

Ensuring no one has to face GIST alone

**BREAKING NEWS**
NEW DRUG RELEASE PAGE 6

In Loving Memory: Ton van Schaik, Terrance Pridmore, Merak Melikian, Stephen Tallau, Vishwas Bawle

The State of GIST Treatments

Current and Emerging Medications

By Pete Knox, LRG Senior Director, Research

Many therapeutic options are available but matching the right patient to the right drug is still a challenge.

Flashback...it's June 2016, and I had just attended the American Society for Clinical Oncology (ASCO) conference in Chicago. Though this was not my first ASCO, a few things were different this time. First, there was a significant increase in posters and presentations about immunotherapy. In prior conferences this topic was represented by about 20% of posters and presentations; now it was closer to 70 or 80%, but nothing specifically for GIST.

That also pointed to the other thing (or lack of a "thing"). Not only weren't there any new drugs being touted for GIST, but there didn't seem to be any on the horizon. Worse yet, when talking to researchers and clinicians

about this, I discovered that there wasn't a lot of interest in research devoted to GIST since there were already plenty of therapies available. There also seemed to be an attitude that other, more immunotherapy-sensitive diseases were "hotter."

This was troubling. While we were lucky enough at that time to have three approved therapies for GIST (Gleevec, Sutent, Stivarga), it was well known that they didn't work for every patient. Also, of note was that these drugs were originally developed with another disease besides GIST in mind, and that GIST had kind of "gone along for the ride."

The future for new GIST treatments looked bleak. However, here's the thing about the future. It's hard to predict, because it can (and often does) change.

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Passion Translates to Advocacy Across the Globe

Leading the Way for GIST Patients

By Carolyn Tordella, LRG Assistant Director, Communications

When facing a diagnosis of cancer, self-advocacy becomes an important survival skill for patients and caregivers. It can be a challenge learning what questions to ask, communicating assertively, and making sure that your needs are a priority in discussions with doctors. For GISTers, the challenge grows exponentially due to the rareness of the disease,

even more so with the rarer mutations like SDH-deficient GIST or D824V.

Out of a need for answers a larger definition of advocacy can be born. Estelle LeCointe-Artzner's story illustrates what an ordinary person can accomplish due to extraordinary circumstances and how she affected change for so many people with GIST.

Estelle LeCointe-Artzner, France

When Estelle LeCointe-Artzner was diagnosed with GIST In 2004 there was no information in French for GIST patients.

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Thank you!

It has become a tradition in our February newsletter to thank all of those who have donated to the programs of the Life Raft Group, to our research, to our educational efforts, to our advocacy.

But that is only the tip of the iceberg. Because of your donation, a husband can reach out at three o'clock in the morning and find his wife sleeping next to him; a grandmother can attend the ballet recital of a granddaughter; and a son can get his dad on the phone to tell him how much he loves him. These and so many other ripples of hope define the impact on countless lives that each donation creates.

So, on behalf of our GIST community in the 67 countries reached by the Life Raft Group, we thank you for these gifts of hope and of time.

- Norman J. Scherzer, Executive Director

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No patient organizations like the Life Raft Group existed in France and when Estelle went to the National Cancer League for answers, she found their response lacking. Describing her feelings at the time, Estelle said, "I felt it was very unfair. I tried to find some information about GIST and the way that it could be treated. When I went to the National Cancer League, the only information I was given was that it was an orphan cancer and you can imagine that I found this kind of definition very disheartening because behind that short definition was the conclusion that there is no solution, just that you will probably die."

Estelle felt that if the National Cancer League had no definition about the disease that a patient would imagine that no doctor knew about GIST. This was untrue, but no patient could know that at that time. She knew that factual information about GIST had to be made available to patients.

"I knew from my oncologist that I was not the only patient with this disease, so this is how the story started. He had another patient who was in touch with The Life Raft Group through the New Horizons meeting which was newly organized by Novartis at that time. I was introduced to other French patients and I went to the next New Horizons meeting where I met people from

Novartis and from The Life Raft Group. It was then that I decided to create a patient organization when I returned to France," said Estelle.



Estelle Artzner & daughter Hazelle

Once she set her mind on creating a way for patients to have the information they needed, she started making connections.

"When I told my oncologist I was about to create a patient organization, he proposed that I meet with Professor Jean-Yves Blay, and that he might possibly be interested in the project. This was how I was introduced to the French scientific community."



Estelle established Association Française des Patients du GIST: Ensemble contre le GIST in October 2005 which has helped hundreds of patients navigate their GIST journey by providing resources and support that were not available when she began her own journey. Estelle's organization has spearheaded research efforts, raised funds for research, and has published articles and educational materials about GIST. As the face of GIST in France, Estelle has brought awareness to the public and to the medical community through conference presentations, panel discussions, and articles throughout the years discussing treatment adherence, research, and social issues, like having a relationship when you have cancer, among other subjects.



The Association Française des Patients du GIST: Ensemble contre le GIST also holds an annual conference for patients and caregivers in conjunction with the French sarcoma patients organization Info Sarcomes, founded in May 2009. The two groups have also recently combined their energies on a common website to provide resources for sarcoma and GIST patients.

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Fortunately for GIST patients, that's exactly what happened, and the last couple of years have seen a plethora of newly approved drugs. Where before there were three just aimed generally at GIST patients, there are now six, many aimed towards specific subtypes that before had nothing, with a seventh on the way. Perhaps even more exciting - a few of these drugs were developed "from the ground up" for GIST specifically. In addition, contrary to what we feared, new research in GIST is happening, and there may be even more therapies around the corner.

The First Wave – Gleevec, Sutent, and Stivarga

The history of GIST could arguably be divided into two phases. Before Gleevec and after Gleevec.

The impact of Gleevec on GIST patients could not possibly be overstated, and one can argue that the impact it had on the general world of cancer was almost as profound, as it ushered in the age of targeted therapy.

What many may not know, however, is that Gleevec was not originally developed for GIST, but for chronic myelogenous leukemia (CML).

Drug/Brand Name: IMATINIB (GLEEVEC, GLIVEC)	
Manufacturer	Novartis
Indication and Treatment Line	CML (later for GIST), 1st Line*
Dosage	100 or 400 mg tablets (400 mg is common starting dose, patients can escalate to 800 mg), Continuous dosage
Approval Year	2001
Status	Generic for CML (as of Feb 2016)**
Molecular Targets	Primarily KIT, PDGFR, BCR/ABL
Side Effects of Note	Edema, eye bleeds, thinning of skin, fatigue, diarrhea, muscle cramps, anemia, bone deformation

****Please note that Gleevec for CML became available as a generic in 2016 in the U.S., however, the patent for Gleevec for GIST expires in 2020. Patients in the U.S. are prescribed the generic as their doctor/healthcare situation indicates. Globally, patents vary by country and your individual situation may be different.**

As mentioned earlier, Gleevec was approved for another indication first (CML). Sutent was actually approved for RCC (renal cell carcinoma) and GIST on the same day. Stivarga, like Gleevec, was approved for another disease (colorectal cancer) first. All of these drugs aimed towards a broad set of molecular targets as opposed to a more narrow, specific one.

The impact of Gleevec is truly remarkable. While the high numbers for median progression free survival (PFS) reported in the trial and afterwards are quite impressive (24 months in the trial, and later numbers trend much higher). Although not the norm, what is even more impressive is the durability of responses. In our LRG Patient Registry we have tracked a handful of patients that have continued to respond for twenty years or more.

One just needs to look at the news even today where oncology drugs that report median PFS numbers of 6 months or less are lauded as "breakthroughs" to see how truly amazing that is.

Gleevec was a game changer, but not all patients responded to this therapy. For patients that progressed while on Gleevec, another option was needed.

That option became available in 2006, when Sutent was approved.

Drug/Brand Name: SUNITINIB (SUTENT)	
Manufacturer	Pfizer
Indication and Treatment Line	Renal Cell Carcinoma (RCC), GIST 2nd Line
Dosage	12.5, 25 mg and 50 mg tablets (typical doses vary from 12.5 to 50 mg), can be continuous or cycled. (4 weeks on/2 weeks off)
Approval Year	2006
Status	Name brand (not yet generic)
Molecular Targets	KIT, PDGFRA, VEGFR and others
Side Effects of Note	Hand and foot syndrome, hypertension, diarrhea, liver toxicity (rarer), thyroid dysfunction

**1st, 2nd, 3rd or 4th line refers to the sequence in which the treatments are given*

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While some of the molecular targets of Sutent were common to those targeted by Gleevec (KIT, PDGFRA), others were not (VEGFR). In addition, some patients with particular submutations (i.e., KIT exon 9 as opposed to KIT exon 11) seemed to do better on one drug than the other.

While the median PFS numbers were not as high as those seen with Gleevec (remembering of course that these patients were primarily 2nd line, so that comparison was not really apples to apples), it was better to have a second option when Gleevec failed than to have nothing at all.

Thus, there was room for yet another weapon in the arsenal, and that came a few years later in the form of Stivarga.

