Rosenau Family
Research Foundation

2023 Annual Report

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A Note from Co-Founder Paul Rosenau

I've had time to do some reflecting on the past, and I'm amazed at all that has taken place in the Krabbe disease space. When The Legacy of Angels Foundation (TLOAF), now Rosenau Family Research Foundation (RFRF), was founded, there was no organized effort to move forward with research. It was hard for new families that came into the disease space to find relevant information and support to help them manage their children's disease. I remember how it felt when Makayla came into our family; like we were out in the middle of the ocean on a small rubber raft with no one in sight to help.

Today, our organization and the many investigators we are partnered with are striving to change the future. We could rewrite the future. It will take a lot of work, but it is starting to make a noticeable difference. We are grateful for the hard work and continued collaborations with our partner organizations: KrabbeConnect, Hunter's Hope, Cystic Fibrosis Foundation, and United Leukodystrophy Foundation.

Today, RFRF is working with researchers, universities, colleges, and anyone who is interested in finding the answers we need to combat this hideous disease that turns families upside down. There is an ongoing effort to bring new researchers into the field and expand the knowledge base. We have new treatments that are being tested to see both how they work and if there are improvements that can be made. A big thank you to Gabriel Cohn, his staff and the RFRF Board for all of their effort in the research area.

We are moving forward slowly. Each time someone in the community becomes involved and invests their time, talents, knowledge, financial support, or whatever they can offer we move one step closer to the answer we are all looking for.

To all those who are or have been involved in this process, I want to say THANK YOU for all you have done.

Best regards,
Paul

From the Desk of the Executive Director

Gabriel Cohn, MBA
Executive Director

When I joined TLOAF at the end of 2022, it was with great excitement and enthusiasm. TLOAF had been instrumental in supporting some of the most innovative developments in Krabbe disease and Cystic Fibrosis, including:

- support for basic science research which has led to a deeper understanding of Krabbe disease;
- support for translational research which has led to investigational products currently under preclinical and clinical evaluation as potential new therapies;
- support for Krabbe disease diagnostic and screening technologies which has led to a refinement in the assessment of risk related to positive newborn screens;
- sponsorship for the annual KTRN meeting which has fostered invaluable exchange and collaboration among the nation's leading Krabbe disease researchers and experts;
- support of basic research which has led to a better understanding of Cystic Fibrosis;
- and indefatigable advocacy.

As the Foundation's new Executive Director my role is to serve as a steward of this wonderful Foundation and help shepherd it to new heights while remaining firmly anchored in and committed to the core values and principles that have been fundamental in driving the Foundation's success and accomplishments. Toward that end, 2023 was a year of great transition; one that saw a nearly, entirely new staff lead this organization.

With the unwavering support of Paul and the Rosenau family, as well as the Foundation's Board, we have successfully transitioned TLOAF to Rosenau Family Research Foundation (RFRF) and re-dedicated our focus and efforts to support work that improves the lives of patients impacted by Krabbe disease and Cystic Fibrosis through research funding and disease advocacy. Our newly formed marketing and communication team's re-branding and social media campaigns have helped increase awareness of our Foundation's mission, work, and accomplishments. The KTRN steering committee was invaluable in identifying speakers and topics for the 2023 KTRN meeting that highlighted new learnings in Krabbe disease, including the emerging knowledge of the role of immunopathology, the overlap this has with other more common neurological disorders, the potential and limitations of new therapeutic modalities, and the benefits and challenges in newborn screening. RFRF's Scientific Advisory Committee identified and recommended support of some of the most robust and innovative grant applications to date, including support of grants for new investigators who will help expand the field of scientists and physicians dedicated to the study of Krabbe disease and Cystic Fibrosis.

