

# Ensuring no one has to face GIST alone

In Loving Memory: Leigh Woodward, Barry Hawkins, Cliff Bechard, Floyd Pothoven, Christine Boyd

## Collaboration Lays the Groundwork for Breakthroughs in GIST

By **Brielle Urciuoli**  
CURE Today

Less than two decades ago, Gleevec (imatinib) drastically changed the frontline treatment for patients with gastrointestinal stromal tumors (GIST). However, as patients develop resistance to Gleevec, as well as second and thirdline treatments, such as Sutent (sunitinib) and Stivarga (regorafenib), they often have no more options left.

The Life Raft Group, a patient advocacy group, is teaming up with researchers

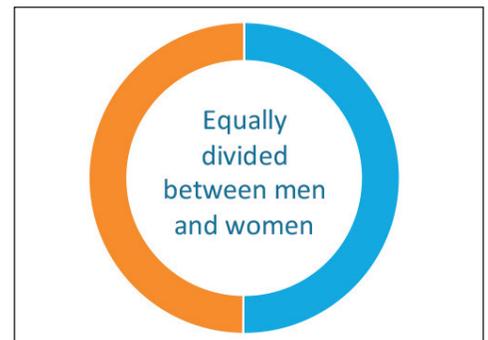
to address this problem and lay the groundwork for more personalized – and effective – treatments for patients with this rare cancer.

“There’s an absolute need in the GIST community for a new drug approach that will allow a new therapeutic way of seeing therapy for this cancer,” Gary K. Schwartz, M.D., chief of the Division of Hematology and Oncology at New YorkPresbyterian/Columbia University Medical Center, said in an interview with CURE.

See **GIST** on page 4

## The Life Raft Group Patient Registry In Review: 2016

By **LRG Patient Registry**



The graph above shows the gender distribution of patients in the LRG Patient Registry.

## Support from the Heart

By **Suzy Burke**  
Caregiver

My name is Suzy Hetheron Burke, I’m 66 years old and happily retired! I grew up in New Jersey, graduated from Rosemont College in PA and moved to NYC after graduation. Charlie and I met, dated and got married when we were living in the West Village and working in the city. We stayed in New York

for two years after marrying and then decided to try New England. We both got jobs, rented and then decided to put down roots, so we bought a home in Massachusetts. We started a family and raised our son and daughter there. It was in Massachusetts when Charlie was 52, that our good friend and family doctor raised the alert at a check-up for a possible colorectal tumor.

See **SUPPORT** on page 5

The Lift Raft Group Patient Registry has over 15 years of self-reported and clinical data encompassing 35 years of patient history crossing institutional boundaries. The Patient Registry has 1703 patients from 67 countries with the youngest patient diagnosed at 5 and the oldest at 92 and represents 12 different mutational types. Continue to learn more about the Patient Registry and where it is today.

See **REGISTRY** on page 6



# *We Love our Donors!*

Over the last 16 years, we have worked toward increasing patient survival and ensuring a better quality of life for GIST survivors. I consider it an incredible privilege to wake up each day to support GIST patients and caregivers. Though we have come so far, it is not always easy to see the direct results of your efforts, your donations, or your time.

The LRG has just announced a collaborative research project with Columbia University Medical Center to investigate a new approach for identifying the best treatment options for people with advanced GIST. This project has extraordinary potential for GIST patients and shows teamwork between a major research center and a patient group in action.

And it would not be possible without your support.

Following the inspiration of the Cancer Moonshot Summit, this venture also presents a great opportunity to showcase the impact patient-powered science can make on cancer research

I wanted to thank you again and demonstrate how your contribution has helped to accelerate research and support the GIST community for years to come.

Sincerely,



Norman Scherzer  
Executive Director

# Countdown to Rare Disease Day 2017



Rare Disease Day is celebrated on the last day of February each year. The day is celebrated in order to bring attention to the general public and decision makers about rare diseases and how they affect patients' lives. The day was first introduced by Eurordis and its Council of National Alliances in 2008.