Drug/Brand Name: REGORAFINIB (STIVARGA)	
Manufacturer	Bayer
Indication and Treatment Line	Colorectal cancer (metastatic), Hepatocellular carcinoma, GIST; 3rd line
Dosage	40 mg tablets, typical max dose 160 mg (typically cycled 21 days on/7 days off)
Approval Year	2013
Status	Name brand (not yet generic)
Molecular Targets	KIT, PDGFRA, VEGFR and others
Side Effects of Note	Hand and foot syndrome, hypertension, diarrhea, liver toxicity (rarer)

As is shown in the table above, Stivarga hit many of the same targets as Sutent, but again, in some cases patients would respond to one drug but not to the other. This was often due again to a specific submutation. The problem, however, was that in many cases, GIST patients were never given mutational testing in the first place, meaning they simply moved through each of the approved lines as they progressed. Obviously, this was not the optimal way to treat patients, and that situation needed to change.

The Life Raft Group continued to speak to clinicians and standard-settings bodies, and little by little, the rates of testing increased, but at a much slower rate than anyone wanted. Luckily, some changes in therapeutic approaches were on the horizon that would help drive home the necessity of mutational testing – changes we will profile in the next section.

The Next Wave – Hyper-Targeted Therapies

It is important to keep in mind that many rare cancers have no therapies available. By 2013, GIST patients were fortunate enough to have three to choose from – or at least, some GIST patients were. While there was anecdotal information that some of the approved medications worked better for specific mutation and exon combinations, none of them were approved for something so specific – instead they were based on much more general indications. Here are the indications for GIST taken from the prescribing information for each of the three previously mentioned approved drugs.

Medication	Indication(s)
GLEEVEC	Patients with KIT (CD117) positive unresectable and/or metastatic malignant gastrointestinal stromal tumors (GIST). Adjuvant treatment of adult patients following resection of KIT (CD117) positive GIST
SUTENT	The treatment of gastrointestinal stromal tumor (GIST) after disease progression on or intolerance to imatinib mesylate
STIVARGA	Locally advanced, unresectable or metastatic gastrointestinal stromal tumor (GIST) who have been previously treated with imatinib mesylate and sunitinib malate

As you can see, there are no specific mutation/exon combinations directly mentioned. Basically, they just identify that the patient should have GIST, and then specify which “line” of medication – i.e. when to sequence them based on the failure of the previous therapies.

Luckily, the next round of drugs changed that approach. They all aimed at more specific subsets – in some cases general mutation and exon combinations, and in other cases very specific mutations/genetic events. Perhaps most importantly, these therapies required patients to have specific mutational testing to ensure they had these mutations/genetic events before being prescribed the medication.

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The first of these new drugs was larotrectinib, which when approved would be named Vitrakvi.

This was a unique drug in many respects – first, it was approved not for a specific disease, but instead for a particular target – the NTRK gene fusion.

Drug/Brand Name: LAROTRECTINIB (VITRAKVI)	
Manufacturer	Loxo (Eli Lilly)/Bayer
Indication and Treatment Line	Various, including GIST; patients with a documented NTRK fusion
Dosage	25 or 100 mg tablets, oral suspension; 200 mg/day
Approval Year	2018
Status	Name brand (not yet generic)
Molecular Targets	Various NTRK fusion partners (for GIST, relevant partner is NTRK3/ETV6)
Side Effects of Note	Generally, well tolerated; fatigue, nausea, dizziness, anemia

Gene fusions occur when parts of two genes join or “fuse”. A simple analogy would be if you and your friend decided to switch shirts. Shirt from “A” is with the pants from “B” and vice-versa. Gene fusions occur in various cancers, and Vitrakvi was essentially approved for many of them simultaneously – anywhere where NTRK had a fusion “partner” (which is what the other gene that fuses with NTRK is called).

NTRK fusions exists in GIST are rare. The most common fusion partnership is between NTRK3 and ETV6.

What was also unique about Vitrakvi was that it required the patient to have a specific test verifying the presence of an NTRK fusion before it could be prescribed which helped to usher in the era of more specific drugs tailored to a patient’s unique mutation.

Patients on the trial had extremely high response rates for extended periods of time.

This is not surprising considering that the drug was optimized to the patient’s specific molecular makeup. This would be a recurrent theme in the drugs to come.

Shortly thereafter a second drug for NTRK fusions was approved, which was somewhat similar to Vitrakvi called entrectinib.

Drug/Brand Name: ENTRECTINIB (ROZYLTREK)	
Manufacturer	Ignyta/Genentech/Roche
Indication and Treatment Line	Various, including GIST; patients with a documented NTRK fusion
Dosage	600 mg daily
Approval Year	2019
Status	Name brand (not yet generic)
Molecular Targets	Various NTRK fusion partners (for GIST, relevant partner is NTRK3/ETV6), ALK rearrangement
Side Effects of Note	Generally well tolerated; fatigue, dizziness, weight increase, nausea, constipation, diarrhea

The biggest difference between the two drugs is that Rozyltrek has additional benefit for lung cancer patients, but as far as GIST they are fairly similar, with side effects varying a bit in some cases.

While the two NTRK drugs were a welcome addition, and especially because of their emphasis on the need for mutational testing, NTRK is exceedingly rare, so in reality the landscape had not changed that much for most GIST patients.

Luckily, a pair of drugs were just on the horizon – one has been approved, and another is still in trial with expectations of being approved soon. These drugs seem to have hit the “sweet spot” of utilizing mutational testing to target specific targets that are prevalent in many patients and they have been designed from the very beginning with GIST patients in mind.

Learn more about NTRK fusions:
bit.ly/NTRKfusions

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The first of these is Ayvakit, approved on January 9th, 2020, known as avapritinib and BLU-285 when it was in trial. Its initial approval was for metastatic GIST patients with a PDGFRA mutation (which represents anywhere from 5-8% of all GIST patients) including those with the D842V mutation – a combination that was resistant to all previously approved treatments.

NEW DRUG RELEASE

Drug/Brand Name - AVAPRITINIB/BLU-285 (AYVAKIT)	
Manufacturer	Blueprint Medicines
Indication and Treatment Line	Mastocytosis and GIST; metastatic PDGFRA (incl. exon 18 D842V), other lines pending (4th, potentially 2nd or 3rd line)
Dosage	300 mg daily (at least 1 hr before and 2 hrs after food)
Approval Year	2020
Status	Name brand (not yet generic)
Molecular Targets	PDGFRA/D842V (metastatic patients), KIT (later lines)
Side Effects of Note	Nausea, fatigue, anemia, edema, diarrhea. Cognitive impairment has been highlighted (appears to be dose-dependent; it is unclear if reversible); intercranial hemorrhage occurred in a small number of patients on the trial. <i>See note below*</i>

Ongoing trials are underway for everything from 2nd through 4th line, including patients with KIT mutations. Blueprint Medicines required patients to undergo mutational testing as part of their trial protocol, and now that it is approved, they will also require testing to receive the approved drug. Because of this, it is again perhaps not a surprise that the response rates observed in the trial were significantly higher for some patients (specifically D842V) than we are accustomed to seeing. To further underline the importance of mutational testing, Blueprint Medicines has also created a campaign that can be accessed via [mutationmatters.com](https://www.mutationmatters.com).

* We have met with Blueprint Medicines to discuss these side effects as they navigate their GIST journey. It is our intention to keep patients informed about all new treatments.

Currently, these are all of the approved drugs for GIST

Considering that just a few short years ago there was concern that no new therapies would be available, that is quite good news. In addition, to these approved drugs, there is another drug currently in trial that has the potential to help a lot of GIST patients.

Like Ayvakit, ripretinib from Deciphera is also a targeted drug designed from the ground up for GIST that requires a mutational test to be utilized effectively. What differentiates it is that it hits a wider variety of targets, so it has the potential to be of even greater benefit.

Drug/Brand Name - RIPRETINIB/DCC-2618	
Manufacturer	Deciphera
Indication and Treatment Line	Mastocytosis, gliomas, solid tumors, GIST; currently in trial for 2nd, 3rd, and 4th line for GIST
Dosage	150 mg daily
Approval Year	Still in trial (expected approval date is mid/late 2020)
Status	Currently in trial
Molecular Targets	KIT/PDGFRA Exon 9, 11, 13, 14, 17, 18 (incl. D842V)
Side Effects of Note	Alopecia, muscle pain, fatigue, hand-foot syndrome

As illustrated above, ripretinib hits multiple targets and is generally well tolerated, so it has the potential to be a true game-changer for GIST patients.

In summary, the landscape for GIST has changed a great deal since I left that ASCO meeting in 2016. Where we had once had only three available treatments aimed broadly at GIST patients and not addressing some subtypes, we now have six, a few of which are designed to target specific subtypes, with a seventh potentially to be released soon. The rationale for mutational testing has also become much clearer, and pharmaceutical companies are beginning to require it as a precursor to prescribing their medications (and in some cases offering to pay for it if it is not covered by insurance) – a development we can all be happy about.

I am fairly confident we will continue to see progress for GIST patients for the near future and beyond. Although we embrace the progress being made, we recognize we still have a long way to go while there are still no treatments available for rarer subsets of GIST.

One Perspective on the BLU-285 Trial (avapritinib/Ayvakit)

Owen Moore, GISTer, PDGFRA D824V

LRG member Owen Moore began participating in the Phase I BLU-285 trial at OSHU with Dr. Michael Heinrich in 2016. After three other drug treatment lines and faced with several growing tumors, the BLU-285 (avapritinib) trial was Owen's best option at the time.

