The Life Raft GROUP

December 2018

Ensuring no one has to face GIST alone

In Loving Memory: Karen Ammons, Gary Lee Napier, Brenda Lee Kuckelburg, Samantha Wexler, Hilda Montenegro-Abarca, Judy Bright

Searching for Hope

A Mother's Mission to Save Her Son

By **Glenda Swinbourn,** LRG Contributor

In this article, Mom Glenda Swinbourn of Australia shares the story of her son Mitchell, a 13-year old with Carney-Stratakis Dyad. It is for patients like Mitch that the Pediatric & SDH-Deficient GIST Consortium was founded.

Mitchell has faced many battles in his young life, even prior to birth, including being diagnosed with high-functioning autism. Little did we realise, our hardest journey was yet to come.

Mitch's GIST journey began with a troubling set of symptoms over several months, that began with uncontrollable vomiting daily, a small breast lump, and ongoing severe headaches for which he was hospitalized in April of 2015.

▶ see MITCH on page 4

Patient Advocacy Reaps Change

Patients & Caregivers March

By **Piga Fernández,**
LRG Global Consultant

On December 4, 2018, President Sebastián Piñera, of Chile, signed the National Cancer Plan 2018 – 2028 and the National Cancer Law. LRG Global Consultant and Executive Director of Fundación GIST Chile, Piga Fernández, and other cancer survivors and thrivers, were at the heart of this story of advocacy in action!

▶ see CANCER MARCH on page 10

Paraganglioma* Preclinical Project

by **The LRG Science Team**

The Pediatric SDH-Deficient GIST Consortium, which was launched at the Biden Cancer Summit, consists of an impressive team of researchers, clinicians, academics, and patient advocacy groups.

A member of our consortium, Dr. Joshua D. Schiffman of Huntsman Cancer Institute, is featured in this newsletter. Dr. Schiffman is conducting a research study on SDH-mutated paragangliomas (PGLs) and other SDH-deficient tumors.

▶ see PROTOCOL on page 7

From our Executive Director - "Closing the Cancer Survival Gap"

Surviving the Gauntlet

So, after an investment of hundreds of millions of dollars and irreplaceable time, a new drug is approved by the FDA for the treatment of a subtype of a rare cancer like GIST. It's time to celebrate a mission accomplished.

Not so fast. The sad reality is that despite this monumental achievement, too many patients who should have benefited from this breakthrough will instead die. Not because the mutational test that should have connected them to this new treatment did not work. Not because the drug that should have attacked the target designated did not work.

The underbelly of cancer is not pretty. Despite the availability for over ten years of mutational tests to identify different subtypes of GIST, the vast majority of GIST patients still do not receive them. Why not? The simple answer is that their oncologists did not order them. Most likely because they were not specialists for this rare cancer and instead fell back on consensus rather than targeted treatment.

The good news of the advent of cancer treatments via oral drugs has had the unintended consequence that patients who previously went to a specialized center of excellence now often go to a local oncologist, who may not have the experience to deal with this, but does not realize it.

Fast forward to the next survival gap obstacle. This patient has now received the right mutational tests and has been matched to the new breakthrough drug.

He is instructed to go home and fill this prescription and then take these four pills every day until told to stop. By the way, your out-of-pocket costs will be $9,000 per month.

Let's assume that the patient and his family can overcome this new disease called financial toxicity and can finally begin treating their cancer. I use the word "their" because the diagnosis of cancer is really a family diagnosis for a malady that affects everyone and the financial toxicity that many have to endure can impact the caregiver's and families' lives across the board.

All right, we should now be home free. Right mutational test. Right treatment prescription. Right out-of-pocket payment.

The likelihood is that the drug has side effects, sometimes tolerable, but sometimes not. Manageable, if the patient had been given a real-life follow-up support plan by both the prescribing physician and the pharmaceutical company that made the drug. That plan would extend over the life span of the treatment which often means many years (side effects change over time and do not expire when the drug patent ends). That always happens right? Well, by now you should be getting the message "not always". The consequence: the patient does not take all the medication prescribed. Really? Yes, really.

In 2019, the Life Raft Group intends to add to its comprehensive global education program a parallel tactic to change behavior; that of the patient and that of the physician. We intend to acknowledge those medical providers and pharmaceutical companies that provide patients with the support they need to stay alive throughout their entire treatment journey and confront those that do not.

We are tired of seeing our friends die unnecessarily. ■

- Norman Scherzer

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LRGCONNECT

BREAKING NEWS

Vitrakvi

New Drug Approved for Patients with Rare Gene Fusion

By **Sara Rothschild,** MPH, Vice President, Program Services & **Jerry Call,** Data Analyst

The Food and Drug Administration granted accelerated approval on November 26th to larotrectinib (VITRAKVI) for adult and pediatric patients with solid tumors that have a neurotrophic receptor tyrosine kinase (NTRK) gene fusion without a known acquired resistance mutation, are metastatic, or where surgical resection is likely to result in severe morbidity and have no satisfactory alternative treatments or that have progressed following treatment.

