



The Legacy of Angels
FOUNDATION

10 YEAR ANNIVERSARY



Continuing the Momentum



THE LEGACY OF ANGELS FOUNDATION
2018 ANNUAL REPORT



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From the Co-Founder

Another year is in the books, completing our 10th year as a 501(c)(3) private giving family foundation established in 2008. Our mission remains untouched; to improve the lives of children by working to promote the expansion of newborn screening (NBS), and to further education, awareness and research of Krabbe disease (KD) and Cystic Fibrosis (CF) to provide a better treatment and a cure. I'm truly proud of this organization my late-wife, Sue Rosenau, and I co-founded. We've witnessed remarkable progress in advances for KD, CF and NBS.

As I take a moment to reflect on 2018, our 10th year provided our organization with some significant trials and triumphs. After a decade of impactful work, our board of directors and staff began reevaluating priorities, our strategy, and timelines. This crucial work allowed our organization to grow cohesively and will collectively prepare us for future developments.

In reviewing our trials, TLOAF endured a significant loss, the passing of our Co-Founder, Sue Rosenau, in July 2018. This remarkable woman was at the forefront of the organization until the day her body was no longer able to be with us. Since October of 2015, she fought the ugly cancer battle for nearly 3 years, with a short remission in 2017. Through it all, Sue remained closely engaged in the organization's efforts in person or remotely from her home office. To honor her life, the board of directors unanimously approved a yearly award named, "The Sue Rosenau Legacy Award." The board selected recipient will be an inspirational leader who collectively creates lasting change and measurable differences in one or more areas of our mission. For the full details on this award and to read our inspirational article "Sue's Legacy Marches On," please visit page 18.

Another trial, we bid a somber farewell to a dear board member, Robert Hoffman. Bob joined the organization in 2014 and was instrumental in navigating the rules and regulations within educational institutions. His leadership and jolly spirit will be missed. We wish him the best on his new endeavors.

Last, but not least, TLOAF's triumphs for 2018. We added two individuals to the organization. Heather S. Techmeier accepted the position of Director of Finance and comes with more than 15 years of experience in finance and accounting. Please check out the full article "TLOAF Welcomes Heather S. Techmeier," on page 13. TLOAF also welcomed Julie McCarrier to the Board of Directors in September of 2018. Julie come to TLOAF with more than 18 years of experience as a board-certified Genetics Counselor. Our "Welcome Aboard" article features more on her credentials and role within the organization, which can be found on page 15.

In closing, The Legacy of Angels Foundation will "Continue the Momentum" for our spirited Co-Founder and for beloved individuals impacted by Krabbe disease and Cystic Fibrosis. I'm happy to report the organization remains strong and cohesive. Our board of directors and staff are determined to forge ahead, identify potential collaborators, and provide seed funding to advance developments in KD and CF. Through our invaluable efforts we will stay committed to executing a strong grant program, extending our network of rare disease innovators, and facilitating exchanges of information in hopes of adding years of quality of life to those impacted by our mission.



Sincerely Yours,
Paul G. Rosenau

About TLOAF

The Legacy of Angels Foundation is a private grant-making organization with a preference for leveraged, strategic funding. Letters of Inquiry are accepted year-round; full applications by invitation only.

BOARD OF DIRECTORS

PAUL ROSENAU
Co-Founder / President

HEATHER TECHMEIER
Board Treasurer

STACY PIKE-LANGENFELD
Board Secretary

BRETT ROSENAU
Director

JULIE MCCARRIER
Director

STAFF

STACY PIKE-LANGENFELD
Director of Programs and Administration

HEATHER TECHMEIER
Director of Finance

CONSULTANTS

JARED DUFALT
Tax Accountant

OUR MISSION

The Mission of The Legacy of Angels Foundation, a 501(c)(3) private giving family foundation established in 2008, is to improve the lives of children by working to promote the expansion of newborn screening, and to further education, awareness and research of Krabbe disease and Cystic Fibrosis to provide a better treatment and a cure.

OUR VISION

The TLOAF vision is to direct, fund and promote research to develop and enhance treatments and cures for Krabbe disease and Cystic Fibrosis.

TLOAF will promote through education and awareness the expansion of newborn screening, Krabbe disease and Cystic Fibrosis; benefiting children with the increased probability of early identification and effective access to treatment.

OUR GUIDING PHILOSOPHY

To invest in research, programs, and initiatives that further the mission and goals of The Legacy of Angels Foundation. Our Board of Directors believes in collaborative efforts, leveraged funding, and commitment. With these attributes, we can collectively change the outcome for those impacted by Cystic Fibrosis and Krabbe disease.

Our Focus Areas

The Legacy of Angels Foundation supports programs and initiatives that advances research, education, and treatment options for those impacted by Cystic Fibrosis (CF) and Krabbe disease (KD). We invest in these two rare diseases as they affect the Co-Founder's, Paul and the late-Sue Rosenau, family genetics.

Newborn Screening (NBS) is another arm of our mission. NBS is key to receiving an early diagnosis and prompt treatment or intervention, allowing for the best chance for survival. Our NBS initiative completes the philanthropic focus of our private family grant-making foundation.



