



# **Rosenau Family**

Research Foundation

## **Newsletter**

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**December 2023**

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## A Message from the Executive Director

It has been a busy fall at the Rosenau Family Research Foundation, and we have some exciting updates to share. In 2023, we approved eight grants, three of which were new investigator grants! This is especially exciting to us as an organization because of our focus on bringing new people and ideas to the table. The topics of research covered by this year's grants cover a wide range, from deciphering the underlying neuropathology and immunology in Krabbe disease to better understanding the disease burden among those individuals with Krabbe disease who have and have not undergone transplant. We look forward to seeing the fantastic work that all of our investigators do to support the patients and families living with Krabbe Disease and Cystic Fibrosis.

Moving forward, we are going to begin featuring grant awardees and the research that has been done over the years. With fifteen years of grants in the books, there is quite a lot to cover! In this newsletter, we will start off by featuring the work of Dr. Philip Farrell and his team at the University of Wisconsin School of Medicine and Public Health, which you can find on page 4. Dr. Farrell was also the recipient of the 2020 Sue Rosenau Legacy Award and has played an enormous role in progressing Cystic Fibrosis research specifically, so it is fitting that we begin by highlighting him.

Thank you for your continued support of RFRF. We will see you in the new year!



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



# What Purpose Does An Annual Board Retreat Serve?

By Mary Kay Delvo

Why and how Boards of Directors do things is a bit of a mystery to most. Unless you have previous board experience, most people agree to board service with little to no understanding of what boards do and why boards matter to the organization. Boards are generally made up of 5-20 people who come from different walks of life, experiences, professional backgrounds and yes, personalities.

The general course of board work takes place in monthly or quarterly board meetings, committee meetings and an annual full-day retreat. While the board and committees meetings are used for ongoing business, the board retreat has a higher purpose, which includes time for making connections and relationship building among board members, re-inspiring focus on the mission, and building confidence in the board's role contributing to that mission. Additionally, the retreat is utilized to develop board culture, educate on the role the board plays and to look ahead to the upcoming year and evaluate any strategic adjustments necessary.



A portion of RFRF's in-person Board of Directors' annual retreat is dedicated to the review of the Scientific Advisory Committee's recommendations for grant applications to be funded in the upcoming funding cycle. At the September retreat, the board approved a slate of board policies, roles, responsibilities, and charters. The remainder of the retreat was spent on 1) training the board on the governance roles and responsibilities and 2) defining the desired attributes, experiences, and background of board member candidates as we look ahead to filling out the board and its executive committee.

The RFRF board meets virtually four times and in-person one weekend a year and has a goal of adding two to five new board members in 2024.

Contact Mary Kay Delvo, [marykaydelvo@rosenaufoundation.org](mailto:marykaydelvo@rosenaufoundation.org) if you have questions about or interest in opportunities to serve on RFRF's Board of Directors!

Watch for more information on Board of Director development in the 2023 Annual Report.



# Featured Board Member



## Julie McCarrier



### WHAT IS YOUR FAVORITE THING ABOUT THE HOLIDAYS?

I enjoy getting together with family and friends, sending and receiving holiday cards, as well as enjoying some great holiday meals.

### HOW DID YOU LEARN ABOUT RFRF?

I worked with a family member professionally in the past, and I was contacted about serving on the Board of Directors as they thought I might be interested.

### TELL US HOW YOU ALIGN WITH ONE OF OUR VALUES: COLLABORATION, SCIENCE-DRIVEN, ETHICAL?

While all of the core values speak to me, collaboration is crucial to the success of RFRF.



### YOU ARE CURRENTLY THE LONGEST-SERVING RFRF BOARD MEMBER. WHAT IS MOST FULFILLING ABOUT SERVING ON THE RFRF BOARD?

I have really enjoyed the progress and forward momentum of RFRF, which will ultimately have a tremendous impact on Krabbe disease and CF. There has been a lot of growth and positive change since the early days!

### WHAT HAS KEPT YOU ON THE BOARD FOR ALL OF THESE YEARS?

As a member of the board, it is always exciting to see the wonderful grant proposals. The research proposals for work to be done are inspiring and will ultimately change the course of these disorders for the patients and families who are impacted by them.



### WHAT DEVELOPMENTS AND CHANGES IN THE BOARD MOST EXCITE YOU?

The expansion and growth of the board provides a breadth of knowledge and varied experience, which is ultimately helpful in providing guidance to the organization, and critical in terms of the sustainability of RFRF.

### WHAT WOULD YOU TELL PEOPLE THAT MIGHT BE CONSIDERING BEING ON THE BOARD?

It is very rewarding to serve on the board, which is extremely well run. Board members have a good understanding of their role and responsibilities, and it is a great way to make a meaningful contribution to an organization that has played a big role in funding research for CF and KD, and hopefully will result in a cure for Krabbe disease.



# Featured Research

Dr. Philip Farrell and his research team at the University of Wisconsin School of Medicine and Public Health recently completed a three-year grant with The Rosenau Family Research Foundation in 2022 titled, "Assessing the added value of whole genome sequencing in cystic fibrosis newborn screening," (2017-2022).

The key words here are "added value". The study had some key successes that may be helpful to similar programs in the future. According to Dr. Farrell, the results have been very impressive and revealing, and they expect to work another year or so on the study.

## Novel Findings

♥ Parents were willing to give consent for whole genome sequencing to find cystic fibrosis (CF) genetic modifiers. About 90% gave consent which is quite a big deal. It should be noted that before parents gave consent they knew the test had to stay focused on CF and not look at other disease genes nor ancestry.