"It most definitely saved his life," said Jennifer Moore, Owen's wife. "We were thinking he might only have another year before he was in real trouble. And that he was able to get a treatment that was actually helping him without bankrupting us too was a great thing."

When Owen started the trial, he had several large tumors. Five or six were tracked specifically and most of them shrank a little bit by the first CAT scan, which took place eight weeks after starting the drug. One of the larger tumors took about six months before it showed any change. Owen went up to the maximum dose. "I think he was one of the patients on which the research team determined what the maximum dose should be," Jennifer said.

"He's stable now, meaning for most of the tumors there's no growth or an 'acceptable level of change'. I don't think they are ever going to disappear completely, though I think the BLU has stopped others from developing. The last report we got literally just said no change for some of the tumors instead of listing their before and after dimensions."

Jennifer says the trial has been a good experience overall. The Moores feel great support, guidance, and patience from Dr. Heinrich's staff, and a side benefit of the travel between Illinois and Oregon is

that they made friends and memories in the process.

"Our situation was unique in that at the time of Owen's diagnosis we were both self-employed, so we were able to make the travel between Illinois and Portland work for us. We are quite fond of Portland and would move out there in a heartbeat if we could," said Jennifer.

The trial, while rendering a good result, has not been without its downside. BLU-285's side effects have included things such as watery eyes, rash, edema, diarrhea, persistent fatigue, infections, and cognitive issues. Some are persistent; others come and go. Discovering the correct therapeutic dosage that is tolerable, and differentiating what is a side effect and what conditions your body may now be susceptible to are also challenges, but all in all, they both feel that participating in the trial has been worth it.

According to Jennifer, Heinrich's team has said that, if he chooses, Owen might remain in the trial for indefinitely for now so that Blueprint Medicines can continue to track patients' progress on the drug.



Owen & Jennifer Moore

**For more information on the newly released
Ayvakit (avapritinib/BLU-285):
<https://liferaftgroup.org/ayvakit-for-gist/>**

Thank you to our Major Donors for December & January!

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The same year, Estelle and other European GIST and sarcoma patients organizations co-founded the European Sarcoma Patients Coalition “Sarcoma Patients Euro Net (SPAEN).

Estelle listed some of the current challenges for GIST patients and how her organization’s education and awareness efforts are not over.

“There is information out there about the disease thanks to our organization and they know where they can be treated and that is a tremendous change since 2004. Today’s challenge is the quality of surgery. Also, the fact that non-GIST specialists can prescribe Glivec but we know that the disease is much more complex than that. This is an issue especially for people dealing with progression because most of the time, these non-specialist doctors do not know what to do in cases of progression,” said Estelle.

Her organization is also dealing with the issue of new government regulations and the uncertain future of the continued affordability of treatment for patients. In January, the government determined that if a generic exists, then a patient must receive the generic or pay out-of-pocket for Glivec which would be very difficult for patients to afford. Estelle explained that legally, if the generics are the same price as the name brand Glivec, patients should be allowed to receive Glivec without extra cost but most of the patients ignore this.

Furthermore, for pharmacists there is obvious confusion between the end of the patent of Glivec for CML (which is already expired and allow pharmacists to replace princeps by generics) and the end of the patent of Glivec for GIST (which is planned in October 2021 and protects patients from receiving generics before this date. She has been fielding questions from patients that are experiencing confusion and fear. The organization will be gathering additional information for their patients.

Another issue for French patients is concerning mutational testing which is not always routinely done in GIST cases. For example, one patient diagnosed with GIST experienced two recurrences in a short period of time before her oncologist ordered mutational testing and it was discovered that she did not even have GIST, though she was being treated for the condition for years.

“It is routinely done in cases of where there are recurrences but unfortunately not always at that the time of diagnosis,” said Estelle.

At this time there is not a French patient registry run by a university or a patient advocacy organization due to regulatory issues, so there is not an accurate public count of GIST patients or a breakdown of their

mutations. Through her contact with the medical community and patients, Estelle estimates there are approximately 1,000 GIST patients diagnosed with GIST each year in France.

Estelle, though currently on maternity leave, continues to strongly advocate for GIST and sarcoma patients in France and is concentrating on a new awareness campaign focused on diagnosing sarcomas and GIST since onset of first symptoms.

Piga Fernández, Chile

Across the globe and several years prior to Estelle’s diagnosis, LRG Global Consultant Piga Fernández found herself in similar circumstances. Piga was misdiagnosed in 1995 with leiomyosarcoma and had a second occurrence of liver metastasis in 2002. She’d endured surgeries, radiotherapy, and was experiencing a poor quality of life. A second opinion rendered a correct diagnosis: GIST.



Piga Fernández

Piga feels she was very fortunate that she was able to get Glivec through a patient program run by Novartis. Others were not so fortunate, having no access to treatment, and though her own situation was resolved, Piga felt led to help others thrive on their cancer journey. Although after starting taking Gleevec she didn’t want to know anything about cancer, after a few months she started to search the internet about this rare disease and found The Life Raft Group. After many back and forth emails with Sara Rothschild, she finally accepted to participate in a meeting in Monterrey, México in which Alianza GIST was born, and where she decided that she needed to do something to help GIST patients and their caregivers, as a way to express gratitude for the gift she had received being able to take the treatment that had kept her alive.

Back in Chile, she helped to create a new GIST and CML patient support group. Years later, after being part of the Board of Directors, she realized that she needed another scenario to develop all the projects she had in mind, especially after being aware of all the resources that LRG had available for GIST patients. When her period as Vice-President was finished, she created Fundación GIST Chile to work in four main areas with patients and caregivers: Psychological support, education and information, advocacy and support to investigation, through publications, presentations, videos, and support groups as well as connecting patients with specialists.



More recently, Piga joined with others to change the laws regarding healthcare for cancer patients in Chile. In a large collaborative

effort, patient organizations, the medical community, healthcare stakeholders, and the Chilean Health Ministry, fueled by the relentless advocacy of people like Piga, worked together advocating to have access to the treatments many cancer patients needed through the Ricarte Soto Law, and one of the big achievements was to have imatinib and sunitinib covered by this law. Several years of advocacy work through marches, discussions, presentations, and speeches resulted in National Cancer Law being passed together with the National Cancer Plan. Piga, as member of ACHAGO (the Chilean Association of Cancer Groups), the medical community, and politicians, like Chilean Senator Carolina Goic worked together to accomplish this goal.

As our Global Consultant, she has also been instrumental in the formation of the GIST Regional Registry Latin America for patients. This move will increase the amount of data available to researchers working on new treatments and allow more data analysis to help with better survival outcomes for patients.

What's next for Piga? Fundación GIST Chile was recently given the task of working with Dr. Marcelo Garrido to provide mutational testing for every GIST patient free of charge. This will help to ensure that patients are receiving treatments that are available and add to the database of knowledge for research and analysis. Also on the advocacy to-do list: spread awareness of GIST beyond Santiago and reach patients along this "long and skinny" country named Chile.



Debra Melikian, California, USA

The path to patient advocacy is a complex and diverse one. It is not uncommon for the catalyst to be that a loved one is diagnosed with a disease, catapulting someone into the world of advocacy out of both caring and necessity. It is rare that the path would also lead to a powerful partnership with a medical professional, and the drive and determination to find ways to further research for a rare disease.

For Debra Melikian, mother of a young patient with SDH-deficient GIST, that path would first lead her to The Life Raft Group and to a GIST Day of Learning (GDOL) that would alter the course of her and her son's life forever.

At that GDOL, she met Dr. Jason Sicklick of Moores Cancer Center at the University of California in San Diego. Sicklick is a surgeon, GIST specialist and clinical researcher. It was the beginning of a unique partnership.

"Merak was the initial inspiration to Dr. Sicklick in his SDH research. He was patient zero on temozolomide a few years back, and his tumor cells were one of the first human SDH-deficient GIST cell lines to be cultured in a Petri dish. Using these cells, for the first time ever, Dr. Sicklick and his team have been able to test thousands of FDA approved drugs and compounds against Merak's cell line, as well as those subsequently developed from other patients' tumors," said Debra.



Dr. Jason Sicklick and Deb Melikian

Continuing she shared, "From there, a short list of drugs has been gleaned, for further research in his laboratory. There is progress being made in real time. We must continue to fund this all-important research at UC San Diego in memory of Merak and all the other young SDH-deficient GIST patients that have battled this disease. While I have lost my entire immediate family to SDH-deficient cancers in a mere two-plus years, I remain laser-focused as a mother on a mission to fund/find a cure for SDH-deficient GIST because I do not want any other family to go through the despair and destruction that SDH-deficient GIST brings."

You can read more about this unique caregiver/ researcher partnership in a longer article in RARE Revolution Magazine at: bit.ly/UniquePartnership

Debra's son, Merak Melikian, died from GIST on February 3rd, 2020.



Whisky Lovers Gather at WOLF

Raising a Glass for #GISTresearch

By Jessica Nowak,
LRG Director, Outreach
& Engagement



Whisky aficionados gathered for the 3rd Annual Water of Life Fall event (WOLF) to enjoy whisky and raise funds for #GISTresearch. Since 2015, Dr. Matt Lurin has hosted this event; a fundraiser he created from his love of good whisky and a desire to help the LRG. Since the first Water of Life event, the fundraiser has evolved into two yearly events, with the fall gathering typically attracting a smaller, more intimate crowd. This year, however, the event was oversold with 60 brand ambassadors and participants in attendance.