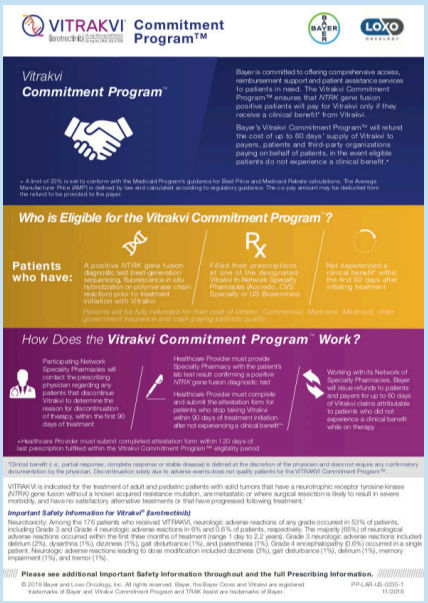
What is an NTRK fusion?

The Trk receptor family comprises three proteins referred to as Trk A, B and C (TrkA, TrkB and TrkC) receptors, and are encoded by the NTRK1, NTRK2 and NTRK3 genes, respectively. These receptor tyrosine kinases (TK) are expressed in human neuronal tissue and play an essential role in both the physiology of development and function of the nervous system through activation by neurotrophins (NTs).

When an NTRK fusion occurs, a portion of a gene gets swapped with another gene (whether the same chromosome or different chromosomes). These fusions are reported in cancers, with the most common being salivary gland tumors, soft tissue sarcoma, infantile fibrosarcoma, and thyroid cancer.

How is this important to GIST? Who should get tested?

Although these mutations are rare, the response rate to larotrectinib is very high (75 percent overall response rate across different types of solid tumors). **This makes testing extremely important.**



In patients where no mutation was found after basic mutational testing (KIT and PDGFRA mutation testing), advanced testing should be performed and should test for a number of different mutations as well as SDHB staining (IHC). Advanced testing is most efficiently done with testing panels that test for many different mutations at one time including NTRK fusion mutations. Laboratories that have panels that have been specifically designed to include the rare mutations that occur in GIST include Knight Diagnostic Laboratories and Foundation Medicine (there may be others as well). For patients with previous negative testing (no mutation found) in multiple genes, they should check to ensure that NTRK fusions were included in that testing and if not, should be retested for NTRK fusion mutations.

Dr. Jason Sicklick, a GIST specialist currently with Moores Cancer Center, University of California, San Diego, presented data several years ago, in a webinar for the LRG, from a project he did in collaboration with Foundation Medicine and observed that patients with these fusions typically range in age between 30-50s. His data also showed that the fusions are more common in men than women and that the tumors can occur anywhere in GI tract. Additionally, one-third of tumors are associated with lymph metastases and 40% are associated with distant metastatic disease.

Patient Assistance

The Vitrakvi Commitment Program is managed by Bayer Oncology. NTRK gene fusion positive patients will pay for VITRAKVI only if they receive a clinical benefit from VITRAKVI. Full or partial refunds (for up to 60 days) will be issued to patients and the primary payer when lack of a clinical benefit occurs.

To learn more about support through TRAK Assist, call 1-844-634-8725 or visit https://www.vitrakvi-us.com.

▶ MITCH continued from cover

At that time, we requested blood and urine tests and were told, "If you don't hear back from us, you know everything is ok." Nothing was heard. My husband's brother had been diagnosed with paragangliomas years before, and I knew the vomiting and headaches were symptoms he had suffered. There was mention of a potential hereditary risk after his diagnosis, but that was never explained to us as a serious risk.

Our GP diagnosed anxiety as the cause of the vomiting due to Mitchell's autism, and advised he take a week off school. I wasn't convinced, especially when days later Mitch begin vomiting blood. I urged the doctors to check last April's blood results to confirm everything was ok but was told they couldn't be located.

When I discussed my concerns regarding our family history of paragangliomas, there was always a blank stare. We were sent home with medication to hopefully help Mitchell's stomach lining settle. Two days later, undeterred and desperate, I went back to Emergency Department (ED) and saw another doctor. I poured out my concerns, and he ordered x-rays.

Still unable to find the prior blood results, the doctor diagnosed constipation. Mitch had an enema and was sent home with a seven-day course of medication. What I didn't know until months after the first tumour removal was that this doctor had identified a mass in the stomach, but knowing Mitchell had autism, decided it was a mass of hair Mitchell had eaten.

To this day my husband and I can never understand why the doctor made that assumption and that he never even asked me if Mitchell tended to eat strange things. When we saw Mitchell's paediatrician, I again poured out his history and my concerns, asked her to find the results from the hospital for me, which she did. I sat there in her office with Mitch building with Legos at a table next to me.

The paediatrician got through to the head of pathology and after a minute or two said those words I will never forget "So, the parents should have been informed..." and began writing figures. Adrenaline was the first one noted and the number was in the thousands.

I knew it suggested a paraganglioma. I looked at Mitchell and felt panic rising within me. Mitchell's paediatrician told me she would refer us to an endocrinologist and we would need to wait for them to get in contact.

That night Mitchell woke with a severely swollen abdomen and I rushed him to hospital. I told them about our results and that prior blood tests potentially indicated a paraganglioma. I told them I was scared and that something was terribly wrong. A senior doctor came to examine Mitchell, and told me to stop the constipation medication, that it had done its job and Mitchell no longer needed it. I told him Mitchell had not been passing much at all, to which he replied, "It's possible he hasn't passed it yet - perhaps continue with the seven-day plan." I glared angrily at him and told him this was our third time to ED in one and a half weeks and I wanted answers, real answers. After relating the risks of more radiation, he agreed to order an x-ray. The junior doctor later told us his x-ray showed no constipation issues, so we could stop his medication and we were free to leave.