The theme this year is "With Research, Possibilities are Limitless." Events are planned by rare disease organizations around the world. <http://www.rarediseaseday.org/events/world>

The LRG was recently featured on the NORD (National Organization for Rare Disorders) Facebook page in their Countdown to Rare Disease Day.

The Life Raft Group since its inception has been dedicated to research. From the early days when our unique Patient Registry was created, through the development of our GIST Collaborative Tissue Bank, to the establishment of our collaborative research team, we have always been about patient-powered science. This has led to innovative research collaborations like the one with Columbia University Medical Center, NewYork Presbyterian mentioned in our lead article.

We have been celebrating the countdown to Rare Disease Day by posting on social media on "Research Fridays," the unique ways in which we engage in research initiatives that continue to impact patients' lives.

One of the suggested activities for Rare Disease Day is to raise and join hands to show solidarity with rare disease patients around the world. We invite you to take photos, whether with your family, friends, coworkers or at an event, where you raise or join hands and either send them to us or post them on the Life Raft Group Facebook page. You can also upload them to the Rare Disease Day website. <http://www.rarediseaseday.org/join-your-hands>

We will be joining hands on February 28, and posting our photo to show our support for this important cause.

Why not join us?



## GIST from page 1

Schwartz is spearheading the clinical side of the project, where researchers are using the VIPER algorithm software to analyze tissue samples from the Life Raft Group's patient database to find "bottleneck" pathways, called master regulators, where the pathways driving cancer cells converge into one or two pathways. Identifying these would give drug developers an easier target to block.



*Norman Scherzer & Dr. Gary Schwartz*

"GIST is one of the tumor types we're focusing on, but frankly, we're using the same technology to look at other cancer types as well, because the bottleneck approach isn't unique to GIST. It's common to all cancers," Schwartz said, mentioning that there are similar projects in the works for triplenegative breast cancer and neuroendocrine tumors. However, the GIST project is currently the most advanced.

Both Schwartz and Norman Scherzer, the executive director for the Life Raft Group, agree that this project would not

be possible without the collaboration between the groups.

"Hospitals interact with patient groups, but usually in a 'soft' kind of way, where information is shared," Scherzer said. "This is different because we have a different mission that drives the Life Raft Group. The best way I can describe it is 'patient-powered science.' We're all about making patients happy and giving them psychologic and informational support, but mostly, we're about using the power of patients to drive the scientific process."

Scherzer explained that the collaboration was the "perfect storm" where the Life Raft Group was able to become the "honest broker" and provide tissue samples to research centers such as Columbia that might have otherwise had difficulty obtaining tissue samples because of HIPPA regulations. But, by deidentifying all samples, the Life Raft Group makes the samples fair game.

Since GIST is considered an orphan disease by the FDA – less than 200,000

patients currently living with this disease – researchers need all the tissue samples that they can get to accurately find these pathways that could lead to new treatment strategies.

"Unless you have coordination with organizations like the Life Raft Group, you can't do clinical trials," Schwartz said. "It takes multiple centers and it takes the support of patient advocacy groups to inform patients of what's going on and educate them in the process, because we need lots of tissue from a lot of patients to design these maps."

The project is also collaborating with other academic institutions across the nation: Fox Chase Cancer Center, Oregon Health & Science University, University of California San Diego, University of Miami, Washington University and Stanford University. Schwartz said that he hopes to have the pathway maps within the next year or two, and start studies within the next two or three years.

"Hopefully this methodology will transform the way we select drugs for cancer care, not just in GIST, but across all solid tumors," he said.

<http://www.curetoday.com/articles/collaboration-lays-the-groundwork-for-breakthroughs-in-gist->

# Help Us Further GIST Research

**If you would like to participate in the Life Raft Group's Tissue Bank, please contact:**

Kathrena Aljallad  
Patient Registry Director  
973-837-9092 Ext. 114  
[kaljallad@liferaftegroup.org](mailto:kaljallad@liferaftegroup.org)

**SUPPORT** from page 1

He suggested we consider one of five local general surgeons.