WOLF was held on December 2, 2019 in the intimate ambiance of the Morrison Room at America Cut Steakhouse in Tribeca, NYC, where attendees enjoyed appetizers such as the signature salad “OG 1924 Hotel Caesar” and shrimp scampi. Brand ambassadors entranced the crowd with illustrative stories about each bottle they poured. Dinner included a 29-day wet-aged Porterhouse steak, among other entrees.

Dr. Lurin’s passion is a great illustration of how to take something you love and turn it into an event everyone will enjoy, plus have it benefit a cause that has personal meaning. Over the decades many people have used their passion to fuel fundraisers for #GISTresearch by holding comedy nights, walks, runs, creating cookbooks, and poker events.

Thank you to Dr. Lurin for his motivation and perseverance in holding this great event!

Do you have a passion that you can turn into a fundraiser?

Contact Diana Nieves to brainstorm at dnieves@lifaftgroup.org.

For some ideas, you can read more about recent fundraisers and fundraising ideas here:

<https://lifaftgroup.org/ways-to-fundraise/>

<https://lifaftgroup.org/2019/06/the-first-gist-do-it-walk-nj-held-in-loving-memory-of-ted-wolf/>

<https://lifaftgroup.org/2019/10/lrg-cookbook-fundraiser/>

Your Donation to the LRG makes an IMPACT!

By Joanna O'Rourke, LRG Outreach & Engagement Associate

At the heart of The Life Raft Group are our donors. We would like to give a shout out to all our donors, and those who selflessly give to the LRG. Without support from you, the LRG would not be able to provide all of our services to help patients survive and thrive.

Donations are vital to the LRG, and we are always looking for more ways to increase funding to help patients with GIST. We encourage you to reach out to your family, friends and business colleagues to ask for their support.



Not sure how? We can help. Potential donors may ask - Where do donations go?

A majority of the money we receive from donations goes directly towards funding research in order to find a cure for GIST. Your money is truly making a difference in cancer research. Funds that we receive go directly towards providing data from our observational studies to researchers to uncover trends, directly providing researchers with tissue from our GIST Collaborative Tissue Bank, collaborative efforts like our Pediatric & SDH-Deficient Consortium, and direct funding of individual projects by researchers.

Funds are also allocated towards information, education, advocacy, and outreach and engagement. These are the programs and tools we provide for patients to help them survive and thrive.

If you or someone you know is interested in donating to The Life Raft Group, visit our website at: <https://lifaftgroup.org/donate/>

<https://lifaftgroup.org/library/> (for the handout on the left.)

Andrea Shedler – Major Donor

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

In July of 2017, Andrea Shedler attended a GIST Day of Learning (GDOL) in San Diego shortly after her best friend, Nikki Morales, was diagnosed with GIST. Like most people, Andrea had never heard of GIST so when Nikki received this diagnosis, Andrea searched the internet for information. The LRG was one of the first websites she encountered and learned that we were holding a GDOL not far from her home in southern California. While at the GDOL, she connected with GIST specialists, patients, caregivers, and the LRG staff. After meeting the LRG community, Andrea knew that this was something that she should be a part of.

Andrea understood that because GIST is so rare most people have never heard of it unless they are affected by it personally. She became determined to spread as much awareness about GIST as possible. Her 'GIST awareness' journey began with Nikki when they did a joint fundraiser for the LRG and a local mental health organization in honor of a close friend.

This event was successful because Nikki and Andrea came from a large community of friends which included many from their 'Greek life' in college. Nikki was beloved by friends and family so it was not difficult getting people to support the cause. Andrea attributes their success in spreading GIST awareness to this large network of friends and family.



Nikki Morales, Andrea Shedler

In addition to engaging a large network of people, Andrea says the most successful way to fundraise is to make fundraising relatable. A few years back she participated in an event for her alumni sorority group to raise funds for children's hospitals. The event was called The Quatrefoil Queen Pageant for women old and young. Participants made donations to vote for their favorite contestant. It was fun for all and engaged participants and attendees. Andrea wanted to do something similar to this to help raise more funds for the LRG. She decided to follow her passion.

Two years ago Andrea began doing stand-up comedy. This led her create the idea for GIST Laugh. In early 2019, Andrea hosted a GIST Laugh comedy night at Town Tavern in Washington, D.C. Several local comics entertained the crowd who then voted for their favorite comedian of the night. Even though this was the first GIST Laugh, the event sold out, raising over \$4,000 for #GISTresearch!

Andrea found a unique way to host an event doing something she loves while also raising money for someone she loved. Though her dearest friend passed away later in 2019, Andrea is determined to continue to fundraise in Nikki's memory with the hope that one day there will be a cure for GIST.

The LRG is very grateful for people like Andrea Shedler who use their creativity to raise awareness for GIST and contribute to #GISTresearch!

You can read about Andrea's GIST Laugh here: <https://lifteraftgroup.org/2019/03/gist-laugh-headliner-andrea-shedler-delivers-a-comedic-success/>

Ways To Fundraise One of the best qualities of the LRG is that we spend a lot of time collaborating fundraising events to help raise money for GIST. By donating to The Life Raft Group, you can be rest assured that you're making an impact on GIST research and helping us find a cure! Support from our donors is vital to the LRG, and we are honored to work with you on any fundraising efforts to support finding a cure for GIST.

We are always looking for new and innovative ideas from our volunteers to help with fundraising! Contact Diana Nieves, dnieves@lifteraftgroup.org with your ideas!

Make a Donation

Go to lifteraftgroup.org/donate

Or contact: DNieves@lifteraftgroup.org



Do you have a fundraising story to share?

Contact Mary Garland, LRG Director, Communications, mgarland@lifteraftgroup.org

Fighting Anxiety & Depression on Your GIST Journey

By Rob Taylor, GIST Thriver, LRG GIST Mentor

I was diagnosed with GIST in December 2016. I had two surgeries that could not be resected. My wildtype tumor is wrapped around a main artery. I was told by my oncologist that I probably only had nine months to live. I had scan after scan and whether the tumor was interpreted as growing depended on the which radiologist analyzed the scan. I thought about my impending death at least twice a week. If I was in a store purchasing a shirt or something, I would either think "I am only going to get a couple of months of wear out of the shirt," or "Would it be a nice one to be buried in?." I was very confused about GIST because my oncologist knew very little about it.

I was having a tough time emotionally and asked my primary doctor for anti-anxiety medication. She suggested that I join a support group. I joined the LRG and met some very wonderful people that were kind and committed to helping me. I was overwhelmed by the compassion, commitment, kindness, and selflessness of my Chicago group leader Jim Hughes as well as Sara Rothschild and Laura Occhiuzzi. They set me straight and convinced me to get a GIST specialist and mutational testing.

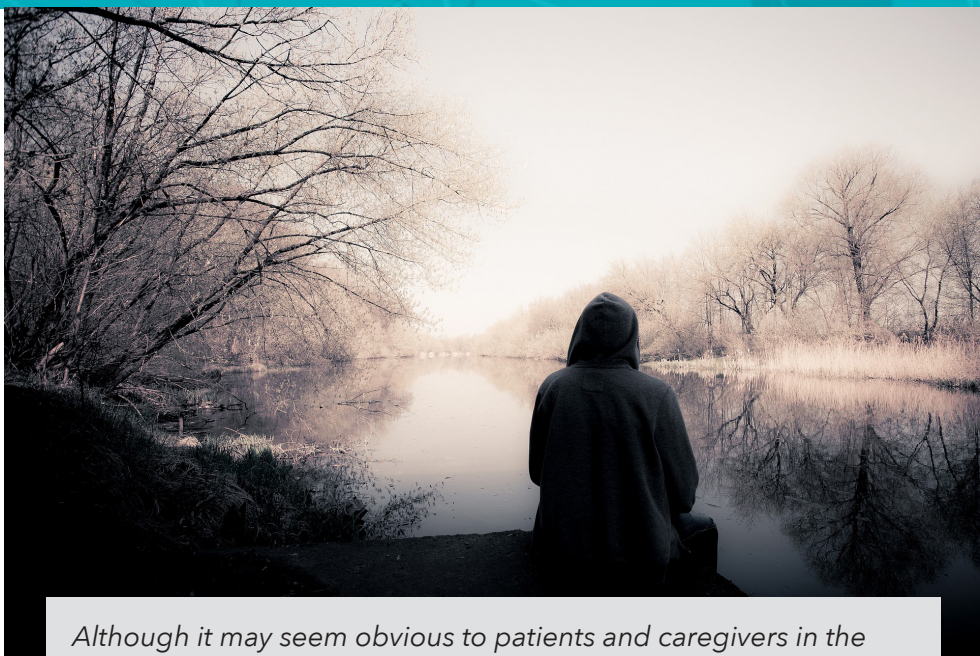
I became educated about my challenge and began to advocate for myself and developed a better outlook on life. Once my concerns about my GIST were minimized, I began to fight the anxiety that went along with it. I realized that we are fighting GIST on three fronts, physically, emotionally, and in our soul. The following are principles that I have adapted on my road to being happy and fighting anxiety while living with GIST.

Intention Being happy and having a pleasant state of mind has to be intentional. It has to be something that we set our minds and heart to do and go after. There can be no compromise for your joy. It must be given a proper place in your life. You must continuously remind yourself of your intent. Speak to your life concerning joy. Tell yourself that "I am going to be full of joy today. I will not allow anything disturb my joy."