> "I felt rage coursing through me; it took every bit of self-control I had not to scream. Mitchell was quite literally a ticking time bomb. I was desperate and so very scared.

I felt rage coursing through me; it took every bit of self-control I had not to scream. I should have. I should have refused to leave, ordered them to get another doctor, and made a scene. But I didn't, I did what they told me to do and I took Mitchell home. It went against my upbringing to stand up to doctors, but I didn't believe them. I felt betrayed and knew if I was right, Mitchell was quite literally a ticking time bomb. If a paraganglioma was there it could secrete enough adrenaline to cause a stroke or kill Mitchell outright by causing a heart attack. I was desperate and so very scared.

I received a call from an endocrinologist who reviewed Mitchell's case and apologized for the trauma our family had endured. She told me the symptoms and tests indicated that Mitchell did have a paraganglioma. I felt so many emotions flooding through me: relief that I was being taken seriously, overwhelming grief, anger, fear and exhaustion.

A 24-hour urine screen performed twice by the endocrinologist confirmed I was right, as it gave all the signs a secreting paraganglioma was present. An MIBG scan identified a mass in his abdomen and Mitch underwent two weeks of blockers to lower his blood pressure.

Read more about mutational testing: bit.ly/MutationalTesting

Without blockers, the para had the potential to cause an adrenaline surge during surgery, enough to awaken Mitchell and cause a stroke or even death.

During the two-week initial wait in hospital undergoing the blockers, Mitchell continued to vomit. During an ultrasound, a second tumour was identified in Mitchell's stomach. Logically we assumed it was another para and but after its removal, we were told it was potentially a GIST. I don't think my husband, or I will ever forget the excitement in the well-meaning oncologist's voice. "This would be the first GIST ever diagnosed at the hospital. Mitchell was an extremely rare case; doctors were lucky to ever come across this cancer in their lifetime". The enthusiasm was not shared by us. We felt afraid and concerned that due to this rarity, we again, had no cure or protocol treatment. We didn't understand how we could be battling two types of rare tumours at the same time.

Research led me to Carney-Stratakis Dyad - paras and GIST, caused by the SDHB mutation and it was therefore no surprise when suggestions were made that this was the diagnosis. We realized that for Mitch to have this mutation, my husband must possess it and therefore our two other boys could also be at risk. Mitchell's tests came back three months later, following his first tumour removal, positive for SDHB mutation. This then enabled my husband to be tested. With the positive result our other two sons were tested. Our youngest son is positive and our oldest is not. This meant my husband and youngest son need to be screened on a regular basis.

The surgeons claimed that Mitchell's GIST had been removed with clear margins, but that the paraganglioma which had attached to the abdominal aorta had also grown into the vena cava, so it was regarded as an incomplete resection. With regular MRI's, the occasional CT and PET scans, things were clear until May 2016, when two new lesions were discovered in Mitchell's liver.

Our oncologist, Dr. Rishi Kotecha from Perth, reached out to experts around the world to find the best treatment options. Dr. Kotecha felt Dr. Lee Helman and Dr. Katherine Janeway's suggestion to use the tyrosine kinase inhibitor, Pazopanib, was the best course of action. I knew there was limited evidence to support the use of these drugs and quite frankly, my husband and I were terrified of what the drug might do to Mitch's little body. I researched extensively and emailed GIST support groups for information on their own experiences.

In the end, we opted for radiofrequency ablation. It was at this time Mitch became extremely ill and it took around a week for doctors to find the cause. Ultimately, he underwent a second laparotomy at the beginning of August to rectify bowel adhesions which had developed as a result of his first laparotomy. It was a difficult recovery but at the end of August, when Mitch was strong enough, he underwent a radiofrequency ablation procedure to destroy the two liver lesions.

At the time the ablation was performed a biopsy was also taken, and although we suspected GIST, we also realized the possibility of the tumour being a paraganglioma. Knowing that a biopsy was considered unsafe if the tumour was a para, we refused a biopsy to be performed on their discovery. It was with great angst we allowed them to take the tissue simultaneously while the burning occurred. We needed to know with certainty what we were dealing with. The results confirmed our suspicions that GIST was indeed our diagnosis.

In a follow-up 12 weeks later, we were devastated to discover Mitchell had three new lesions in his liver and in December 2016, he underwent a second radiofrequency ablation procedure. At the next 12-week follow-up after this procedure we were again shattered to find Mitchell now had a total of five new lesions present in his liver. Ablation was now considered unfeasible due to positioning of tumours and current damage to the liver. In the beginning of 2017, it was decided that our best course of action would be to place Mitchell on Pazopanib.

Thankfully, the only side effects our little boy encountered was a change in his hair colour and constant nasal congestion.

Mitch's story continued on page 6

▶ MITCH continued from page 5

In July of 2017, our family undertook a huge journey to travel from Perth in Western Australia to the National Institutes of Health in the U.S., to take part in their annual Pediatric and Wild-Type GIST Clinic. Mitchell was examined by top experts and our whole family provided DNA, something which we felt was extremely important, not only for Mitch, but with the hope of helping others already affected and those yet to be diagnosed.

Over a year, Mitchell's five liver lesions continued to grow, and in February, Mitch was placed in the PRISM Trial at Princess Margaret Hospital. He was hospitalised to undergo a fine needle biopsy which would use tumour tissue with the aim of individualizing treatment based on the specific genetic make-up of his tumours. The following month, after yet another MRI, we were informed not only had the biopsy removed unviable tissue, but that all his tumours had grown significantly, plus he now had a sixth lesion close to the outer surface of the liver.

