It's All About THE KIDS

The Legacy of Angels Foundation
2015 Annual Report
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IT’S ALL ABOUT THE KIDS!

Reflecting back on 2015, I’ve come to the realization that everything The Legacy of Angels Foundation (TLOAF) has done is “All About The Kids!” Every step taken, every dollar spent, every grant awarded leads to helping kids. Whether they are newborns, juveniles, or adults, TLOAF strives to put the lives of all those fighting krabbes and cystic fibrosis disease first!

2015 proved to be a challenging year with an increasing need for grant dollars. While we will never have an infinite amount to support every potential grant, The Legacy of Angels Foundation tries to prioritize and support wherever the greatest need exits.

RESEARCH - It’s All About the Kids!

In 2015 TLOAF funded more than $2.2 million dollars in research. Grants were supported for all three TLOAF mission initiatives: Krabbes Disease, Cystic Fibrosis and Newborn Screening.

KRABBES DISEASE

 جميل NDRD - The Legacy of Angels Foundation continues to support the Program for the Study of Neurodevelopment and Rare Disorders at Children’s Hospital of Pittsburgh of UPMC. Dr. Maria Escolar is the world renowned clinician and researcher for krabbes disease and other leukodystrophies and treats patients from across the United States and around the world.

 جميل KRABBES RESEARCH - It’s a very exciting time in research of krabbes disease. Currently the standard of care for new patients of early diagnosis of krabbes disease is a cord blood transplant (CBT). While this transplant has shown great improvement in the lifespan of patients with krabbes, it is evident that the peripheral nervous system is not being rescued through this transplant. Gene therapy when used in conjunction with CBT is showing tremendous promise in the laboratory. TLOAF has funded projects at various labs throughout the US experimenting with several methods of using gene therapy. One very promising TLOAF funded project is working with FDA to get this treatment into clinical trials.
Cystic Fibrosis

CF Research - The Legacy of Angels Foundation continues to support projects that improve the diagnosis of cystic fibrosis. Projects that explore better testing methods for newborns and second-tier sequencing for improving diagnosis of CF by lessening the false negatives and false positives will provide families with a solid diagnosis.

Newborn Screening

NBS Research – The Legacy of Angels Foundation supported newborn screening projects to help in the diagnosis of both krabbes disease and cystic fibrosis. The foundation met with a Minnesota senator to discuss krabbes newborn screening and also attended the Minnesota Newborn Screening Advisory Committee meetings. TLOAF representatives Sue Rosenau and Micki Gartzke recently were appointed to the Duchenne Muscular Dystrophy Newborn Screening National Steering Committee, broadening exposure of TLOAF on a highly respected national committee advancing efforts for NBS.....the right way!

Additional Initiatives

Hormel Institute – Dr. Zigang Dong, Executive Director of the Hormel Institute gave us an overview and history of the Hormel Institute. We were treated to a tour of the research facility and discussed potential partnership opportunities.

We feel blessed to be on the cusp of such exciting and potentially life-saving research. To have a promising treatment that may dramatically improve the quality of life for patients with krabbes disease; to improve the diagnosis of cystic fibrosis; and to improve and discover better newborn screening methods for krabbes disease and cystic fibrosis are all goals that TLOAF sees becoming reality. It’s exciting times and The Legacy of Angels Foundation is thrilled to be a part of this research and looking forward to moving closer to these goals in 2016!

Sue Rosenau, Co-Founder
The Legacy of Angels Foundation
IT’S ALL ABOUT THE KIDS!
TOTAL GRANTS AWARDED BY YEAR

2009 – 2015 $9,998,255

- 2009 -- $500,000 2 grants
- 2010 -- $1,289,000 5 grants
- 2011 -- $1,576,253 7 grants
- 2012 -- $1,332,994 6 grants
- 2013 -- $1,475,013 9 grants
- 2014 -- $1,603,663 10 grants
- 2015 -- $2,221,332 15 grants

TOTAL GRANTS AWARDED BY PROGRAM IN 2015

% rounded to the nearest full percentage point*

- Krabbes = $1,487,240
- CF = $372,384
- NBS = $361,708

GRANT CYCLE

If our initial review of a grantseeker’s Letter of Inquiry indicates that there may be a priority in one of our focus areas, we work to learn more about the organization and its programs. If a grantseeker remains under consideration, our due diligence, application and grant cycle is:

- March 1 Letter of Inquiry Deadline
- April 1 Application Invitations
- June 1 Application Deadline
- June-July Application Review Process
- September Grant Approval and Award Announcements
- September-October Grant Disbursement
TOTAL GRANTS AWARDED: $2,221,332.00