Cystic Fibrosis: Kendall Alauf, daughter of Casey and Tara Alauf of WI, was diagnosed with Cystic Fibrosis at 13 days of age. Cystic Fibrosis is a life-threatening genetic disorder that causes the accumulation of thick sticky mucus and secretions generating issues for the lungs, pancreas and digestive system. This disorder is caused by a mutation of the Cystic Fibrosis Transmembrane Conductance Regulator Gene (CFTR) and is considered rare, despite affecting nearly 30,000 people in the U.S. Today, Tara explains Kendall as, “A spunky 7-year-old, competitive gymnast and a loving big sister.” Kendall’s therapy regimen consists of 10 daily medications requiring her to swallow 30 plus pills a day, 3 respiratory inhalers and 2 rounds of chest therapy every day. Kendall’s parents are thankful for the research efforts in Cystic Fibrosis and state, “we hope one day, Kendall will have the opportunity to live life without boundaries or hesitation.” TLOAF supports education and scientific research in the Cystic Fibrosis space.



Krabbe Disease: Makayla Lynn Pike, granddaughter of TLOAF Co-Founders, was diagnosed with Krabbe disease in October of 2001. At 3.5 months of age, the family learned Makayla would live a shorten life. The disease is a rare neurodegenerative disorder inherited in an autosomal recessive manner, causing a mutation of the GALC gene. Without the GALC lysosomal enzyme, the myelin sheath of the nervous system lacks the means to properly develop. To-date, there’s no cure. TLOAF supports scientific research in KD to advance survival and quality of life for those affected. Makayla, who lost her life to the disease in May of 2003, will forever be part of the foundation’s legacy.



Newborn Screening: Quinton James Nitahara was born to Ryan and Laura Nitahara of Illinois. Quinton underwent a newborn screening test, using a few drops of blood from a heel poke, shortly after his arrival. NBS is a vital public health program that’s utilized internationally to identify disorders in infants. Quinton was identified with late-onset Krabbe disease through their state implemented screening program. As a result of NBS, Quinton will have treatment opportunities to prevent intellectual and physical disability from this life-threatening disease. Today, Illinois is 1 of 9 states testing for Krabbe disease. The core of TLOAF’s mission is to strengthen and improve testing for CF and KD, to ensure all babies born receive a prompt and accurate diagnosis.

2018 Grant Recipients

Each year, The Legacy of Angels Foundation spends a great deal of time and energy on supporting initiatives through their grant program. Our grant program works to strengthen the research within institutions, aiding successful securement of long-term support for treatment advances or clinical trials from NIH, FDA, and industry. The Foundation accepts Letters of Inquiry (LOI's) year-round but full applications are by invitation only. Programs or projects involving collaboration among multiple stakeholders are strongly encouraged to apply.

Since 2009, TLOAF's grant program has awarded almost \$16 million to aid research and educational programs. The

foundation strives to invest in collaborate efforts to help sustain their financial portfolio for years to come. In 2018, the Foundation awarded 5 research projects totally **\$1,027,896.94**. We often receive projects far in excess of our available funds each year. This leads to difficult decisions in prioritizing our funding and requires several projects to be declined.

Yet, we don't focus on what we can't do as an organization but what we can. Each year we look forward to seeing the progress made from each grantee and we're thrilled to be a part of moving research forward. The 2018 grant recipients were as follows:



Krabbe Disease Patient Care and Research (NDRD)

Maria Escolar, M.D. –
Children's Hospital of Pittsburgh

Treatment of Krabbe Disease Using Gene Therapy: Continuation

Maria Escolar, M.D. –
Children's Hospital of Pittsburgh



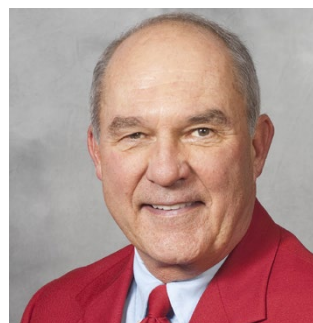
Development of Pharmacological Chaperone Therapy for Krabbe Disease

Chris Lee, Ph.D. –
Biomedical Research Institute of New Jersey



Optimizing the Treatment of the Mouse Model of Krabbe Disease for Translation into Human Trial

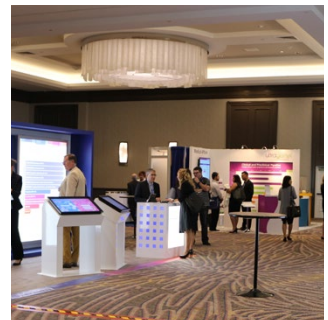
David Wenger, Ph.D. –
Thomas Jefferson University, Philadelphia, PA



Assessing the Added Value of Whole Genome Sequencing in Cystic Fibrosis Newborn Screening

Philip Farrell, M.D., Ph.D. –
University of Wisconsin, Madison

2018 WorldSymposium



The WORLDSymposium, attracting leading scientists from around the world, provides a forum for cutting-edge science on lysosomal storage disorders (LSD's). This conference, held at the Manchester Grand Hyatt in San Diego, has grown exponentially over the last 5 years, with record attendance reaching nearly 1800 attendees this year. Stacy Pike-Langenfeld, Director of Program and Administration for TLOAF, had the pleasure of attending the conference.

The program started off with the Council of Patient Advocates (COPA) workshop, addressing unmet needs in lysosomal storage disease space. One of the unmet needs in many rare disease groups revolves around the demand for a Natural History Study (NHS). A Natural History Study consists of an aggregate of data that tracks the course of disease over a period of time (often years) and the study helps identify variables that correlate with disease development and outcomes. A study of this magnitude requires time, funding and planning yet, it's absolutely necessary in order to advance a new therapy to the next stage – a clinical trial.

