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Five years have now passed since we began our philanthropic endeavors with the creation of The Legacy of Angels Foundation. During this span of time, the Foundation has funded more than $6 million dollars to 18 grantees undertaking potentially life-saving research projects in our programmatic areas. As we reflect back, we are generally pleased with both the accomplishments of our grantees and the grantmaking process that has guided our efforts.

In many respects, five years is a short time for a private Foundation to establish itself in the philanthropic community. While we have gained a great deal of experience over these first five years, we remain convinced that we still have many research hurdles in front of us and the path that we continually strive to achieve needs to be pushed forward, persistently.

As there is a dynamic tension by having more grantee requests than available funding, this favorable environment should continue to foster an aggregate outcome consistent with our philanthropic goals.

Will we ever reach a steady state of grantmaking? We do not know, which is probably good, as a portfolio of grantees positive outcomes is akin to a portfolio of investments where the results will flourish over time with active and effective tending!

Our 5th anniversary year provided an excellent challenge from a grants planning and investment standpoint, one in which we made significant progress. Through this effort, we were able to confirm that our grant dollars contributed in helping many grantees produce notable and often impressive results of which they can be proud.

Currently, we are funding nine labs throughout the USA, many working collaboratively to find the necessary breakthroughs needed to advance the field of Krabbe’s disease research.
2013 proved to be another rewarding year for the work of The Legacy of Angels Foundation. More than a million and a half grant dollars were awarded to research projects that have the potential to positively impact the lives of children, born with Krabbe’s disease and Cystic Fibrosis, and their families.

Newborn screening has been at the forefront of discussion legislatively in many states to include screening for Krabbe’s disease in their newborn screening program. We continue to fund translational research projects in improved treatments and knowledge, to reach that objective to help advance the timeline that Krabbe’s will be added to the federal recommendations for Newborn Screening nationwide. While we believe the inclusion of Krabbe’s to states’ NBS program to be important and exciting, it is our belief that we need Krabbe’s on the national Recommended Uniform Screening Panel (RUSP). As it’s imperative for that to happen, a better treatment of this disease is necessary as well as a better understanding of all the mutations and the disease process, before giving false hope to families that have this tragic disease in their families. Research continues to improve current treatments and discover additional treatments and we are very hopeful that researchers are closing in on this goal.

“After losing a granddaughter to Krabbe’s disease, we know how important early detection is, but also recognize that with that diagnosis, an effective treatment is vital.”

**Total Grants Awarded By Year**
2009 - 2013

- 2013 -- $1,475,013  
  - 9 grants
- 2012 -- $1,332,994  
  - 6 grants
- 2011 -- $1,576,253  
  - 7 grants
- 2010 -- $1,289,000  
  - 5 grants
- 2009 -- $500,000  
  - 2 grants

**Total Grants Awarded By Program**
*%s are rounded to the nearest full percentage point*

- **2009**
  - $500,000 TOTAL
    - Krabbe’s (1)
    - CF (1)
  - 50%
  - 50%

- **2010**
  - $1,289,000 TOTAL
    - Krabbe’s (2)
    - CF (2)
    - NBS (1)
  - 58%
  - 22%
  - 20%

- **2011**
  - $1,576,253 TOTAL
    - Krabbe’s (4)
    - CF (2)
    - NBS (1)
  - 69%
  - 18%
  - 13%

- **2012**
  - $1,332,994 TOTAL
    - Krabbe’s (4)
    - CF (1)
  - 77%
  - 23%

- **2013**
  - $1,475,013 TOTAL
    - Krabbe’s (8)
    - CF (1)
  - 86%
  - 14%
In 2013, the Foundation funded $1,475,013 in grants for the following:

Krabbe’s Translational Research Network (KTRN) Annual Meeting
Maria Luisa Escolar, M.D. – University of Pittsburgh Medical Center, Pittsburgh, PA

Krabbe’s Translational Research Network (KTRN) Collaborative Lab
Maria Luisa Escolar, M.D. – University of Pittsburgh Medical Center, Pittsburgh, PA

