

# *Ensuring no one has to face GIST alone*

In Loving Memory: John Lundon, Garnette Draper, John Henry Adams, Ted Wolf, Elsie María Hernández Saborío

## **The Healing Power of Adventure:** **How First Descents Helps Young Adults Impacted by Cancer**

By **Liz Skree**, LRG Contributor

A cancer diagnosis at any age is life-changing, daunting, and difficult. It can be especially challenging for young adults, people ages 18-39, who are dealing with cancer while also navigating college, first careers, new relationships, starting families, and other young adult milestones.

Every year, 70,000 young adults are diagnosed with cancer, and that doesn't include those diagnosed as children who are now young adult survivors and thrivers. As a young adult with cancer, I know it can be hard finding other people who "get it."

▶ see Healing on page 4



## **The Magic of Clinical Trial Math** **How to Make $2/3 = 1$ (100%)**

by **Jerry Call**, LRG Data Analyst

In a recent discussion about the frequency of mutations in GIST, we noted that the most common mutation is KIT exon 11, which makes up about 2/3 of all GISTs (LRG data). We also noted that in the original phase II trial, this percentage might have been as high as 85 percent.

These mutations are very responsive to Gleevec. In fact, the average KIT exon 11 patient with advanced/metastatic disease will remain progression-free for about 31 months (2.5 years, LRG data).

▶ see Magic Math on page 5

## **New Horizons Convenes in Vienna**

By **Denisse Montoya**, Director, Patient Registry

On September 6<sup>th</sup>, Executive Director, Norman Scherzer and I attended the New Horizons GIST meeting in Vienna, Austria. Forty-three delegates, including GIST experts, advocacy groups, and patients/caregivers from 18 countries, gathered to share valuable medical information and scientific data regarding treatments.

The main goal of this conference is to unify and solidify collaborations among the GIST community, with the objective of discovering ways of increasing patient survival worldwide.

▶ see New Horizons on page 3

## A Few Words from Our Executive Director..



**Norman J. Scherzer**

It is 18 years since the Life Raft Group began on a few kitchen tables, and two years since I lost my soul mate, Anita.

I would like Anita's story to continue to inspire all those who struggle with GIST. Anita was given six months to live in 1994, but made it to 2000 after nine surgeries, two rounds of traditional chemotherapy and one round of radiation. But

time had run out. Anita was clearly dying. All that remained was for us to exchange our wedding vows once again (we had married when Anita was 19 and I was 21) so that Anita could say goodbye to our family and friends.

Fast forward to 2000 with Anita entering the first group of 30 in the Novartis clinical trial for Gleevec (then called STI-571). On day 28, an excited radiologist was to take me by the hand to see her films and to exclaim that he had never seen anything like this in his life: "half her tumors were gone and the rest were dissolving into liquid." Anita remained stable on Gleevec for 16 years only to die from a recurrent infection in 2016.

We have unfinished business, including the Consortium I spoke about at the Biden Cancer Summit recently to address those patients with SDH-Deficient GIST, a subtype that particularly affects children and young adults.

We have to continue the search for new treatments, and ultimately a cure, to overcome the resistance that stalks too many treatments.

In the interim, we intend to launch a new initiative to address the unacceptable reality that too many patients are dying and suffering unnecessarily because:

- They do not receive the diagnostic tests that have existed for many years to help target their treatments.
- They do not receive the specific treatments that are currently understood to work to halt or delay progression.
- They do not receive support to manage the side effects that accompany such treatments and often change over time.
- They do not take the drugs prescribed either because they cannot access them, or because they fail to take them (most new cancer treatments are oral drugs that need to be taken daily for long periods of time, often, years).

There are a number of reasons for this unacceptable situation, including the failure to recognize solutions and for patients to seek physicians with sufficient specialty knowledge to manage rarer types of cancer.

We intend to change this type of behavior because patients are dying too fast to do otherwise.

Stay tuned for new developments.■

*- Norman Scherzer*

## The LRG Welcomes New Board Member

By **Carolyn Tordella**, LRG Web and Design Associate

The Life Raft Group welcomed Ron Agypt to the Board this month. Ron is a retired insurance executive, father of three, and grandfather of five. Ron and his wife, Kim, have learned about GIST firsthand. After Ron's GIST diagnosis, he began treatment with Gleevec only to discover this typical first-line treatment was ineffective for him.

The couple searched for answers and contacted the LRG, which recommended mutational testing and a GIST specialist. Mutational testing

revealed that Ron was exon 9, which usually does not respond to a 400mg dose of Gleevec.