Mindfulness We have to become aware of the impact of our thoughts and choose which ones will dwell within us and which will not. There are two sets of thoughts - one includes doubt, defeat, selfishness, ill will, and cruelty, and another that includes kindness, contentment, success, and compassion. We have a choice as to which will dwell within us. Choose positive thoughts. Dwelling on negativity has no positive impact on our situation.

Gratitude We become better grounded as well as having a 'macro' view of our lives when we find things for which we are grateful. When one bad thing happens in our life, we tend to focus in on that one thing and forget about all of the good things that are happening in our lives. We have to learn to count our blessings.

Finding Joy In difficult times, we have to learn how to "keep it going". Apply steps that will help you. Repeat your intention to yourself, deal with the hard things but keep your mind on the good things for a longer period. Yes, I have cancer, but it does not define me. I have family, friends, a job, etc. Don't let the difficult times overcome you, you overcome it with gratitude, intention and mindfulness. Pain is inevitable, suffering is optional.



Although it may seem obvious to patients and caregivers in the cancer community, depression is often a problem that is left untreated, put on the "back burner" to concentrate on the physical aspects of cancer treatment. The first and foremost thing that you need to know right now is that whatever you are feeling at this moment is completely normal. Do not feel ashamed or worried about your reaction to the news that you have cancer. Chances are that others have felt the very same way.

Integrity Be true to your inner self. Stand up and represent what is true. Create a motivation wall with people that positively impact your life. When you need encouragement to be true, look at the wall and think of what the people would tell you of the situation. My 'motivation wall' includes the LRG staff and many members of our group that I really adore.

Generosity Being generous positively impacts the giver as well as the receiver. It is an expression of selfless love that does more for the receiver than the giver is ever aware of. It prevents us from making gods out of stuff and appreciate the what we have.

Learning to love yourself Learn to accept your shortcomings and embrace the positives. We are all different and not made to be the same. Don't compare yourself to others but to your progress. Work on acceptance of things that you cannot change and learn to love and appreciate the life that you have.

Dealing with others I have learned to love people by not being judgmental, minding my own business, accepting people for who they are no matter if I agree with them or not. I have also learned to avoid drama and 'drama queens.' I have blocked people close to me on social media as well as my phone. I don't have the landscape to deal with them or their negativity. My landscape is crowded with fighting GIST and living life joyfully.

Compassion Caring for others and sacrificing makes your burden seem light. Compassion helps to keep you grounded and opens your heart to care for others.

Joy and happiness have to be awakened in you so that you can live life to the fullest. It must be intentional, practiced and not compromised. Love for yourself as well as others. Find joy, keep a song in your heart, smile and share smiles, learn to live freely. As fellow GISTer, John Abrams, has shared with me, "Don't sweat the small stuff, and it is ALL small stuff."

A cancer diagnosis has a significant impact on patients, caregivers and their families. It is normal for someone diagnosed with GIST to feel a whole host of emotions ranging from anger to fear, sadness and anxiety about the future. It is important, however, to be able to differentiate between the normal range of emotions and clinical symptoms of depression and anxiety, which require swift intervention.

If you or your loved one have any of these symptoms, notify a treatment professional or reach out to a support hotline such as the National Suicide Prevention Lifeline: 1-800-273-TALK (1-800-273-8255).

- Suicidal thoughts or thoughts of hurting yourself
- Lack of interest in normal activities
- Unable to sleep or sleeping excessively
- Strong emotional states that interfere with daily activities
- Severe fatigue or loss of energy
- Mental confusion or a diminished ability to think or concentrate

**1-800-273-TALK
(1-800-273-8255)**

Do you have a coping issue that you want to share about or have us write about?

Contact Mary Garland,
LRG Director, Communications,
mgarland@liferaftgroup.org

Building a Network of Rare Specialists

By Carolyn Tordella, LRG Assistant Director of Communications



Dr. Margaret (Margo) Shoup

Margo Shoup is a doctor on a mission. She moved from the Chicago area to Connecticut almost a year ago and has been the catalyst behind the creation of specialized disease teams targeting cancer care in a health system that covers an extensive area from western Connecticut up into the Hudson Valley of New York State.

In this unique situation, seven hospitals became NuVance Health, a merger between Western Connecticut Health Network and Health Quest in early 2019. These hospitals are Putnam Hospital Center (Carmel, NY), Northern, Dutchess Hospital (Rhinebeck, NY), Vassar Brothers Medical (Poughkeepsie, NY), Sharon Hospital (Sharon, CT), New Milford Hospital (New Milford, CT), Danbury Hospital (Danbury, CT), and Norwalk Hospital (Norwalk, CT).

Dr. Shoup is creating collaborative disease teams throughout the system comprised of physicians that are focused on a specialty.

"Disease teams were the first initiatives I set up when I got here. We had some specialists who were treating GISTs in some locations but not all," said Dr. Shoup. Shoup is the executive leader of the cancer program for the entire system. "I still see patients," she continued, "but my role is largely administrative as I put together disease teams across the system to focus on specific cancers."

How does this affect GIST? "For GIST, as an example, we have put together a GI disease team which is made up of our physicians that specialize in medical oncology, surgical oncology, gastroenterology, and our **Navigators**."

Navigators are RNs whose goal is to be the point person for the patient, assisting with the coordination of appointments and answering questions and concerns about their care. Shoup feels the Navigators are a great asset to the system because "the patients have enough on their minds and coordinating all the aspects of cancer diagnosis, surgery, monitoring, and treatment is complex."



The teams are formed by physicians who have decided on a focus area. The teams collaborate frequently, meeting about once a month to discuss processes, and what clinical trials to open. Cases are presented at the tumor boards and they discuss next steps in treatment.

"Generally, in community practice, oncologists don't specialize but we feel strongly that cancer is complicated, and it can be struggle to keep up on the latest and most effective techniques and testing for the all cancers. We want our team to focus on one area that they can become an expert in," said Dr. Shoup, "We need specialists that are knowledgeable about GIST and can detect it earlier."

Danbury, Norwalk, and Vassar Brothers Medical are comprehensive cancer centers where surgeries take place while the others are community cancer centers where follow up care can be done so that patients don't have to focus on so much stressful travel and can count more on their local support system of family and friends.

"We are building an academic quality cancer program for our patients with first-rate technology, highly trained specialists, and clinical trials (at the three larger sites). And although we do clinical trials, we're not focused on individual research and grants. We are patient-focused, offering everything a major metropolitan cancer center has so that patients don't have to travel long distances. Patients have enough on their minds, traveling long distances should not be necessary. We want to take care of the scheduling and coordination of care for them so they can concentrate on their health," said Dr. Shoup.

For more information on Dr. Shoup and the team of oncologists/surgeons at NuVance Health see: <https://nuvancehealth.org/>

Want to find a GIST specialist in your area?

See our GIST specialists database with doctors in the U.S. and International
<https://lifteraftgroup.org/gist-specialists/>

Need a checklist to take to your Doctor's Visit?

A pdf is available to print out at:
<https://bit.ly/DoctorsChecklist>

Making New Connections in NJ

The LRG Visits Rutgers Cancer Institute

By Sahibjeet Kaur, LRG Patient Registry Supervisor



Left to right: Sahibjeet Kaur, Denisse Montoya, Dr. Roman Groisberg, Laura Occhiuzzi, Sara Rothschild, Diana Nieves

Earlier this month, members of the LRG staff including Senior VP, Laura Occhuzzi, Patient Registry Director, Denisse Montoya, Patient Registry Supervisor, Sahibjeet Kaur, Vice President, Strategic Planning, Sara Rothschild and Senior Director of Outreach and Engagement, Diana Nieves were invited to the Rutgers Cancer Institute of New Jersey by sarcoma specialist Dr. Roman Groisberg.

Dr. Groisberg, a sarcoma specialist, is actively pushing research to new levels and providing the best treatment for GIST patients. This institution is especially important because it is the only NCI (National Cancer Institute) designated cancer center in New Jersey.

Along with colleague Dr. Shridar Ganesan, Dr. Groisberg, shared some challenges community hospitals face in terms of treating rare cancers like GIST. There was a discussion about how we can work together to educate patients and medical professionals about rare cancers more effectively and how to make treatments accessible to all patients. Select cases from the institution's Tumor Board were presented and it was interesting to compare Rutgers' review board to LRG's own Virtual Tumor Board.

The LRG is excited to have another GIST specialist in New Jersey and to have the opportunity to work with Rutgers Cancer Institute. For more info: <https://www.cinj.org/>

Q&A

Dr. Groisberg shared about collaborating with the LRG, his interest in sarcomas, and his extensive bowtie collection.

What made you decide to focus more on sarcomas?

I had a mentor during fellowship (Vivek Subbiah at MD Anderson) who had a lot of sarcoma patients treated on early phase clinical trials. It became apparent to me that this is an understudied and underrepresented group of diseases that deserved more.

Can you provide a brief statement about your vision with working with the Life Raft Group?

As the only sarcoma focused oncologist at the only NCI designated cancer center in NJ, I hope to provide the best medical care for GIST patients. With the help of Life Raft Group patients will have not only cutting edge medical care, but the support, education, and community to help them live life with GIST rather than be defined by their disease. With the Life Raft Group, we are developing innovative research programs to study mechanisms of resistance to next-generation Kit inhibitors.