Program for the Study of Neurodevelopment in Rare Disorders
Maria Luisa Escolar, M.D. – University of Pittsburgh Medical Center, Pittsburgh, PA

MRI for Program for the Study of Neurodevelopment in Rare Disorders
Maria Luisa Escolar, M.D. – University of Pittsburgh Medical Center, Pittsburgh PA

Improving IRT/DNA Newborn Screening for Cystic Fibrosis to Reduce False Positives by a New Molecular Strategy
Mei Baker, M.D. – University of Wisconsin, Madison WI

UK Krabbe’s Disease Scientific Workshop - Travel Grant for International for Krabbe’s Disease Expert
Pat Roberts – Save Babies Through Screening Foundation UK

Identification of Drugs for the Treatment of Krabbe’s Disease
Magdalena A. Petryniak, M.D. – Oregon Health Sciences University, Portland OR

Novel Approaches to the Treatment of Krabbe’s Disease
Mark Noble, Ph.D. – University of Rochester Medical Center, Rochester NY

Intracerebroventricular and Intravenous Injections of AAVrh10-CGALC into the Dog Model of Krabbe’s Disease
David Wenger Ph.D. – Thomas Jefferson University, Philadelphia, PA

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 grant seeker remains under consideration, our due diligence, application and grant cycle is:

March 1  Letter of Inquiry Deadline
April 1   Application Invitations
June 1    Applications Deadline
June-July Applications Review Process
August    Grant Approval & Award Announcements
September Grant Disbursement
The 3rd Annual Krabbe’s Translational Research Network (KTRN) meeting was convened through a grant from TLOAF at the beautiful historic Biltmore Hotel. Maria L. Escolar, M.D. the world-wide recognized premier physician who directs The Program for the Study of Neurodevelopment in Rare Disorders (NDRD) at the University of Pittsburgh Medical Center and has created a Virtual Medical Home and Clinic for Children with Krabbe’s Disease located on the web at: http://ndrdvirtualmed.com/index.shtml, hosted the meeting.

More than twenty-nine doctors, researchers, clinicians, consultants, advocates, and family members participated.

For nearly three full days, the Krabbe’s Translational Research Network, a consortium of the leading scientists and clinicians who are dedicated to reducing the time it takes for research discoveries to become clinical therapies, met to fight the rare childhood disease, Krabbe’s. Along with related world-renowned experts and clinical practitioners, these members work together, integrating knowledge from clinical experience and basic science and developing unique studies to bring the latest research into the clinic where it can improve the lives of children with Krabbe’s Disease. The goal of the latest meeting, which was held in the inspiring surroundings of the historic Biltmore Hotel in Coral Gables, Florida, was to share knowledge about Krabbe’s disease by increasing communication among researchers and identifying specific projects that have the potential to develop into new therapies for patients. Some of the important topics that were addressed included: Natural Progression and Treatment Outcomes of Krabbe’s Disease, Unresolved Questions; Hematopoietic Stem Cell Transplantation for Krabbe’s Disease;
Neuroimaging, What a Scan Shows and Does Not Show; Molecular Mechanisms of Demyelination and Gene Therapy in Peripheral Nerve Disease, Combining Cellular and Molecular Analysis with Unbiased Drug Discovery to Identify Potential New Treatments for Krabbe’s Disease; Combination Therapy.

When asked to summarize the 2013 KTRN meeting Dr. Escolar noted, “We have now several laboratories that in the past worked independently, successfully collaborating and sharing knowledge towards improving therapy. The experiments and the challenges have been discussed, resulting in a more in-depth understanding of Krabbe’s disease. Clinicians have helped basic scientists refocus their research to make their experiments more relevant to clinical challenges that will ultimately benefit patients.”

