



# Evaluation of a BDNF-like Molecule as a Potential Therapeutic Agent for KAND

Speaker: Aditi Falnikar, Ph.D., Vallee Lab

August 16, 2019

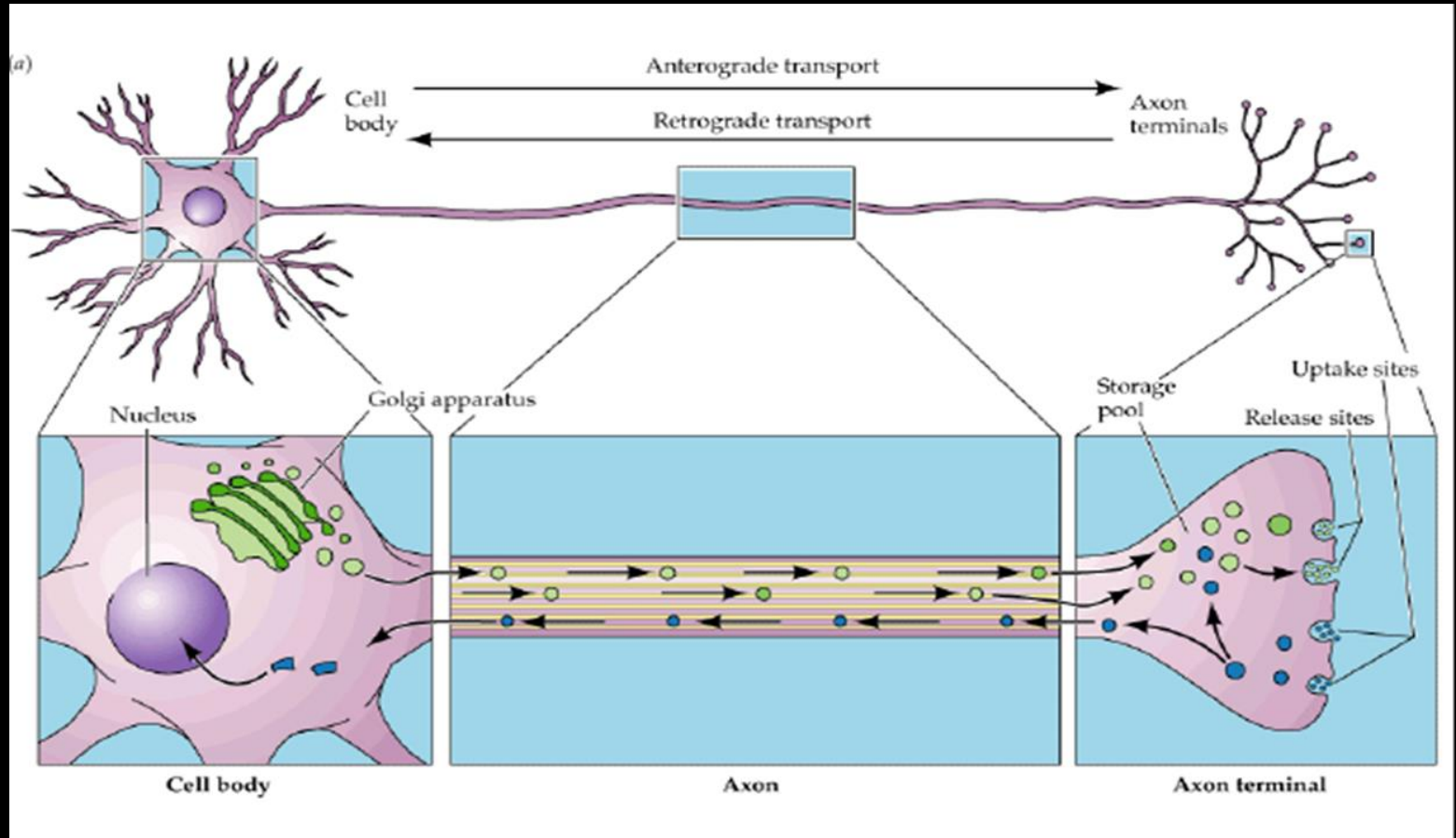