After trying to absorb the shock of a cancer diagnosis and what it might mean for us, I went into overdrive and called my former boss who was a Boston consultant who had recently started a medical-related consulting firm in Boston. Within a few days I was speaking with a well-known and well-connected physician at Mass General Hospital and Harvard Medical School. He explained the necessity of having a good surgeon, and recommended five to us. After chemo, surgery and additional chemo, Charlie was cancer free! We moved all our doctors to MGH physicians over fears of referral and insurance issues. The drive was worth the sense of security that we had access to the best doctors.

Fast forward to when Charlie was 64 and had lost some weight and wasn't feeling well. We went to our MGH primary who did blood work, ordered a scan and set up an appointment with his MGH oncologist. We had become quite close to his oncologist, and had a number of friends in common. When he

walked into the examining room after viewing the scan and the lab work he delivered the news that Charlie had a large and very rare GIST tumor. He had us stay in the room while he brought the MGH GIST specialist in to meet with us. Not only was it cancer again, but now very rare! When we received the very rare GIST diagnosis, I was confident we had the best doctors to guide us. I truly recommend that all going through this journey go the extra distance to find GIST specialists to help them.

Charlie's tumor was large and the decision was made to go on Gleevec for five to six months to shrink the tumor and then operate. It worked so well that the tumor shrunk from the size of a Nerf football to a small kiwi. Charlie then had his successful surgery and stayed on Gleevec for four years. Cancer free is where we are! Life is good. Charlie's GIST oncologist introduced us to the Life Raft Group

and we have both become involved with them. Support systems are great! I have been the one who believes that if I am to be supportive to Charlie, I have to take care of myself first, and for me that means taking care of my own health. I am a regular at the gym, and if that's not available, I walk. Actually, Charlie and I usually walk every morning for three to four miles. It clears my head and helps me feel good. There are some things over which we have no control, but we can take care of ourselves with a program of regular exercise and healthy diet.

Life is good. We are now both retired and live in New Jersey, halfway in between our two grown children and closer to our granddaughter. Our adult children are a huge source of support for both Charlie and me. They're good listeners and a great source of positive energy!

Our mantra is YOLO, you only live once. We do enjoy every single day and share our appreciation for life with all we meet. No guarantees, every day is a gift!



Suzy & Her Husband Charlie

**CANCERVERSARY**  
10 YEARS



**Kimberly Glass**

I celebrated my ten-year "cancerversary" on January 31st. I can't believe it's been 10 years! I will celebrate by spending some intentional time with my family, including my beautiful 16 month-old daughter, Katelyn. My wish for all of you battling GIST is to really live each day, as none of us are promised tomorrow. Forgive often, give and receive love, and enjoy the little things in life. Thank you to the Lift Raft Group – without you, I may not have been here to celebrate this ten-year mark.

**CANCERVERSARY**  
14 YEARS



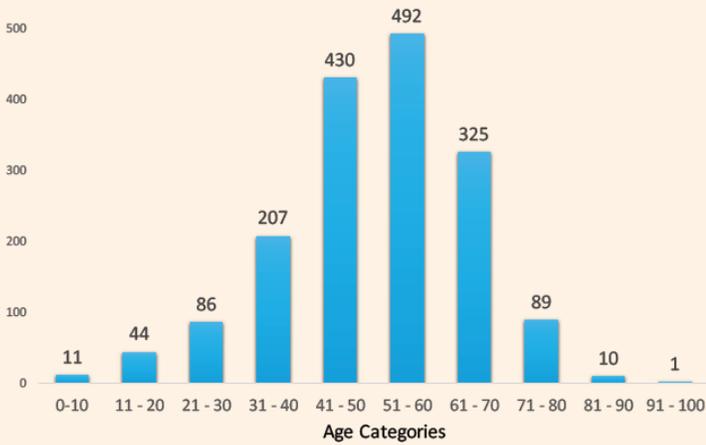
**Martha Zielinski**

On January 14, 2003 when I had surgery to remove my GIST I never dreamed I would still be here 14 years later! So little was known about this cancer, at that point the most important thing was to be C-Kit positive and hope that Gleevec worked! The tumor was positive and the Gleevec has! A lot of pills later I can celebrate everyday with John, my husband of 43 years and my family. I am grateful to the many friends I share the raft with and to all the researchers who keep learning about GIST. I have learned to be my own advocate and to be grateful for all the things having cancer has taught me.