Ron is currently participating in a clinical trial run by GIST experts and doing very well. He and Kim believe the LRG's help was "critical to his survival." In turn, they are eager to help others who are facing a GIST journey and want to do whatever they can to help GIST patients – especially encouraging them to see a GIST specialist and advocating for mutational testing for all GIST patients.



**Ron & Kim Agypt**



## ► New Horizons continued from cover

The Life Raft Group presented the importance of Real World Evidence (RWE) and Real World Data (RWD) at the conference.

RWE utilizes observational data to determine the perceived benefit of treatments to increase survival and improve quality of life for cancer patients. The rich diversity of data collected from patients will yield more precise, better targeted, and therefore, more effective health care. RWD and the resultant RWE is being utilized to enhance and complement traditional research by providing a broader picture of the patient's GIST journey.

The power of data was truly shown during this meeting, but the real question was "How can we transform that data into something more powerful?" The answer is simple - collaboration. The importance of collaboration in the GIST community is crucial due to the rarity of this cancer. Unifying and collaborating with the scientific community, advocacy groups, and patients/caregivers will lead to a faster cure.

More subtypes of GIST are being discovered (such as SDH-Deficient GIST). Currently there is a lack of effective treatment for such subtypes. It is important to collaborate in order to strengthen and advance research for effective treatments for patients from subtypes that have been unresponsive to the current standard treatments. The Life Raft Group and a roster of leading GIST experts have created the Pediatric & SDH-Deficient GIST Consortium.

The objective of the Consortium is to identify at least one effective treatment within three years through initiation of new clinical trials for the Pediatric and SDH-Deficient GIST population.



Adjuvant treatment and metastatic disease were among other topics discussed, as well as new treatments for GIST. For years only three lines of drug treatments were known to be effective. However, with the advance of scientific investigations and clinical trials, more options are available for patients that failed the three standard treatment lines. Leading GIST experts who attended this conference were able to show many case studies of adults with advanced GIST who took a clinical trial drug that showed very promising outcomes. Such findings fill healthcare practitioners, patients, and caregivers with hope and optimism for the future of GIST treatments.

Participants also had the amazing opportunity to share information regarding the role of Psycho-Oncology in GIST, for patients and caregivers. Different ways of coping and helping to recognize the unique psychological needs of cancer patients were discussed, as well as the importance of safeguarding the psychological health of caregivers.

The New Horizons GIST Meeting was a great conference, with many things learned and shared by GIST experts and advocacy groups. Delegates from all over the world were present with the primary objective of collaboration. This is proof of the international dedication to patient advocacy and research. ■

## The Life Raft Group

**Who are we, what do we do?** The mission of the Life Raft Group is to enhance survival and quality of life for people living with GIST through patient-powered research, education and empowerment, and global advocacy efforts. The LRG vision is to champion patient-powered science and drastically increase long-term survivorship for all cancer patients.

**To accomplish this, we rely on four key pillars:**

- Leverage the patient perspective to drive innovative solutions in cancer research
- Educate and empower patients to take a larger role in their care
- Accelerate research outcomes through collaborative efforts
- Increase access to effective treatments worldwide

**How to help?** Donations to the Life Raft Group, a 501(c)(3) nonprofit organization, are tax deductible in the United States. You can donate by credit card at [lifaftgroup.org/donate](https://lifaftgroup.org/donate), or by check, mail to: The Life Raft Group, 155 US Highway 46, Suite 202, Wayne, NJ 07470.

**Disclaimer:** We are patients and caregivers, not doctors. Information shared is not a substitute for discussion with your doctor. Please advise Mary Garland, Director, Communications, [mgarland@lifaftgroup.org](mailto:mgarland@lifaftgroup.org), of any errors.

## ▶ Healing Power continued from cover



Photo credit: First Descents

I was diagnosed with GIST at age 18 and had my first surgery right before college. Like other young adults with cancer, I found a way to make it work. I switched campus jobs so that I wouldn't have to lift anything heavy after surgery. I scheduled CT scans around classes. I determined what foods I could eat without getting sick. I showed off my scar at parties. For the most part, I was a normal college student.

Soon after starting my first job in a new city, at age 22, I had surgery again. Now, more than 15 years after my initial diagnosis, my cancer is stable. But it can be hard for even close friends to understand what it's like to always live with cancer.