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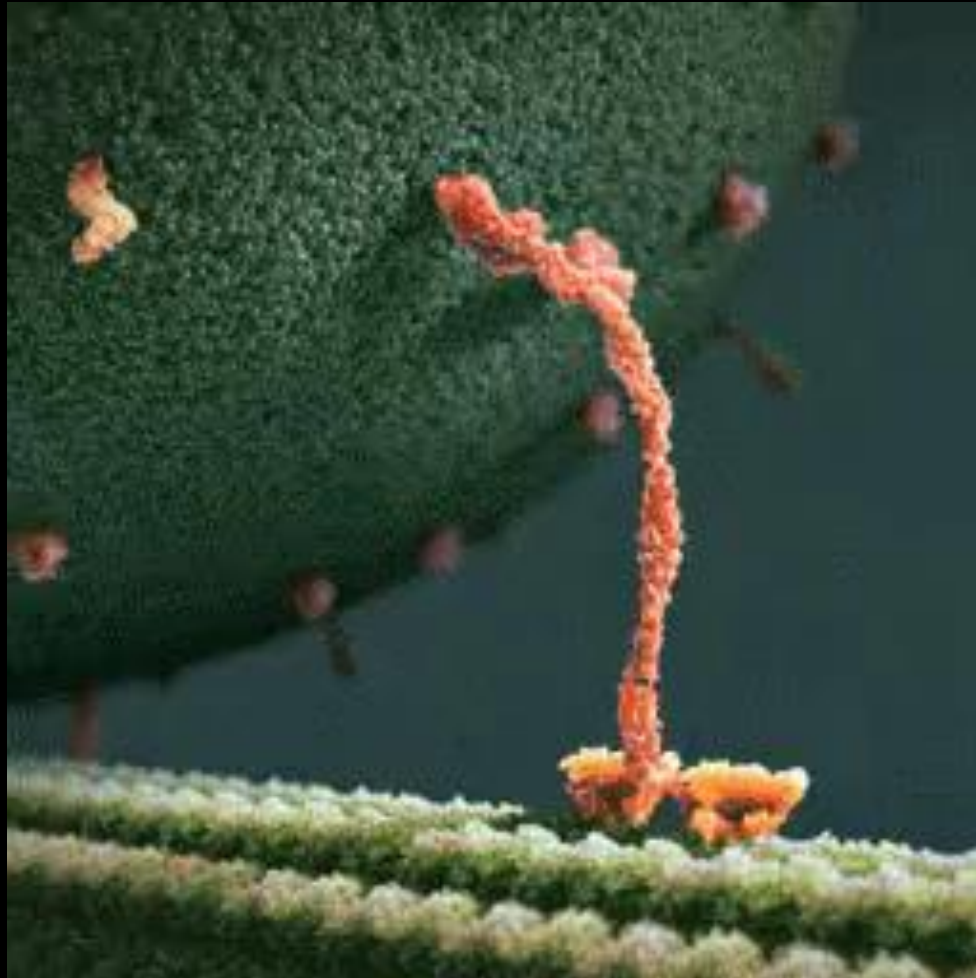
# EVALUATION OF A BDNF-LIKE MOLECULE AS A POTENTIAL THERAPEUTIC AGENT FOR KAND

