

Special Edition • August 2018



This edition is dedicated to the memory of Ted Wolf and Elsie María Hernández Saborío



2018 Life Fest Celebration
GIST Awareness Day

Reflections from our Executive Director

We have some unfinished business.

Since our initial founding in the year 2000 around a kitchen table, we have had extraordinary success in improving the survival and well-being of patients with GIST. The advent of Gleevec, followed by Sutent and Stivarga, have provided physicians with three FDA approved drugs. When combined with mutational testing that facilitates the choice of drug and dosage, these patients now have a trio of effective treatments that have opened the doors to still more personalized drugs in development.



On a parallel front, we have developed real world patient-driven databases that have contributed new data on side effects and drug selection. Our patient registry, backed by a tissue bank at Stanford University, has facilitated clinical trial development and recruitment. Our side effects management tool, SideEQ, has opened the door to an unprecedented collaboration bringing Pharma, the FDA, and the Life Raft Group into the same space. Many other cutting-edge collaborations have removed the barriers to sharing life-saving information.

But we have some important unfinished business. There is a subset of GIST which particularly affects children and young adults with a resistant form of this cancer for which the current battery of drugs do not work well. Because the number of

these patients is comparatively small and the nature of the cancer comparatively complex, progress has been difficult.

What to do? Suppose we brought together specialists from prestigious institutions and advocacy groups that shared our passion for helping these young patients to survive, forming a consortium to collaborate in finding effective new treatments and eventually a cure?

Experts such as Dr. Jonathan Trent, Sylvester Comprehensive Cancer Center; Dr. Venkata Ramesh Bulusu and Ruth Casey, PhD, Cambridge University Hospitals; Drs. Suzanne George and Katherine Janeway, Dana-Farber Cancer Institute; Eyal Gottlieb, MSc, PhD, Israel Institute of Technology; Dr. J. Keith Killian, Foundation Medicine; Dr. Michael Heinrich, OHSU; Dr. Lee Helman, Children's Hospital Los Angeles; Dr. Michael LaQuaglia, Memorial Sloan Kettering Cancer Center; Dr. Jason Sicklick, USC, San Diego; and Drs. Fernanda Arnaldez, Markku Miettinen, Karel Pacak, and Constantine Stratakis from the National Institutes of Health.

And patient advocates who on a daily basis work to help this vulnerable population such as Jayne Bressington from GIST Support UK; Phyllis Gay and Becky Owens of GIST Support International and SDH-Deficient Cancer Research Advocates; Sara Rothschild, Senior Director of Program Operations for the Life Raft Group and myself.

Well, we just did. Along with the incredible gathering of patients and caregivers, GIST experts and researchers at Life Fest, and amid the excitement of our successful first GIST DO IT Walk, we assembled this extraordinary convocation of experts in Pediatric/Wildtype/SDH-Deficient GIST to begin what may prove to be the incubator for effective treatments for our youngest patients.

In a conversation at Life Fest with Dr. Lee Helman, he reminded me that the idea for the NIH Pediatric Wildtype GIST Clinic was born through conversations at ASCO in 2006, with him, Dr. Alberto Pappo and myself. The clinic, which has become an extraordinary resource for patients and their families, was Phase I. In Phase II, we hope to take it to the next level, taking care of this vital unfinished business.

Let it be so.

- Norman Scherzer



Pediatric GIST patients presenting the Jeroen Pit Science Award with Norman to the NIH



July 13th 2018

GIST Awareness Day is celebrated on July 13th every year. It was created to raise awareness about GIST.

The theme this year is GIST DO IT. Around the world, GIST Awareness Day was celebrated with educational seminars and celebrations with our GIST DO IT theme.



Chile



Kenya



Bethpage, NY



Blueprint Medicines Team,
GIST DO IT Walk, Miami, FL



Japan



Hungary



Deciphera Team, GIST DO IT Walk



India

Life Fest 2018 Miami July 13th-15th



By Diana Nieves, Outreach & Engagement Director

The Life Raft Group was proud to celebrate the 9th anniversary of our biennial Life Fest conference. At this unique event, patients and caregivers have the opportunity to gather for a weekend of camaraderie and access to medical and research leaders in the field of GIST. Each meeting brings a wealth of new information about the latest advancements, treatments, and side effects management that patients eagerly await to learn.