And the bowties?

I did medical school in New Orleans; they are quite fashionable there and some of my favorite professors wore them. When I moved to New Haven for residency, I missed New Orleans and thought this would be a good way to remind myself. Also, they are much more hygienic than regular ties.

Management of Adults with SDH-Deficient GISTs

By **Dr. Andrew Blakely**, Program Director, Surgical Oncology Research Fellowship, National Cancer Institute, National Institutes of Health



The vast majority of gastrointestinal stromal tumors (GISTs) contain mutations in the KIT or PDGFRA genes. These are sporadic tumors, without a genetic predisposition. GISTs with SDH mutations, with resultant SDH enzyme deficiency, are different from GISTs with KIT or PDGFRA mutations for several reasons. SDH mutations are inherited, predisposing patients to developing GISTs throughout their lives. SDH-deficient GISTs tend to be indolent yet unrelenting¹. Such GISTs are more likely to also involve the liver and/or the lining of the abdominal cavity as opposed to remaining localized to the stomach or small intestine. Nowadays, patients diagnosed with SDH-deficient GISTs at a young age are much more likely to live well into adulthood than years ago. Increasingly, patients are diagnosed for the first time as adults². Regardless of area of the first presentation, patients with SDH-deficient GISTs are likely to develop multiple areas of involvement requiring repeated treatment over their lifetimes.

Treatment for SDH-deficient GISTs is primarily surgical. Systemic treatment options are experimental and are often associated with either significant side effects and/or poor response rates². Surgery is typically offered to patients with limited disease, patients with growing tumors, or patients with tumor-related symptoms such as pain or bleeding². A recent study of the National Institutes of Health (NIH) experience in surgical treatment of SDH-deficient GISTs showed increased time to tumor progression among patients able to undergo surgery¹. Of note, the use of systemic treatment was not associated with survival. Given the lifelong predisposition to developing GISTs among patients with SDH mutations, it is not uncommon for patients to require multiple surgeries to remove areas of disease.

Given the unique aspects of SDH-deficient GISTs, the Surgical Oncology Program at the NCI believes there is an unmet need for comprehensive assessment by a multidisciplinary surgical team able to care for tumors in the stomach, liver, and peritoneal surface. The surgical oncology program at the NIH is comprised of specialists in gastric (Dr. Jeremy Davis), liver (Dr. Jonathan Hernandez), peritoneal surface (Dr. Andrew Blakely), and endocrine (Dr. Naris Nilubol) diseases.

Together, this team provides comprehensive care of adult patients with SDH-deficient GISTs of any stage. This includes care of associated tumors such as paragangliomas, as in the case of Carney Triad and Carney Stratakis syndrome.

The need for better systemic therapy for SDH-deficient GISTs is clear. Tumors removed at the NIH are routinely sent for next generation genomic sequencing (TruSight Oncology 500, Illumina, Inc.) to identify mutations that available treatments may target. In addition, Dr. Hernandez's lab is actively developing a platform for patient tumor tissue by which a broad range of treatments can be tested for potential response. On this platform, tissue perfusion is maintained for several days in order to keep the tissue alive and test the treatment effect of a panel of drugs. This platform is enhanced by NIH access to a bank of all FDA-approved and many investigational treatments. Using genomic sequencing and this platform, we aim to use tumor removed from patients to identify new, personalized treatments and to expand potential treatment options for all patients suffering from SDH-deficient GISTs.

This team's goal is to expand the existing research in SDH-deficient GISTs. Combining basic science research, translational research, and clinical trial outcomes builds on the NIH's history of robust, expert care of patients with SDH-deficient GISTs. The Surgical Oncology Program is proud to work with partners such as The Life Raft Group and investigators at the University of California, San Diego (Dr. Jason Sicklick), in order to better serve patients with GISTs. We are planning to develop a joint research venture that combines the basic science progress made by Dr. Sicklick's laboratory and the facilities and resources available at the NIH. In this way, we will fully evaluate patients' tumor tissue, and we hope to advance the care of patients with SDH-deficient GISTs.

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REASONS TO BE AT 20 LIFE FEST

2020 is here, and It's Time to get ready for Life Fest in New Orleans. We are celebrating 20 years of targeted treatment for GIST and 20 years of relationships formed to help GISTers survive and thrive.

To kick off the celebration, here are 20 compelling reasons for you to join us in New Orleans for Life Fest 2020!

NEW ORLEANS JULY 10-12, 2020

1 It's *The Big Easy*! From streetcar rides to architectural gems and the mighty Mississippi in the background, New Orleans is filled with attractions to appeal to everyone.

2 You can join a gathering of the world's foremost GIST experts in an atmosphere that encourages personal interaction.

3 Enjoy the food! One of the world's foremost food cities, from delicious gumbo, po-boy and muffuletta sandwiches to the airy beignets at Café du Monde, NOLA is a foodie's idea of heaven.

4 Come meet other GISTers for the first time! Nothing tops sharing your journey while sharing food and fun!



5 Be amazed by the music! Strolling along Bourbon Street, you can pop in and out of venues, experiencing everything from Jazz to Cajun. Street performers add to the fun. And don't miss Preservation Hall!

6 Build your GIST knowledge by learning about the latest research findings and treatments. Educational sessions are tailored to patients and caregivers.

7 Take in the beauty of Jackson Square. With the St. Louis Cathedral at its center, this is the heart of NOLA. And there's great people watching, with artists and street performers adding to the ambience.



8 Get to know the LRG staff. One of the best experiences for our staff is meeting patients and caregivers face-to-face.

9 Go shopping on Magazine Street. Known for antiques and art, it is a great place to find a special souvenir.

10 Learn about *The Path to Survival* developed

over 20 years by LRG Executive Director Norman Scherzer, is a must for GIST patients and caregivers.

11 Visit museums. From the National World War II Museum to the Southern Food and Beverage Museum, NOLA has a wealth of museums for every interest.

12 Celebrate with us in a special 20-year anniversary ceremony.



13 Turn up the suspense with a Ghost, Voodoo and Vampire Night Time Walking Tour.

14 GIST Do It! as we walk to raise awareness and funds for #GISTresearch.

15 Grab your chapeau or parasol and ride on a steamboat up the Mississippi.

16 Meet our 2020 Life Fest Honorees. We celebrate and honor the individuals and organizations who are our Partners for Survival.

17 Visit the Garden District with its beautiful Victorian homes and gardens along with antebellum mansions and weeping willows line the streets.



18 Interact with long-term GIST survivors and hear stories of hope.

19 Visit Mardi Gras World. This warehouse will provide an inside look into the building of the floats and the excitement of Mardi Gras.

20 "Laissez les bon temps rouler!" means 'Let the good times roll!' Wherever two or more GISTers gather, fun will follow.

Laissez les bon temps rouler!

Resources for Your Cancer Journey

By Angela Edson, LRG Outreach & Engagement Associate

Many resources related to cancer diagnoses are disease specific. Unfortunately, being diagnosed with a rare cancer like GIST, which most people have never even heard of, can make finding resources more challenging. While this is not an exhaustive list of resources available, these organizations can provide a wealth of information and assistance.

Lodging

Hope Lodge through the American Cancer Society (ACS) is available in 36 major cities across the United States. Hope Lodge provides free lodging accommodations for patients and caregivers traveling far from home for outpatient medical care related to the patient's cancer diagnosis. To qualify for a stay, you must reside more than 40 miles or one hour away from the cancer treatment center. Each patient must be accompanied by a caregiver. The request must be initiated by the treatment facility doctor or social worker. For more information visit cancer.org or call 1-800-227-2345.

Transportation

ACS has partnered with Lyft to provide free rides for those battling cancer. It is part of the society's Road To Recovery program. The rides are available to and from treatments and are free of charge, courtesy of ACS. Road to Recovery offers patients who do not have a ride or are unable to drive themselves, free rides to cancer-related medical appointments. To find out if the Road to Recovery is available in your area contact the ACS at 1-800-227-2345 or online at cancer.org

Uber has partnered with healthcare organizations to offer rides for patients through *Uber Health*. Ask your healthcare provider/hospital about this partnership to access its benefits.