Finally, RFRF has also partnered with other advocacy groups and organizations such as Hunter's Hope and Krabbe-Connect to continue to advocate for universal Krabbe disease newborn screening. All this would not have been possible without the guidance of the Foundation's Board and the tireless work of the Foundation's Investment Committee, financial advisors, and accountants who have helped navigate our finances and budget through turbulent financial times and markets and helped assure the financial health necessary for the continued support of the Foundation's mission. I wish to thank all those who have helped support RFRF in 2023 and all the scientists, physicians, patients, families, and advocacy groups who have worked tirelessly to improve the lives of those impacted by Krabbe disease and CF. During this year of transition, we have successfully reinforced the foundations of this great organization and are well positioned to move forward, along with our scientific, medical, and advocacy partners, in supporting individuals to live life undefined by Krabbe disease and Cystic Fibrosis.

Our Vision

Living a life undefined by Krabbe disease and Cystic Fibrosis

Our Mission

Improving the lives of people impacted by Krabbe disease and Cystic Fibrosis through research funding and disease advocacy

Our Values

Collaboration
Ethical Decision-Making
Science-Driven

2023 TEAM

Staff

Gabriel M Cohn, MD, MBA
Executive Director & Medical Director

Heather Techmeier
Finance Director

Contractors

Renea Muellerleile
Fractional Operations Leader, Hood & Associates

Mary Kay Delvo
Strategist, Certified Coach & Facilitator, INspiring SIGHT

Heidi Carrozzella
Fractional Marketing Leader, Zella Marketing

Rachel Jackson
Fractional Marketing Director

Board

Paul Rosenau
Chair

Gillian Hauboldt
Treasurer

Gabriel M Cohn, MD, MBA
Ex-Officio

Phil Christianson
Director

Karlita Blackwell
Director

Julie McCarrier
Director

Li Ou
Director

Marci Sontag
Director

Heather Techmeier
Director

With Support From: BELIN MCCORMICK ATTORNEYS AT LAW · Dufault CPA CERTIFIED PUBLIC ACCOUNTANT · FOSTER GROUP

Board Members Keep the Foundation Relevant

Mary Kay Delvo
Strategist, Certified Coach & Facilitator, INspiring SIGHT

As RFRF continues to mature, I am happy to report its leadership has a full appreciation for how important board strategy and on-boarding are to developing a board that takes seriously its legal duties of obedience, loyalty, and care. By law, non-profit organizations need a Board of Directors. But why are they so important?

Board members are the tentacles that extend throughout the community and keep the Foundation relevant. We count on their voices to ensure well-rounded strategic discussions take place. We count on them to be inquisitive and ask questions from a variety of perspectives. We remain proactive when board members bring fresh eyes to the Foundation for planning into the future. We expand our reach and opportunities when board members take seriously their role as ambassadors of the Foundation in their existing networks.

As the Foundation continues to mature and put processes and operational foundations in place, the stage has been set for prioritizing a strategy for creating a robust board of directors. The 2024 board development priorities include:

1) adding two to four new board members
2) filling the board's leadership positions of chair, incoming chair, and secretary
3) providing board coaching to ensure new and existing members are equipped to succeed in their board roles
4) creating a robust on-boarding and training program to ensure the board is fully supported in serving the Foundation

Rosenau Family Research Foundation is committed to its mission and the community it serves. If you or someone you know is too, we would love to talk with you.

The Rosenau Family Foundation Board of Directors meets virtually, five times, and in-person, one weekend a year. Contact Mary Kay Delvo, marykaydelvo@rosenaufoundation.org if you know of someone who may be interested in serving the rare disease community in this way.