This year's Life Fest conference was held July 13th-15th at the Downtown Miami Hilton in Florida. It was a spectacular event filled with inspirational stories, laughter, and tears shared by GISTers, caregivers, medical professionals, pharmaceutical representatives, staff, board members, and many friends. Over 180 people participated throughout the weekend.

We are grateful to our Board of Directors and donors who made possible the Life Fest Scholarship Fund again this year. Scholarships were awarded to families who needed help to participate in Life Fest. Almost \$20,000 was raised to provide 28 families with support to attend the conference.

We are so grateful for our expert speakers and presenters who shared with us not only their knowledge but also their dedication and passion for the GIST community.

“The best part of the weekend was the support, camaraderie and new relationships I created with other GISTers.”



Left to right: **Jon Treder, Norman Scherzer, Jerry Cudzil**



Left to right: **Vicki Ossio, Carolina Ponce, Sara Rothschild, Michael Josephy**



Left to right: **Tammy Wolf, Dr. Suzanne George, Ted Wolf**

“The involvement of so many clinicians has been inspirational!”

The weekend kicked off with a GIST 101 session followed by an informal gathering for all the participants on Friday evening. Our weekend was filled with general sessions designed to inform, educate and provide tools for patients and caregivers alike. Saturday morning's main program consisted of two tracks – one for the adults and another for pediatric patients and their caregivers. The remainder of the weekend's program involved all of the participants.

This year's event included an expanded Pediatric/SDH-Deficient GIST track. Experts covered such topics as *Understanding Pediatric & SDH-Deficient GIST*, *Current Research Findings*, *The Genetics of Carney-Stratakis Syndrome and Carney Triad*, *What Every Patient Should Know About Paraganglioma*, and a Q&A with a panel of GIST specialists.

Separately, the leading experts in this rare subset of GIST convened for the first-ever research symposium to share their latest findings and individual research. This meeting resulted in the formation of the Pediatric & SDH-Deficient GIST Consortium. By pooling resources and sharing data, the Consortium hopes to make significant progress in solving the puzzle of SDH-driven tumors and to develop better treatment strategies.



Valuable sessions were held for patients & caregivers



Left to right: JILL and JASON DeLORENZO, DAVID JOSEPHY, MICHAEL JOSEPHY

The Pediatric & SDH-Deficient GIST Consortium



Front row, left to right: Katherine Janeway, MD, Dana-Farber Cancer Institute; Dr. Ruth Casey, PhD, Cambridge University Hospitals, UK; Jonathan Keith Killian, MD, PhD, Foundation Medicine; Suzanne George, MD, Dana-Farber Cancer Institute; Dr. Jonathan Trent, MD, Sylvester Comprehensive Cancer Center; Eyal Gottlieb, MSc, PhD, Laura and Isaac Perlmutter Metabolomics Center, Technion – Israel Institute of Technology, Israel; Phyllis Gay, GIST Support International; Becky Owens, SDH-RA Cancer Research.

Back row, left to right: Karel Pacak, D.Sc., MD, PhD, NIH; Michael P. LaQuaglia, MD, Memorial Sloan Kettering Cancer Center; Constantine Stratakis, MD, NIH; Jason Sicklick, MD, Moores Cancer Center, UCSD; Fernanda Arnaldez, MD, NIH Pediatric and Wildtype GIST Clinic; Michael Heinrich, MD, Knight Cancer Institute, OHSU; Venkata Ramesh Bulusu, MD, Cambridge University Hospitals, UK; Lee Helman, MD, Children's Hospital Los Angeles; Jayne Bressington, PAWS-GIST Patient Director, GIST Support UK; Markku Martti Miettinen, MD, National Cancer Institute; Norman Scherzer, Executive Director, The LRG; Yu Wang, Data Scientist, The LRG; Sara Rothschild, Programs Operations Senior Director, The LRG.

Life Fest 2018 Honorees

By Laura Occhiuzzi, Outreach & Engagement Senior Director

Jeroen Pit Science Award - Dr. Fernanda Arnaldez and the NIH Team - Presented by Norman Scherzer

The Jeroen Pit Science award was presented to the NIH Pediatric & Wildtype GIST Clinic for their dedication, passion, and contribution to research. It was an emotional presentation as many of the GIST Kids that they treat helped Norman Scherzer present the award. These amazing physicians serve as a model of support for the youngest and rarest GIST patients through education, support, and advocacy efforts.