Aditi Falnikar, Ph.D.

8/16/19

Kif1a is a motor protein that transports essential substances from the nerve cell body into the axon for normal neuronal function.





[Transport inside the brain: The basic mechanisms of neuronal trafficking](#)

# The BDNF Hypothesis

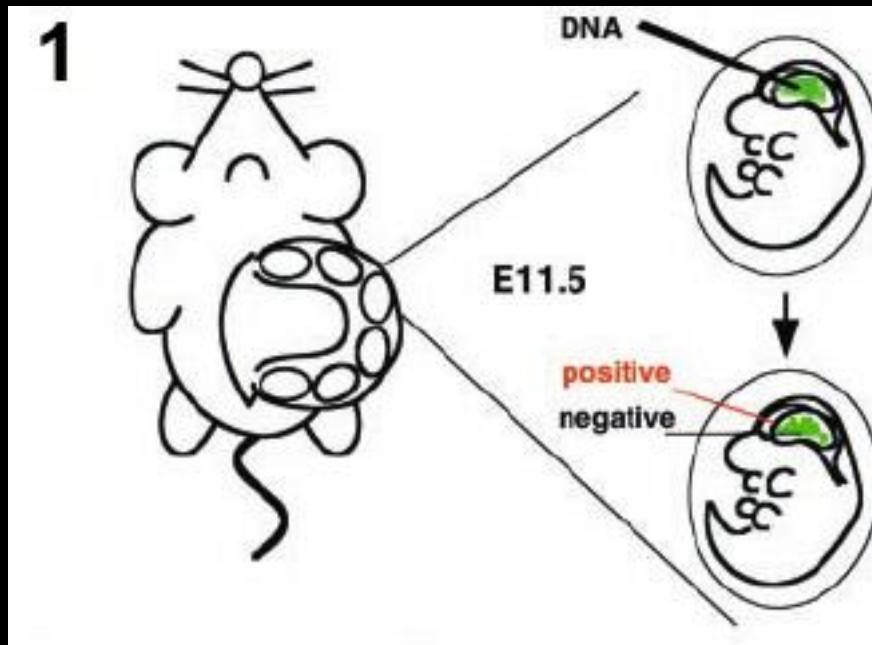
- Kif1a carries large vesicles filled with Brain Derived Neurotrophic Factor (BDNF) from the cell body into the axons.
- BDNF is essential for nervous system development, maturation and function.
- Lack of sufficient amounts of BDNF contributes to KAND.