RFRF IS LOOKING FOR PEOPLE WHO ARE:

- Inquisitive & personable
- Thoughtful in their approach to working with others and challenging the foundation
- Committed to RFRF's mission
- Interactive and engaged
- Experienced in or have knowledge of bio ethics, non-profit or medical law, the rare disease space, philanthropy, pharmaceuticals, non-profits supporting families of those affected by rare diseases, advocacy, healthcare policy, clinical research bench science, newborn screening, and other areas relevant to the foundation's mission

A Thoughtful Planning Approach

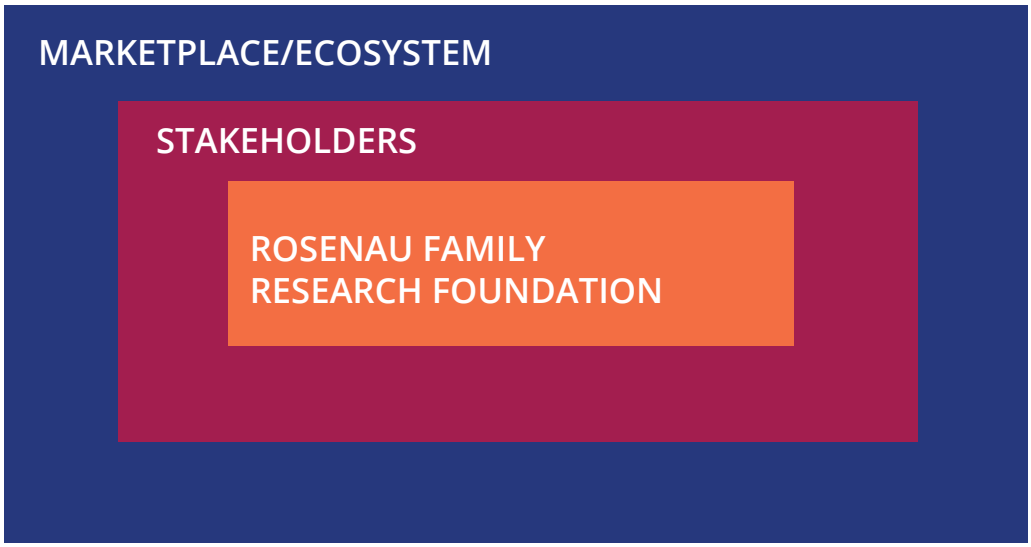
In February 2023, the TLOAF team set out to create a strategic marketing and communications plan to further their mission of improving the lives of people impacted by Krabbe disease and Cystic Fibrosis through research funding and disease advocacy.

Our process was first to review the recently completed strategic plan to ensure we were clear on all marketing related outcomes that were identified by the broader team.

From there, the team completed a series of conversations focused on the marketplace/ecosystem, stakeholder groups and finally Rosenau Family Research Foundation.

Heidi Carrozzella
Fractional Marketing Leader, Zella Marketing

Market Playbook Process



The key to this format is starting with discussions around the big picture environment in which we exist. It was important to understand the context of the current environment and the needs of our stakeholders before we started creating a laundry list of marketing tactics that should be executed. This format supported an ongoing dialogue with internal stakeholders to ensure we were getting various points of view, and the team was in alignment as we worked through the process.

During the stakeholder sections, we interviewed twelve individuals from different external stakeholder groups to ensure that we were listening to their needs and ideas, and to ensure that our findings were aligned with one another.

Through the process, it became clear we had a common goal of growing and expanding our network to recruit more investigators to ensure the legacy that the Rosenau family started continues to live strong. This includes programs to engage new investigators to work with us.

In 2023, the team laid the foundation for embarking on this big goal by transitioning from The Legacy of Angels Foundation to Rosenau Family Research Foundation. A new website was created and new communications tools were set up including a quarterly newsletter featuring research and news about our network. And this is just the beginning! We look forward to highlighting more of your stories in ways that will help our network recruit the next generation of investigators.

From the Desk of the Finance Director

Heather Techmeier
Finance Director

In 2023 the first quarter started strong, and there was hope that the market was on a robust path to recovery from 2022. However, most of those gains were washed away in the second and third quarters. The financial markets rallied in the fourth quarter, resulting in a strong year overall. While everything was positive, the degree of positivity varied. Large growth US stocks was up over 40% last year, which was remarkable. Most of the large growth was driven by the Magnificent Seven (i.e. Alphabet, Amazon, Apple Inc., Meta, Microsoft, Nvidia, and Tesla), which overshadowed the fact that everything else did well by historical standards. International markets performed incredibly well, which was nice to see because they had been lagging for a while, and bond markets started to perform better. There was a lot of news on the investment front, including activity within capital markets, inflation, and federal fund rates.

