



Rosenau Family
Research Foundation

November
2025
Newsletter

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Grant Recipient Highlight: Laura Kirkpatrick



Laura Kirkpatrick, MD, has been awarded a \$375,000 RFRF grant for her project, Disclosure of Newborn Screening Results for Krabbe Disease in Pennsylvania, through the University of Pittsburgh.

This project will assess challenges to optimal disclosure of Newborn Screening (NBS) results for Krabbe disease in Pennsylvania. The findings of this study will be impactful for improving communication about Krabbe disease and NBS

results in states that currently include Krabbe Disease on the NBS as well as those that will include Krabbe disease on the NBS in the future.

Dr. Kirkpatrick is an Assistant Professor of Pediatrics and Neurology at the University of Pittsburgh, a pediatric neurologist at UPMC Children's Hospital of Pittsburgh, and the Associate Director of Child Neurology Research for the Division of Child Neurology. Her research focuses on improving communication between child neurologists and families about difficult topics, including diagnosis disclosure and psychosocial or quality-of-life implications of pediatric-onset neurologic conditions.

Click below to watch our introductory interview with Dr. Kirkpatrick, and follow along with her grant journey at rosenaufoundation.org/kirkpatrick-2024.

A video thumbnail with a teal border. On the left, there is a pink and white abstract graphic. The text in the center reads: "AN INTERVIEW WITH" in pink, "Laura Kirkpatrick, MD" in large blue font, a red play button icon, and "Grant Recipient" and "University of Pittsburgh" in blue. At the bottom, there is a logo for the Rosenau Family Research Foundation, which consists of a pink heart with a white outline and the text "Rosenau Family Research Foundation" in blue.

AN INTERVIEW WITH
Laura Kirkpatrick, MD
Grant Recipient
University of Pittsburgh
Rosenau Family
Research Foundation



Grant Recipient Highlight: Daesung Shin



Daesung Shin, PhD, has been awarded a \$250,000 grant for his project, Modulating NF- κ B as a Therapeutic Strategy for Krabbe Disease, which aims to investigate the role of hyperactive NF- κ B signaling in Krabbe disease and its impact on inflammation. This research will enhance the understanding and aid in developing new therapeutic strategies.

Read on for our introductory interview with Dr. Shin, and learn more about his research at rosenaufoundation.org/shin-2024.

What is a brief summary of the project you'll be working on with your grant funding?

Oligodendrocytes are special cells in the brain that are mainly affected in Krabbe disease, a serious genetic disorder that damages the nervous system. Krabbe disease usually starts in babies and often causes death by around age two. Right now, the only treatment is a stem cell transplant, which can only slow the disease down a bit, but there is no cure yet. In our study, we want to see if blocking a protein called NF κ B in oligodendrocytes can help reduce the damage from Krabbe disease. This is important because activating NF κ B in other brain cells, like astrocytes and microglia, is already known to be harmful. If NF κ B in oligodendrocytes also makes things worse, then stopping it could be a new and better treatment.

How did you hear about Rosenau Family Research Foundation's grant program?

I became acquainted with the Rosenau Family Research Foundation when I started working on Krabbe disease at the Hunter James Kelly Research Institute in Buffalo, New York. I have heard much about the Foundation's valuable support for research and the many success stories, which I greatly appreciate.

How did you get into rare disease research/Krabbe specifically?

I was trained for many years in neuroscience, with a specialization in genetic brain disorders, particularly myelin disorders. After completing my postdoctoral training, I had the opportunity to join the Hunter James Kelly Research Institute. I saw it as a great chance to apply my expertise to help individuals and families affected by these conditions. Since then, I have continued my research on Krabbe disease.

What about your research gives you hope?

We are getting closer to understanding how Krabbe disease progresses, which will enable us to develop effective strategies to slow down or halt its progression.

What are the best/most challenging parts of your job?

First, always strive to understand the disease mechanism, a challenge many have attempted but have yet to fully overcome. Second, funding for rare diseases is quite limited. Support from foundations like Rosenau Family is truly helpful in enabling continued progress toward better diagnosis and potential therapies for affected individuals.

What advice would you give to students pursuing the field of rare disease research?

Although rare, diseases like Krabbe share many aspects of pathogenic mechanisms with other conditions, making them valuable models for understanding common diseases. Every individual deserves access to potential cures, regardless of how common or rare their disease is.

What is one thing you wish you would have known earlier in your career?

I wish I had realized that good questioning is the key to better science and benefiting more people.



Board Hellos

Rosenau Family Research Foundation is excited to announce that it has added two new Directors to its board: Zac Leeker and Greg Potter!



Zac's first experience with Krabbe Disease was in 2000 when his brother, Trevor, was diagnosed at 10 months of age with early infantile Krabbe Disease. Though he was only 6 years old when his brother passed, an advocacy journey was sparked. For the better part of the next 25 years, annual family vacations were spent at medical symposiums learning more about Krabbe Disease, summers were spent hosting "Trevor's Tournament", a charity softball tournament raising money for scholarships for those going into the medical field to raise awareness for Krabbe Disease, and any free other free time was mostly spent advocating with state representatives on expanding newborn screening.