# The Life Raft Group Patient Registry In Review: 2016

## Demographics

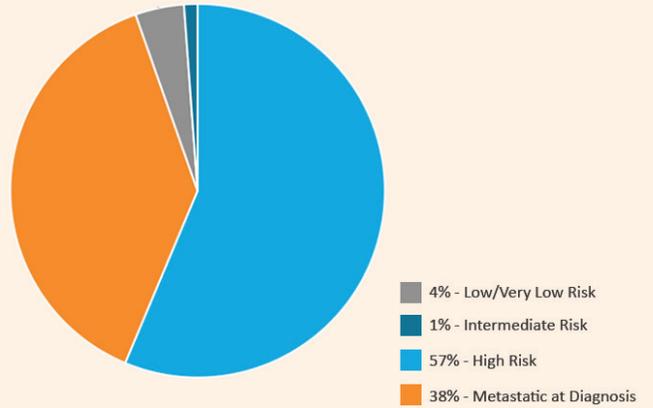
**Distribution of Age at Diagnosis**



\*8 Records are missing due to missing date of birth

GIST is mainly diagnosed in people above the age of 50, however people of all ages are affected.

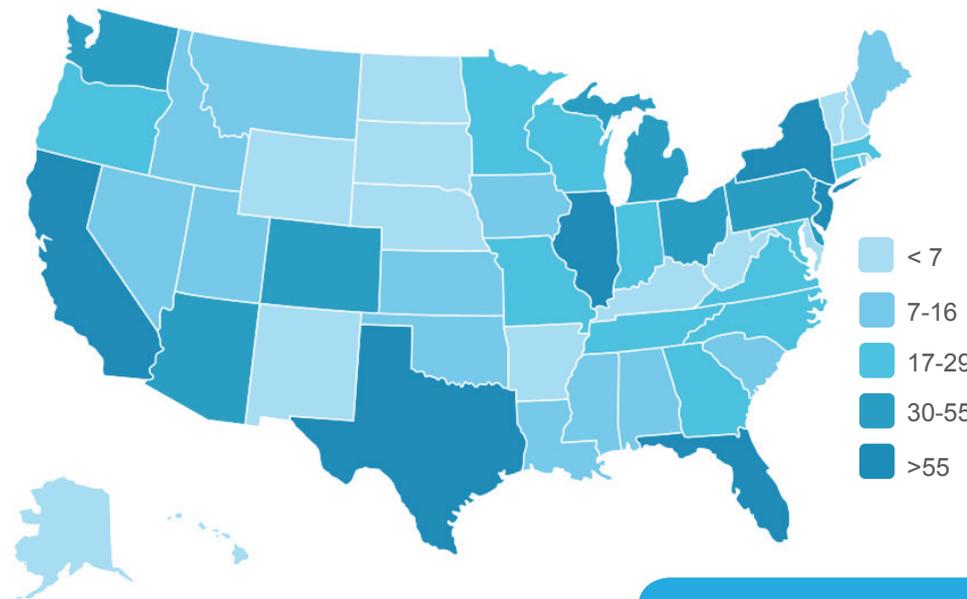
**Risk of Recurrence of Those Who Know Their Risk**



71% of patients know their risk of recurrence. Of those who do know their risk of recurrence, the majority of them have a high risk. There are several different methods used to classify the risk of recurrence in GIST. The Patient Registry uses the Modified NIH Method, which looks at primary tumor size, mitotic count, and location.

### Number of GIST Patients in the Registry by State

The LRG Patient Registry has the largest patient population in California and the smallest population tied between Vermont and Wyoming. Be part of a state or country support group by contacting your Local Leader or Country Representative.