After much discussion, we ceased Mitchell's medication and in April, a Sydney paediatric liver specialist flew to Perth and conducted yet another laparotomy to remove the new lesion. Unfortunately, the remaining lesions were

> "...our whole family provided DNA, something which we felt was extremely important, not only for Mitch, but with the hope of helping others already affected and those yet to be diagnosed.

deemed inoperable, due, not only their location, but the inability for the remaining liver to function viably. We were very relieved when around six weeks later we were told the painful procedure had been successful and the tissue was viable - thus allowing us to proceed in the trial.

The follow up in July indicated the five remaining tumours had again grown significantly and heartbreakingly, another two lesions had arisen, taking his total to seven active lesions. After the results of the first part of the trial were revealed. we were advised to try a second TKI called Regorafenib. This drug proved to be harsher and resulted in Mitch developing terrible foot pain with severe blistering and peeling.

As with the Pazopanib, the results of Mitchell's latest MRI showed once again a failure to achieve stability in his tumours, instead seeing a continued increase. We were also able to distinguish that the growth rate had slowed, and we made the joint decision to lower the dosage and

The Swinbourn Family - Darcy, Mom Glenda, Harrison, in front, Dad Matt, and Mitchell

hope the side effects would be reduced. Mitchell started the lower dose of Regorafenib over the last few days. We are holding our breath and waiting, hoping our little boy, who has suffered so much, will suffer as little as possible for the time we have left with him.

Our family lost my husband's brother in September 2016 due to metastatic paraganglioma and pheochromocytoma caused by the SDHB mutation. He was 36, and even though ill, he was a great support for our family as we battled our own rareness. Our family lost my Dad in 2017 and my Mum this past August.

Death seems ever present, and anxiety, depression, and grief have impacted my life heavily over these last few years. I continue to find all my strength in this little boy, who has endured so much pain, and continues to smile and fight this relentless disease. Watching a child fight cancer is unbelievably painful; watching a child fight an incurable cancer is soul-destroying.

We try to live one day at a time, and I continue to research trials, still hoping that tomorrow we have options to give our little boy as much time as possible. If you would like to follow Mitchell's story, he has a Facebook page, Team Mitchell, www.facebook.com/myrarecancer. ■

"I will always remember Mitch & his family from the NIH Clinic. A sweet boy, who is numb to moving in and out of hospitals, and his mother Glenda, fighting her son's invisible predator of childhood cancer. It is unsettling to know that we have not found the right treatment at the right time for Mitch. We were compelled to take action and launch the Pediatric & SDH-Deficient GIST Consortium for kids like Mitch."

- Sara Rothschild, Vice President, Program Services

▶ PROTOCOL continued from cover

* *Paraganglioma is a rare tumor, a collection of cells, that develops in the peripheral nervous system, closely related to pheochromocytoma. It can present as part of Carney-Stratakis Dyad (paraganglioma and GIST). For more info on paraganglioma and GIST, see bit.ly/Carney-Stratakis.*

Dr. Joshua Schiffman, a member of the Pediatric & SDH-Deficient GIST Consortium, is conducting a research study on SDH-mutated Paragangliomas (PGLs), Pheochromocytomas (PCCs), and other tumors, entitled **The Paraganglioma Preclinical Project.** The project is underway and Dr. Schiffman is looking for support.

What's the project about?

To quote from a recent appeal from Dr. Schiffman: Paragangliomas (PGLs) and pheochromocytomas (PCCs) are extremely rare in the general population (along with other SDH-driven tumors like some gastrointestinal stromal tumors [GISTs] and Renal Cell Carcinomas [RCCs]). When these rare tumors are found in the healthy person in the population, they are often slow growing and benign, especially PGLs.

However, when PGLs and PCCs develop due to an underlying genetic and inherited cause such as a germline mutation in the SDH family of genes (like SDHB mutations), these tumors can become quite aggressive and difficult to treat.

In the setting of an underlying genetic cause, these tumors can spread, or metastasize, and they can be very resistant to any type of treatment. Unfortunately, once these PGL or PCC tumors spread throughout the body, we have very little to offer patients in the way of successful therapy.

One of the challenges of treating these tumors is that they are so rare that it is difficult for doctors and scientists to help choose the correct therapy and run a large-scale clinical trial. This effort is being funded in order to improve the lives of patients with PGL/PCCs and hopefully to find a treatment for this disease.

What's the goal?

As part of this project, we have established a PGL/PCC team of collaborators at HCI that will work to:

1) collect PGL/PCC tumors from patients when they are removed surgically,
2) grow these tumors in the laboratory,
3) perform genomic analysis on the tumors,
4) implant these tumors into mice,
5) maintain these mice so that tumors can be expanded into more mice, and
6) test novel therapies and combination of drugs to identify what can effectively work to stop these tumors from growing in mice.

Can I participate?