Zac's educational background includes a Bachelor's degree in Kinesiology and Master's Degree in Healthcare Administration. After years of service on the Kansas Newborn Screening Advisory Council and a short stint in local public health, Zac took over as the program manager for newborn screening in his home state of Kansas. 3 years later, Zac was presented with an offer to transition as program manager for newborn screening for the state of Iowa and help lead a quad-state collective of screening programs including North Dakota, South Dakota, and Alaska.

Greg is currently Associate Director of Molecular Biology at Precigen Therapeutics, where he leads research programs developing next-generation gene and cell therapies. Previously, he was a Scientist at Denali Therapeutics, where he helped advance a small molecule therapeutic program from the lab bench into clinical trials. Over the past 25 years, he has built a career that bridges academia and industry, with expertise spanning neuroscience, immunotherapy, biologics, and drug development. Greg received his Ph.D. in Neuroscience from Johns Hopkins University School of Medicine.

During his postdoctoral work at UCSF, Greg discovered the underlying mutation in a novel Krabbe disease mouse model. He and his wife later established a small research group at OHSU in Portland, Oregon, where they continued to investigate the molecular mechanisms and disease processes of Krabbe disease using this model. As part of this work, Greg's group received a research grant from the Legacy of Angels Foundation (now RFRF), funding that was instrumental in supporting their lab. He remains grateful for that support and is honored to return to the Foundation as a Board member. Greg brings a combined scientific and industry perspective to support strategic planning and to help accelerate therapies for rare diseases.



Board Goodbyes

Rosenau Family Research Foundation would like to extend its heartfelt thanks and gratitude to two departing members of its board: Karlita Black and Phil Christianson.



Karlita served as a Board Director from 2022-2024. As the mother of a child with a rare disease, her extensive experience with patient advocacy was invaluable in guiding the work of the board and the direction of the foundation, especially in the area of Newborn Screening. We are grateful for her time and knowledge on both a personal and professional level.

Wishing you all the best in your future endeavors, Karlita!

Phil served as both a Board Director and Chair-Elect in his four years with RFRF. His substantial knowledge of the health care industry and experience with the improvement of mid-sized companies and organizations contributed greatly to RFRF's continuing success in growing our grant program into what it is today.

Thank you, Phil; it has been a true privilege to work alongside you!



Serve on Our Board of Directors

Rosenau Family Research Foundation is seeking candidates to fill additional roles on our Board of Directors.

We are looking for a wide range of expertise to complement the knowledge of our current Board. Our Board members play a crucial role in the functioning of our organization, and ensuring that we are serving our grantees and the rare disease community in the most efficient, empowered way possible.

Interest in the
Board of Directors

Serve on Our Investment Committee

Rosenau Family Research Foundation is looking for individuals for our Investment Committee who are:

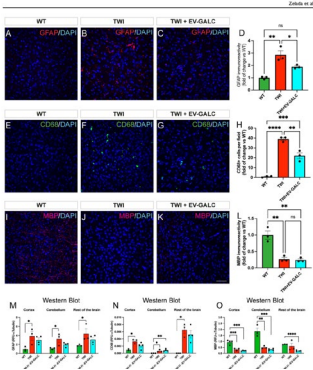
- Committed to RFRF's mission
- Thoughtful in their approach to working with others and challenging the foundation
- Interactive and engaged
- Inquisitive & personable
- Experienced in relevant fields such as investment, accounting, banking, financial planning, corporate finance, or non-profit financial sector

Click below to get in touch about joining the Investment Committee, or email info@rosenaufoundation.org.

Investment Committee
Interest

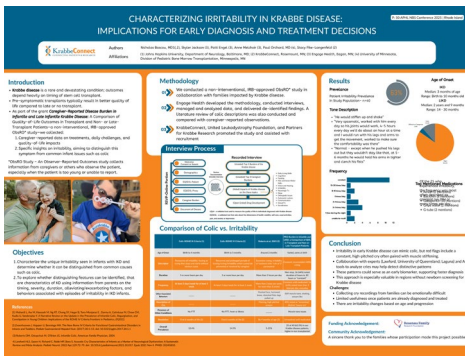


Grant Research Findings



Diego Zelada, Natalia Saldivia and Maria Irene Givogri, PhD, with others, have published a manuscript in the International Journal of Nanomedicine with findings related to an RFRF grant awarded in 2020 titled “Vesicular Delivery of GALC for ERT of Krabbe’s Disease”.

Read the
Manuscript



KrabbeConnect presented a poster at the APHL Newborn Screening Conference in Rhode Island in early October on findings from research made possible by their RFRF grant, “Caregiver-Reported Disease Burden in Infantile and Late Infantile Krabbe Disease: A Comparison of Quality-of-Life Outcomes in Transplant and Non- or Late-Transplant Patients.”

See the
Poster

Save the Date for KTRN 2026!

KTRN 2026 will take place March 18-20 at the Hilton Minneapolis/Bloomington!



Registration will open in January.
More details, including registration and sponsorship information, to come!

Call for Abstracts

We are now accepting abstracts for consideration to present at the KTRN. Please fill out the form by clicking the button below and email it to ktrn@rosenaufoundation.org.

Download the
Abstract Form

All abstracts are due by Wednesday, 12/31.



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