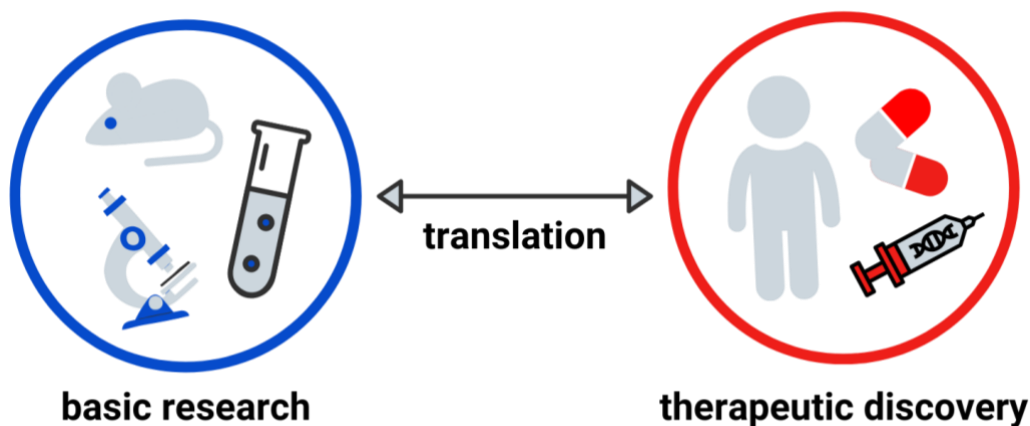




Research Roundtable Community Summary – September 24, 2020

The third KIF1A.ORG Research Roundtable meeting focused on “Therapeutic Strategies for KAND.” Before diving into the summary of this meeting, let’s take a step back to discuss the purpose of the KIF1A.ORG Research Network. The ultimate goal of the Research Network is to accelerate the path from basic research in the lab to treatments for people living with KAND. This is known as “translational” or “bench to bedside” research.

Scientists could spend their entire careers running and analyzing experiments to learn how KIF1A functions. Their work will never end. **Our objective is to identify gaps in knowledge we need to fill, not just to learn more about KIF1A, but to discover how to treat KAND.** To learn more about the spectrum of translational science, visit [this page](#) from the NIH National Center for Advancing Translational Sciences (NCATS).



Our goals for September’s Research Roundtable meeting were to 1) Review current KIF1A/KAND therapeutic strategies, especially for new members, and 2) hear insights on rare disease therapeutic discovery from three leaders in the KIF1A.ORG Research Network. Having Research Network members be informed and on the same page is key to our mission of accelerating research to find treatments for KAND!

Attendance



14 INSTITUTIONS



25 RESEARCHERS
from **3** CONTINENTS



3 KIF1A.ORG REPS

Presenters & Summaries

Dominique Lessard, PhD | KIF1A.ORG

- We opened the floor with a brief presentation from Dr. Dom in which she covered recent advancements such as [Ionis](#) and [Ovid](#) KAND therapeutic development announcements. She then went on to outline our short-term and long-term therapeutic goals, highlighting where our Research Network fits into the process.

Wendy Chung, MD/PhD & Lia Boyle | Columbia University

- Next, Lia Boyle gave a brief clinical overview of KAND, highlighting results from their recently submitted [paper](#) on our Natural History Study. As most of the Research Network members do not come from a clinical background, this presentation was essential to bring everyone up to speed.
- Dr. Chung led a recorded discussion of KIF1A/KAND treatment strategy and approaches. Many topics were covered in this presentation such as current hypotheses, potential treatment options, and an extensive amount of discussion on gene therapy.

Cat Lutz, PhD/MBA | The Jackson Laboratory (JAX)

- Dr. Lutz gave a presentation on the history of KIF1A mouse models and available resources at JAX. As many of our Research Network members are interested in using KIF1A mouse models, this presentation has already fostered new collaborative conversations about how researchers can work together to answer experimental questions, leveraging JAX resources.

Hans Christinger, MBA | Ovid Therapeutics

- VP of Business Development and Alliance Management at Ovid Therapeutics and KIF1A.ORG board member Hans Christinger ended our session with a discussion on the logistical and business-aspects of therapeutic development. Wearing hats as an Ovid employee and a KIF1A.ORG board member, he was able to communicate a unique perspective about how rare disease organizations and biotech companies can work together by leaning on each other's strengths to march forward towards the clinic.