Patients with PGL/PCC tumors (and any SDH-driven tumors) regardless of their location can participate and will be invited to be enrolled on the Cancer Genetics Study at HCI, an Institutional Review Board (IRB)-approved study that consents patients with rare cancers due to genetic causes to share their tumors for research like that being done as part of the PPP. **Patients with PGL/PCCs that will require surgery who are interested in participating in this project can call (801)-587-4768 for more information and ask about participating in the Cancer Genetics Study.**

As the Pediatric & SDH-Deficient GIST Consortium continues to work collaboratively to share research projects, we will continue to feature studies where patient participation and data can move the research forward.■

Dr. Schiffman is a Professor of Pediatrics and Investigator at Huntsman Cancer Institute, the Medical Director for the Family Cancer Assessment Clinic, and the Education Director for the Program in Personalized Health at the University of Utah. He is also a Pediatric Hematologist-Oncologist at Intermountain Primary Children's Hospital in Salt Lake City, Utah.

He oversees a research laboratory at Huntsman Cancer Institute at University of Utah where he focuses on genomic development of cancer in children and studying animals that develop more cancer (like pet dogs) and other animals that are naturally protected from cancer (like elephants and whales).

Dr. Schiffman's work revolves around genetic risk for cancer and its application for cancer diagnosis, treatment, and prevention. Dr. Schiffman's clinical and research efforts focus on how to translate scientific discovery from the laboratory to his patients at increased risk for cancer.

Read more about the Consortium: bit.ly/Consortium101518

Night to Fight Cancer

Annual Fundraiser

By **Jessica Nowak**, Director, Outreach & Engagement

The 15th annual "Night to Fight Cancer" (NTFC) benefiting the Life Raft Group's research programs took place November 7th, 2018 at Midtown Loft and Terrace. Jerry Cudzil, LRG Board President, was joined again by co-host Matthew Knopman. Matthew has considered Jerry a mentor and friend for over fifteen years and continues to support the Life Raft Group.

Jerry and Matt were overwhelmed by the enormous amount of support and generosity from friends and family who attended. Each year we are blown away by the number of participants, and this year's numbers did not disappoint! With 225 participants, we were able to raise close to $220,000 for the Life Raft Group! NTFC attendees enjoyed the no-nonsense barbecue food provided by Pig Beach NYC, which included assorted sliders, mac n' cheese, coleslaw, peanut butter buckeyes and brownies.

The poker event took place on the main floor, while other guests enjoyed casino games on the Terrace. The winners of the night were Lyon Carter III in 1st place, Michael Cudzil in 2nd place, and Stephen Cudzil in 3rd place!

We would like to thank our NTFC Ambassadors, Brian Behrens, Lyon Carter III, Benji Cheung, Armando Petruccelli, and Netty Tsai for their continued support year after year. A special thank you also goes out to our corporate sponsors: our Club Sponsors, who donated $10,000, include Bank of America, Morgan Stanley, Goldman Sachs, Pfizer, RBC, and Tradeweb. Our Hearts Sponsors, who donated $5,000, include Cantor Fitzgerald, Fenics, and Nomura.

Many thanks to our major donors, Jerry Cudzil, Matt Knopman, Laird Landman, Jess Ravich, J.P. Morgan, and the Dan and Stacie Allen Charitable Fund, for their very generous contributions. We also appreciate Guggenheim Securities, Deutsch Bank, Citibank, and Natixis, for their continued support. In addition, we would like to thank Lyon Carter III, our beverage sponsor, Michelle Mattioli for donating photography services, volunteers Anthony Cashin, Cora Ramadan, Michael DeStefano, and Merilyn Marmora, and awards donors Murray Rosenthal, Michael Lamb and Robert Scherzer. ■

LOOKING FORWARD TO 2019:
SAVE THE DATE
NIGHT TO FIGHT CANCER
OCTOBER 24, 2019

Left to right: Co-Host Matthew Knopman, Norman Scherzer, and Host Jerry Cudzil

Left to right: Matthew Knopman with 3rd place winner, Stephen Cudzil; 2nd place winner, Michael Cudzil; 1st place winner, Lyon Carter III; and Jerry Cudzil.

Left to right from back: LRG staff members Lorraine Ramadan, Sahibjeet Kaur, Laura Occhiuzzi , Allison Russo, Pete Knox, Merilyn Marmora, Norman Scherzer, Board Member Teena Petersohn, Board Member Jerry Cudzil, Angela Edson, Matthew Mattioli, Helena Mattioli, Volunteer Fred Chamanara Front row: Diana Nieves, Jess Nowak, Denisse Montoya, Chelsea Gottfried, and Sara Rothschild.

See more NTFC 2018 Photos: liferaftgroup.org/ntfc-2018

Our 2018 Holiday Campaign features YOU - The Faces of Courage

We will be featuring stories throughout the holiday season illustrating the many Faces of Courage. Please donate today! You can also setup up a crowdfunding fundraising page through the Donate link below. If you do not have the ability to donate, or just want to do more, crowdfunding can help to generate a lot more donations to go towards finding a cure. We have proven this with our GIST DO IT Walk in Miami this past July. One member reached out to friends and family on social media using their own LRG crowdfunding fundraising page and raised over $10,000!

Learn more & donate today at liferaftgroup.org/donate2018

See More Faces of Courage: liferaftgroup.org/2018/12/donate2018

▶ CANCER MARCH continued from cover

In Chile, several types of cancer, including GIST, are not covered by the health systems, (public or private), which means that many patients do not have access to the treatments they need to live, and most of them do not have the resources to pay out of pocket. Therefore, they need a law that will protect them. What we are asking is that the law will:

- Strengthen cancer prevention campaigns
- Strengthen primary care attention to be able to make early diagnosis
- Incentivize oncology specialists: We have 100 oncology specialists and we need at least 400
- Create a National Cancer Patient Registry
- Guarantee health coverage for all types of cancers
- Assure multidisciplinary attention from diagnosis to the end of life for patients and caregivers
- Incentivize investigation
- Create more Oncology Centers of Excellence

The project of law is an initiative of Carolina Goic, a senator who is also a cancer survivor. We presented it to the President for the first time in 2013, and since then, we have been advocating for its passage.