*Carabalona et al., 2016, Nat.Neuro.*

*Lo et al., 2011, Neurosci. Lett.*

# Analyzing the Brain

In utero electroporation

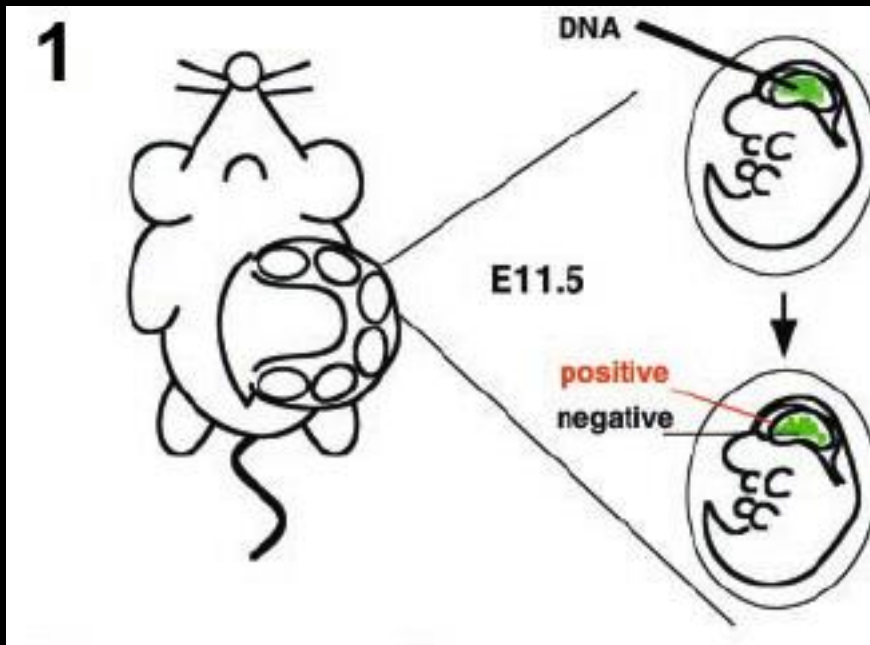


Mutant mouse

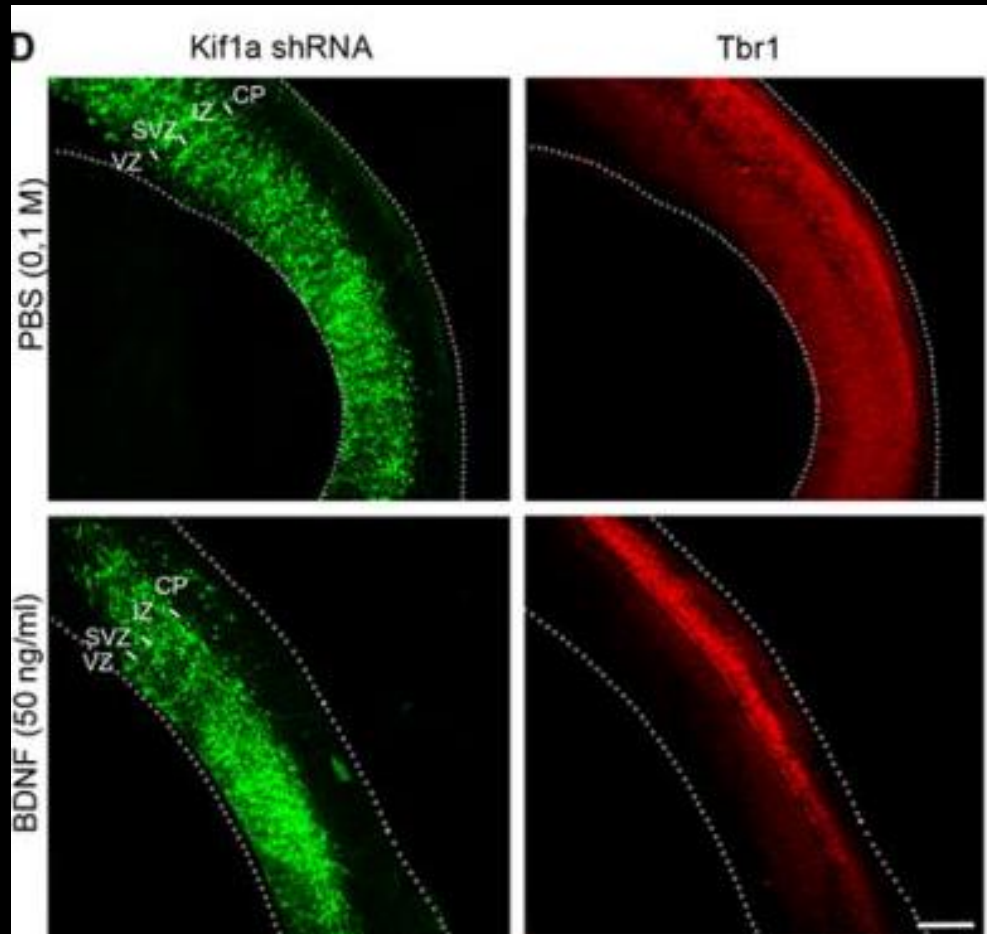