Luckily, there's a great organization that understands the unique challenges young adults impacted by cancer face and seeks to help them be more than their diagnosis, to get busy "out living it" and experience the healing power of nature and adventure.

First Descents is a nonprofit organization that takes young adults impacted by cancer on free outdoor adventure trips. The organization addresses what the National Cancer Institute identifies as a major determinant of long-term survivorship health: ongoing psychosocial supportive care. First Descents' programs help young adults with cancer improve their body image, self-compassion and self-esteem, while also helping reduce fatigue, depression, and alienation. In short, First Descents is amazing.

I was lucky enough to go on a "FD1" trip with First Descents in August 2016. FD1s are week-long programs for anyone ages 18-39 diagnosed with cancer after age fifteen. Participants can select from rock climbing, whitewater kayaking or surfing programs in numerous locations across the U.S.

My FD1 was a rock-climbing trip in Estes Park, CO. I'd never been rock climbing outdoors before in my life, and I was nervous about spending a week with strangers. But when my friend dropped me off at the cabin, all those fears went away and I felt at home.

At First Descents, everyone has a nickname. It can be a name you already had or a new one. But it's a chance to shed your former skin and be someone else for a week. I wasn't "Liz with cancer." I was Decoy. And it was empowering! When I was nervous on the rock, it helped to hear my new friends cheering me on, yelling "You can do it, Decoy!"

In addition to FD1 programs, First Descents has local groups in major cities around the country called "tributaries." These "FDtribs" provide weekend outdoor experiences for FD1 alumni and those new to First Descents. FDtribs go rock climbing, hiking, biking, kayaking and so on. First Descents also provides international adventure programs for FD1 alumni, called FDX.

I recently returned from FDX Italy, a 10-day adventure near the Dolomites. I was joined by 14 other young adult cancer survivors and thrivers, and we had an awesome time sailing, hiking, kayaking, rock climbing, caving, biking and the ultimate outdoor adventure experience:

a *via ferrata*\* in the Dolomites.

While our days were filled with outdoor adventures, our evenings were spent talking around the campfire. Campfire time is a special part

“The gratitude, love, and compassion shared there is invaluable. I didn't realize what I was holding onto within myself until I was able to share it out loud with others who understood.”

of any First Descents experience. The gratitude, love, and compassion shared there is invaluable. I didn't realize what I was holding onto within myself until I was able to share it out loud with others who understood, and I've made lifelong friends through First Descents.

If you're a young adult who's been impacted by cancer or know or someone who would benefit from a First Descents experience, visit [FirstDescents.org](http://FirstDescents.org) to learn more. ■

**\*A via ferrata (Italian for "iron path," plural vie ferrate or in English via ferratas) is a protected climbing route found in the Alps and certain other locations. The term "via ferrata" is used in most countries and languages except notably in German-speaking countries including Switzerland and Austria, which use Klettersteig (German for "climbing path," plural Klettersteige). (Source: Wikipedia)**



## ► Magic Math continued from cover

In the original phase II trial with more advanced patients, it was about 24 months. Most of those patients will remain on first-line Gleevec for about 45 months (3.8 years, LRG data). Half will do better than this; some much better.

Imagine - a treatment where effectiveness can be measured in years. For many cancers, the effectiveness of drugs that actually get approved are measured not in years or even in months, but in weeks!

### An Exercise in Thinking

Here is a fictitious example, based on what would actually happen if you did this: Imagine how powerful the effect could be when 2/3 of patients in a clinical trial have the type of response we noted above.

If you were comparing two groups of patients with the same makeup (2/3 KIT exon 11 mutations, 1/3 something else) and one group was given Gleevec and the other group a placebo, the Gleevec group would obviously do much better! The patients with the sensitive KIT exon 11 mutations would have such a great response that it would not matter how the other 1/3 of patients were comprised.

You could even place breast cancer patients in both groups (as the 1/3) and the Gleevec group would do better because of the exon 11 patients. Of course, the breast cancer patients in either group would not be getting any benefit, but as a group, the group with Gleevec would do better.

Present this group to the FDA and the drug could easily be approved for all patients in the group, including the 1/3 that may or may not have responded (if they were breast cancer patients, they would not have responded).

In reality, the trial that resulted in the approval of Gleevec did not have a placebo. In fact, Gleevec was so effective that there was really no comparison group at all (other than 400 mg Gleevec vs. 600 mg Gleevec). Very rarely is a drug approved without a comparison, but very rarely was a cancer drug as effective as Gleevec. However, the effect of 2/3 or more of the KIT exon 11 patients still existed and was the primary reason for the effect being measured in years.