Clinician of the Year Award - Dr. Suzanne George - Presented by Ted Wolf

In honor of her unique approach to bringing research into her practice while maintaining support for her patients throughout the continuum of care, Dr. Suzanne George was presented with the Life Raft Group 2018 Clinician of the Year Award by her patient, Ted Wolf. The Wolf family traveled from across the country to watch Ted present the award. Dr. George accepted with open arms. Tears of joy and gratitude on both parts made for a very special moment. Dr. George is personally responsible for increasing patient survival. Her dedication to her patients and her work in research has impacted the GIST community tremendously.

GIST Center of Excellence Award - Sylvester Comprehensive Cancer Center - Accepted by Dr. Jonathan Trent - Presented by Ron Agypt

We are so grateful for their commitment to the GIST community and to the LRG. As a university-based cancer center, Sylvester physicians and scientists apply research breakthroughs from the laboratory to the patient's bedside. They closely evaluate the effectiveness of new treatments, and share clinical insights with laboratory investigators, leading to improvements benefiting patient care. This translational research is the cornerstone of their comprehensive cancer center, providing Sylvester with the ability to accurately treat GIST from stage one to stage four. Patients also have the opportunity to participate in groundbreaking clinical trials that offer new treatment approaches and help the scientific community answer questions about new ways of using existing drugs to fight GIST.



Allan Tobes Volunteer of the Year Award -

The MacLea Family and the Dillon & Morales Families - Presented by Erin MacBean

Michelle and Nancy Maclea have taken on a role this past year that they never thought they would. As a mother and grandmother, their determination to make a difference has been unwavering. Increasing awareness and spreading the word across the country has been their mission. From west coast to east, both Michelle and Nancy have raised close to \$20,000 for SDH research for the Life Raft Group. They have told their story - handing out Life Raft turquoise wristlets to everyone and anyone who would listen. Their support system of friends and family have joined in the cause. Michelle and Nancy are 'moms on a mission' - to increase survival, to get the word out, and to raise funds so researchers can do what they do best. The Life Raft Group is truly grateful to them and for all their time and effort.



Nikki Morales is a true friend to the Life Raft Group. She is a champion who has rallied her friends and family to spread the word all in the name of research. Nikki personally raised almost \$20,000 for SDH research and has helped to increase awareness along the way. Her use of social media has increased her reach - from her best friend Andrea to her sorority sisters - they have all contributed to her cause. Her husband John, her mom Jana, and dad Doug are her biggest cheerleaders. Nikki epitomizes the meaning of a volunteer. She is currently our Arizona state leader and is always willing to help her patient community. She was instrumental in helping organize our Scottsdale GIST Day of Learning this winter and jumped at the chance to help at Life Fest. We, at The Life Raft Group, are so grateful to Nikki for her dedication, her passion and her commitment.





By Chelsea
Gottfried,
Patient Registry
Associate

Our first ever GIST DO IT Walk took place on the morning of July 15th, 2018 at Margaret Pace Park in Miami, Florida. Patients, caregivers, doctors, friends, families, and virtual participants walked to raise funds for research to find a cure for GIST.

There were over 100 participants at the GIST DO IT Walk. Fifty percent of the proceeds will be donated to Sylvester Comprehensive Cancer Center.

Thank you to everyone who participated. Your passion is inspiring! It is moving to be surrounded by a community affected by GIST, which brings hope, comfort, and a feeling of togetherness.



On left -

A very special award was given to Ted Wolf during the first GIST DO IT Walk, on Sunday morning, for both his courage and philanthropy.



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We deeply appreciate all those who made this event a success!

A grand thank you goes to our corporate sponsors. Without their sponsorship this weekend would not have been possible. Much gratitude goes to our volunteer photographer and videographer - Don Edgar and Van Russell. And many, many thanks go to several other volunteers* and staff who gave so much of their time, energy and talent to making this a fantastic weekend for all.

**Volunteers - Anthony Cashin, Michael DeStefano, Julie Durkee, Carolyn DeWalt, Sam Evans, Erin MacBean, Hannah Marcus, Karen Meyers, Marlene Nei, Alyssa Occhiuzzi, Carolina Ponce, and Kay Stolzer*

Thank you to our Major Donors for May, June, and July.

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Questions or comments? Please contact Mary Garland, Communications Director, mgarland@lifteraftgroup.org
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