# Analyzing the Brain

In utero electroporation

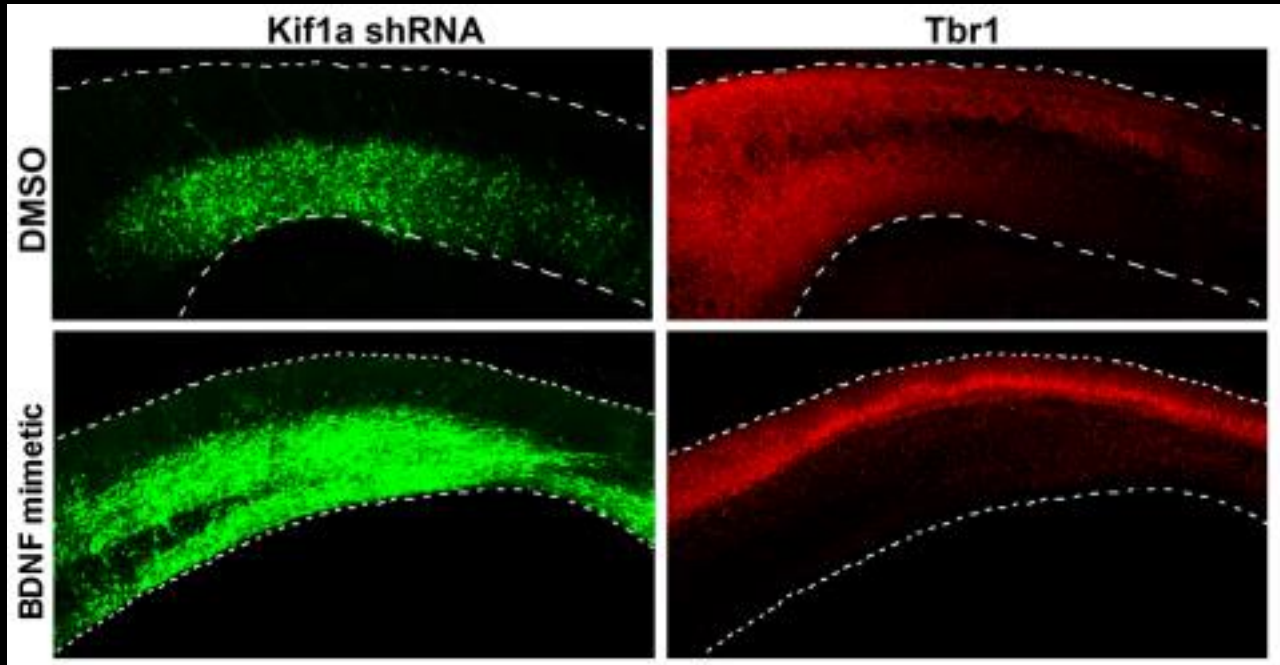


# BDNF Restores Certain Abnormal Features in the Developing Brain





# BDNF-Like Small Molecule Also Restores Certain Abnormal Features in