What type of mutation do the other 1/3 of patients have?

About 10% of all GIST patients have a KIT exon 9 mutation. These patients likely had some benefit from Gleevec, but later data (from the phase III trials) would show that these patients did much better on a higher dose. Some other rare mutations, such as KIT exon 13 and PDGFRA exons 12 and 14 also are very likely to respond to Gleevec. Conversely, about 22% (LRG data) are not likely to respond.

### One Third Went for a Ride

The 1/3 rode along with the KIT exon 11 mutations straight to drug approval in the USA and around the world. Two thirds of patients with a “super response” equaled all patients in the trial being approved for the drug!

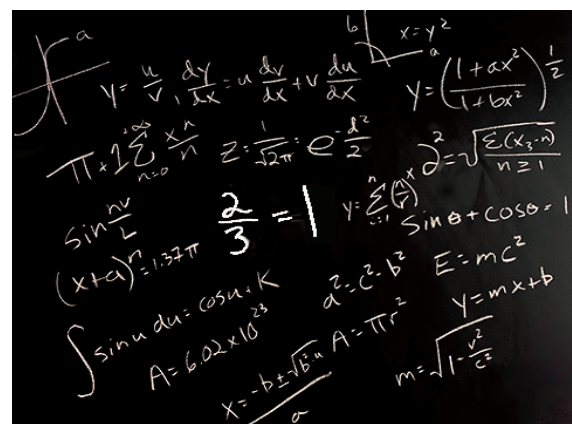
*This is the magic of math:  $2/3 = 100\%$ .*

Because early trials were focused on advanced/metastatic GIST with no other options than Gleevec, oncologists also went along for the KIT exon 11/Gleevec ride. They didn't bother to test for mutations since “almost all GISTs respond to Gleevec” and since there was nothing else to give them anyway.

This should have changed when the effectiveness of adjuvant Gleevec was finally proven (about 2011). Unfortunately, dogma had already set in; no need to test for mutations in GIST!

Never mind that 80% of GIST patients are diagnosed with a single tumor and many are candidates for adjuvant treatment. We know most GIST patients respond to Gleevec, so why test?

When you read anything about GIST, always remember the  $2/3 = 100\%$  ride. Are you in the 2/3 or the 1/3 that is going along for the ride?



Remember that the GIST conversation is driven by KIT exon 11 mutations. It's good to always question whether or not information you are receiving applies to you (even if it is coming from one of our patients).

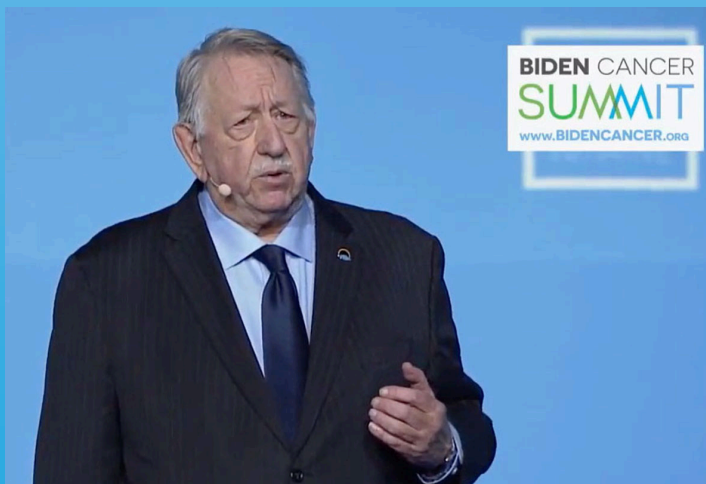
Much of what we know today has evolved over many years of learning more about GIST and how it responds to different drugs. I do not mean to imply that this (approval for all patients) was deliberate or that there was any malicious intent. It's just how things worked out.

On the other hand, what I did intend to be critical of was the practice of not performing mutational testing. It is simply not acceptable to continue to subject patients to ineffective treatments with our current understanding of GIST. Half of living LRG patients don't know their mutation. When 22% of them (LRG data) are not likely to respond to imatinib and 10% of them (exon 9) need a higher dose of imatinib, not performing mutational testing is no longer acceptable.■



On September 21<sup>st</sup>, 2018, Vice President Joe Biden and Dr. Jill Biden convened a national Biden Cancer Summit, with the anchor event in Washington D.C., and more than 450 Community Summits held where people live, work, learn, and worship around the country. The effort brought together thousands of people to focus national attention on the urgency of now to create actionable solutions in the fight against cancer.