Angel Airlines for Cancer Patients (AACP) provides no-cost commercial airline tickets for cancer patients and their families. An information specialist will determine the needs of the patient and make a determination of the best form of travel assistance. For more information please call 1-888-675-1405 or visit <https://angelairlinesforcancerpatients.org>

Mercy Medical Angels may be able to help with transportation costs in the form of gas cards, bus and train tickets, and air flights through commercial airlines and volunteer pilots. For more information please visit mercymedical.org

Open Homes by AirBnB provides free places to stay for people in times of need. The program supports medical patients and their caregivers, evacuees, relief workers, and asylum seekers. <https://bit.ly/AirBnB-eligibility>

Medications

Gleevec - Novartis Patient Assistance for patients taking Gleevec, Novartis offers financial assistance programs for those who have limited or no third-party insurance coverage for their medicines. For further information please call 1-800-282-7630 or visit <https://www.pharma.us.novartis.com/our-products/patient-assistance>

Financial assistance for *Glivec* is also available internationally through The Max Foundation through their The Glivec International Patient Assistance Program (GIPAP). For more information please call 1-888-462-9368 or visit <https://www.themaxfoundation.org/what-does-treatment-access-mean-to-you/>

Sutent - Pfizer RxPathways assists patient with financial challenges through insurance support, co-pay help, and medicines for free or at a savings. Call 1-877-744-5675 or visit <https://www.pfizerRxpathways.com/>

The Max Foundation has partnered with Pfizer for a pilot program to assist international patients with financial barriers gain access to Sutent. For more information please call 1-888-462-9368 or visit <https://www.themaxfoundation.org/what-does-treatment-access-mean-to-you/>

Stivarga - (REACH) Resources for Expert Assistance and Care Helpline offers financial assistance options for those struggling to afford Stivarga. For more information please visit <https://www.stivarga-us.com/getting-and-paying/>

Ayvakit - Your Blueprint™ is a patient support program to assist you through your treatment by providing one-on-one support, financial assistance options, and resources. Connect with a case manager today at 1-888-BLUPRINT (1-888-258-7768). Patient Guide is available at: https://ayvakit.com/wp-content/uploads/AYVAKIT_Patient_and_Caregiver_Guide.pdf

GoodRx is a prescription savings program for those who are uninsured or underinsured. You may not use GoodRx in combination with your insurance plan. If you have insurance and choose to use a GoodRx cost savings card, the price you pay will NOT count toward your deductible or out of pocket maximum. For more information please visit [GoodRx.com](https://www.goodrx.com)

Psychosocial Support

CancerCare provides free individual and group support to anyone affected by cancer. Oncology Social Workers provide short-term, strengths-based emotional support and may also assist in locating practical assistance, including community resources. In-person services are available in New York City, New Jersey and Long Island. However, online and telephone support are available nationally. To learn more about these services please visit [cancercare.org](https://www.cancercare.org) or call 1-800-813-HOPE (4673).

Cancer Support Community has 170 locations worldwide, including 46 licensed affiliates and health care partnerships. They provide in-person support groups, educational sessions and health and wellness programs that are available to patients with a cancer diagnosis as well as caregivers. They have helpline counselors available online and over the phone, Monday-Friday from 9am-9pm whether you are looking for cancer resources and information or someone to talk to about emotional and social worries that come along with a cancer diagnosis. For more information please call 1-888-793-9355 or visit [cancersupportcommunity.org](https://www.cancersupportcommunity.org)

Mary's Place by the Sea is a nonprofit organization dedicated to supporting women who are receiving treatment for cancer. They provide services that complement medical treatment including oncology massages, nutrition education, individual counseling, expressive writing, reflexology, Reiki, guided meditation, prayer, and yoga. These services are offered free of charge. There are several locations throughout the United States. To see the guest reservation guidelines and for more information please visit marysplacebythesea.org or call 732-455-5344.

211 is an organization available 24/7 that connects people with local resources and services. You can call, text, or online chat, with a resource specialist to help you find supplemental food and nutrition programs, shelter and housing options and utilities assistance, emergency information and disaster relief, employment and education opportunities, services for veterans, health care, vaccination and health epidemic information, addiction prevention and rehabilitation programs, re-entry help for ex-offenders, support groups for individuals with mental illnesses or special needs, and a safe, confidential path out of physical and/or emotional domestic abuse. For more information see [211.org](https://www.211.org)

For more resources see: <https://lifteraftgroup.org/financial-aid/>

More Global Advocacy on the Go!

Piga Fernández was recently in Milan, Italy for the SPAEN (Sarcoma Patients EuroNet) Conference. Attending as conference delegate and speaker, she presented information about the challenges of GIST patients in Latin America. The SELNET project which is creating a multidisciplinary network of European and Latin American clinical and translational specialists. The SELNET network will focus on conducting an international registry-based observational study to assess clinical

management and prognosis of sarcomas as well as the development of intercontinental sarcoma biobanks, preclinical models, and a translational study with drugs in rare sarcoma subtypes.

On this trip, Piga also attended the ESMO (European Society for Medical Oncology) Sarcoma GIST Symposium 2020.

Piga Fernandez, meeting with global advocates Anna Costato of Italy, Helga Schnorf of Switzerland and Jayne Bressington of the UK at the SPAEN Conference



Photo credit: Uli Deck, ARTIS

Do you have an advocacy story to share?

Contact Mary Garland, LRG Director, Communications, mgarland@lifteraftgroup.org



Top Ten Tips for Caregivers

By Mary Garland, LRG Director, Communications

1. Acknowledge your feelings. When a loved one is diagnosed with a serious illness, it is normal to feel a whole range of emotions, from fear to anger to sadness.

Allow yourself to feel your feelings. If they are overwhelming, seek support from a trusted friend, a spiritual advisor or clergy, or a treatment professional. Remember, you are not alone.

2. Build a support system. Assure that you are not alone in this journey by gathering together a team of people to support you and your loved one. Your team should include treatment professionals, friends and loved ones, providers of support services and other caregivers via a support group or advocacy organization. Be sure to include people outside the world of cancer, especially those who make you laugh.

3. Accept help from those who offer. Be specific when asking for assistance, i.e. help with shopping for groceries, picking up the children at school, sitting with your loved one while you grab a cup of coffee with a friend.

4. Educate yourself. The world of your loved one's disease is an alien one. Seek sources of reliable information. Empowered patients and caregivers have higher rates of survival. The Life Raft Group is a great source of information.

5. Keep organized medical records. Having all the information about your loved one's treatment is empowering. Be sure to prepare for any medical visits by jotting down questions and concerns in advance. Your loved one may be too stressed at the time of the actual appointment.

6. Let go of expectations. This is hard. It does not in any way mean "abandoning hope." It simply means taking one step at a time and dealing with any information and interventions as they come. Staying in the moment helps to alleviate any disappointment and to avoid any overblown expectations. It means taking the treatment journey one day at a time.

7. Don't avoid the difficult and practical conversations. It is important to plan early on, preferably before a family member becomes ill, for practical issues such as writing an advanced directive and having all important documents in a secure location. All of us, whether we have a serious illness or not, should have in place important paperwork such as a designated healthcare agent, power of attorney and a will.

8. Balance the practical with the hopeful. Talk about and plan things to look forward to. Share moments of gratitude each day together.

9. Take care of your own health. In order to be there for your loved one, it is vital that you tend to any health issues you may have. Be sure to keep up with your own medical appointments, eat well and get enough rest.

10. Don't lose sight of your true self. You are not just a caregiver. Your loved one is not just a patient and is not their disease. As your caregiver journey begins, sit down together and make a list of all the things each of you are -husband, father, golfer, artists, guitar player, poet, craftsman. Find a place to post your lists in plain sight, so that you are reminded of your authentic selves.

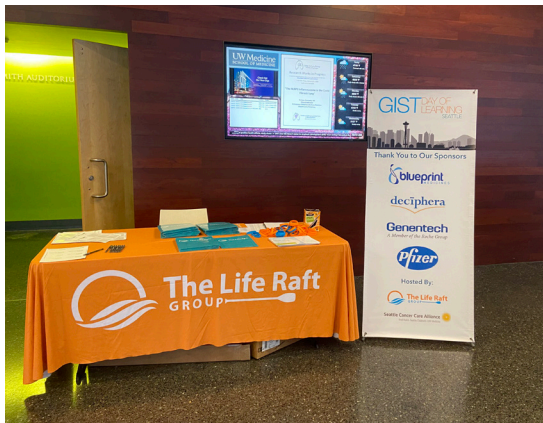


Spokane GISTers Gather for Food & Sharing Along the Journey

By Linda Jackson, LRG Support Group Leader

We enjoyed a fabulous luncheon on January 25. We had five GISTers and four caregivers attend the luncheon at Europa Restaurant and Bakery. Besides a great meal, we were able to meet each other and share our journeys through our GIST lives. I was able to distribute Life Raft Group literature provided by Diana Nieves. Many had never attended a GIST get-together before and expressed great appreciation for making it happen. This is a photo of the group picture. Everyone agreed that they wanted to get together again this fall!

For more info on this group contact: jacksonrh@comcast.net



GIST DAY OF LEARNING SEATTLE



A GIST Day of Learning (GDOL) Seattle took place Saturday, February 15, 2020, and was hosted in partnership with the Seattle Cancer Care Alliance (SCCA) at the Orin Smith Auditorium on the University of Washington campus. Speakers included Dr. Michael Wagner and Dr. Teresa Kim of SCCA and Dr. Michael Heinrich of OHSU and featured topics like *GIST 101* and *New Treatments in GIST*. GDOLs will be held in Tampa, Denver, and San Francisco this year.

Attend a GDOL this year!
See upcoming dates on page 23.

We Love Our Donors!

Without your generous support over the the years, we would not have been able to accomplish all that we have. You, as donors, have been a part of important milestones such as:

- Doubling the rate of patient survival
- Producing real world evidence that side effects diminish over time, leading to greater patient adherence
- Reaching out across the globe to advocate that patients receive access to treatment and medication
- Assembling the greatest GIST experts in the world to provide research and education for our patients
- Being recognized by the Biden Cancer Initiative for assembling a world class consortium of international GIST experts to share data, tissue and resources to find treatments for pediatric and SDH-deficient GIST patients...and so much more.

Your contributions have made these milestones possible. We want to thank you for supporting our efforts and look forward to the great privilege of working together as we continue to search for a cure.