A few other discussion points at the COPA meeting included quality of life, pain management, emotional/social issues, and the role of genetics. One powerful comment, a patient advocate made, resonated with Stacy was, "How does one navigate the ups and downs of childhood (tween, teenager,

young adult) as more treatment allow patients to live longer?" It's evident from the discussion, many questions remain unanswered in treating patients with these disorders.

The remainder of the WorldSymposium focused on scientific presentations providing the latest insights on discoveries and clinical significances of lysosomal storage disorders. To date, nearly 50 lysosomal storage disorders have been identified; 11 posters and a handful of presentations were specifically devoted to Krabbe disease—simply encouraging. Attendees spent 3 days listening to presentations on LSD's and networked with stakeholders in their respective disease community.

TLOAF grant recipient, Steven Gray, presented on AAV capsids for the treatment of lysosomal disorders. His presentation discussed the use of DNA shuffling to create novel AAV capsids with better targeting to the nervous system than "gold standard" vectors such as AAV9. Stacy Pike-Langenfeld stated, "It's exciting to see advances in Krabbe disease with the help of our family foundation grant program. We look forward to this meeting each year as it provides a congregate of research happenings across all LSDs, a venue to connect with key opinion leaders and a robust group to identify new candidates for our grant program." Until next year WORLD.....

Rare Disease Day- University of Minnesota



Each year, many organizations across the world are involved in efforts to advance treatments, raise awareness, and bring resources to those living with a rare disease. In 2008, the European Organization for Rare Disease, better known as EURORDIS,

successfully implemented the last day of February as Rare Disease Day. This worldwide day of observance brings stakeholders in the rare disease space together to coordinate activities, educate congressional leaders, and acknowledge the millions of people suffering with a rare condition.

In honor of those living with a rare disease, Stacy Pike-Langenfeld of TLOAF, attended The University of Minnesota Rare Disease Day event. The half-day event began with an exhibit displaying 28 research posters and 22 patient advocacy tables. The theme this year was, "Gene Therapy for Rare Disease: Promise and Challenges."

After the hour-long exhibit, attendees enjoyed a boxed lunch while listening to 3 presentations related to gene therapy. Each presentation was valuable and informative. Attendees learned about advances in Epidermolysis, received an overview of gene therapy versus gene editing, and most importantly, discussed the power of the patient perspective in advancing science. Dr. Jakub Tolar, Dean of the Medical School at the University of Minnesota (pictured at left), graciously moderated the guest speakers and audience Q & A.

An added value of this 1-day event; the networking and exchanging of ideas that occurs when people come together. These valuable gatherings offer an environment to engage in meaningful conversations and rally for effective solutions to treat rare diseases. As Helen Keller notes, **"Alone we can do so little; together we can do so much."**





A Perfect Angel

On April eighth two thousand and one
A beautiful life of a child had begun.

The birth of my first grandchild from my very own,
Brought a happiness inconceivable the day she was born.

As a new grandma, I was as proud as could be;
With this sweet little girl who joined our family.

She was a perfect angel with ten fingers and toes,
Dark hair, blue eyes and a cute little nose.

As I held my granddaughter like no other,
I got that same feeling like becoming a new mother.

My heart soon was filled with love for this child,
Each day it grew stronger and went totally wild.

But soon we discovered at her very young age,
Her life would be short, with this we must wage.

A dreadful disease now known as krabbes,
Would consume her body and take her away.

How this could happen was hard to understand.
But God was in charge and it was put in His hands.

So we filled every day with all the memories we could;
Living life to the fullest we certainly would.

Give all to this child who deserved so much more,
A lifetime of happiness - to give was no chore.

She had a strong spirit and was always a fighter.
She gave hope to life and made our days brighter.

Her birthdays we celebrated; First was One, and then Two!
But her body was weakening and it was then that we knew.

Her earthly life was soon to give way.
On to another - in heaven she'd play.

On May fourth in two thousand and three
A perfect little angel she was meant to be.

Now enjoying and living in heavenly bliss.
Makayla Lynn Pike will forever be missed.

In Memory of my granddaughter Makayla Lynn Pike



The 2018 KTRN Meeting

The attendees for the 2018 Krabbe Translational Research Network (KTRN) meeting gathered March 14th-16th at the Opal Sands Resort, in Clearwater, Florida. This resort, beautifully situated next to the white sandy coastline of Clearwater Beach, is located on the Gulf of Mexico. More than thirty individuals from multiple institutions and disciplines filled the ocean-front conference room.

Dr. Maria Escolar commenced the meeting by expressing her deep gratitude to The Legacy of Angels Foundation for their ongoing commitment to Krabbe disease (KD). As Dr. Escolar noted, "TLOAF's work and grant program for KD has expedited advances in Krabbe disease." Excitement is building as the KTRN consortium is hopeful a clinical trial will be a reality soon. Sue Rosenau, the late Co-Founder of TLOAF, closed the commencement with a heartfelt address to the attendees, recognizing their hard work and stated, "I look forward to the day when a new treatment option becomes available to the bedside of patients. Let's get this disease cured!"

After Sue's inspirational address, the meeting continued with key researchers presenting on the recent findings in KD research. Some of the interesting findings from the natural history study of Infantile Krabbe disease (IKD) are as follows:

- **80% of patients have some degree of spasticity, peripheral muscle weakness and central hypotonia**
- **Growth was affected in all cases, but a majority are in the normal range for BMI (BMI is your body mass index measure. It provides a means for measuring a healthy weight based on height and weight)**
- **There's a large variability of motor impairment in children with IKD**

Several publications addressing the Natural History Study of Krabbe patients are now available. Check out the information box to the right for a list of recent publications.