“Today, we celebrate the survivors, the caregivers, the doctors, the nurses, the researchers, the scientists. We’re not here just to talk, we’re here to act. We’ve reached an inflection point in this fight against cancer.  
– Vice President Joe Biden



“We have more than doubled progress for patients living with GIST, with a major exception - Pediatric and SDH-Deficient GIST. There are no effective treatments for these patients. That is our unfinished business: Nobody wants to lose any more children.  
– Norman J. Scherzer

**View Norman’s presentation at the Summit: [bit.ly/LRGPresentation](https://bit.ly/LRGPresentation)**





“We will illuminate the emerging national narrative of how people turn their cancer fear into cancer fierce, told through the commitments – new efforts and collaborations – coming from across industries and sectors, and inspired by the call to action of our co-chairs Joe and Jill Biden. They, like so, so many in this community, have endured their worst nightmare and risen above it to lead a movement to double the rate of progress against cancer.

– Greg Simon, President, Biden Cancer Initiative

“If we never spent another dime on finding new cures, we could still save thousands more lives by doing one thing – sharing the knowledge that already exists.

– Dr. Jill Biden



The Life Raft Group was honored to have Norman chosen as one of the highlighted speakers during the Biden Cancer Summit to feature our Commitment to Doubling the Rate of Progress.

Norman introduced the newest initiative of the LRG, the Pediatric & SDH-Deficient GIST Consortium, the collaborative efforts of physicians, researchers and shared data to stimulate results to improve patient experience.



**Left to right:**  
**Dr. Jill Biden, Norman Scherzer, Sara Rothschild, and Vice President Joe Biden**  
*Photo credit: Chuck Kennedy*

# The Pediatric & SDH-Deficient GIST Consortium

## Our Commitment to the Biden Cancer Initiative

By **Sara Rothschild**, MPH, Vice President, Program Services

We are excited to launch an innovative initiative to strengthen and advance research for effective treatments of a rare subset of Gastrointestinal Stromal Tumor (GIST) patients, who have been unresponsive to the current standard treatments.

Historically, most researchers with an interest in Pediatric and SDH-Deficient GIST worked independently, with each seeing very few of these rare disease patients. There is a critical need for a model of collaboration for sharing data, tissue and resources with patient advocacy groups acting as the linchpin.

To incubate progress for this rare disease population, we have formed the Pediatric & SDH-Deficient GIST Consortium, a collaboration of experts with complementary skill sets to team with patients to discover targeted solutions. We aim to identify at least one effective treatment within three years as demonstrated by the initiation of clinical trials for the SDH population.

The initial focus will include: creation of a platform for shared data to answer key questions, establishment of a tissue bank and guidelines for clinical stakeholders, and the generation of cell lines. Expansion and initiation of studies and clinical trials will accelerate results. Access and outreach strategies will include training for community oncologists and educational webinars for patients and families worldwide. ■

“Anyone can bring together a consortium of people. The trick is to make the consortium work, and I think what helps in a situation is that a patient advocacy group like us, we become the honest brokers. We enable researchers who used to compete to share information.