The patient registry reaches **67** different countries

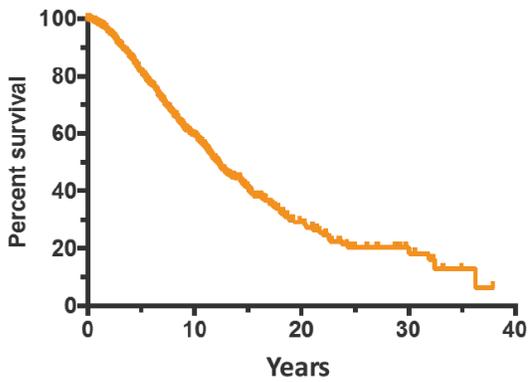
### Top 5 Countries

-  Canada
-  Netherlands
-  Great Britain
-  Australia
-  Chile



To view the full Patient Registry In Review: 2016 please go to [bit.ly/LRGPatientRegistry2016](http://bit.ly/LRGPatientRegistry2016)

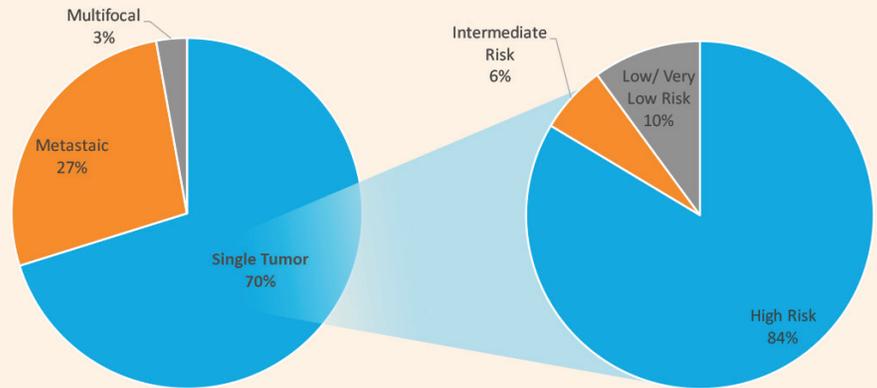
### Median Overall Survival



**12 Years**  
Median Survival Years  
of all Registry Patients

### Tumor Staging and Risk Stratification

Most patients in the Patient Registry are high risk despite being diagnosed with a single tumor. Learn How to Read Your Pathology Report to understand tumor staging and risk.



## Treatment

### Current Treatment of Living Patients



Continued on page 8

### Average Time on Medication

1<sup>st</sup> Line: imatinib (Gleevec)

2<sup>nd</sup> Line: sunitinib (Sutent)

3<sup>rd</sup> Line: regorafenib (Stivarga)

After those treatments are exhausted, off-label drugs used for GIST Treatment

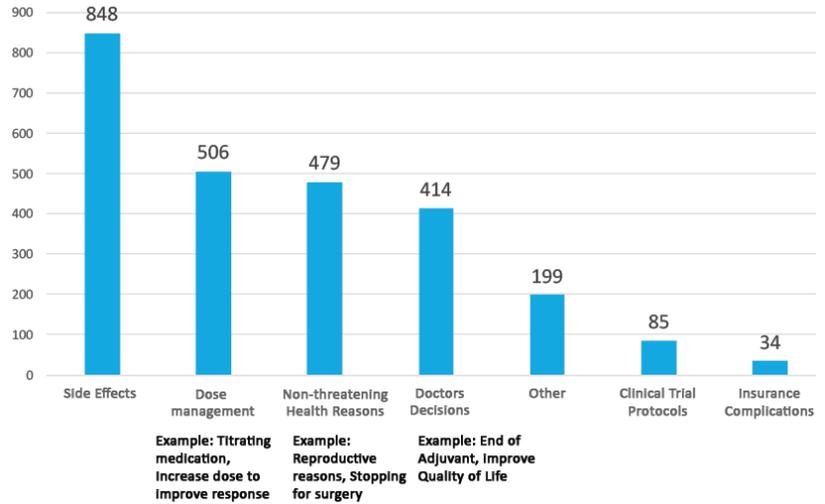
Drug	Average TOM (months)
imatinib (Gleevec)	40.11
sunitinib (Sutent)	14.8
regorafenib (Stivarga)	11.66
Off-label*	7.72

\*Off-label refers to a group a drugs that are taken past the 3rd line drug

### Treatment Interruptions:

Ending or temporarily stopping medication or altering dose.