Other insightful presentations at this meeting were as follows: Dr. Julia Koffler, Assistant Professor of Pathology at the University of Pittsburgh Medical Center, provided an overview of 9 Krabbe biorepositories which included results from transplanted and non-transplanted patients; Dr. David Wenger, from Thomas Jefferson University, presented on the outcomes of gene therapy for KD; Dr. Steven Gray, from the University of Texas, reported on the delivery approach of gene therapy and how it translates into humans; and lastly, Dr. Dolan Sondhi, a Research Professor at Weill Medical College of Cornell University, initiated a health discussion with attendees on balancing the purity, potency, and reliability of vectors – a highly tasking responsibility of vector production.

In reviewing the agenda for the 7th KTRN meeting, each participant offered perceptive evidence and held a keen understanding of the current scientific advances in KD. As Heather Techmeier, Director of Finance stated, "it was encouraging to see and witness the initiation of several new scientific investigations, learn that assessments on the structure of the gene therapy trial are underway, and hear tactics to engage the FDA with the design of a gene therapy trial in KD." Each and every step towards a better treatment in patients with Krabbe disease provides another layer of hope – that a new treatment option for this disease is within reach.

Recent NHS Publications on Krabbe disease:

Beltran-Quintero ML, Bascou NA, Poe MD, Wenger DA, Saavedra-Matiz CA, Nichols MJ, Escolar ML. **Early progression of Krabbe disease in patients with symptom onset between 0 and 5 months.** Orphanet J Rare Dis. 2019 Feb 18;14(1):46.

Bascou N, DeRenzo A, Poe MD, Escolar ML. **A prospective natural history study of Krabbe disease in a patient cohort with onset between 6 months and 3 years of life.** Orphanet J Rare Dis. 2018 Aug 9;13(1):126.

Wright MD, Poe MD, DeRenzo A, Haldal S, Escolar ML. **Developmental outcomes of cord blood transplantation for Krabbe disease: A 15-year study.** Neurology. 2017 Sep 26;89(13):1365-1372.

Note: The above research and publications were made possible due to TLOAF approved grants for Krabbe disease. Please visit our website, TLOAF.org, for more information on our grant program.



Coalition of Patient Advocacy Groups (CPAG) Meeting

The Rare Diseases Clinical Research Network (RDCRN) hosts a meeting each year focused on the needs of Patient Advocacy Groups, also known as PAGS. PAGS are organizations that encompass one or more initiative to advance research, awareness, education and more, in a specific disease space. The Legacy of Angels Foundation is a PAG under the Lysosomal Disease Network. Dr. Chester Whitley is the principal investigator for this consortium; there's currently 21 consortia groups associated with the RDCRN.

In May of 2018, The RDCRN hosted a CPAG meeting at the Hilton in Rockville, Maryland. TLOAF staff member, Stacy Pike-Langefeld, attended this 1-day meeting. The agenda provided an overview of the National Center for Advancing Translational Sciences (NCATS) toolkit, highlighted upcoming funding opportunities, shared insights on patient-derived stem cells for research, discussed genome editing, and encouraged PAGS to leverage collaborative efforts. A favorite of many attendees was the presentation on how to bring patient experiences to the FDA.

So much information packed into 8 hours provided Stacy with a plethora of valuable content to share with staff and board members. Since it's difficult to sum up a meeting involving a wide span of content, it seems most appropriate to provide 5 major takeaways from this meeting, which are as follows:



1

The NCATS Toolkit provides a web based interface with a comprehensive collection of therapy development. This patient friendly resource can be found on their website at <https://ncats.nih.gov/toolkit>.

2

To establish standards of care within your respective disease space, a consensus meeting is a must. It's not possible to develop standards of care through a publication. Establishing standards requires many meetings with leaders in the space, presenting to hospitals through grand rounds, educating physicians, and lots of follow-up.

3

Many PAG organizations would like to see hospitals invest in a rare disease team. Leaders and investigators expressed concern in cultivating such a team of experts. The hospital systems would be faced with proving the cost effectiveness of such a program. Is that possible to do with over 7,000 rare disease currently identifiable?

4

There's a large deficiency in board-certified genetic counselors. In 2017, 16 positions across the U.S. identified a need for a board-certified genetics counselor. In 2018, a total of 30 open positions existed across the U.S.

5

It's vital to utilize the patient's voice whenever possible. A patient's or caregiver's voice can be a powerful tool to assess clinical trial readiness, to evaluate disease specific needs of the community, to provide insights on the shortcomings of existing treatments, and to showcase the need for a new treatment. Yet, the most important value each patient and caregiver perspective offer can be summed up in three words, **"the human element."** Empirical data can't replace the voice and experience of the patient.

* For more information on this meeting or upcoming meeting dates, visit rarediseasesnetwork.org or reach out to the RDCRN-Chair, Joyce Kullman from the Vasculitis Foundation at jakullman@vasculitisfoundation.org.

Welcome Heather Techmeier



Without further a du, we're excited to add another employee to The Legacy of Angels Foundation. On March 1st, Heather Techmeier accepted the Director of Finance position. Heather comes to the organization with more than 15 years of experience in finance and accounting. Her most recent role at American Transmission Company included investment projects and forecasting of budgets, financial reporting and data analysis, plus complete accountability for capital banking and cash management activities. Her attention to detail, analytical skills, and integrity, will be vital in protecting the foundation's assets.

