



# DRUG REPURPOSING AS A STRATEGY FOR KLEEFSTRA SYNDROME

Sept 20, 2017 (12:00pm EST)



Advancing drug development for  
Kids with Intellectual Disability Syndromes

# Submit Your Questions

2

To send in questions, please use  
WebEx's Q&A feature

(located on the right-hand side of your screen, or at the top of your screen if you're in full view mode).

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# What We'll Cover Today

3

- ❑ What is Drug Repurposing/Repositioning?
- ❑ How do drugs get chosen for repurposing?
- ❑ Why do drugs need to be re-tested in a new patient population?
- ❑ What is the latest on repurposed/repositioned drugs in testing for KS?
- ❑ Q&A session

# Today's Webinar Panelists

4



**Braden Root-McCaig** (Moderator)

▣ Executive Director, K.I.D.S. IQ Project



**Dr. Paul Smith**

▣ Scientific Director, K.I.D.S. IQ Project

# What is Drug Repurposing?

- Testing a therapy approved for one disease for its effect on another
- How we choose a drug for repurposing?
  - ▣ Observe trend in people who take the drug
  - ▣ Identify disease biology that existing drug addresses
  - ▣ Select drugs with likelihood of success (i.e., can get into the brain)
  - ▣ Sometimes no selection bias – screen entire FDA library
- What is this different from repositioning?

# Why Do We Have to Re-Test Repurposed Drugs?

- We need to make sure the drug works.
- The biology and symptoms of a different disease may impact drug safety.
- The required dose may be different, which could impact safety.
- It may interfere with current treatment regimen.

# Applied to Kleefstra syndrome (KS)

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- K.I.D.S. IQ Project actively funding a research project looking to screen FDA-approved library
- Also screening drugs/compounds not yet approved by FDA (developed for other diseases/disorders)

# Case Studies

- Frontotemporal Dementia (FTD)
  - ▣ Bluefield project: Identified compound through FDA library screen
- Fibrodysplasia Ossificans Progressiva (FOP) or “stone man’s disease”
- ALS screening
- Parkinson’s drug for dyskinesia



# Bluefield project: Fronto-temporal Dementia (FTD)

## Background

- Progressive neurodegenerative disease that causes profound behavioral and personality changes.
- 3 main forms of fronto-temporal dementia: linked to mutations in six genes.
- Progranulin gene mutations result in lowered levels of progranulin protein and this causes nerves in the brain to die.

## Approach

- High-throughput screening (HTS) of new drug-like chemicals and FDA approved therapies.

**UTSouthwestern**  
Medical Center

+230,000 compounds



National Center  
for Advancing  
Translational Sciences

+590,000 compounds



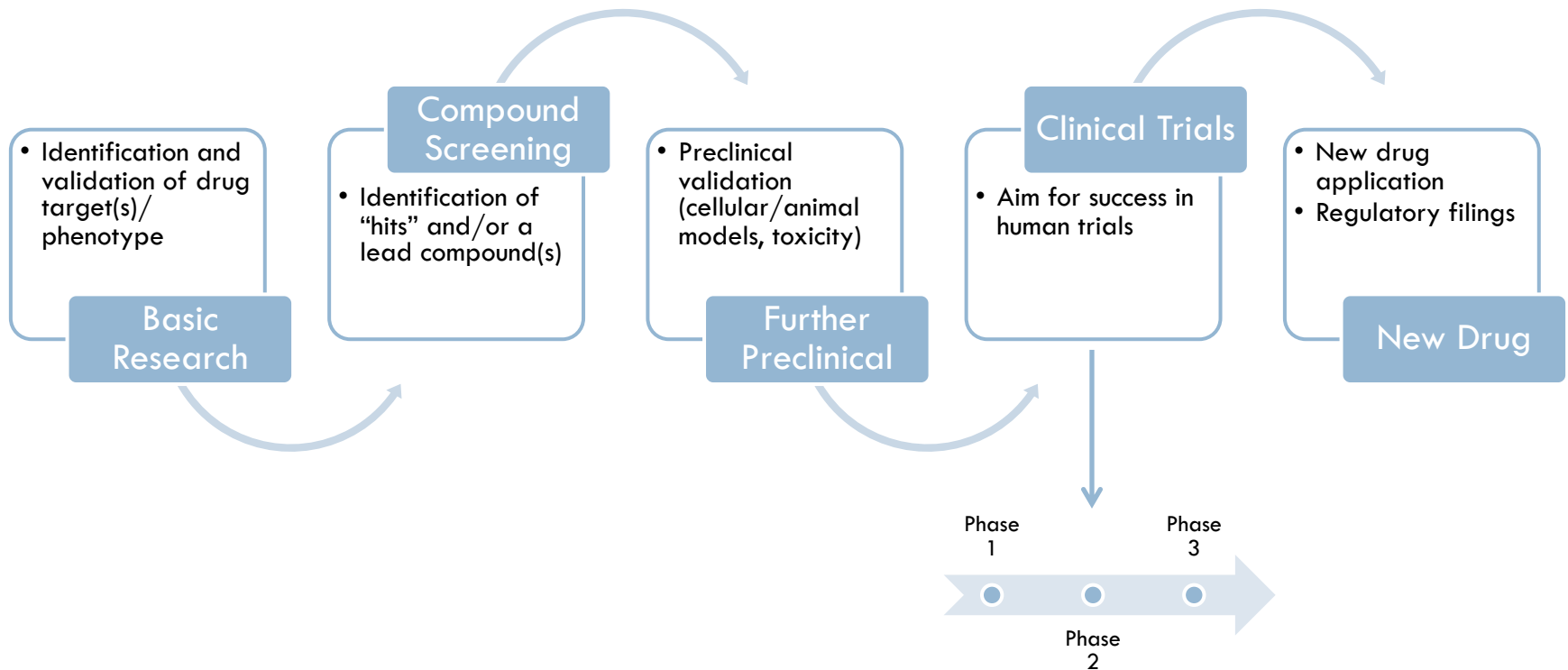
+150,000 compounds

## Status

- Identified Nimodipine. Drug approved for treatment of high blood pressure.
- Recently completed a small clinical trial (dose finding and safety).

# Impacting the Traditional Timeline

13



Accelerated timelines are achievable with rare disorders and drugs that are re-purposed/re-positioned

# Key Takeaways

- Drug repurposing & repositioning can point us to potential KS treatments.
- This approach may move drugs into clinical trials faster but still demands rigorous testing to understand impact and safety in KS.
- KS is an orphan indication (rare) and would receive fast-track review (no medicines) which accelerates timelines.
- A family database improves our knowledge of the KS clinical spectrum and organize any future clinical trial faster.

# QUESTION & ANSWERS

Type your questions into the Q&A text box.

# Upcoming with K.I.D.S. IQ Project

15

Submit Patient  
Data

- [www.kidsIQproject.org](http://www.kidsIQproject.org):
- “Patient registry & database”

Sept 22

- Walk & Roll - Webinar presentation

Oct. Webinar

- KS & Mental Health

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