A month ago a group of patient organization leaders, together with Goic, Dr. Claudio Mora, an oncological surgeon who is a cancer patient himself, and other congressmen, again presented the project of law at "La Moneda" (the Chilean White House). After that we decided to organize a March to create awareness of the reality of cancer patients in Chile, and ask our President to sign the project of law so that it can go to Congress to start the approval process.

The March took place throughout Chile. In Santiago, more than 2000 people participated in the March, most of them patients with multiple needs. It was really touching to see how "a face" was given to each one of the problems cancer patients have. Among them was María José Araya, a young mother of two. A member of our Registry Program, María needs to start her treatment with Regorafenib, but can´t afford the medicine.

I had the privilege to be one of the leaders of the march together with Senator Goic and other patient organization leaders. Fundación GIST Chile was present in Santiago with more than 50 representatives. We were also represented in Valparaiso, La Serena, and Concepción.

Our blue t-shirts made a beautiful contrast with the red balloons, illustrating a message of hope, but also one of determination and strength. During the march we shouted again and again: "We need a cancer law NOW!"

BREAKING NEWS FROM PIGA

Today [12/4], the last, or maybe the first, chapter of this story was written. I was invited, together with other representatives of cancer patient organizations, to La Moneda (the Chilean "White House"), to be present in the ceremony in which President Sebastián Piñera signed two very important documents: The National Cancer Plan 2018–2028 and the National Cancer Law - this last one, a project for which we have been advocating for years and for which we marched last November18th.

We are very happy! This is a very important step that marks the beginning of a project in which we will continue working until we have a law that will protect cancer patients and their families. This is an achievement of the civilian society, of patient organizations. We have been fighting for years to make cancer a priority in Chile. This is our triumph! We thank President Piñera for signing this law project. With his signature, the National Cancer Law will start its approval process in the Congress.

Our commitment is to continue working together participating in the discussion at the Congress. With the same strength that we marched, we will continue working until we have a law that meets the real needs of cancer patients and their families.

Left to right: Piga Fernández, with Senator Carolina Goic and President Sebastián Piñera of Chile

An Exciting Time for GIST Research

News from CTOS 2018

By **Pete Knox,** Senior Director, Research

The annual meeting of the Connective Tissue Oncology Society (CTOS) took place in Rome, Italy, from November 14th through the 17th. This meeting is one of the largest and most influential ones that focuses solely on sarcomas, and GIST was a significant topic of discussion in a number of sessions.

The Life Raft Group sent two senior staff members to the meeting to focus on two main goals: attend informational sessions to learn what was happening in GIST research, and meet with various doctors and patient groups to figure out how best to collaborate on behalf of patients with GIST and other sarcomas.

Left to right: Laura Occhiuzzi, Pete Knox, Alyssa O'Driscoll, Sarcoma Alliance, Annie Achee, National LeioMyoSarcoma Foundation, Denise Reinke, SARC, and Mitch Achee, National LeioMyoSarcoma Foundation.

LRG continues to collaborate with medical experts and advocacy partners

In addition to taking in a number of presentations, a number of collaborative meetings also occurred. While attending the SARC pre-meeting (which traditionally kicks off the CTOS session), we met with Dr. Gary Schwartz of Columbia University Medical Center, who has worked with the LRG on a number of projects, and discussed the potential for future collaborations.

Similarly, we later spoke with Dr. Ramesh Bulusu, a member of the LRG's Pediatric and SDH-Deficient GIST Consortium, of Cambridge University Hospital in the UK about strengthening our collaborations to benefit UK GISTers. We then moved on to speaking with representatives from various patient groups, including Annie and Mitch Achee from The National Leiomyosarcoma Foundation, Denise Reinke from Sarcoma Alliance for Research through Collaboration (SARC), Alyssa O'Driscoll from Sarcoma Alliance, and Peter and Sue Wyckoff from Rein in Sarcoma.

All of these groups are eager to collaborate with us in order to help share findings that can benefit all sarcoma patients. A key topic of these discussions was SideEQ (www.mysideeq.org), an LRG platform that helps patients track and manage their side effects. As the platform is not limited to GIST patients, but instead focuses on patients with all types of cancers, there was a great deal of interest regarding collaboration.

In addition, SARC has created a Sarcoma Coalition Education Workgroup that is designed to help sarcoma organizations collaborate on a number of issues, and the LRG committed to increased participation in that effort as well.