> ***"Forecasts may tell you a great deal about the forecaster; they tell you nothing about the future."***
>
> - Warren Buffett

What's interesting looking back at 2023 is that many experts predicted a recession. According to an article in the Financial Times, 85% of economists who were polled predicted that we would experience a recession at some point in 2023[1]. However, as we know, a recession did not occur. Additionally, those same economists predicted that the unemployment rate would go north of 5.5% at some point in 2023, but the unemployment rate remained around historic lows of 3.5%. The moral of these stories is that it is difficult to predict what will happen in the market. While it may be tempting to act on news or market volatility, it is best to ignore the noise. There will always be headline news, but those who are able to block out the noise tend to have better investment outcomes. An approach the Foundation has embraced over the years. Despite all the ups and downs, headlines, predictions, etc., the Foundation's portfolio with the Foster Group earned a 12.16% return in 2023, with 8.47% of that return coming in the fourth quarter.

Rosenau Family Research Foundation Balance Sheet

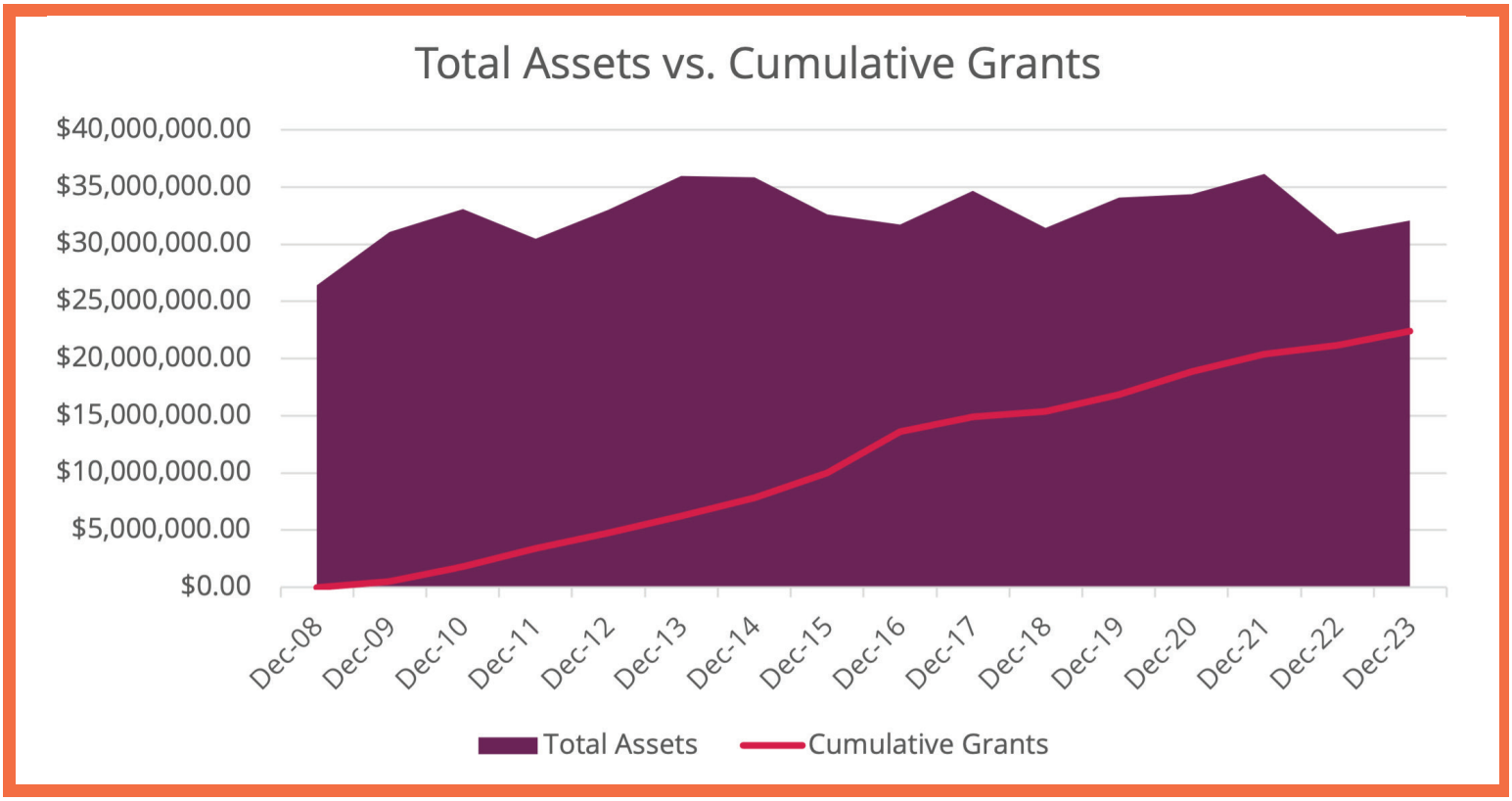
As of December 31, 2023

ASSETS	TOTAL
Current Assets	**$93,583.54**
Other Assets	
Life Insurance	**2,126,629.00**
Pershing Advisor Solutions LLC	29,867,814.09
Total Other Assets	**$31,994,443.09**
TOTAL ASSETS	**$32,088,026.63**
LIABILITIES AND EQUITY	
Liabilities	
Current Liabilities	**$505.22**
Total Liabilities	**$505.22**
Equity	
Retained Earnings Net	30,893,001.53
Income	1,194,519.88
Total Equity	**$32,087,521.41**