These events are from the treatment records of all of our patients. As patients change medication or dose, multiple records are created.

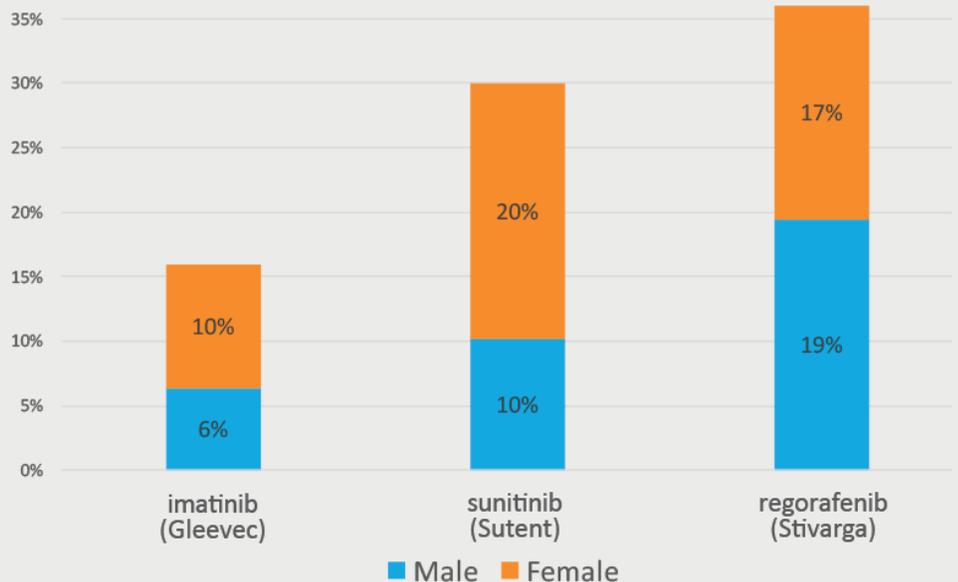


Not included in the above graph are those who had a treatment interruption due to a negative event (i.e. progression or death). This is the primary cause for treatment interruption. Over the past 16 years, there have been 2166 reports of treatment interruption due to a negative event.

### Treatment Interruptions Due to Side Effects\*

Treatment was interrupted 848 times due to side effects. Below is a breakdown of the distribution of these interruptions by the 3 approved drugs for GIST. Side effect management is essential for the success of any treatment. Visit SideEQ for best practices and patient tips on managing side effects.

\* The remaining 18% refers to the off label drugs.



# Tissue Bank

396 Patients

767 Tissues

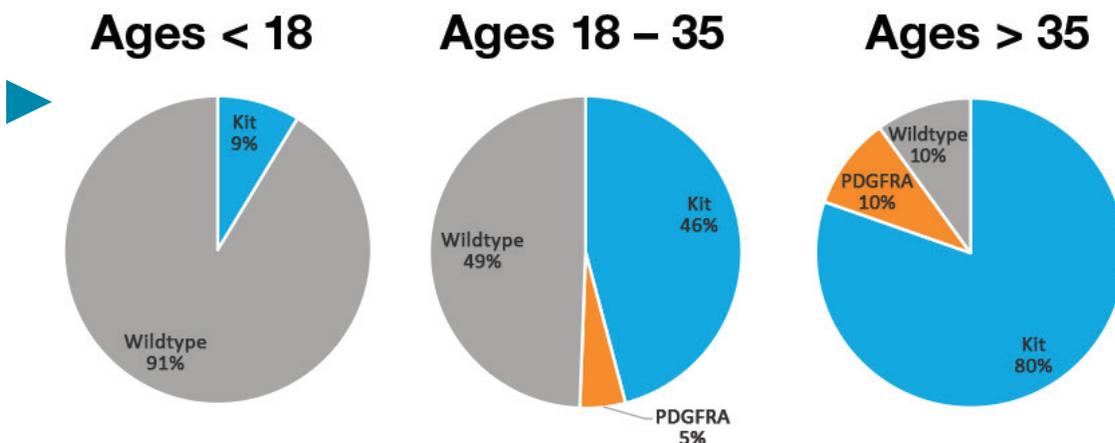
On average a patient donates 2 tissue samples