GIST Research

The heart of the meeting was the presentation of current GIST and sarcoma research. There was a great deal of information, which can be divided into a number of broad categories:

1. A discussion of an important survey that deals with a side effect felt by many GIST patients*

2. Two reports on drugs currently in clinical trial for GIST**

3. Three different presentations covering circulating tumor DNA testing (ctDNA) – a technology that hopes to provide better details regarding treatment options for metastatic GIST patients.***

Read more about CTOS 2018 on our website

Including details on:

* An important survey from Dr. Anette Duensing, a member of the LRG Research Team, from the University of Pittsburgh

** Notes on Avapritinib (BLU-285) and DCC-2618: two drugs currently in clinical trial for GIST

*** A treatment with future potential - ctDNA

More CTOS 2018 at liferaftgroup.org/ctos-2018

GIST DAY OF LEARNING Michigan

By **Diana Nieves**, MPH, Senior Director, Outreach & Engagement

Dr. Baker presenting at GDOL Michigan

Interested in having The LRG come to your city for our next GDOL? Contact Diana: DNieves@liferaftgroup.org

The Life Raft Group held its first GIST Day of Learning (GDOL) in Michigan on October 27th in Ann Arbor. GDOLs are free, one-day programs that provide both education and support to the GIST community. Top GIST specialists present the latest on research and treatment options as well as provide a comprehensive review of the science behind GIST. GDOLs provide an opportunity not only to meet and interact with local expert practitioners in an intimate setting, but also for patients to connect with one another in a supportive environment.

Over 70 patients, caregivers and researchers attended the weekend event. Lawrence Baker, MD, from the University of Michigan's Rogel Cancer Center opened the day with an Introduction and Brief History of GIST. Many of the attendees were newly diagnosed so this session was especially informative to them.The Role of the Cancer Surgeon in Diagnosis and Therapy was led by Will Burns, MD, and and the Role of the Medical Oncologist in Diagnosis and Therapy was led by Rashmi Chugh, MD.

Clinical pharmacist, Rachelle McDivit and Stephanie Taylor, RN discussed Drug Interactions and Other FAQs and Side Effect Management. Many of the participants were very grateful for this session as many had indicated side effects being an area of concern. Scott Schuetze, MD, then discussed New Therapies On the Horizon. Dr. Baker ended the day with a very compassionate and empowering discussion on GIST Patients as Cancer Survivors.

GDOLs provide an opportunity to interact with the physicians and presenters as well as other patients and caregivers. The session was extremely interactive. A huge thank you to all the physicians who spent the day taking questions from the attendees.A very big thank you to generous sponsors - Bayer, Novartis, Deciphera and Pfizer.■

Global Representative Spotlight

Verónica Armand Ugón, Uruguay

I am 45 years old and I live in Tarariras, a small city of Colonia, Uruguay. I am married and I have three wonderful children: Matías (18 years old), Tomás (15 years old) and Francisco (11 years old). I love traveling and making handcrafts and decorations, so I try to do things that make me feel happy in my free time, since I work as a part-time Executive Assistant.

In March 2013, after surgery of the small intestine, I was diagnosed with GIST. My mutation was KIT Exon 11, so since then, I have been using GLIVEC 400 mg per day.

I am a woman of Faith and I do believe that illnesses come to our lives to make some changes in our daily routine, to appreciate the wonderful gifts that God gives us every day and to take the learning of the experiences we face to become a better person. Positive thinking and mental attitude is very important to cope with GIST.

I am very enthusiastic about being the representative of ALIANZA GIST and The LRG in Uruguay, since I can give support to other people that face the same illness and offer them hope that there is a light at the end of the path!

Finally, I would like to thank my dear family and close friends for their unconditional support and love during all these years. I have you in my heart; you are a very important part of my life and I am blessed to share my days with you.

"LIFE IS AN ADVENTURE, ENJOY THE RIDE"

Overcoming the Holiday Blues

By **Mary Garland**, MA, Director, Communications

Everywhere you turn, the sights and sounds of the holiday season assail you. Before the Halloween candy is sorted and the Thanksgiving turkey is cold, we are reminded of what some call “the most wonderful time of the year.” Whether you celebrate Christmas, Hanukkah, Kwanzaa or no holidays at all, there is an expectation that all is to be festive, merry and bright this time of year.

But for some, the holidays are challenging. For cancer patients, the expectations of a consistently happy mood may be unrealistic. If you are newly diagnosed, the realization of what treatment means is probably not fully absorbed. You are adjusting to the new normal, dealing with the physical challenges and perhaps recovering from surgery or adapting to a new medication regime and resultant side effects.

Instead of joy, you may be experiencing an overwhelming sense of sadness or loss. Life has changed for you and your loved ones. In addition to dealing with cancer, there may be other challenges or losses in your life that are adding to your blue mood. This is normal, and it is perfectly appropriate to configure your holidays the way they work best for you. This may mean cutting back on the flurry of activities, and the social expectations that ensue. It may mean finding some new traditions that are less labor intensive, and are more joy-filled.

So how can you ward off ‘the Grinch’, aka cancer, to keep it from robbing you of holiday joy?

Here are some tips from your fellow GISTers to help you Take Joy in this holiday season

1. Looking at holiday decorations, listening to holiday music, looking at the snow falling and eating a holiday cookie. The beautiful silence of a snowy wintertime and the knowledge that “all is calm.” - *Wanda S.*

2. Faith. Choose to rejoice. You always have the agency to choose, and knowing this gives you power & strength. Use it to grow your joy. Seek joy. Choose hope. Trust. We are greater than our disease even when it hampers our daily lives. - *Margaret T*.