A Prospective Study of Newborn Screening for Cystic Fibrosis Using A Novel IRT/Next Generation Sequencing Method
Mei Baker, M.D. -- University of Wisconsin

Global Correction of Krabbe Disease Via Combined Cell, Gene, and Neuroprotective Therapies
Ernesto Bongarzone, Ph.D. -- University of Illinois

Generation and Validation of New Humanized Krabbe Disease Mouse and Induced Pluripotent Stem Cell Models
Ernesto Bongarzone, Ph.D. -- University of Illinois

Lentiviral-mediated Hematopoietic Stem Cell Gene Therapy for Canine Globoid Cell Leukodystrophy
Allison Bradbury, Ph.D. -- University of Pennsylvania

Krabbe Disease Patient Care and Research (NDRD)
Maria Escolar, M.D. -- Children’s Hospital of Pittsburgh

Development Program for the Treatment of Krabbe Disease Using Intravenous Adeno-associated Virus Gene Therapy
Maria Escolar, M.D. -- Children’s Hospital of Pittsburgh

KTRN Annual Research Meeting
Maria Escolar, M.D. -- Children’s Hospital of Pittsburgh

High Accuracy Galactosylceramidase (GALC) Enzyme Assay for Second Tier Screening of Krabbe Disease
Michael Gelb, Ph.D. -- University of Washington
Comparison of Intrathecal AAV9, AAVrh10 and AAV-Oligo001 in Combination with Bone Marrow Transplant
Steven Gray, Ph.D. -- University of North Carolina at Chapel Hill

Low IRT Levels and Characteristics of Persons with False Negative Newborn Screening Tests for Cystic Fibrosis in the United States
Martin Kharazzi, Ph.D. -- Sequoia Foundation

GeneThink: A Revolutionary Educational Website/Application Providing Increased Access to Accurate and Updated Natural History, Diagnosis, and Treatment Information About Genetic Disorders
Dawn Laney, MS, CGC -- Emory University

Completion of a Study to Develop Efficient and Effective Screening Strategies for Lysosomal Storage Disorders, Friedeich Ataxia, Wilson Disease and X-linked Adrenoleukodystrophy
Dietrich Matern, M.D. -- Mayo Clinic

Identification of Drugs for the Treatment of Krabbe’s Disease
Magdalena Petryniak, M.D. -- Oregon Health and Science University Foundation

A Multi-Center Study of a New Method of Sweat Testing: The CF Quantum Sweat Test
Michael Rock, M.D. -- University of Wisconsin

The Paul M. Fernhoff Memorial Lecture Series
Robert Vogt, Ph.D. -- CDC Foundation
REFLECTIONS FROM BOB HOFFMAN
TLOAF Board Member

As I complete and look back on my first year as a member of The Legacy of Angels Foundation Board of Directors, I am so amazed at the scope and significance of the reach of TLOAF. In addition to personally supporting a variety of community activities, the Rosenaus truly focus on underwriting the research necessary to find an effective treatment for krabbes disease. The great strides made in the most recent eighteen months have been most remarkable. From supporting the Program for the Study of Neurodevelopment and Rare Disorders at the Children’s Hospital of Pittsburgh to the awarding of significant grants that underwrite the exciting research being done by Dr. Wenger, Dr. Escolar, and others, TLOAF is playing the major role in identifying and developing a treatment that will enable the diagnosed children to live a longer and a higher quality of life. It is so exciting to be a part of something that will soon bring hope to many.

I truly look forward to being a part of the team that will take this great mission to the next level!
Dr. Mei Baker is a leader in newborn screening (NBS). As a trained biochemical and molecular geneticist, Dr. Baker works in routine NBS functions, communicates the test results to primary care providers and newborn screening clinical consultants, and provides laboratory consultation on atypical testing results. Mei works closely with the medical director of the Department of Health Services to address multiple NBS related policies and procedures, such as updating NBS specimen collection procedure for NICU infants, evaluating the NBS system, and implementing the policy of NBS cards for every birth in Wisconsin. Nationally, Mei serves on the Association of Public Health Laboratories Newborn Screening Molecular Subcommittee; Laboratory Standards and Procedures Subcommittee for the Secretary’s Advisory Committee on Heritable Disorders in Newborns and Children and is a member and co-chair of the Association of Public Health Laboratories NewSTEPs (Newborn Screening Technical Assistance and Evaluation Program) Steering Committee.

