

## LIFE RAFT GROUP

January/  
February 2013

In memory of Doug Horst, Michael Lerner, Eric Pontes, William Clyde Turner, Dan Butt, Richard Bodden, Ann Kremer, Ron McLelland, John Aloise, Nely Jaques, George Logan, Bob Book & Maura Cesarini

Vol 14, No. 1

## LRG Research Team members publish results of imatinib combination study

By Pete Knox

LRG Special Projects Coordinator

**D**rs. Jonathan Fletcher, of Brigham & Women's Hospital, Harvard University and Maria Debiec-Rychter, of the Catholic University in Leuven, Belgium, members of the LRG Research Team were part of a recently published study that looked at the effectiveness of GDC-0941, a PI3K inhibitor when used in combination with imatinib (Gleevec). The study, published in Clinical Cancer Research (see <http://clincancerres.aacrjournals.org> for the abstract), was entitled "A potent combination of the novel PI3K inhibitor, GDC-0941, with imatinib in gastrointestinal stromal tumor xenografts: long-lasting responses

after treatment withdrawal." The study looked to compare each drug separately, as well as both drugs in combination to see which approach was most effective.

The study design was as follows: 136 nude mice (mice with a compromised immune system) had human GIST tumors introduced into their systems (a total of six GIST xenograft models were used). They were then divided into four different groups and had their tumors assessed after four weeks:

1. Control (no treatment)
2. GDC-0941 only
3. Imatinib only
4. GDC-0941 and imatinib in combination

Groups three and four (imatinib and the combined treatments) also had an

## Personalized medicine: is it myth or reality?

Executive Director's report focuses on better treatment options for GISTers

By Norman Scherzer  
LRG Executive Director



SCHERZER

**F**or my Executive Director's report, I usually highlight the many accomplishments of the Life Raft Group. Some of which include:

- Launching a GIST Day of Learning program in Miami, which is being expanded in 2013
- Using our GIST Collaborative Tissue Bank to help physicians in Latin America identify misdiagnosed GIST cases
- Major presentations to the FDA, FasterCures & CTOS

See PI3K, Page 13

See 2013, Page 10

## Lon Garber's Excellent Adventure: facing Gollum, P.T. & Paul Simon with R2D2

By Lon Garber

Namibia LRG Local Group Leader

**I**t all began in Africa with a pain in my backside. My wife, Val, jokingly called it payback time since I'd been a pain in her backside for 37 years. The pain was just an annoyance at first, but it persisted and each



GARBER

new episode grew in duration and intensity. My doctor, probably suspecting colon cancer, recommended a colonoscopy. The scope vindicated my colon, but revealed a troublesome

bulge pressing against my rectum from the abdomen. A follow-up MRI uncovered a 9.5cm tumor of unknown origin. My Namibian surgeon urged me to return to the States for diagnosis and treatment.

Two weeks later (May 2009), a biopsy in Denver returned a GIST diagnosis.

See GARBER, Page 11

# Patients' face numerous obstacles in quest for life-saving treatment

**By Phil Avila**  
LRG Writer

**J**essica Marchionda was desperate for a drug that would help her fight GIST in November 2011.

Her mother, Linda Martin, recalls how Jessica had tried and failed on a series of drugs since being diagnosed in 2007.

"It was such a frustrating time. It was awful," Linda says, recalling how doctors had recommended she give Glaxo-SmithKline's drug Votrient a shot but was unable to get Jessica's insurance company to cover it despite appeals documented by GIST specialists.

"We even sent a scan showing how Votrient had shrunk the tumor," she says.

The family's decision had been to start the drug, which costs around \$60,000 a year, and somehow pay for it on their own through fundraising efforts, until coverage could be obtained.

Jessica died in February 2012 at age 36 while an application for patient assistance with the drug was awaiting approval by Glaxo-SmithKline. Insurance approval never came through.

Susan Farmer, another GIST patient, also was denied coverage by her insurance company for Votrient. When appeals failed she opted to participate in a clinical trial of Votrient to gain access to the drug.

Still another patient, who asked not to be identified, says Votrient is what's keeping her alive. After switching insurance companies she lost coverage only to win it back after lengthy appeals.

The obstacles Jessica and others faced in getting insurance coverage for Votrient are common, according to Dr. Jona-

than Trent, a GIST specialist at Sylvester Cancer Center in Miami. Dr. Trent says he frequently prescribes Votrient to his GIST patients when the first- and second-line treatments of Gleevec and Sutent no longer work. And his patients frequently face denials from insurance companies.

This is partially due to the fact that the drug is being used off-label, not having been approved for treatment of GIST by the Food and Drug Administration (FDA).

Even though clinical trials have not shown the efficacy of Votrient in GIST, Dr. Trent says as many as 20 percent of the patients he puts on the drug respond well. Dr. Trent uses information from his practice to help patients appeal denials by insurance companies.

And sometimes the insurance companies acquiesce. "If you can show the patient is benefitting, the insurance company doesn't have a leg to stand on," Dr. Trent says, saying as many as half of his patients who are denied Votrient coverage eventually win appeals. "You have to get past the first couple of levels and

talk to the medical director," he says.

Frank Burroughs, President of the Abigail Alliance for Better Access to Develop-

mental Drugs, puts part of the blame on the FDA for what he considers an outdated approval process.