TOTAL LIABILITIES AND EQUITY	**$32,088,026.63**

1. Gilbert, Caitlin and Smith, Cody, "US unemployment rate set to surpass 5.5%, economists predict", December 6, 2022, Financial Times, https://www.ft.com/content/c2d4d4b5-cbc8-4b5c-9ea3-44e9742d5b3a

From the Desk of the Finance Director, cont.

I am also pleased to report that it has been a year of great transition for the Foundation. As Dr. Gabriel M. Cohn discussed in the Executive Director report, we have created more governance and structure within the Foundation's financial structure. We developed an Investment Committee to ensure prudent processes are in place and followed for making decisions surrounding our investments. The Investment Committee developed an Investment Policy Statement (IPS) to provide safeguards, establish protocols for managing risk, and ensure that investments are consistent with the organization's risk tolerance. It was through these channels the Board of Directors made slight adjustments to the portfolio to better align with the stated objectives in the IPS. These adjustments, while minor, were implemented because of the additional governance and structure that was put in place this year.



Since joining forces with the Foster Group in 2017, the Foundation has never wavered from the importance of time in the market versus trying to time the market. It has found an investment approach that they are comfortable with and can be used to navigate both good times and bad. The Foundation prides itself on being a disciplined investor, embracing uncertainty while looking beyond the concerns of today to the long-term growth potential of markets. A philosophy the Foundation believes will continue to pay dividends.

