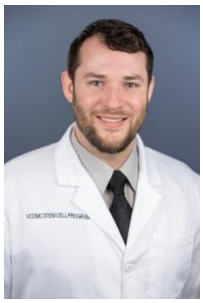
Kyle Fink, PhD, University Of California at Davis



The Research: Gene Therapy/CRISPR

Dr. Fink is working on a new approach in gene therapy using CRISPR/Cas9 technology. His group is developing a method to reactivate the healthy, silenced *MECP2* gene. They want to "turn on" the normal copy of *MECP2* that is on the inactive X chromosome and to be able to control how much *MECP2* is "turned on". They will be creating neurons from cells taken from swabbing patients' cheeks and will use them in this research. Rettsyndrome.org has given his lab a $150,000 award to study this cutting edge technology for the next two years. We are proud to welcome Kyle as a new Principle Investigator for Rettsyndrome.org.

For a more in-depth look at the research visit https://www.rettsyndrome.org/file/18-research-files/In-Depth-Kyle-Fink.pdf

The Hope

Dr. Fink shares, "We are living in time in which addressing underlying genetics may now be achievable. The strides that have been made in gene therapy give hope to not only the patients, but also the researchers. Beyond traditional gene therapy, the discovery of molecules that are capable of changing the way DNA is expressed gives great hope to everyone involved that a useful treatment may be in the near future."

The Answers to Your Questions

Why is this work important to helping my child?

This work holds the potential to gain valuable insight into treating Rett syndrome. Our group is focused on translating the field of genome engineering/gene editing to clinical trials. The field is moving rapidly and we hope that we can assist in keeping therapies for Rett syndrome on the forefront of these discovers.

Is there any way for families to help with your project?

The Rett community has done a wonderful job of creating disease repositories and having the affected children's genome sequenced. These existing databases are publically available and make our research design and methods easier to accomplish. If families are interested in helping to fund the specific work that is ongoing at the UC Davis Institute for Regenerative Cures a link to the giving page is provided. https://give.ucdavis.edu/HDIS

Does the knowledge gained help treat Rett or cure Rett?

The hope would be that this study will help further the treatment and understanding of Rett Syndrome. These studies are still in their infancy and a clinical trial to test a therapeutic of this nature is still in the future.

What is the timeline of your work?

We hope to have established and validated our therapeutic constructs within the first 6-12 months of the award. In the second year of funding we will be utilizing Rett patient iPSC derived cells to test the "molecular rescue" and effect on the cells.

The Researcher

Dr. Fink received his Ph.D. in Neuroscience from Central Michigan University and the University of Nantes. His research team focuses on neurological disorders such as Rett syndrome, CDKL5 deficiency, Angelman's Syndrome, and Huntington's disease that are targetable with gene editing. His lab is identifying common genetic variants and looking for "actionable domains" where gene therapy can be employed.