– Norman J. Scherzer

## Members of the Pediatric & SDH-Deficient GIST Consortium

### Clinical/Research Partners

Fernanda Arnaldez, MD  
National Cancer Institute

Sosipatros Boikos, MD  
VCU, Massey Cancer Center

Venkata Ramesh Bulusu, MD  
Cambridge University Hospitals, UK

Ruth Casey, PhD  
Cambridge University Hospitals, UK

Suzanne George, MD  
Dana-Farber Cancer Institute

Olivier Giger, MD  
Addenbrookes' University Hospital, UK

Eyal Gottlieb, MSc, PhD  
Technion – Israel Institute of Technology, Israel

Michael Heinrich, MD  
Oregon Health and Science University

Lee Helman, MD  
Childrens Hospital Los Angeles

Katherine Janeway, MD  
Dana-Farber Cancer Institute

Jonathan Keith Killian, MD, PhD  
Foundation Medicine

Michael P. LaQuaglia, MD  
Memorial Sloan Kettering Cancer Center

Markku Martti Miettinen, M.D.  
National Cancer Institute

Karel Pacak, D.Sc., MD, PhD  
National Institutes of Health

Maria Pantaleo, MD  
University of Bologna, Italy

Alberto S. Pappo, MD  
St. Jude Children's Research Hospital

Joshua D. Schiffman, MD  
Huntsman Cancer Institute

Jason Sicklick, MD  
Moores Cancer Center, UCSD

Constantine Stratakis, MD  
National Institutes of Health

Jonathan Trent, MD  
Sylvester Comprehensive Cancer Center

Margaret von Mehren, MD  
Fox Chase Cancer Center

Christopher B. Weldon, MD, PhD  
Boston Childrens Hospital

### Patient Advocacy Partners

Dr. Victoria Bassett  
GIST Support UK

Jayne Bressington  
GIST Support UK

Phyllis Gay  
GIST Support International

Sara Rothschild, MPH  
The Life Raft Group

Norman J. Scherzer  
The Life Raft Group

Yu Wang, MB, PhD  
The Life Raft Group

Becky Owens  
SDH-RA Cancer Research Advocates



## In Memoriam

### Elsie María Hernández Saborío (1953-2018)

A tribute by **Michael Josephy**, LRG Contributor

My dear wife, Elsie María Hernández Saborío, passed away August 7<sup>th</sup> after a long battle with GIST. I miss her greatly.

Elsie was born and raised in Costa Rica. She grew up in San Ramón de Alajuela, where much of her extended family still lives. Her mother, Enilda, was a high school French teacher, her father, Sergio, a tailor with a small shop in downtown San Ramón.

Elsie studied mathematics education at the Universidad de Costa Rica (UCR). In 1973, she and her sister, Ana Isabel, moved to lodging near the campus to complete their degrees. After graduation, Elsie taught at public high schools before being hired by the mathematics department at the Instituto Tecnológico de Costa Rica (the Tec) in Cartago where she remained for the rest of her career. She was a well-loved professor, who won many teaching awards and was admired by her students.

Elsie and I met at events in the UCR mathematics department. After a short courtship, we were married in 1984 at the San Antonio Church in Guadalupe.

We have three children, Daniel, Sylvia, and Irene. Daniel (born 1986) studied at the Universidad Nacional in Heredia, earning a BA in English Teaching and two Masters degrees. He then completed a PhD in Translation Studies at the University of Ottawa. Sylvia (1988) graduated in medicine at the UCR, completed a Masters in Neuroscience at McGill University in Montreal, and is currently a neurology resident at UMass in Worcester, Massachusetts. Irene (1997) is in her final year as a political science major at the UCR.

Sylvia married her longtime boyfriend Bobby Giglio at Villa Blanca, near San Ramón, on July 28<sup>th</sup>, a date chosen long ago. Bobby is a New Jersey native. Many of his friends and family came from the States for the event. Elsie was there to enjoy the ceremony.

In 2000, Elsie was diagnosed with a gastrointestinal stromal tumour (GIST), a rare sarcoma.

During an operation in Costa Rica, the doctors found liver and peritoneal metastases. The prognosis was poor, with no effective treatments. But, just when we needed it, a new experimental drug, STI-571, was being tested, and in January 2001, with much help, we went to Columbia Presbyterian Hospital in New York City, where Elsie entered a clinical trial. She was assigned to the higher 800 mg dose. The medication was so effective that, a year later, it gained FDA approval and became the standard treatment under the brand name Glivec or Gleevec from Novartis. Elsie continued taking it faithfully.

After years of stability, Elsie started to face a series of medical complications. In 2008, a toxic colon infection needed surgery and she required a colectomy. In 2015,

kidney failure obliged her to enter a program of hemodialysis three times a week at our local public hospital. Last year, a subdural hematoma required further surgery. Elsie fought and won all these battles. She received excellent medical care at several Costa Rican hospitals. But this year, further metastases, now to her spinal column, left her in a wheelchair. It was a final struggle she could not withstand.

We were among the first members of the Life Raft Group, a support group for patients with GIST, as readers

know. Its founder, Norman Scherzer, helped Elsie enter the trial and remains a friend. My brother David continues as president of the Life Raft Group Canada.

Despite having to confront cancer and subsequent illnesses, and despite undergoing severe medical treatments, Elsie always maintained a fighting spirit, eager to overcome her problems and always looking for ways to help out her fellow patients. She was a woman who displayed great tenderness and respect towards everyone she knew.

She was a wonderful wife, sister, mother, friend and teacher. We cherish her memory. ■



# Check Out Our New Look!

By **Thomas Cordasco**, LRG Web Manager

If you haven't visited the Life Raft Group's website recently, you may have missed some of the big changes.

