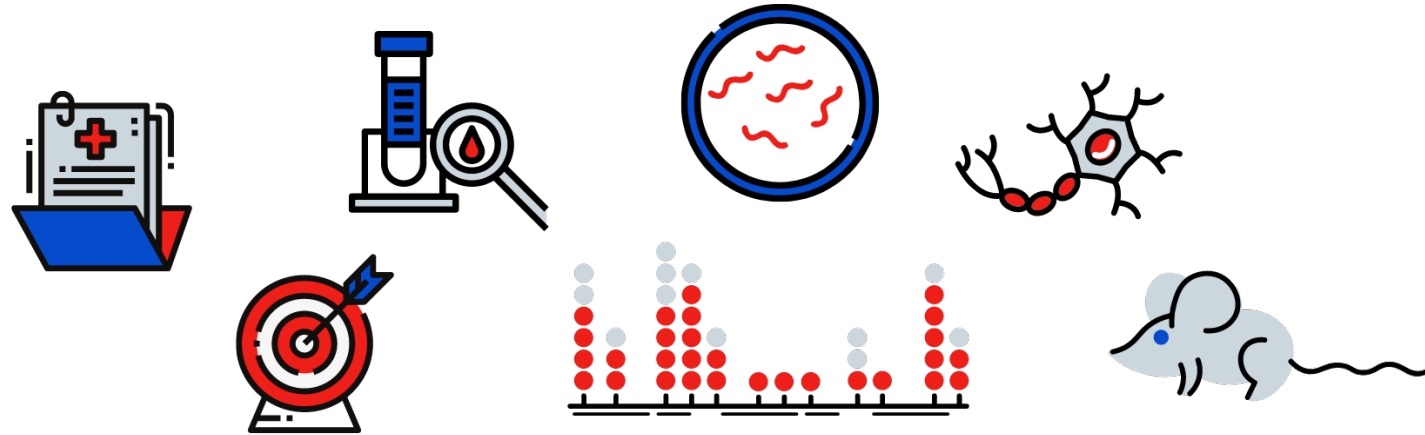


# WHAT IS THERAPEUTIC DEVELOPMENT?

“A collection of preclinical strategies to support the development of products intended for use in the cure, mitigation, diagnosis, or treatment of disease”



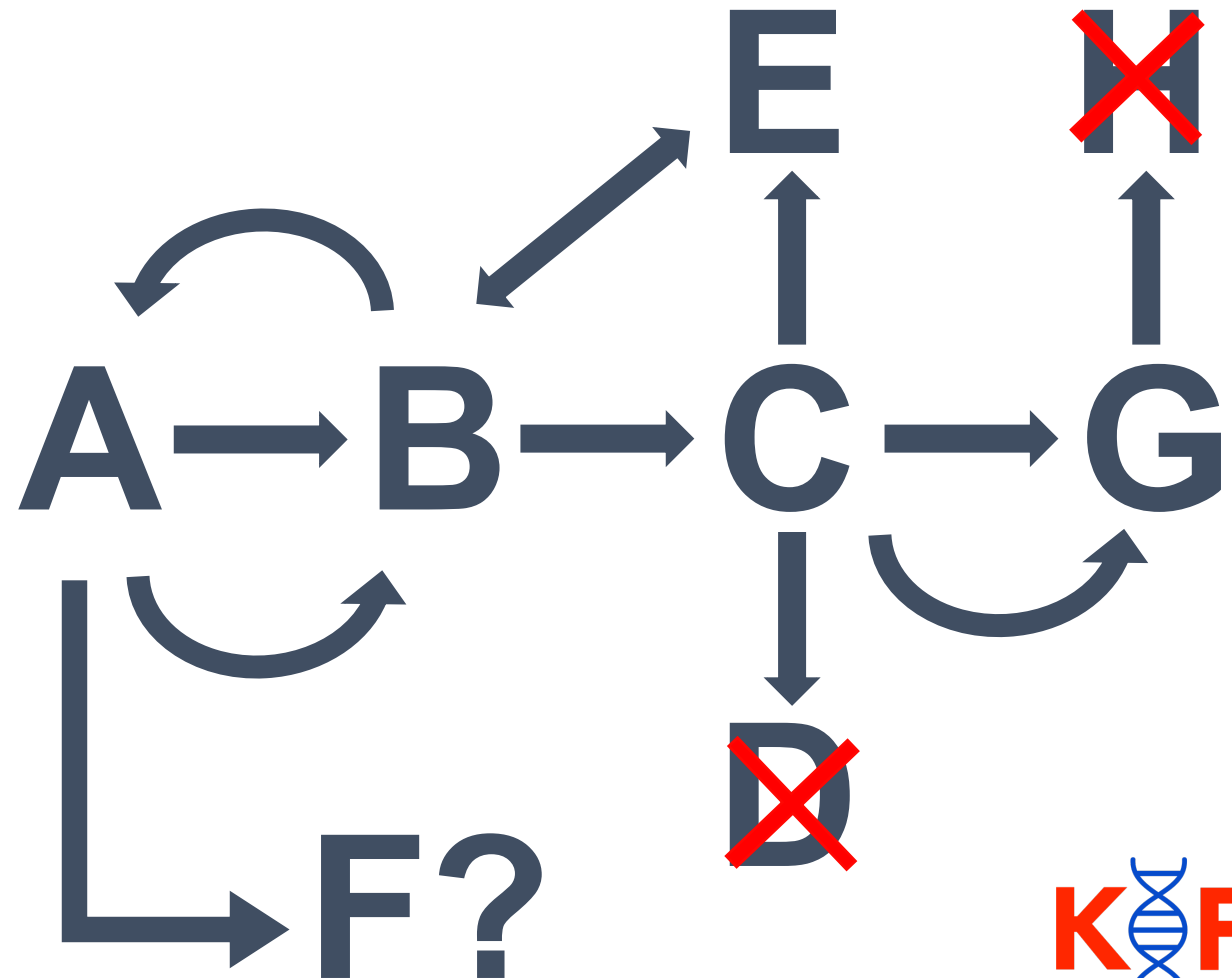
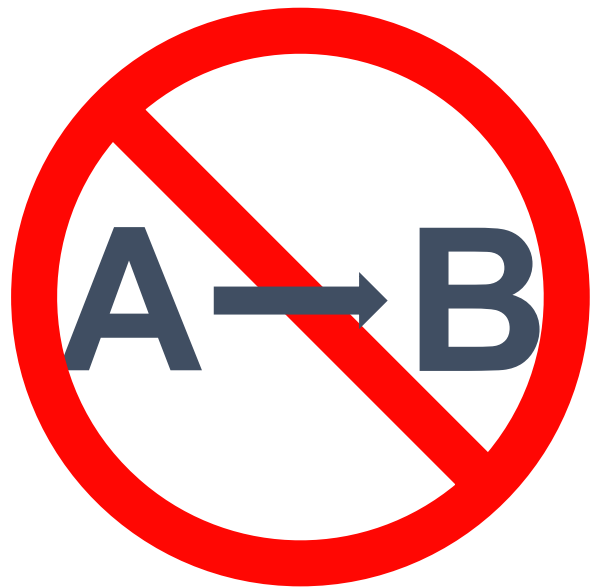
Our goal: gather and openly share resources that scientists and clinicians need to develop treatments for KAND