Heather's experience in the rare disease is personal in nature. Heather was introduced to the rare disease space upon welcoming her niece, Makayla Lynn Pike, into the world while studying at the University of Wisconsin, LaCrosse. Heather noted, **"Learning my niece would live a shortened life provided a fierce reminder how the world we live in can be so heart-breaking."**

After the passing of her niece, Heather married a year later. As Heather and her husband, Kurt, planned for a family, they quickly learned their fate in bringing healthy children into this world would be complicated. Genetic testing revealed Heather was a carrier for Krabbe disease and a life-impacting mutation of Cystic Fibrosis (CF); both rare and not generous to a child's longevity. Normally this wouldn't conflict or cause much issue since both diseases are recessive. Yet, luck was not on their side as Kurt's result revealed he was a carrier for CF also.

As the couple forged ahead in their family planning, they decided to take a more scientific approach to starting a family. Heather and Kurt began the journey of In Vitro Fertilization (IVF) with Pre-Implantation Genetic Diagnosis (PGD) testing. PGD is a procedure used prior to implantation to help identify genetic defects within embryos. This serves to prevent certain genetic diseases or disorders from being passed on to the child. Thanks to IVF with PGD testing, Heather and Kurt enjoy an active family household with 3 children, Mason (10), Emma (7), and Ashlyn (7) and live in the Milwaukee area.

Heather is thrilled to be a part of The Legacy of Angels Foundation. Her role within the foundation will entail guiding the foundation towards profitability and long-term success by maximizing the return on financial investments. She will monitor the financial reports of grants, enhance financial policies and procedures of the foundation, institute an employee handbook, and assist with strategic and operational planning.

In Heather's spare time she finds great joy in being a mom of twin girls and a sports-driven son. When she's not busy with the family, Heather enjoys reading, baking, boating, volunteering at the kids' school, being the PTO Co-President, and getting together with family and friends. Heather can be reached at HeatherTechmeier@tloaf.org.

Researcher Spotlight—Dr. Philip Farrell



Each year we take a moment to shine a spotlight on a researcher doing remarkable work in one or more areas of our mission. This year, the staff at The Legacy of Angels Foundation selected Dr. Philip M. Farrell, a board-certified neonatologist and pediatric pulmonologist. Dr. Farrell, a kind-hearted investigator, is a nationally recognized leader in newborn screening for Cystic Fibrosis.

Cystic Fibrosis is a condition many folks have some understanding of or can at minimum say, “I’ve heard of that.” To date, it’s the most common rare disease of European decent. It’s caused by a mutation in the cystic fibrosis transmembrane conductance regulator or CFTR. The CFTR is an important protein in the

human body. It’s responsible for regulating the viscosity, or in layman’s terms, the stickiness of mucus, that lines the ducts of several organs. The extra mucus clogs passage ways or ducts, especially in the lungs and pancreas, triggering a breeding zone for infections.

Dr. Farrell’s passion for CF began during his 3rd-year as a medical student at St. Louis University. He witnessed firsthand how CF affected the life of an 8-year old girl. This child left an enormous impression on Dr. Farrell personally and professionally which he harnesses for inspiration and motivation in his everyday work. He stated, “I still remember that 8-year old girl in 1966 who died suffocating, I felt that no child should ever experience such suffering.”

Fast forward to 2018, Dr. Philip Farrell currently directs the Wisconsin Cystic Fibrosis Neonatal Screening project as an Investigator on a variety of newborn screening studies; works with the Center for Disease Control and Prevention on quality assurance in CF newborn screening; serves as the CF Foundation’s national facilitator for quality improvement in CF newborn screening; and lastly, investigates the ancient origin of CF with a team of researchers in France. Phew, that’s a whole lot of responsibility and titles. It’s evident his passion for CF will perfect screening and open new therapeutic interventions, a priceless contribution to parents hoping for the best life for their newborn CF child.

In addition to his leadership roles, he’s entered the 2nd year of a 3rd grant titled, “Assessing the Added Value of Whole Genome Sequencing in Cystic Fibrosis Newborn Screening,” funded by TLOAF. Although definitive results for this study won’t be available until 2021, there’s hope genome sequencing will add value to patient care in the near future. The Legacy of Angels Foundation looks forward to sharing the publication and results of this project.

FUN FACTS ABOUT DR. FARRELL

- Dr. Farrell is fond of spending time with his wife, Alice, especially while participating in beach activities in Florida
- He thoroughly enjoys bicycling and swimming, especially in the WI Northwoods
- Spending time with 9 wonderful grandchildren
- Is a football and baseball fanatic
- Published a book with two of his grandchildren called, “An Illustrated History of Siesta Key.”

HONORS AND AWARDS

2019-Docteur Honoris Causa Honorary Degree in Genetics
Universite’ de Bretagne Occidentale, Brest France

2013 George Cunningham Visionary Award in Newborn Screening
Association of Public Health Laboratories

2011-CDC Award of Excellence for Leadership and Outstanding Efforts in Cystic Fibrosis Newborn Screening
Quality Assurance Centers for Disease Control and Prevention

2010-Presidential Citation Award
Wisconsin Medical Society

2008- Edwin L. Kendig Award
American Academy of Pediatrics and American College of Chest Physicians

2005- Paul di Sant’ Agnese Distinguished Scientific Achievement Award
U.S. Cystic Fibrosis Foundation

Robert Hoffman: Thank You!