The screenshot shows the homepage of The Life Raft Group. At the top is the logo and a navigation bar with links: International Patient, About GIST, About LRG, Get Support, and a DONATE button. A search icon is also present. Below the navigation bar is a large hero image of a woman, Anita G., smiling while sitting on a motorcycle in a workshop. Overlaid on the left side of the hero image is the text "Patient Powered Science For Life". On the right side, a dropdown menu is open, showing links: GIST Specialists Database, GIST Clinical Trials, Patient Resource Toolkit (highlighted with a mouse cursor), Join Our Community, Financial Aid for GIST Patients, Find a Support Group, Coping With GIST, Important Links, and Contact Us. Below the hero image, there is a section titled "Patient Resource Toolkit" with four circular icons: "NEWLY DIAGNOSED" (purple), "FAQS" (blue), "LEARN" (green), and "COPING" (orange). Each icon has a brief description and a "Read More" or "View FAQs" link. At the bottom of the toolkit section are four buttons: "UPDATE" (Update your Patient Registry Information), "JOIN OUR COMMUNITY" (Join The Life Raft Group), "LEARN MORE ABOUT TRIALS" (Available GIST clinical trials), and "VIEW SPECIALISTS" (Search our database of GIST Specialists).

Located on the homepage is a new area called the Patient Resource Toolkit. The Toolkit has quick access to important links for newly diagnosed patients, FAQs, and coping strategies for patients and caregivers. The Learn area on this page helps to educate, empower, and learn more about the science of GIST. There are also links to join our community, update your registry information and find a GIST specialist from our database. The Toolkit can also be accessed from the Get Support and the About GIST dropdown menus in our global navigation.

Toolkit can be found at:  
[liferaftgroup.org/patient-resource-toolkit/](http://liferaftgroup.org/patient-resource-toolkit/)

**27**

**LRG CALENDAR**  
OCTOBER 27<sup>TH</sup> 8AM-3PM

**GIST DAY OF LEARNING**  
*Michigan*

**University of Michigan, Michigan League**  
911 North University Avenue,  
2nd floor, Hussey Room,  
Ann Arbor, MI 48109-1265, United States

## Thank you to our Major Donors for August & September

- |                               |                      |
|-------------------------------|----------------------|
| Carlos Baldor                 | Gerald Knapp         |
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| Cantor Fitzgerald Relief Fund | Clinton Matter       |
| Chad Christensen              | Pfizer Oncology      |
| Deciphera Pharmaceuticals     | David Pichler        |
| Dublin's Pub                  | Tradeweb Markets LLC |

View events: [liferaftgroup.org/events/](http://liferaftgroup.org/events/)



# THE LIFE RAFT GROUP RESEARCH MODEL

## Real World Evidence in Action



### Real World Data

"Data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources."

-FDA



### Real World Evidence

"The clinical evidence regarding the usage and potential benefits or risks of a medical product derived from the analysis of real world data (RWD)."

-FDA

Traditionally, data used in drug research & development has come from the gold standard – Randomized Clinical Trials (RCTs).

With the onset of the 21<sup>st</sup> Century Cures Act, there has been an increased demand for real world data and real world evidence – the post-market insights into drug effectiveness and safety after clinical trials.

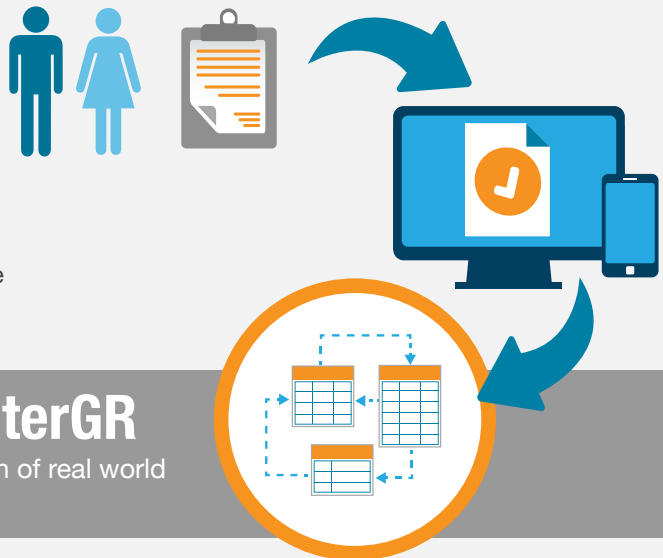
Born out of a desire to learn more about this rare cancer called GIST, our **Patient Registry** started by gathering patient information on index cards.