Mei has been one of TLOAF’s grant recipients for the past several years. Her continuing effort and success in conducting research, and in translating that research into routine newborn screening practice, has further validated her dedication to maintaining, improving, and advancing the field of newborn screening, and solidify her scholarship and leadership position in newborn screening.

Mei’s contributions have been widely recognized. In 2014 she received the Harry Hannon Laboratory Improvement Award in Newborn Screening at the Association of Public Health Laboratories (APHL) 2014 Newborn Screening and Genetic Testing Symposium, held in Anaheim, California.

Dr. Baker was the principal investigator for a research project entitled “Improving IRT/DNA Newborn Screening for Cystic Fibrosis to Reduce False Positives by a New Molecular Strategy” funded by The Legacy of Angels Foundation. A Next Generation Sequencing (NGS) assay capable of simultaneously detecting 250 CFTR mutations using routine dried blood NBS specimens has been successfully developed. This research work was published in Genetics in Medicine. Currently Dr. Baker is working on another grant project that The Legacy of Angels Foundation awarded to evaluate the
usefulness of expanded DNA analyses using a panel of 250 CFTR disease-causing mutations in NBS for CF in a real-world NBS environment.

In addition to TLOAF funded projects, Dr. Baker serves as principal investigator for an ongoing NIH/NICHD funded project for “Establishing a Newborn Screening Process for Early Identification and Treatment of Infants with Pompe Disease.” Her responsibilities include organizing a monthly virtual interdisciplinary NBS pompe pilot study conference, and managing the database that records population screening test data and all presumed pompe screening positive case information. They have fully validated an enzymatic fluorometric assay to screen for pompe disease using digital microfluidic technology (DMF).

Dr. Baker is a scientific leader in SCID NBS. Mei is a co-author for an influential publication “Newborn Screening for Severe Combined Immunodeficiency in 11 Screening Programs in the United States” (JAMA, 2014). She is one of three expert advisors serving on an APHL awarded $4 million project through a two-year cooperative agreement with the Genetic Services Branch of the U.S. HHS. Dr. Baker has been working collaboratively with Dr. Marsha Mailick on FMR1 pre-mutation related projects. Their successful collaboration is evidenced by two recent publications (Am J Med Genetics Part B: Neuropsychiatric Genetics, 2012 and Am J Med Genetics, 2013). Furthermore, as a co-investigator, Mei continues to work with Dr. Mailick on a newly awarded NIH RO1 grant to characterize the FMR1 premutation phenotype in permutation carrier mothers of children with and without fragile X syndrome (FXS) diagnoses and controls.

Building on her seminal contribution to developing SCID (severe combined immunodeficiency) screening methods and processes that are amenable to the high throughput required by public health NBS laboratories, Dr. Baker continues to seek scientific improvements. She recently constructed a synthetic nucleotide that contains both T-cell receptor circle (TREC) and reference gene sequences named as SCID gBlock. Mei is in the final stage of validating a much-improved SCID screening assay using a combination of SCID gBlock and her invented DNA isolation method (The patent for this invention, claimed by WARF, has been granted in the US and Europe), which will have fewer false positives and simpler process.

The Legacy of Angels Foundation is excited about the research Dr. Baker has done and continues to do. She has become a world leader and widely recognized researcher in the newborn screening field.
This year’s Krabbes Translational Research Network (KTRN) meeting, was held March 4-6th, 2015 at ’Tween Waters on Captiva Island, FL. Recognizing previous progress and celebrating the 5th anniversary of this TLOAF signature grant sponsored meeting, a new dimension was added; a select group of parents of children born with krabbes disease. The purpose of this new group is to fill a much needed market gap, previously identified through extensive research.

Paul and Sue Rosenau Co-Founders, and TLOAF board members Stacy Pike and Bob Hoffman participated in this exciting meeting, along with TLOAF research consultant, Micki Gartzke.

The foundation keenly believes engaging families and other stakeholders in creating a family-centered network: to develop a patient advocacy group that can raise public awareness of krabbes disease and play a key role in all levels of educational opportunities and increase participation in studies of potential new treatments that could benefit affected children; and to form a governance structure and six subcommittees to address the most important topics for the network and develop tasks and deliverables for the meeting and the following 12 months. This new Family-Centered KTRN (FC-KTRN) will help to contribute research ideas, share data, adhere to protocols and participate in observational studies and randomized clinical trials for krabbes disease.