Robert Hoffman (Bob), retired Vice President of Minnesota State University, has resigned from the TLOAF Board of Directors to pursue other opportunities. The Foundation is forever grateful for his vital work and passion for our mission. Bob noted in his letter of resignation, "I am so impressed with the influence and the success of The Legacy of Angels Foundation and have been honored to be a part of it."

Bob was instrumental in developing leadership roles and providing procedural insight into educational institutions. His spirit and jovial demeanor as a Director on the TLOAF Board will be missed. We wish Bob the very best with his future endeavors and thank him dearly for all his hard work and time!



Julie McCarrier Joins TLOAF Board



The Legacy of Angels Foundation is proud to announce the addition of Julie McCarrier to its Board of Directors. Julie comes to TLOAF with more than 18 years of experience as a board-certified Genetics Counselor and is currently the Director of Pediatric Genetics at the Medical College of Wisconsin. Her decorated Curriculum Vitae reveals many published articles, her commitment to community service, and a list of professional affiliations.

Julie began her education at the University of Wisconsin, Stevens Point, where she received her BA in Biology. She then went on to graduate school at the Sarah Lawrence College, Bronxville, NY and became board certified in 2002.

The TLOAF Board of Directors believes Julie's skill set will bring tactical insights in the field of genetics and genomics to the organization. Her ability to assess hereditary conditions and provide comprehensive services including but not limited to analysis on whole exome sequencing, risk assessment, as well as outreach, will add intellectual depth to our board. As the complexity of care rises with patients affected by rare hereditary conditions, the role of the genetic counselor has become an increasing vital component of the care team.

Julie expressed the following upon invitation to the board, "I am thrilled to be part of an active and passionate board dedicated to advancing therapeutic treatments for Cystic Fibrosis and Krabbe disease." She goes on to say, "Being part of this board provides me with another opportunity to be part of a team dedicated to advancing therapeutic developments in 2 rare diseases."

When Julie's out of the office or away from business, she can be found with her partner, Lance, rock climbing, or doing any of the following: cooking, baking, reading, and traveling.....that is, when she's not participating in her other passion, road cycling. Please help us welcome Julie to the board by sending her a message at jmccarrier@mcw.edu

Celebrating 10 Years!

It's been an astounding 10 years since the inception of The Legacy of Angels Foundation. What a tremendous ride it's been for Co-Founders Paul and the late-Sue Rosenau. They embarked on their dream to help those living with Cystic Fibrosis and Krabbe disease, two rare diseases that affect the Rosenau family genetics, after receiving a large check from one little lottery ticket.



In a relatively short timeframe, the much-admired couple worked tirelessly to develop the infrastructure for the organization; Sue focused on the mission and vision segments and Paul mindfully activated a strong financial portfolio. The first 2 years of operation, Paul and Sue, engrossed themselves in the non-profit space, met with key opinion leaders in CF, KD, and NBS, and initiated a research driven grant program. A remarkable feat as the hard-working middle-class couple overcame the uncomfortable convictions that comes with automatic wealth.

As time moved forward, Paul and Sue forged ahead with vigor, building strong working relationships with key opinion leaders, researchers, and academic institutions that could impact their mission. With the help of a strong board and a knowledgeable scientific advisory board, great progress has been achieved. Some of the highlights over the past 10 years are as follows:

- 1. In 2010, TLOAF helped establish a scientific consortium called, The Krabbe Translational Research Network meeting, with Dr. Maria Luisa Escolar.**
- 2. In 2011, Save Babies Through Screening Foundation debuted "One Foot at a Time," a newborn screening educational video made possible from TLOAF funding.**

- 3. In 2012, a state-of-the-art MRI was installed at The Program for the Study of Neurodevelopment in Rare Disorders (NDRD) clinic to assist in detecting changes in the brain of Krabbe patients. Funding was provided by TLOAF in partnership with the University of Pittsburgh Medical Center.**

- 4. In 2012, the foundation funded Dr. Wenger's grant: "Intracerebroventricular and Intravenous Injections of AAVrh10-cGALC into the Dog Model of Krabbe Disease" for the purpose of moving research studies closer to human clinical trials for the treatment of Krabbe disease.**

- 5. In 2014, next generation sequencing technology was installed at the Wisconsin State Screening Laboratory of Hygiene to improve newborn screening in Cystic Fibrosis.**

- 6. In 2015, at Thomas Jefferson University, under the direction of Dr. David Wenger, encouraging studies in the twitcher mouse were providing very favorable results. This study, describing the successful treatment of twitcher mice with BMT plus a single IV injection of AAVrh10, was published in the November 2015 edition of Molecular Therapy.**