**Contribute your data to research today**

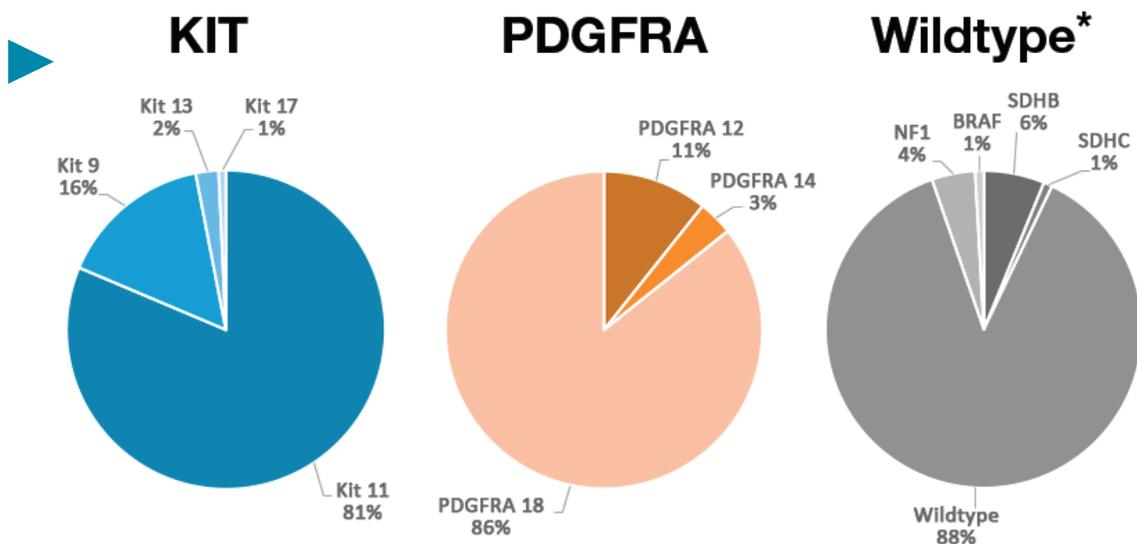
**Join the Patient Registry go to [bit.ly/JoinLRG](http://bit.ly/JoinLRG)**

## Breakdown of GIST Mutation

Out of those 43% who know their mutation, this represents the breakdown of the gene mutation by age categories at diagnosis.



Out of those 43% who know their mutation, this represents the breakdown by gene.



\* Wildtype mutation refers to GIST tumors having no detectable KIT or PDGFRA mutations that have not gone through testing for further classification (e.g. SDHx, or RAS pathway indicators).

# In Memoriam



Barry with his beloved sister, Sally Hawkins

Barry Hawkins, age 46 of St. Paul passed away peacefully August 13, 2016 at home in St. Paul, surrounded by his parents and siblings. Barry will be remembered as an irreplaceable spirit: a man of strong faith who was genuinely interested in and would remember each person he met, a gentle jokester who knew just how to put a smile on everyone's face, and a courageous fighter who faced his GIST sarcoma cancer with strength and optimism. Barry was an avid walker, dedicated family historian, and deeply proud Irishman who never let anyone forget how much he loved them. He was a long-time member of Lumen Christi Catholic Community, and had recently

retired from the Highland Park Lunds & Byerlys.

Barry is survived by his parents, George and Jeanine Hawkins, and siblings Betsy (Dennis) Evans, Jane (John) Scherer, Sally Hawkins, Brian (Linda) Hawkins, and Mark Hawkins. He was the "favorite uncle" of Troy, Ben, Megan, Liz, Kathryn, Shannon, Spencer, Patrick, Brie, and Matthew, and a GREAT great uncle to Greta, Owen, Matilda, Amelia, and Finn. Dear aunts and uncles, cousins, friends, and co-workers are among the many others who loved and were loved by Barry. We are blessed to have known our Irish Rover, whom we'll never forget and will always love!