WORLD LYSOSOMAL STORAGE DISORDER CONFERENCE
Orlando, FL February 12-15, 2013

Co-Founders, Paul and Sue Rosenau, TLOAF Board Member, Stacy Pike and TLOAF Consultant, Micki Gartzke participated in the 9th annual WORLD. This conference is co-presented by the Lysosomal Disease Network and the National Institutes of Health and is a multidisciplinary forum presenting the latest information from basic science, translational research, and clinical trials for lysosomal diseases. Dr. Maria Escolar was one of the key presenters and poster exhibitors. Maria’s presentation was “DTI as a Test for Early Infantile Krabbe’s Disease.” The presentations at this conference cover many of the lysosomal disorders. It is a good conference to meet and hear other researchers who work on diseases that align closely with Krabbe’s. It also included presentations on Newborn Screening of some of the LSDs and the effects of adding more of these diseases to the NBS panel. The next WORLD LSD conference was held in San Diego, February 10-13, 2014.
50TH ANNIVERSARY OF NBS
Atlanta, GA

It was an exciting honor to be invited to celebrate what many consider to be the most effective Public Health Program in the history of the United States, Newborn Screening. Co-Founders Paul and Sue Rosenau, TLOAF board member, Stacy Pike and TLOAF consultant, Micki Gartzke attended the 50th Anniversary of Newborn Screening Celebration at the Joint Meeting of the American Public Health Labs Association and the International Society for Neonatal Screening: Newborn Screening and Genetic Testing Symposium held in Atlanta Georgia May 5-10, 2013. Presentations included a wide variety of high-level important aspects of Newborn Screening, including its history; common issues and solutions in NBS; quality assurance/quality control methods and developments; implementing new conditions to NBS; financial, ethical, legal and social issues; electronic data systems, etc. Also included were patient/parent panels and how their diseases were diagnosed and their treatments. Highlights included presenters including Mei Baker from the Wisconsin State Laboratory of Hygiene speaking on her project “Improving IRT/DNA Newborn Screening for Cystic Fibrosis to Recue False Positives by New Molecular Strategy.” This is a TLOAF-funded project and we were gratified with the generous credit and acknowledgements given to TLOAF at the conclusion of her presentation. Another highlight was a tour of the Centers for Disease Control, where we we shown many quality assurance/quality control methodologies that are vital to effective and efficient Newborn Screening. The conference also included poster and manufacturer exhibits. During the award luncheon, another TLOAF grantee Dr. Phil Farrell received the prestigious George Cunningham Visionary Award. This was a very fitting and deserved award for all the visionary work Dr. Farrell has done to improve care for those affected by CF.

WISCONSIN NEWBORN SCREENING (50TH NBS ANNIVERSARY CELEBRATION)
Madison, WI

Micki Gartzke represented The Legacy of Angels Foundation at the Wisconsin 50th NBS Anniversary Celebration, July 12, 2013 at the Wisconsin State Newborn Screening Lab in Madison Wisconsin. The meeting was co-presented by the State of Wisconsin Dept. of Hygiene - Newborn Screening Lab and the University of Wisconsin. This collaborative effort made for a memorable event: speeches about the importance of NBS, and celebrating the 50 years of its success, illuminated that possibly Newborn Screening is the most effective public health program in the history of the United States. Additionally, a large segment of the program was an honorary tribute to Dr. Gary Hoffman, the world-renowned WI NBS Lab Director, who recently retired. This hour-long portion of the presentation was emceed by TLOAF grantee Dr. Philip Farrell, and of note was a tribute presentation by Dr. Harry Hannon, CDC NBS Lab Director, Emeritus. Additionally, TLOAF grantees Dr. Michael Rock and Dr. Mei Baker participated. Tours of the WI NBS Lab were provided and a highlight was the Cystic Fibrosis area, where a recent screen positive was identified and a call placed out to the newborn’s hospital to arrange for prompt follow-ups.
Newborn Screening for Krabbe’s Disease Issues and Recommendations—Hunter’s Hope Medical Symposium

Micki Gartzke represented The Legacy of Angels Foundation at the “Newborn Screening for Krabbe’s Disease Issues and Recommendations,” hosted by Hunter’s Hope during its annual Family and Medical Symposium, July 23–24, 2012 in Ellicottville, NY. This two-day meeting focused on the issues that have arisen since Krabbe’s disease Newborn Screening was started in New York State, August 7, 2006, by the Executive Order of then Governor George Pataki. It was reported that since New York Krabbe’s NBS started, five cases of early infantile Krabbe’s disease were identified and referred for transplant. Four of the five infants transplanted. Only one of those children is currently alive.