Sincerely - **The Life Raft Group**

WELCOME

The Life Raft Group Announces Steve Pontell, Esq. as the newest addition to our Board of Directors

Steve has served as our volunteer attorney since 2017, and has been a valuable part of our family since then. 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



"A couple of years ago, I readily welcomed the opportunity to serve as counsel for LRG. I had some knowledge of the mission, saw first-hand the devastating effects of the disease and enjoy giving back through the provision of pro bono service. It was easy to accept the position. My decision to serve on the Board, however, is the result of observing the commitment and drive of the officers, staff, Board and volunteers at LRG, as well as the remarkable efforts and progress made towards enriching the lives of those effected and finding a cure for GIST. I look forward to following these examples and contributing in a meaningful way."



Steve and Marcy Jo Pontell

We are excited that Steve has agreed to take on this new role and look forward to his continued guidance and support!

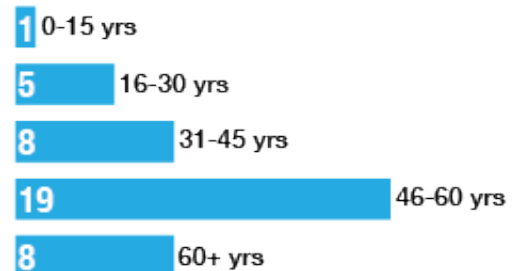
Patient Registry Data

41 new members joined the LRG Patient Registry since January 1, 2020

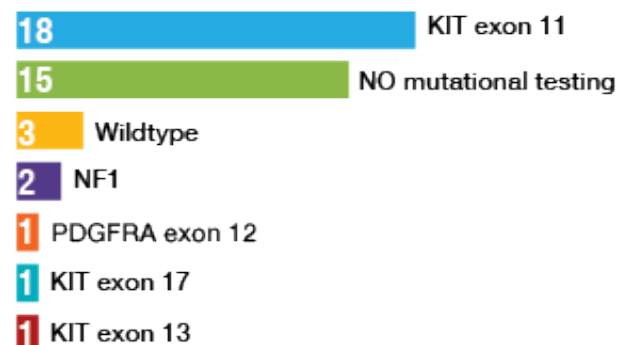


Australia
Aruba
Brazil
Canada
Cuba
Denmark
Germany
Mexico
Netherlands
U.K.
U.S.
Uzbekistan

Age at Diagnosis



Mutations





See the full list of events:
<https://lifteraftgroup.org/event/>

Read more about GDOLs (GIST Days
of Learning) and Life Fest here:
<https://lifteraftgroup.org/powered/>



March 28

Register: <https://lifteraftgroup.org/event/gdol-tampa-2020/>



May 16

Register: <https://lifteraftgroup.org/event/gdol-denver-2020/>



August 29

Register: <https://lifteraftgroup.org/event/gdol-sanfrancisco-2020/>

For events in Canada visit: **GIST Sarcoma Life Raft
Group Canada** at <https://lifteraftgroup.ca/welcome/>

In Loving Memory of Ted Wolf

**GIST
DO IT**
The Life Raft
GROUP

New Jersey

May 2nd, 2020 • 8am
Verona Park • Verona, NJ

GIST Do It Walk - New Jersey
Register: lifteraftgroup.org/event/gist-do-it-walk-nj/



Life Fest 2020 - New Orleans
Register: lifteraftgroup.org/event/lifestest2020/

In Loving Memory of Ted Wolf

**GIST
DO IT**
The Life Raft
GROUP

New Orleans

July 12th, 2020 • 7am
New Orleans, LA

GIST Do It Walk/Life Fest 2020
Register: lifteraftgroup.org/event/gist-do-it-walk-new-orleans/



Chicago Meeting Dates: May 9th, Sept. 12th
lifteraftgroup.org/event/chicago-support-group/

The Life Raft Group Global Community

LRG Staff

Executive Director	Norman Scherzer
Senior Vice President	Laura Occhiuzzi
Vice President, Strategic Planning	Sara Rothschild
Senior Director, Research	Pete Knox
Senior Director, Outreach & Engagement	Diana Nieves
Director, Communications	Mary Garland
Director, Marketing & Operations	Matthew Mattioli
Director, Patient Registry	Denisse Montoya
Director, Outreach & Engagement	Jessica Nowak
Controller	Lorraine Ramadan
Assistant Director, Communications	Carolyn Tordella
Patient Registry Supervisor	Sahibjeet Kaur
Data Analyst	Jerry Call
Operations Manager	Helena Mattioli
Outreach & Engagement Associate	Angela Edson
Patient Registry Associate	Stephanie Gachette
Patient Registry Associate	Jennily Eshak
Marketing & Operations Associate	Jayda Jones
Outreach & Engagement Associate	Joanna O'Rourke
Executive Assistant	Allison Russo
Consultants	
Global Relations Coordinator	Piga Fernández
Grants Consultant	Kathleen Gronet
IT Consultant	James Lee

LRG Volunteers

Pediatric GIST	Erin MacBean
Official Greeter	Gail Mansfield
Clinical Trials Coordinator	Jim Hughes
Staff Photographer	Michelle Mattioli
General Counsel	Steven Pontell
State Liaisons	Charles Burke
	Rick Isaías
Science Team	Erin MacBean
	Paula Vettel
	David Josephy
	Michael Josephy
	Joan Krakowsky
	Jim Hughes
National GIST Mentor Director	Santy DiSabatino
GIST Mentors	Carolyn Dewalt
	Jason DeLorenzo
	Julie Durkee
	Eric Lindberg
	Karen Meyers
	Marlene Nei
	Kay Stolzer
	Rob Taylor

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Executive Committee
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Stan Bunn – Past President
Gary Glasser – Secretary/Treasurer
Jim Hughes
Teena Petersohn
John Poss
Rodrigo Salas
Eric Biegansky
Steve Pontell
Mia Byrne (Ex Officio)
Ray Montague (Ex Officio)

Interested in Volunteering?

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

Interested in serving on the LRG Board of Directors?

Contact: Laura Occhiuzzi, LRG Senior VP
locchiuzzi@lifteraftgroup.org

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Life Raft US Representatives Find info for a local & global reps at lifteraftgroup.org/find-a-support-group/

Alabama	Kyle Brown	Maine	Jodi Merry	Puerto Rico	Eileen Rolon
	Gina Smith	Maryland	Wendi Lax	South Carolina	Paula Stover
California, Northern	Martha Zielinski	Michigan	Ellen Rosenthal	Tennessee	Alice Sulkowski
California, Southern	Dina Wiley	Michigan - West	Cliff Kopp	Texas	Dee Hawkins
Colorado	John Abrams	Minnesota	Tom Dupont	Utah	Mike Ginsberg
	Donald Hurd	Missouri	Katie Bloss		Carrie Callister
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Florida	Marian Ritter	New Hampshire	Jenna Colby	Virginia	Sally Jackson
Hawaii	Erin Rakes	New York	Pat Bonda-Swenson	Washington	Jack Tinnea
Idaho	Janet Conley		Frances Wenderoth	West Virginia	Susan Caperton
Illinois	Jim Hughes	North Carolina	Jennifer Higgins	Wisconsin	Rick Ware
	Marlene Nei		Carolyn Dewalt	Wyoming	Alan Quille
Iowa	Barbara Kepple	Oklahoma	Jane Rowan	At Large	Charles Burke
Kansas	Christine Engel	Pennsylvania	Jeffrey Bernstein		
			Tiffany Werner		

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Life Raft Global Liaisons See global news & resources in our **International section** on our website.

Argentina	Mariel Garibaldi	Hong Kong	Thomas Chow	Poland	Piotr Fonrobert
Australia	Roy Neil	Hungary	Zoltan Kalo	Romania	Simona Ene
Austria	Amy Bruno-Lindner	India	Nikhil Guhagarkar	Russia	Dmitry Bukhtenkov
Belgium	Gerard van Oortmerssen	Iran	Negar Amirfarhad	Samoa	Leasi John Galuvao
Bolivia	Vicky Ossio	Israel	Avi Zigdon	Saudi Arabi	Mohamed-Elbagir Ahmed
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Bulgaria	Juliana Popova	Japan	Sumito Nishidate	Singapore	Amelia Yeo
Canada	David Josephy	Jordan	Sameer Yaser	Spain	Luis Herrero
Chile	Piga Fernández	Kenya	Florence Thwagi	Sudan	Mohamed-Elbagir Ahmed
China	Minying Zheng	Macedonia	Dejan Krstevski	Switzerland	Helga Schnorf
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Costa Rica	Michael Josephy	Namibia	Lon Garber	Tunisia	Hanen Bouamoud
Cyprus	George Constantinou	Nepal	Atul Upadhyay	Turkey	Haver Tanbay
Czech Republic	Jana Pelouchová	Netherlands	Gerard van Oortmerssen	Ukraine	Larisa Kutovenko
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Finland	Saila Mattila	Norway	Frode Homb	Uruguay	Verónica Armand Ugón
France	Estelle LeCointe-Artzner	Pakistan	Sobia Wali Muhammad		
Germany	Markus Wartenberg	Peru	Karla Ruiz de Castilla		
Greece	Lefteris Patapis	Philippines	Roy Mijares		
Guatemala	Silvia Castillo de Armas		Ron Padua		

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