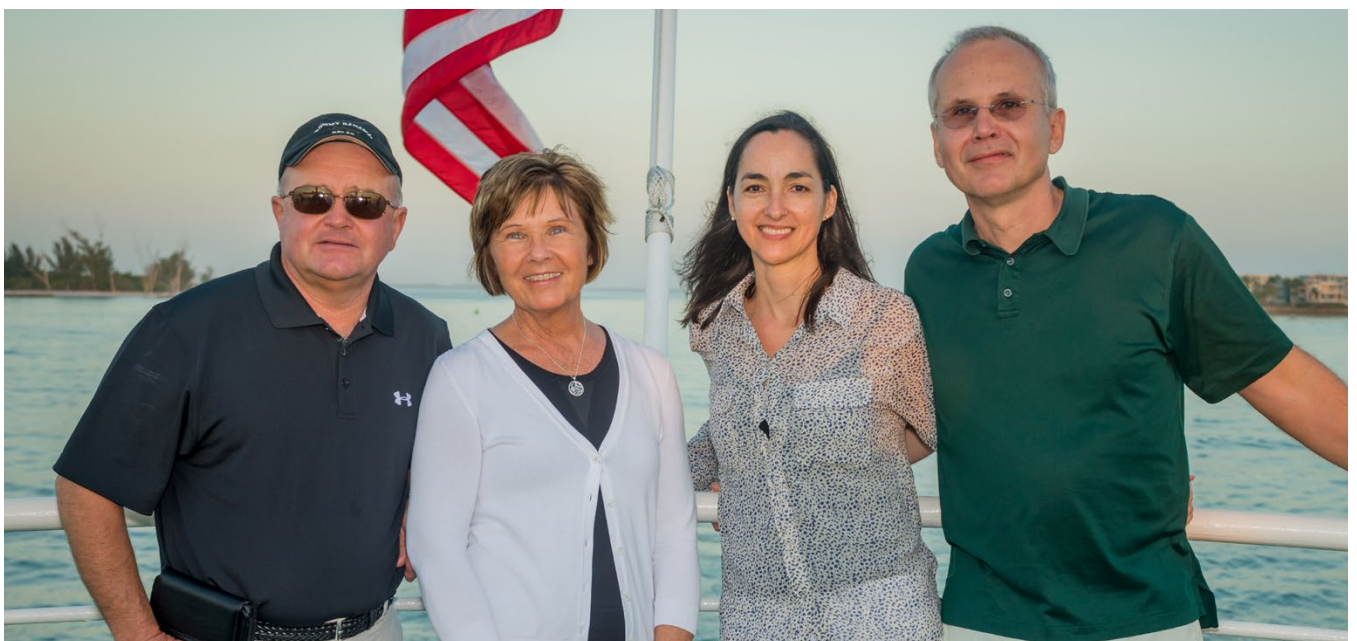
- 7. In 2016, Co-Founders, Paul and Sue Rosenau were selected as one of the finalists for Minnesota Business magazine's 5th annual Leaders in Health Care Awards. Dr. Donna Block was presented with the Champion Award that evening.**

- 8. In 2018, Cystic Fibrosis gains momentum with whole genome sequencing. This technology will help provide personal precision care for those CF patients. Final results of this project won't be available until 2021.**

- 9. In June of 2018, Co-Founders, Paul and Sue Rosenau received the Paul M. Fernhoff Endowment Fund Award for their outstanding contributions to Newborn Screening and Infant Health.**

In closing out a decade of work in the rare disease space, we're thankful for the progress and remain hopeful our work will bring therapeutic advances to the bedside of patients. Our fiercely committed Board of Directors will remain focused on the mission and continue to work until the words, **"CURE FOUND,"** are obtained for CF and KD. This would truly be the ultimate legacy to an organization that endured the heart-breaking loss of their dear granddaughter and the loss of their strong-spirited Co-Founder. Until then, we will strive to continue the momentum.

10 YEAR ANNIVERSARY



Sue's Legacy Marches On

Co-Founder and Chief Operating Officer of The Legacy of Angels Foundation lost her 3-year battle of multiple primary cancers, ovarian and serous endometrial, on July 31st, 2018. For those of you who've followed her journey since her devastating diagnosis in October of 2015, this remarkably driven wife, nana, and philanthropic extraordinaire threw every ounce of energy and strength into beating the odds. Sue was poked, scanned, operated on, and hospitalized countless times, yet she remained engaged and focused on the organization's goals.

At the beginning of 2018, Sue implemented TLOAF's executive succession plan. She worked with Stacy Pike-Langenfeld and Heather Techmeier daily to ensure the long-term sustainability of the organization. Stacy, as the Director of Programs and Administration, will oversee the grant program, manage and produce creative assets, and guide operating methods. Heather, as Director of Finance, will develop and monitor all budgets, analyze financial data, disperse grant funds, and manage the foundation's benefits.

Sue, as the Chief Operating Officer, was the cornerstone and trusted leader of the foundation. The TLOAF staff and Board of Directors will forever be grateful for Sue's vision and commitment to Cystic Fibrosis, Krabbe disease, and Newborn Screening. The loss challenged us emotionally, physically, and at times, operationally. There's no level of succession plan that can completely prepare you for the grief, heartache, and emptiness our organization endured when we were forced to say our final goodbyes on Sunday, August 5th.

Sue fought hard and refused to let any piece of her cancer journey interfere with her commitment and dedication towards TLOAF's mission. Cancer is an insidious disease requiring difficult decisions at every turn, yet Sue's perseverance and brightly shining personality will be her legacy, not the cancer. To keep Sue's legacy alive within the foundation and community at large, TLOAF will honor an individual for his\her achievements in Sue's name.

The Board of Directors will announce each spring the recipient of the "Sue Rosenau Legacy Award." This yearly award will recognize an inspirational leader who collectively creates lasting change and measurable differences in one or more areas of our mission. The individual selected will be honored for their extraordinary contributions, ability to propel progress, and altruistic support for the patient community; a replica of the late-Co-Founder, Sue Rosenau.



So here we are, with the next decade in our hands, with more goals to reach and work to do. Although nothing prepares you for the death of a person firmly by your side since inception, our foundation remains focused and determined to march on, utilizing the blueprint created by our dear Co-Founder, Sue. The foundation so graciously inherited a great example and a name to forever remember.