Per the opening remarks of Patti Duffner, retired Director of the Hunter James Kelly Research Institute, “this meeting is totally dedicated to NBS.”

Some of the important topics of discussion included:

• The risks and gaps in knowledge surrounding newborn screening. Including, but not limited to, that parents continue to advocate for state by state Krabbe’s NBS and it is not helping and increases the risk for future harm. (This was further emphasized by a presentation by the Hunter’s Hope Newborn Screening Director who went over all the tools and support HHF can offer to support parents who advocate in their state for Krabbe’s NBS.
• The near future need for a multi-state collaboration that will need funding
• Today’s technological problems, for example today Missouri is mandated to screen for Krabbe’s, but does not have the capacity, thus outsourcing to New York. Currently a child, if identified with Infantile Krabbe’s cannot be identified in time to transplant on the already well accepted necessary timeline.
• Untruthful press in different states that have been pressured to add Krabbe’s to NBS.
• Improved confirmatory testing is imperative per Dr. Rodney Howell
• No existing proficiency testing or quality assurance exists
• Limitations of commercially available substrates
• Standardization topics are numerous and a robust discussion with all engaged experts identified some next steps.
• Psychosine assay development collaboration with Maria Escolar, Dieter Matern and Joe Orsini was discussed and should be executed this year
• Dr. Escolar stated that she is following 105 children with Krabbe’s Disease and has done 340 evaluations; has a large Database now
• Dr. Escolar stated that she published a staging system for Krabbe’s in 2006
• Dr. Escolar is dispersing information for others to use 3T Siemens DTI for staging disease, training others, this will help as NBS expands for more accurate diagnosis of the stage of disease progression.

The Legacy of Angels Foundation was well represented as a number of scientific presentations were made by TLOAF grantees and it was wonderful that these presenters made direct gratitude acknowledgements thanking The Legacy of Angels Foundation for its direct impact and current unparalleled funding support to make scientific advancements in the fields of basic, clinical and translational research of Krabbe’s disease. We are grateful to see results coming forward from our ever expanding research portfolio.
Minnesota Breath of Life Gala

Paul and Sue Rosenau along with consultant, Micki Gartzke attended the annual Cystic Fibrosis Breath of Life Gala on Saturday, November 16, 2013 held at the Hyatt Regency in Minneapolis. This always amazing event is an opportunity to connect with leaders from the Minnesota chapter of The Cystic Fibrosis Foundation and also with patients and families affected by CF. This is a premier Cystic Fibrosis Foundation fund-raising event; with nearly one million dollars raised that night. The Legacy of Angels Foundation continues to partner with The Cystic Fibrosis Foundation and researchers of CF to fund research. Through these joint efforts, the life expectancy of a child with CF has doubled in the last 30 years. For the first time ever, a drug – Kalydeco – is now available to treat the underlying symptoms of CF in a small group of people with this disease.

Reflecting Back

Reflecting back on 2013, we are grateful for all the opportunities and events that The Legacy of Angels Foundation has been able to fund and participate in as we expand into the future. In looking forward to 2014, we are energized by the bounty of possibilities that lie ahead as we strive toward our goals!

“We wanted to focus our philanthropy on the education and awareness of this disease and ultimately help fund research for better treatments and improved health outcomes for those now and in the future with Krabbe’s disease.”

~Sue and Paul Rosenau
BOARD OF DIRECTORS

Paul Rosenau
President and Co-Founder

Sue Rosenau
Vice President and Co-Founder

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Heather Techmeier/Paul Rosenau
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Dale DeRaad
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CONSULTANTS

Micki Gartzke
Research Consultant

Jared Dufault
Foundation Tax Advisor

John Priebe
Financial 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 Krabbe’s 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 Krabbe’s disease and Cystic Fibrosis.

TLOAF will promote through education and awareness the expansion of Newborn Screening, Krabbe’s disease and Cystic Fibrosis; benefiting children with the increased probability of early identification and effective access to treatment.
moving AHEAD

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