### Early on...

We conducted a Side Effects Survey, where we applied our own quality of life scale. We discovered valuable evidence that in our patient population, side effects improved over time. This data was shared with the pharmaceutical company that developed the primary treatment for our patients. This complemented the data from the early clinical trials.

Since the Year  
**2000**

this patient-provided, patient  
focused data has been the  
core of the LRG's own research



## Now, we developed Project InterGR

A way to foster collaborative research through the collection of real world data on multiple platforms.



**Patient Registry** - The largest GIST registry in the world with over 1800 patients from more than 60 countries.

Tracks patients over time across institutional and international boundaries.



**Pediatric & SDH-Deficient GIST Consortium** - Collaborative effort of leading Pediatric and SDH-Deficient GIST experts aiming to dramatically impact patient survival and quality of life through data and tissue sharing.



**SideEQ** - Interactive side effects management platform that provides further insights into key issues that impact patient compliance and treatment outcomes.



**GIST Collaborative Tissue Bank** - Housed at Stanford University, vital tissue samples that provide researchers with valuable insights into this rare disease are paired with detailed clinical histories from our Patient Registry.



**GIST Clinical Trials Database** - Provides current information about clinical studies focused on GIST in an easy to use format to aid patients in making key decisions about their disease management.



**Project Surveillance** - A collaborative platform where GIST experts can share real world, real time observations.

Read more: [bit.ly/ResearchModel](https://bit.ly/ResearchModel)

# The Life Raft Group Global Community

## LRG STAFF

Executive Director	Norman Scherzer
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Vice President, Program Services	Sara Rothschild
Senior Director, Research	Pete Knox
Senior Director, Outreach & Engagement	Diana Nieves
Director, Communications	Mary Garland
Director, Operations	Matthew Mattioli
Director, Patient Registry	Denisse Montoya
Director, Outreach & Engagement	Jessica Nowak
Data Analyst	Jerry Call
Patient Registry Supervisor	Sahibjeet Kaur
Real World Data Scientist	Yu Wang
Communications Associate	Danielle Boone
Website Manager	Thomas Cordasco
Office Manager	Angela Edson
Patient Registry Associate	Jennily Eshak
Patient Registry Associate	Chelsea Gottfried
Operations Associate	Helena Mattioli
Director, Finance	Lorraine Ramadan
Executive Assistant	Allison Russo
Web and Design Associate	Carolyn Tordella
Consultant	Marisa Bolognese
Global Relations Consultant	Piga Fernández
Grants Consultant	Kathleen Gronet
IT Consultant	James Lee
	Yash Patel

## LRG VOLUNTEERS

Pediatric GIST	Erin MacBean
Official Greeter	Gail Mansfield
Clinical Trials Coordinator	Jim Hughes
Photographer	Kim Tallau
Staff Photographer	Michelle Mattioli
Special Projects Asst.	Hannah Marcus
General Counsel	Steven Pontell
State Liaisons	Charles Burke
	Rick Isaías
Science Team	Erin MacBean
	Paula Vettel
	David Josephy
	Michael Josephy
	Joan Krakowsky
	Jim Hughes

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Ray Montague (Ex Officio)

### Interested in Volunteering?

Contact: Diana Nieves, Senior Director, Outreach & Engagement,  
dnieves@lifaftgroup.org

### Interested in serving on the LRG Board of Directors?

Contact: Laura Occhiuzzi, Senior Vice President,  
locchiuzzi@lifaftgroup.org

**Life Raft Regional Chapters:** Find info for a local rep at [lifaftgroup.org/find-a-support-group/](http://lifaftgroup.org/find-a-support-group/)

Alabama	Kyle Brown	Kansas	Christine Engel	Oklahoma	Jane Rowan
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**Life Raft Global Liaisons:** Learn more about the Global GIST Network & find contact info for your rep at [globalgistnetwork.com](http://globalgistnetwork.com)

Argentina	Mariel Garibaldi	Honduras	Andrea Torres	Pakistan	Sobia Wali Muhammad
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Austria	Amy Bruno-Lindner		Andras Szeckdu	Philippines	Rod Padua
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155 US Highway 46, Suite 202  
Wayne, NJ 07470

phone: 973-837-9092

fax: 973-837-9095

email: [lifaft@lifaftgroup.org](mailto:lifaft@lifaftgroup.org)

website: [www.lifaftgroup.org](http://www.lifaftgroup.org)



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