The foundation is excited to see the progress this group will make in all areas; especially now is the vital need for the Krabbes Tissue and Brain Repository which will be housed in the Pittsburgh Brain Bank, developed under the direction of Dr. Maria Escolar, et al.

The 2016 meeting will once again be held at ‘Tween Waters on Captiva Island, FL and will be a scientific meeting similar to those held in previous years.
Meeting with a number of TLOAF grantees and krabbes researchers is always a highlight! Paul Rosenau, Co-Founder, attended this year’s WORLD Symposium, along with Micki Gartzke, TLOAF research consultant. Together, they participated in the program of Basic Science and Translational Research presentations, The Council of Patient Advocates meeting, as well as taking in the vital poster sessions.

This annual NIH-funded event is designed to help researchers and clinicians to better manage and understand diagnostic options for patients with lysosomal diseases, identify areas requiring additional basic and clinical research, public policy and regulatory attention, and identify the latest findings in the natural history of lysosomal diseases.

The interdisciplinary forum of this meeting provides many unique and educationally important channels to the foundation. It explores and discusses specific areas of interest, research and clinical applicability related to lysosomal diseases, including krabbes disease.

“This meeting is like one great big KTRN meeting.”
~ PAUL ROSENAU, CO-FOUNDER
MEETING THE AMAZING MICE

A key meeting took place in April 2015 at Thomas Jefferson University in Philadelphia, Pennsylvania between The Legacy of Angels Foundation Co-Founders Paul and Sue Rosenau, research consultant Micki Gartzke and TJU LSD Lab Director, Dr. David Wenger, his outstanding team, and his amazing mice!

“To see a 300+ days old treated twitcher mouse swinging from the top of his cage, as if it were a jungle gym is no minor scientific achievement; and we saw plenty of that in the two days of meetings, exceptional success and excellent use of TLOAF funds,” commented Sue Rosenau. This is especially true when that performance is contrasted with an untreated twitcher mouse 200 or more days younger, dragging its hind legs and barely able to move.

In 2012, the foundation funded Dr. Wenger’s grant: “Intracerebroventricular and Intravenous Injections of AAVrh10-cGALC into the Dog Model of Krabbes Disease” for the purpose of moving research studies closer to human clinical trials for the treatment of krabbes disease. These very encouraging studies in the twitcher mouse model of krabbes disease are amazing and unprecedented. The gene therapy vector AAVrh10 was chosen because it has shown widespread distribution after IV, as well as distribution throughout other organs, including peripheral nervous system - a problem that needs to be fixed in people with krabbes disease.

This is a protocol that could be used in human patients since hematopoietic stem cell transplantation (HSCT) is the current standard of treatment for individuals with pre-symptomatic infantile krabbes disease. The study describing the successful treatment of twitcher mice with BMT plus a single IV injection of AAVrh10 has been published in the November 2015 edition of Molecular Therapy.

Dr. Wenger was most generous with his time and explained his work in great detail. His lab partners were as highly enthusiastic with their remarks on the project as well. The foundation is fortunate to work with such a wonderful group, achieving excellence and success with TLOAF grant dollars.
Camden’s Concert was held at the Hopkins Center for the Arts in Hopkins, MN on July 13, 2015. TLOAF Co-Founders, Paul and Sue Rosenau joined TLOAF board member Bob Hoffman and his wife Judy at the annual event.

Camden’s Concert is a fundraiser for thousands of kids like Camden as well as adults that suffer with cystic fibrosis. Camden is the son of Kirk and Chelsey Mona and the only grandchild of Dave and Linda Mona and Robert and Gail Carter.

Entertainment featured the Wright Brothers. They have been entertaining Minnesota audiences for many years with a repertoire that ranges from Elton John to the Eagles. RespirTech sponsored Camden’s 2015 concert. During the concert, the Wright Brothers performed Shake, Rattle and Roll while wearing the vests those with CF wear twice a day so they can breathe. Proceeds from a silent auction and the concert raised over $51,000 to help fight cystic fibrosis.

“The Mona’s do a wonderful job of organizing this event and the Wright Brothers provide great entertainment,” reflected Sue Rosenau, Co-Founder.
MEETING WITH NDRD AND HUNTER’S HOPE FOUNDATION

Key members of The Legacy of Angels Foundation team traveled to Pittsburgh in June to meet with Dr. Maria Escolar and her team at the Program for the Study of Neurodevelopment and Rare Disorders, as well as the Hunter’s Hope Foundation.

The foundation’s goal for the meeting with Hunter’s Hope was to determine how best to use all the assets of the krabbes market for the benefit of the children. TLOAF’s desired outcome was to identify some possible partnership opportunities.