New Grants Funded in 2023

EFFECTS OF MOTOR LEARNING ON EFFICACY AND SAFETY OF AAV-GENE THERAPY IN KRABBE DISEASE

New Investigator

Natalia Saldivia Soto, PhD — University of Illinois, Chicago

DYSFUNCTION OF BRAIN SYNAPSIS IN KRABBE DISEASE

New Investigator

Diego Zelada, PhD — University of Illinois, Chicago

ELUCIDATING THE MECHANISMS UNDERLYING MACROPHAGE DYSFUNCTION IN KRABBE DISEASE

New Investigator

Sai (Venkata) Chaluvadi — University of Pennsylvania

A MALADAPTIVE IL-1 F3-ACID CERAMIDASE-INFLAMMATORY PATHWAY INVOLVED IN EARLY PATHOGENESIS OF KRABBE DISEASE

Maria I. Givogri, PhD — University of Illinois, Chicago

IMPROVING DEVELOPMENTAL OUTCOMES IN MOUSE MODELS OF KRABBE DISEASE

Steven LeVine, PhD — University of Kansas

PARENT-REPORT DISEASE BURDEN IN INFANTILE AND LATE INFANTILE KRABBE DISEASE; A COMPARISON OF QUALITY-OF-LIFE OUTCOMES IN TRANSPLANT AND NON-OR-LATE TRANSPLANT PATIENTS

Nicholas Bascou, MD, Stacy Pike-Langenfeld — KrabbeConnect

CHALLENGING THE CAPACITY OF AAV-GALC GENE THERAPY TO COPE WITH MODIFIERS OF ADULT BRAIN FUNCTION

Ernesto Bongarzone, PhD — University of Illinois, Chicago

IMMUNE RESPONSES IN PERIPHERAL NERVES AFTER HEMATOPOIETIC STEM CELL TRANSPLANTS FOR KRABBE

Anthony Filiano, PhD — Duke University

CLICK HERE TO LEARN MORE ABOUT THE RFRF GRANT PROGRAM

Caregiver and Clinician AwaREness for early Cystic Fibrosis diagnosis (C-CARE CF)

Research Spotlight

Cystic Fibrosis

"We are lucky to be able to utilize Rosenau Family Research Foundation dollars in conjunction with a grant for chronic disease education and awareness from the Centers for Disease Control. Between the two, we are able to increase the scope of our projects and, hopefully, the impact of our work on both patients and the physicians caring for them."

Susanna McColley, MD

About the Study

In 2023, Dr. Susanna McColley and her team at Ann & Robert H. Lurie Children's Hospital of Chicago began a two-year study to better understand delays in diagnosis and treatment of Cystic Fibrosis (CF). Additionally, the RFRF grant supports educational efforts to ensure higher knowledge and awareness in both the community and primary care doctors.

2023 Progress

Over 1,600 parents with children 13 years old or younger were surveyed about their knowledge of newborn screening and CF. Results showed that a large proportion of parents did not know about newborn screening, even though they had children born in the United States. Along demographic lines, many fewer Black and Hispanic parents than White parents had heard of CF.

Additionally, results showed that it took longer than guidelines recommend for patients with positive results to be seen by primary care physicians, and to consequently start receiving treatment.

See page 13 for a selection of data from the survey.

Looking Forward

The next step from this study is to create focus groups and a public awareness campaign through Lurie Children's Hospital, aimed at the general public, particularly underserved populations. This campaign will disseminate information about CF and newborn screenings. The hope is that physician practices will be positively impacted as awareness grows.

Caregiver and Clinician AwaREness for early Cystic Fibrosis diagnosis (C-CARE CF)

DATA

Results from a survey of 1,600 parents with children 13 or younger, regarding their knowledge of newborn screening and Cystic Fibrosis (CF):

- 79% of parents from the general population knew what CF is
- There were differences by race and ethnicity in this category:
  - White, non-Hispanic: 87%
  - Hispanic: 66%
  - Black: 68%
  - Asian: 63%
- 48% knew that CF is a disorder that can be detected with newborn screening
- 51% said that they knew what tests are included in newborn screening
- 58% knew that a newborn screening test can raise concern for a disease when the child does not have the disease (false positive)
- 54% knew that there can be a normal result when the baby actually has the disease
- 25% of parents did not remember their youngest child having a newborn screening

Results from a survey of 380 parents with children who had been diagnosed with CF, regarding their experience with the diagnosis and treatment process:

- The number of parents whose child was seen for a test or a clinical evaluation:
  - 21% were seen within the first three days after notification
  - 38% were seen between eight and 14 days
  - 6% were seen after 15 days or more
- 35% of parents thought it took too long to get information about next steps and treatment
- About a quarter of people also reported that after their initial visit, it took more than two weeks to get a final diagnosis, and thus, start treatment
- 30% of parents did not agree or strongly agree that getting a diagnosis was smooth
- 26% of people thought that their child's diagnosis might be delayed based on their race or ethnicity

Research Spotlight

Krabbe Disease

Evaluation of Immunomodulatory Therapy for the Treatment of Krabbe Disease in a Mouse Model

About the Study

Dr. Stephen Crocker and his team at the UCONN School of Medicine began their first year of a three-year Rosenau Family Research Foundation grant in 2023. The purpose of their research is to identify whether medication used for multiple sclerosis can be repurposed to treat Krabbe disease. The medication in question blocks T Cells, which have been proven to cause damage to the brain in both multiple sclerosis and Krabbe disease. Additionally, investigators will be studying how these types of treatments affect leg muscles and use electrophysiological measurement to determine the ability of mouse muscles to twitch, and the strength of those muscles within the context of the above treatment paradigm.

2023 Progress

Investigators have tested a medication named Natalizumab, which binds to the T Cells and blocks their ability to attach to blood vessels, which is necessary for them to enter the brain. Testing was first done at low concentrations, once a week, and continuing on to increase both the dosage and frequency of application. Preliminary results have shown that the medication is having the intended effect, though more testing needs to be done.

Looking Forward

An important component of this research is exploring the potential connection between Krabbe disease and multiple sclerosis, Alzheimer's disease, Parkinson's disease, and more. Discerning the role T Cells play in these diseases, and how certain treatments might be used across a wide variety of conditions, could have implications that reach much farther than only Krabbe disease and other leukodystrophies.

"The grant funding we have received from Rosenau Family Research Foundation for specific projects within our study dovetails nicely with other grants we have received, including funding from the National Institutes of Health, and they will complement and support each other as we further our research."

Stephen Crocker, PhD

Awards & Remembrances

2023 Sue Rosenau Legacy Award

Maria I. Givogri, PhD and Ernesto Bongarzone, PhD

Dr. Ernesto Bongarzone, PhD was named the recipient of the 2023 Sue Rosenau Legacy Award.

Dr. Bongarzone obtained his PhD in Buenos Aires, Argentina and is Professor in Neuroscience at the University of Illinois at Chicago. His studies focus on the pathogenic mechanisms of Krabbe disease, and the development of potential therapies including gene therapy and substrate reduction therapy for this disease.

At the 2023 KTRN meeting, Dr. Bongarzone gave a presentation entitled "Inspiration that Mattered: My Life Dedicated to Krabbe Disease", during which he defined inspiration not only scientifically, but also as a synergistic by-product. He reviewed his life, and highlighted the early motivation he had in his childhood to explore and to go further in the humble world he grew up in. He was devoted to reading books, play with his new chemistry-set and exploring the micro-world that is not directly visible with the eyes.

Congratulations to Dr. Bongarzone on his award!

Remembering Dr. Laura Feltri

Laura Feltri, MD

Rosenau Family Research Foundation would like to honor the memory of Laura Feltri, MD, whose internationally recognized research moved the needle on scientists' understanding of Krabbe disease.

Dr. Feltri was a crucial partner to RFRF, serving on the KTRN Steering Committee in 2022 and 2023, and receiving two RFRF grants spanning from 2021 to 2023 at The Research Foundation of SUNY at The University of Buffalo.

Dr. Feltri's legacy will live on through her many contributions and discoveries in the fields of Krabbe disease, multiple sclerosis, and Charcot-Marie-Tooth disease, as well as through the continued work of her colleagues, with whom she was in the midst of collaborating with on multiple projects. She was an incomparable scientist who left a lasting effect on everyone she worked with, and she will be greatly missed.

Connect With Us!

General Information

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Grants

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