Every life  
leaves something  
beautiful behind

Contact the Life Raft Group  
at [liferaft@liferaftgroup.org](mailto:liferaft@liferaftgroup.org) for ways to  
honor your loved one.



## Become an LRG state leader!

Have you ever wanted to become more involved in giving back to the GIST community but don't know how?

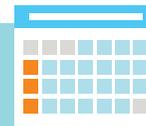
### Become an LRG State Leader!

State leaders are an important part of the Life Raft Group network, providing important person-to-person contact to help members know they are not in this journey alone.

Responsibilities include:

- Being a point of contact for members in your state
- Welcoming new members
- Planning meetings and get togethers for your state

If you are interested or just want to learn more, please contact Laura Occhiuzzi at [locchiuzzi@liferaftgroup.org](mailto:locchiuzzi@liferaftgroup.org)



## Calendar

**February 23<sup>rd</sup> 2017**

New Insights into Gene Fusions in GIST Webcast

**February 28<sup>th</sup> 2017**

Rare Disease Day

**March 25<sup>th</sup> 2017**

Miami GDOL

Check  
[www.liferaftgroup.org](http://www.liferaftgroup.org)  
for more info

# Welcome to Steve Pontell, the LRG's new volunteer Attorney



The LRG is proud to welcome Steve Pontell, Esq. who has joined the LRG team as our volunteer attorney.

Mr. Pontell is a member of the firm of Verde, Steinberg & Pontell, LLC, established in 1986, which maintains offices in Hackensack, New Jersey and New York City. He graduated from Tufts University in 1979, and obtained his JD from George Washington University National Law Center in 1982.

Steven concentrates his practice in the field of litigation and the firm provides diverse legal services for the business community.

He is certified as a civil trial attorney by the Supreme Court of New Jersey, Board of Trial Advocacy; AV Preeminent Peer Review rated by Martindale-Hubbell, and was voted a Super Lawyer for the years 2005-2016. Steven is admitted to practice law in New Jersey, New York, Washington, D.C and before the Supreme Court.

“The cause could not be more worthwhile, and the facilitation of treatment and prospects for a cure are inspirational. I am excited about being involved in an organization which is so meaningful and look forward to assisting in any way I can,” Pontell stated.

# Life Raft Group hosts Open House for local members



By **Laura Occhiuzzi**  
Program Outreach Director

On Sunday, December 4, 2016, The Life Raft Group hosted a gathering to introduce members to staff and provide a tour of our newly renovated facilities. Over brunch, attendees mingled and got to know one another.

After viewing a short video, attendees took turns sharing their personal stories. Forgoing a formalized agenda, our goal was to give everyone the chance to get to learn more about each other.

What did we take away from the meeting? Patient power is both comforting and heartwarming, as well as impactful. We are hoping to offer these meetings throughout the country to give members the opportunity to share and mingle. If you are interested in hosting a meeting in your state, please reach out to Laura Occhiuzzi at [locchiuzzi@liferaftgroup.org](mailto:locchiuzzi@liferaftgroup.org).



## Latest GIST and Sarcoma Journal has arrived!

The GIST and Sarcoma Journal is the first journal specifically focused on Gastrointestinal Stromal Tumors (GISTs) and other Sarcomas.

**ALL INQUIRIES:**  
Pete Knox  
Director of Strategic Planning  
973-837-9092 x123  
[pknox@liferaftgroup.org](mailto:pknox@liferaftgroup.org)

# THE LIFE RAFT GROUP COMMUNITY

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Program Operations Senior Director	Sara Rothschild
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Staff Photographer	Michelle Mattioli
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Special Projects Asst.	Eileen Glasser
Lawyer	Steven Pontell
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## Life Raft Regional Chapters: Find your reps info at [liferaftgroup.org/find-a-support-group/](http://liferaftgroup.org/find-a-support-group/)

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Kentucky	Cindy Betz				

## Life Raft Country Liaisons: Learn more about the Global GIST Network & find contact info for your rep at [www.globalgistnetwork.com](http://www.globalgistnetwork.com)

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