the Developing Brain

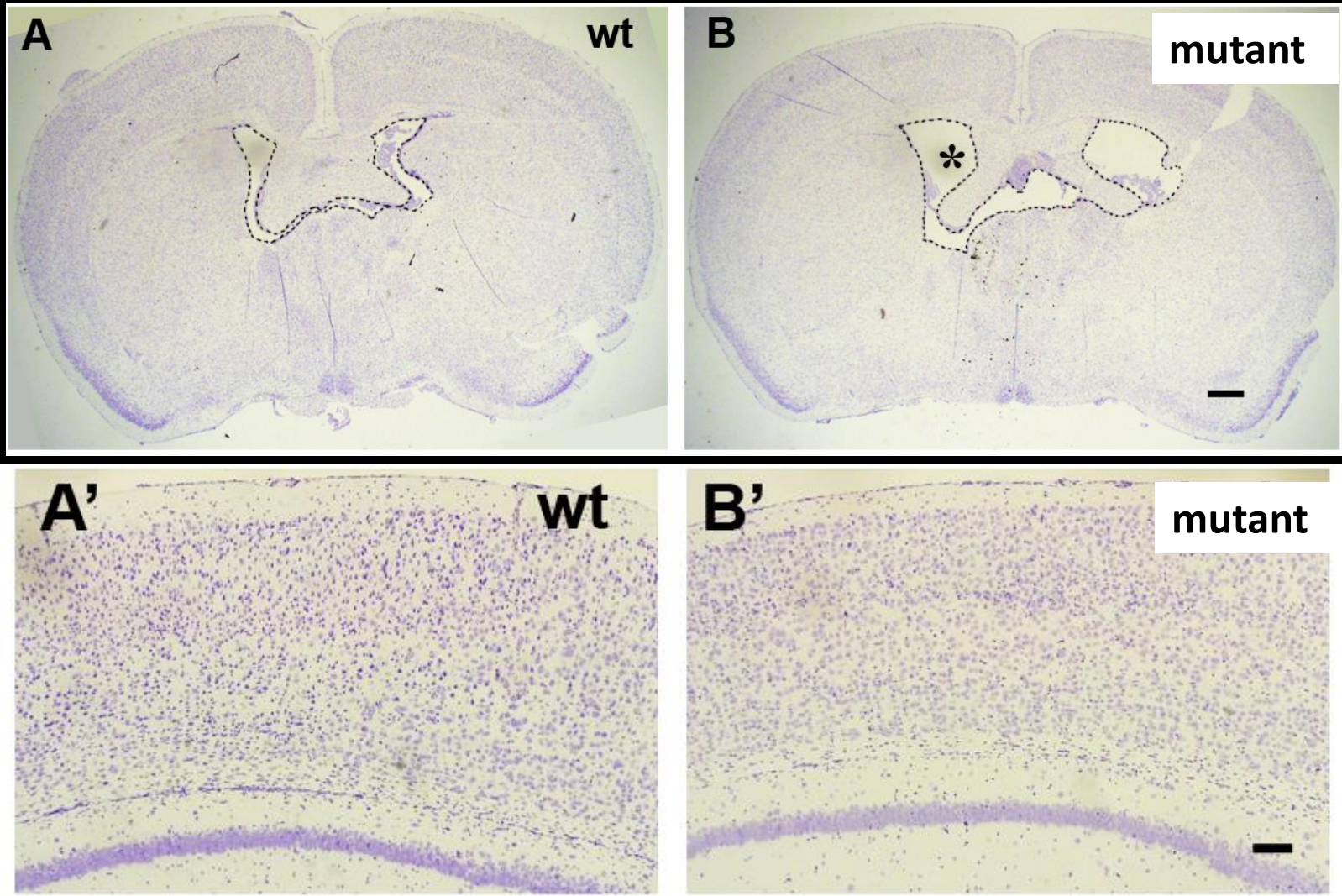


# Analyzing the Brain

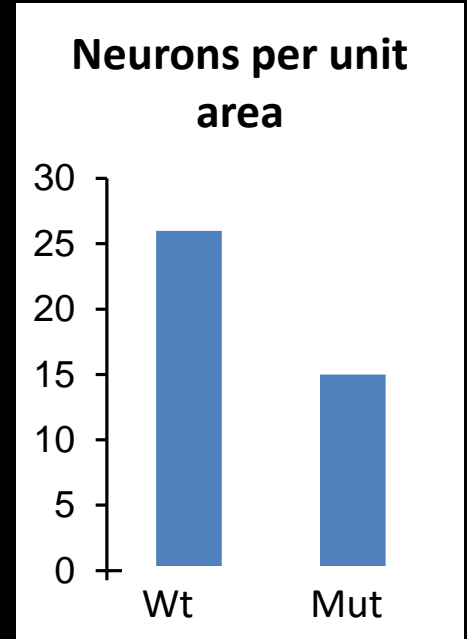
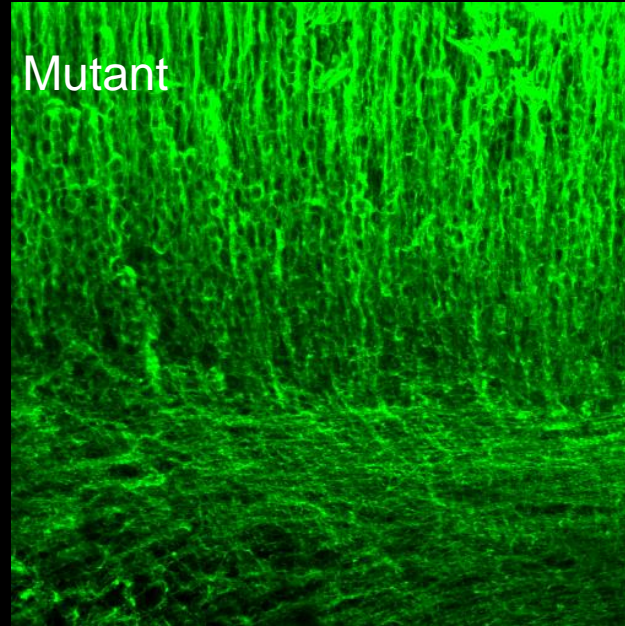
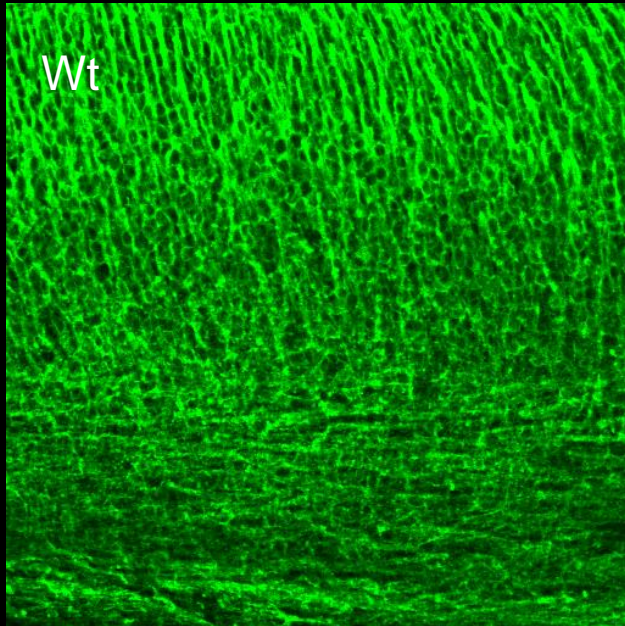
Mutant Mouse