# HOW DOES THERAPEUTIC DEVELOPMENT WORK?

Expectation

Vs.

Reality



# HOW DOES KIF1A.ORG APPROACH THERAPEUTIC DEVELOPMENT?

- What is our approach?
  - “De-risk” KIF1A/KAND research and development
  - Cast a wide net of opportunities and pivot quickly
  - Hold a seat at the table of important stakeholder meetings
  - Be *relentless*
- Why is it unique?
  - Resources are openly available for scientific partners
  - Patients are at the center of every conversation
  - Dedicated KIF1A.ORG science team working with developers

# WHERE ARE WE NOW?

- Multiple active partnerships

# ACTIVE THERAPEUTIC PARTNERSHIPS



Albert Einstein College of Medicine

AI drug discovery



Gene therapy &  
RNAi gene targeting



Targeted high-throughput  
& epilepsy-focused screens



iPSC characterization & screening  
platform

Single drug repurposing efforts with  
multiple academic/biotech collaborators

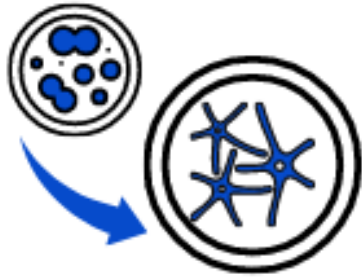


Target identification & screening

## WHERE ARE WE NOW?

- Multiple active partnerships
- Relationships that have evolved:
  - MCRI/CMRI (Kaur/Christodoulou/Gold): Received grant from Australian NHMRC focused on epilepsy treatment for KAND
  - Gennerich lab: Winner of 2022 Xseed award focused on identifying drugs targeting the KIF1A protein
  - Rarebase: Concluded axonal degeneration ASO study; currently reviewing data
- TAP/NeuCyte compounds screening starting August 2022

# TREATMENT ACCELERTOR PROGRAM – 2022 UPDATE



## DIFFERENTIATE

Create neurons with KIF1A mutations to model the disease



## PHENOTYPE

Collect data on observable characteristics of healthy neurons vs. KAND neurons



## SCREEN

Test promising treatments for KAND, using insights from phenotyping to look for measurable improvements



**IDENTIFY  
THERAPEUTIC  
CANDIDATES FOR  
CLINICAL TRIALS**

# WHERE ARE WE NOW?

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- Launched KOALA and EEG Study



# HOW DOES KOALA STUDY ADVANCE THERAPEUTIC DEVELOPMENT?

“Generates clinical outcome measures and endpoints”



Determine how effective potential therapeutics will be in patient population



KIF1A  
Outcome measures,  
Assessments,  
Longitudinal  
And endpoints Study

# HOW WILL WE BRING TREATMENT TO OUR COMMUNITY?

- We are working on both short-term and long-term solutions at the same time
- We develop tools and resources that are expediting our transition into clinical trials
  - Pre-clinical tools for development
  - Patient-centered studies (NHS, KOALA, etc.)



It is our mission to find treatment for  
*every single patient.*