♥ The team was pleased to have a 100% success rate on the 104 specimens from children, even those as small as .5 ml of blood! Due to a technical difficulty in the lab, they were not able to achieve the same success rate with the adult specimens.

♥ The team was able to use a method called polygenic risk scoring (PRS) which is often used with cardiovascular diseases and cancer but has never been used with Cystic Fibrosis or other lung diseases. Specifically, they were able to aggregate the potential genetic modifiers, using a PRS score to learn if it does indeed have a strong association with a variety of important aspects of CF, one of which is the early CF lung disease phenotype. The PRS was novel and worked quite well.

## The Results

So far, three papers have been published on this study with several more in process. You can read more about these results in the links below. For an extended version of this article, with more details and information about the process and results of this study, [click here](#).

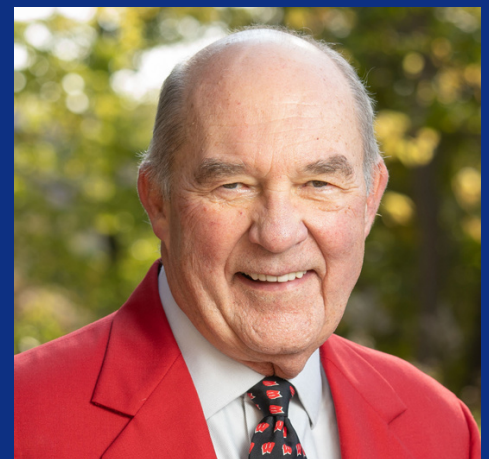
[Genetic factors help explain the variable responses of young children with cystic fibrosis to vitamin D supplements](#)

[Impact of intrinsic and extrinsic risk factors on early-onset lung disease in cystic fibrosis](#)

[Defining and identifying early-onset lung disease in cystic fibrosis with cumulative clinical characteristics](#)

**"Getting the grant from Rosenau Family Research Foundation (RFRF) was so important. I don't think we could have gotten one from the National Institute of Health. It's so advanced and on the leading edge that it might have been considered too exploratory. We were happy that the RFRF review committee was open minded and trusted us to do something so valuable."**

*Philip M Farrell, MD, PhD*



# Spotlight: Marketing Update

By Heidi Carrozzella

I had the opportunity to meet with many of you at the 2023 KTRN. I'll admit, it was intimidating talking with professionals in this space but each of you were very giving of your time, and very passionate about what you do, the KTRN and your relationships with Rosenau Family Research Foundation (RFRF).

It quickly became clear that RFRF and our investigator community have a common goal of growing and expanding our network and we are excited to get to work!

With our new brand, new website and this very newsletter launching, we have the right avenues in place to tell your stories and share them within our network to reconnect in between the KTRN each year. Perhaps even more importantly, we can share these stories outside of our network to help attract new people to work on Krabbe disease and Cystic Fibrosis research.

As we launch this first newsletter, I ask you to share your thoughts and feedback with us. Consider:

- What types of stories would you like to see more of?
- Do you have a story to contribute for our next issue?
- Are there aspiring researchers you can share this newsletter with?



Please share your thoughts with us at: [info@rosenaufoundation.org](mailto:info@rosenaufoundation.org).

As we get into 2024, we are excited to capture more stories from our network, both in video and written formats. Please follow us on social media and look for our email updates!

Finally, please welcome Rachel Jackson to the RFRF marketing team! She will be handling day-to-day marketing operations as we go forward. Those of you that attend the KTRN meeting will get to meet her in March!

## RFRF Social Media Handles/Links

Please follow us at our new handles on social media!

 [www.linkedin.com/company/rosenaufoundation](https://www.linkedin.com/company/rosenaufoundation)

 [www.facebook.com/RosenauFoundation](https://www.facebook.com/RosenauFoundation)

 [@rosenaufoundation](https://www.instagram.com/rosenaufoundation)

 [@Rosenau\\_Fdn](https://twitter.com/Rosenau_Fdn)

## Updated Website

[www.rosenaufoundation.org](http://www.rosenaufoundation.org)



# Soundbites

Our Co-Founder, Paul Rosenau, celebrated his 70th birthday in November! His generosity, wisdom and wit are just a few things we celebrate about him as we work to continue his legacy of supporting research that helps families lead lives undefined by Krabbe Disease and Cystic Fibrosis.



Happy birthday, Paul! 🎉

We are accepting Letters of Inquiry (LOI) for the 2024 funding cycle! A LOI is the first step in the process to apply for grant funding. LOI's for the current funding year must be received by 1/31, using our new online grant management system.

NOW ACCEPTING  
**Letters of Inquiry**  
FOR 2024 FUNDING

[Learn more and apply here.](#)

## SAVE THE DATE!



We are excited to announce that the Krabbe Translational Research Network's 2024 meeting will be held at the Hilton Bloomington in Minnesota on March 19-21!

Details and registration will be available at the beginning of January.

PRESENTED BY:  Rosenau Family Research Foundation

We have several sponsorship levels available, which you can find below. We are especially excited to work with sponsors on custom options this year, to make sure we are connecting with our partners and giving you the most valuable opportunity possible.

Please email [info@rosenaufoundation.org](mailto:info@rosenaufoundation.org) with any questions. We look forward to seeing you in Bloomington! [Learn about sponsorship opportunities here.](#)

This fall, we were proud sponsors of KrabbeConnect's A Million Dreams Gala and the Cystic Fibrosis Foundation's Breath of Life Gala.

We look forward to our continued sponsorships in 2024.