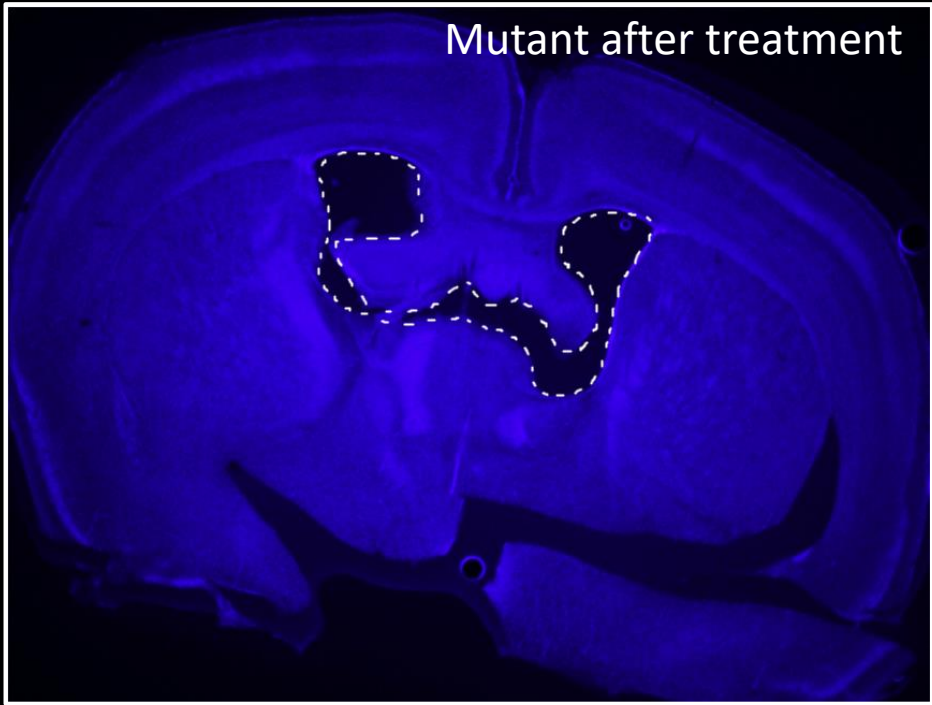
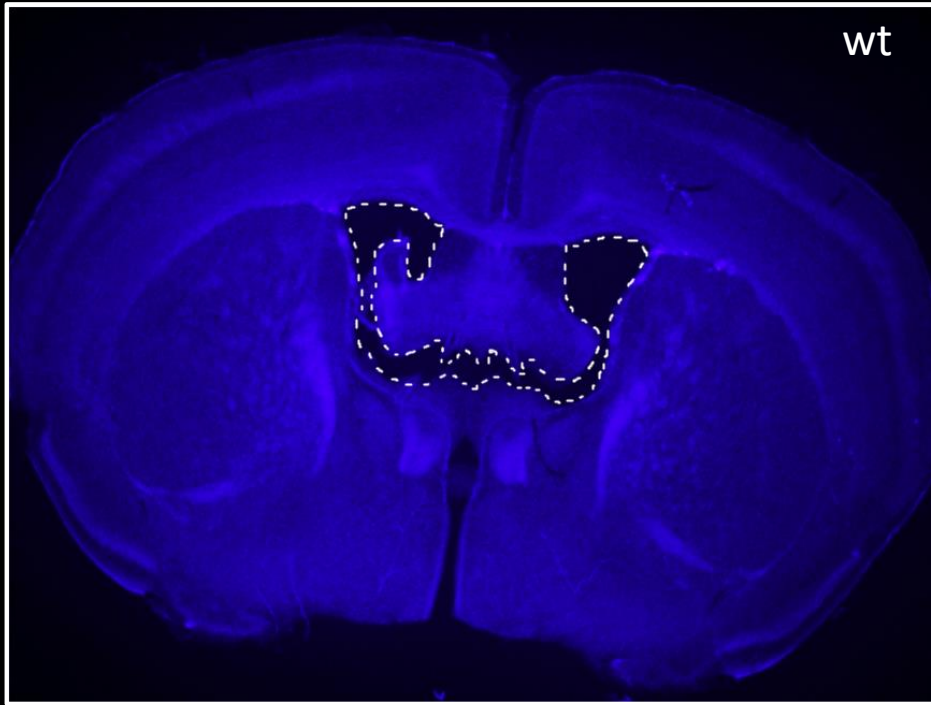
# Mutant Mouse Brain Has Enlarged Fluid-Filled Spaces and Thinner Cortex