3. After my GIST diagnosis in 2003, I became depressed, but I eventually recovered. Then when Christmas came, I had a strong case of the holiday blues, and couldn’t shake it. The next Christmas, however, I planned a trip away from home with my husband. That helped tremendously! - *Dina W.*

4. I found the best way to cope is to socialize with family and friends and stay positive. - *Jeff D.*

5. Focus on serving somebody else. Maybe do some volunteer work or help a friend or family member in need. Sometimes seeing other people’s problems puts your own into perspective. - *Mary H.*

6. Count your blessings. Try keeping a notebook naming each and every one. - *Margaret C.*

7. Look for small things to brighten each day, like sunshine or warm feet. - *Carolyn D.*

8. Live to your fullest on your good days, and be compassionate to yourself on the rest. - *Karen D.*

9. Create a new tradition. Make it one that requires little preparation or effort. Take a walk as snow begins to fall. Watch a favorite holiday movie.

10. Attend a concert. This time of year many organizations have free concerts. Immersing yourself in music can provide a welcome retreat from your thoughts.

11. Don’t isolate. You may feel like hiding under the covers. Don’t. Take the time to talk to an old friend. Reach out to your LRG community on the ListServ or Facebook.

12. Get plenty of rest. You may need more that your normal amount this time of year.

13. Exercise in a way that works for you. Exercising releases endorphins, feel-good chemicals that will help elevate your mood.

14. Do something that gives you joy. Bake a favorite family recipe and take it to a friend. Play with your grandchildren. Take a walk. Enjoy holiday lights. Snap photos of family and friends. Find a favorite toy from your childhood.

15. Tend to your spirit. Consider attending a religious service. Visit a sacred spot. Commune with nature. Seek the larger meaning of life through reading or discussion. Meditate. Take a yoga class.

GIST Resolutions

by **Mary Garland,** MA, Director, Communications

With the start of a new year, there is an opportunity to make positive changes. Cancer patients face dramatic change in their lives, and chances are they have already experienced a time of reflection and self-evaluation.

What better time than the ending of the old year to move forward with positive changes that will impact both your health, and your state of mind? At a time when you may feel a loss of control, this is a way to feel empowered.

Top Ten Resolutions for GISTers

1. Eat a healthy diet designed with you in mind. Eating more unprocessed food, bearing in mind the limitations your cancer imposes (medications, post-gastrectomy, etc.) will lead to an optimal state of health. Consult a nutritionist for a plan customized for your individual needs.
2. Exercise more. Exercise not only leads to greater energy and strength, but also to better mental health. Find an exercise routine to which you can adhere and adapt it to your needs.
3. Promise to practice radical self-care. If you are tired, rest. Allow others to help when you feel overwhelmed. Taking breaks and cat naps are loving ways to recharge your batteries.
4. Don't isolate. Reach out to others when you feel depressed. Spend more time with family and friends.
5. Find time to help others. By sharing your strength, hope and experiences you will derive great benefit. Volunteering with the LRG provides a lifeline for new patients and can be rewarding for you.
6. Maintain your GIST treatments. Keep your appointments with your medical team, schedule the appropriate tests and scans, and maintain adherence to your medication regimen.
7. Spend time in nature. Being in touch with the elements relieves stress and anxiety and connects you to something larger.
8. Try something new. Learn a new language. Try crocheting. Take a class. Keeping the brain active can help with brain fog triggered by medication.
9. Count your blessings. Keeping a gratitude journal and writing what you are thankful for each night before bed can keep negativity away.
10. Live for the day. We don't know what tomorrow will bring, but we are given the gift of today. Make each moment of the coming year count.

Happy New Year from The Life Raft Group!

Thank You to our Major Donors for October & November

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UPCOMING EVENT

16

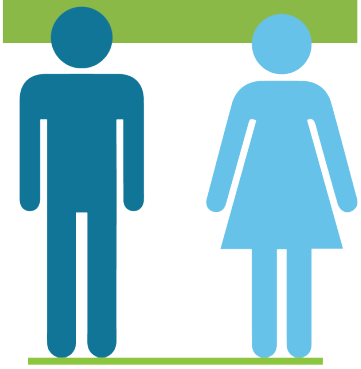
FEBRUARY 16, 2019
8AM-5PM

Ronald Reagan UCLA Medical Center
757 Westwood Plaza
Los Angeles, CA 90095, United States

View events: liferaftgroup.org/events

Patient Registry Data

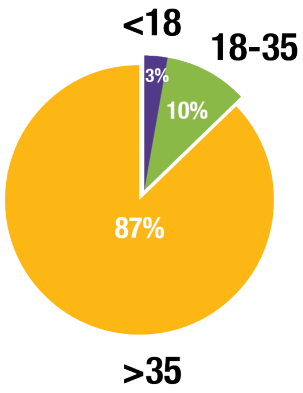
1911 Members



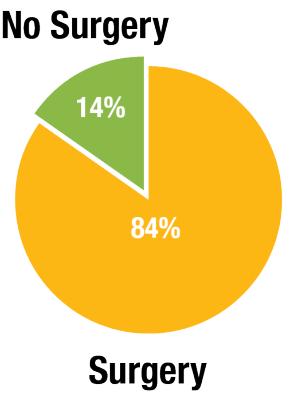
950 961

United States
1341
International
570

Age at Diagnosis



Surgery on Primary Tumor



Reported Mutations

832 patients reported

KIT	650
PDGFRA	69
NOS*	113

*No other specified

Top 5 Medications

1438 patients reported

The Life Raft Group Global Community

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

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Contact: Laura Occhiuzzi, Senior Vice President,

34 Life Raft Regional Chapters: Find info for a local rep at liferaftgroup.org/find-a-support-group

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