Financial Update

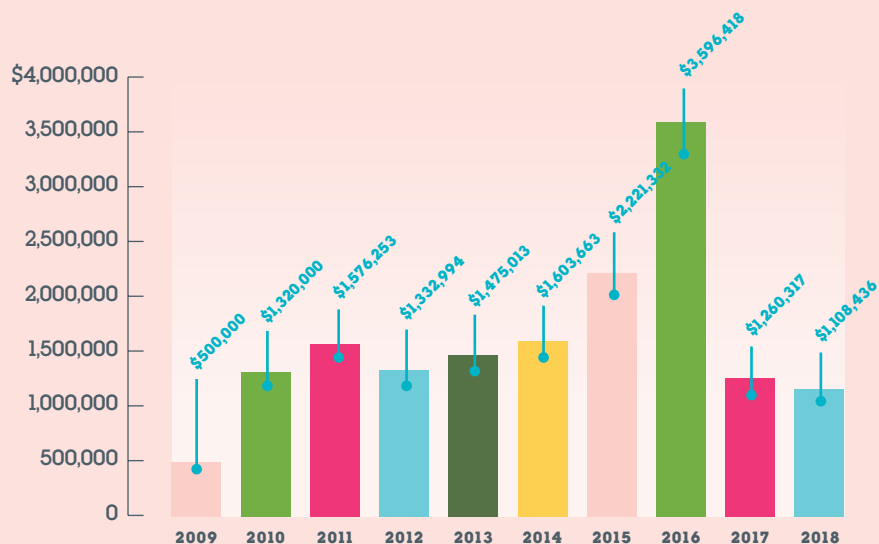
As we reflect, we can learn a thing or two from the past when it comes to our selection of grants. Throughout the past decade, TLOAF grants have been, and will continue to be, competitive. From a financial perspective it's important we re-evaluate our goals and ensure we continue to fund for impact. The Legacy of Angels Foundation is always looking for opportunities to partner with others to maximize our funding support. Why spend time trying to pave your own path when others have done it?

To help us get there we rely on our Scientific Advisory Board (SAB) which is composed of rare disease medical experts who generously donate their time to help TLOAF's mission. The grant selection process is unbiased, independent, and awards are based solely on scientific merit and how it relates to TLOAF's mission, as determined by TLOAF's SAB.

It's been no small feat managing TLOAF's investment portfolio; it's a balancing act of risk versus performance, growth versus safety, amid granting nearly \$16 million towards research for the past decade. While the Foundation has witnessed exciting advances in research from its dedicated grant program, it's important to reassess the portfolio from time-to-time. Therefore, Heather Techmeier, TLOAF Director of Finance, recently initiated the following to ensure the Foundation's longevity: meticulously monitoring of funds, the restructuring of investments, and year-over-year budget analysis. We're thankful our Foundation remains strong and economically sound as we have one very important mission to achieve:

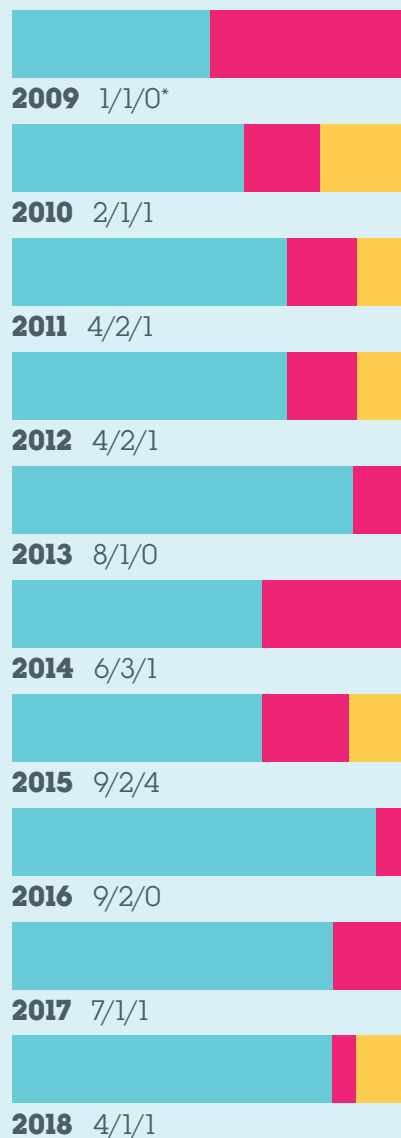
Improving the lives of children by working to promote the expansion of newborn screening, and to further education, awareness and research of Krabbe disease and Cystic Fibrosis to provide a better treatment and a cure.

GRANT FUNDING: 2009-2019



GRANT FUNDING: 2009-2019

■ Krabbe Disease
■ Cystic Fibrosis
■ Newborn Screening



* Number of grants awarded to each field of study

Please take note: As you can see above, a larger portion of our grant funding has been allocated to Krabbe disease. The TLOAF Board of Directors works diligently to identify vulnerable areas of research needs in Cystic Fibrosis. Specifically, research areas not receiving support or seed funding from the nationally recognized Cystic Fibrosis Foundation, established in 1955.

NOTE:

NEW
ADDRESS

THE LEGACY OF ANGELS FOUNDATION

PO BOX 413, ROSEMOUNT, MN 55068
WWW.TLOAF.ORG



**The Legacy of Angels
FOUNDATION**