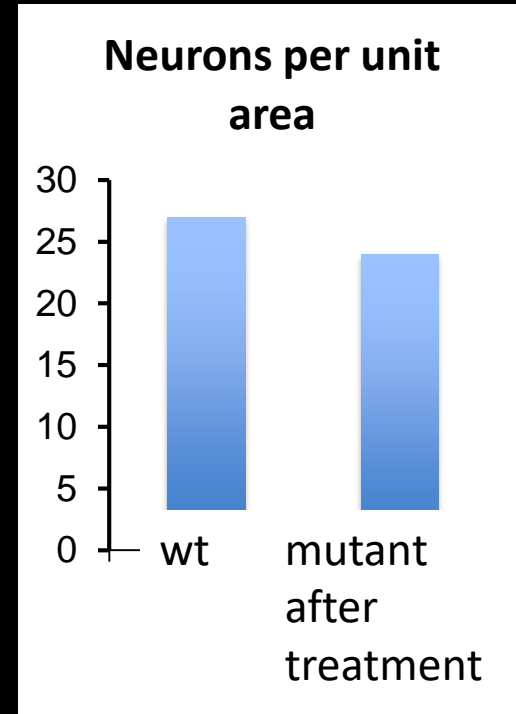
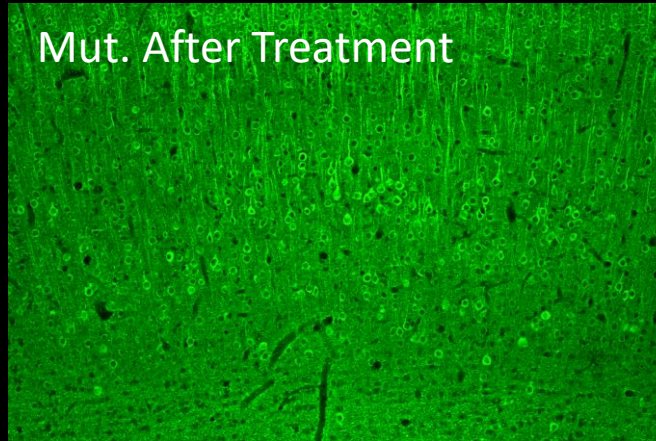
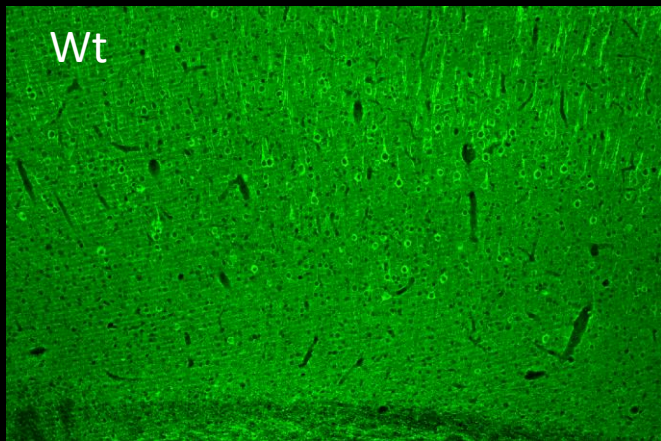
# Staining Shows a Decrease in Neuronal Number



# Treatment With BDNF-Like Molecule Reduces The Ventricular Enlargement



# Staining Shows Normal Neuronal Number



# Conclusion

Further studies are needed to assess the role of a BDNF-like molecule as a treatment for KAND.

Thank you !!!

Dr. Richard Vallee

Dr. Wendy Chung

Vallee lab members

Chung lab members