  
Decentralized Trials,  
Phase 1/2, Replace  
Placebo with Natural  
History

## Phase II

## Phase I/II

## Phase II/III

## Phase III

Compassionate  
Use →

## Phase IV/V (POST MARKET RESEARCH)

Track long-term safety  
and benefits  
• Pricing  
• Reimbursement

## Access and Distribution

Approved

TRY  
AGAIN

- Abbreviated New Drug Application (ANDA)
- Breakthrough Therapy
- Fast Track
- Orphan Drug Status
- Priority Review
- Pediatric Priority Review Voucher Program
- Regenerative Medicine Advanced Therapy Designation (RMAT)

STANDARD  
REVIEW

ACCELERATED  
REVIEW

LINE STARTS  
HERE

## HOW IS RESEARCH FUNDED?

- Federal grants
- Patient organization grants
- Industry support
- Venture philanthropy