TLOAF board member Robert Hoffman graciously agreed to facilitate the meeting and present TLOAF’s mission, vision and research portfolio. Ms. Jacque Waggoner from Hunter’s Hope Foundation presented their marketing kit for fundraising.

Using all the typical tools for reaching agreement - proposals were put forth. Listing and brainstorming, clarification, prioritizing and advocating led to what was to be a joint funding opportunity of an additional physician in Dr. Escolar’s clinic. TLOAF has provided grant funding to support this position and were hopeful that Hunter’s Hope could partner with The Legacy of Angels Foundation to support that position. This would greatly help Dr. Escolar in treating the krabbes patients along with patients with other leukodystrophies.

Unfortunately Hunter’s Hope Foundation was not able to provide funds for the partnership opportunity.
PAUL M. FERNHOFF MEMORIAL LECTURE SERIES
“A Miraculous New Era for Cystic Fibrosis: Impact of Molecular Screening and Therapy”

This year’s keynote speaker at the Paul Fernhoff Memorial Lecture Series was Philip M. Farrell, M.D. Ph.D. Dr. Farrell shared new and exciting information regarding important advancements in cystic fibrosis newborn screening. The groundbreaking cystic fibrosis work developed through a grant Dr. Farrell and Mei Baker received from The Legacy of Angels Foundation, provided the basis for this new improved life-saving methodology. This prestigious lecture series is a key feature of the CDC Foundation’s Newborn Screening Translational Research Initiative. The Legacy of Angels Foundation partnership with the CDC is important to TLOAF’s mission.

This event premieres the cutting edge work of the valuable research The Legacy of Angels Foundation has funded. TLOAF’s financial sponsorship for this event promotes our mission to an ever-broadening group of high-level key individuals in nationally relevant institutions and programs, both public and private.

The Legacy of Angels Foundation was promoted favorably by more than one of the speakers for its value to both the cystic fibrosis and the LSD community with its work in krabbes research. Attention was brought to the audience about the value and expansion of the TLOAF Research Grant Program. Attendees were encouraged to check out The Legacy of Angels Foundation website to learn more about the breadth and depth of the TLOAF grant work since inception.

TLOAF Co-Founders, Paul and Sue Rosenau and research consultant, Micki Gartzke participated. They attended both the evening reception the night before the lecture series as well as the lectures at Emory University.

Paul Fernhoff, for whom this lecture series is named, was the first Lysosomal Storage Disease geneticist. The Legacy of Angels Foundation met with in 2009. This meeting was in conjunction with TLOAF’s initial meetings with the Newborn Screening Branch and CDC Foundation at the Centers for Disease Control and Prevention in Atlanta, GA. Paul’s sudden death in September 2011 shocked and saddened the LSD community worldwide.
2015 FOUNDATION EVENTS

CYSTIC FIBROSIS BREATH OF LIFE GALA

The Minnesota Cystic Fibrosis Breath of Life Gala was held Saturday, November 21, 2015 at the Hyatt Regency in Minneapolis, MN.

TLOAF board member Bob Hoffman and his wife Judy represented The Legacy of Angels Foundation. Others attending included: TLOAF financial advisor, John Priebe; TLOAF accountant, Jared and Stacy Dufault; Aaron and Tina Wirtz; and Dale and Cindy DeRaad.

The 2015 Breath of Life Gala received the financial support of more than 30 sponsoring companies. This event drew nearly 600 attendees, ranging from corporate sponsors to CF family members to CF patients.

The evening began with silent and live auctions followed by dinner and an evening hosted by award winning and former Minneapolis WCCO news anchor, Don Shelby. The gala raised over $800,000.
BOARD OF DIRECTORS

Paul Rosenau
President and Co-Founder

Sue Rosenau
Vice-President and Co-Founder

Stacy Pike/Brett Rosenau
Secretary

Heather Techmeier/Paul Rosenau
Treasurer

Robert Hoffman
Board Member

CONSULTANTS

Micki Gartzke
Research Consultant

John Priebe
Financial Advisor

Jared Dufault
Foundation Tax Advisor

OUR MISSION

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

OUR VISION

Is to direct, fund and promote research to develop and enhance treatments and cures for Krabbes disease and Cystic Fibrosis.

TLOAF will promote through education and awareness the expansion of Newborn Screening, Krabbes disease and Cystic Fibrosis; benefitting children with the increased probability of early identification and effective access to treatment.