"There's a need for earlier access, for provisional or early approval of drugs," he says. "What [the FDA] calls the fast



**TRENT**



**ABIGAIL**  
alliance

## The Life Raft Group

### Who are we, what do we do?

**The Life Raft Group (LRG) directs research to find a cure for a rare cancer and help those affected through support and advocacy until we do.**

The LRG provides support, information and assistance to patients and families with a rare cancer called Gastrointestinal Stromal Tumor (GIST). The LRG achieves this by providing an online community for patients and caregivers, supporting local in-person meetings, patient education through monthly newsletters and webcasts, one-on-one patient consultations, and most importantly, managing a major research project to find the cure for GIST.

### How to help

Donations to The Life Raft Group, a 501(c)(3) nonprofit organization, are tax deductible in the United States.

You can donate by **credit card** at [www.liferaftgroup.org/make-a-donation/](http://www.liferaftgroup.org/make-a-donation/) or by sending a **check** to:

The Life Raft Group  
155 US Highway 46, Suite 202  
Wayne, NJ 07470

### Disclaimer

We are patients and caregivers, not doctors. Information shared is not a substitute for discussion with your doctor.

Please advise Erin Kristoff, LRG Communications Director, at [ekristoff@liferaftgroup.org](mailto:ekristoff@liferaftgroup.org) of any errors.

track is the slow line for people fighting for their lives."

Burroughs says his organization has been pushing the FDA to follow a White House panel that in 2007 recommended a process for speeding approval of off-label drugs. "This would be a big help in getting insurance approval of these drugs," he says.

A spokesperson for the FDA said the panel's recommendation is under consideration, but didn't comment further.

Burroughs suggested that the FDA take into consideration evidence from spe-



## Research Articles on the Science of GIST

### LRG & colleagues make their mark on 17<sup>th</sup> annual CTOS meeting in Prague

By Roberto Pazmino

LRG Administrative Director

**R**epresentatives from the Life Raft Group recently traveled to Prague, Czech Republic to attend the annual Connective Tissue Oncology Society (CTOS) meeting. CTOS is an international group comprised of physicians and scientists with a primary interest in the tumors of connective tissues. The goal of the society is to advance the care of patients with connective tissue tumors and to increase knowledge of all aspects of the biology of these tumors, including basic and clinical research (see [www.ctos.org](http://www.ctos.org) for more information).

The LRG was honored to present a poster that summarized our recently completed mutational and plasma level

testing survey. The poster from the study, entitled “The Investigation of Routine Mutational Testing and Routine Plasma Level Testing: A Survey of Patients, Caregivers, and Physicians” was displayed alongside numerous other GIST-related posters throughout much of the conference.

A number of these posters were of interest to the GIST community. Among them was one from Dr. Mikael Eriksson from Skane University Hospital in Sweden, who presented a poster along with one of his colleagues, entitled “Low Dose Imatinib May Be Active in Many GIST Patients with Intolerance to Standard Dose.” The study looked at a retrospective analysis of five cases of GIST patients, all Exon 11 and with metastatic disease, who have responded satisfactorily to very low doses of imatinib – from 200 mg daily to as low as 100 mg every

fourth day. These patients were able to experience low levels of side-effects and have not had further progression. While this study did deal with a small number of cases, it is interesting to note that these patients did respond to doses that would be considered lower than normal. If this were to be validated through further study, the potential exists to prescribe patients experiencing significant side-effects even lower doses of imatinib before switching them to other drugs.

In addition to the standard poster session, there was also a juried poster session and a number of presentations. A number involved members of the LRG Research Team. One of these, from Dr. Maria Debiec-Rychter and her colleagues at the Catholic University in Leuven, Belgium, entitled “MIR-17, MIR-20A and MIR-222 Target KIT and

**See CTOS, Page 15**

### LRG Research Team holds quarterly meeting

By Pete Knox

LRG Special Projects Coordinator

**T**he LRG Research Team recently held a teleconference that focused primarily on a study the team will collaborate on and intends to publish. The LRG Research Team consists of nine members, with seven at institutions throughout the United States and two in Europe (Germany and Belgium) and meets four times a year, either via teleconference or in-person. The team works in a truly collaborative fashion, sharing resources and ideas when working on their various projects, all of which focus on understanding the nature of GIST and developing compounds that can be transformed into drugs used to treat the disease.

The creation of this paper is a perfect

example of this collaboration. The paper will be examining the role of a molecule that is active in a particular metabolic pathway relevant to GIST – in order to study this, a number of things need to be accomplished – including providing relevant clinical data through patient histories, doing tests for the presence of the molecule, performing analysis on tissue samples, and writing a draft of the results. **The LRG, through its patient registry, was able to provide patient histories to the team,** and the other tasks were divided up among the various team members, all of whom generously offered their time and equipment.

This all occurred in real time through conferencing software – this is part of what makes the LRG Research Team unique, as normally this process does not occur in such a timely and efficient fashion, but instead slowly and often



The LRG Research Team in Leuven, Belgium.

only after multiple layers of approval from the various institutions involved.

The LRG providing information to the researchers directly via the patient registry is also not something typically found in other research environments – in addition to sponsoring the research of its team members, the LRG is also actively participating in it by directly providing data.

The paper is currently in the process of development and we all anxiously await the publication of its results, and will be sure to inform you as soon as that occurs.



# LRG launches new & improved website, convenient mobile app & online store!

By **Erin Kristoff**, LRG Communications Director & **Matt Mattioli**, LRG Office Manager

**A**fter two years of planning, toiling, and more planning, the LRG unveiled its redesigned website on January 1 at our same web address: [www.liferaftgroup.org](http://www.liferaftgroup.org). Finding the balance between providing life-saving information and explaining GIST in a concise, understandable way, has always been a struggle for the Life Raft Group. This new site was created to offer both forms of information with a couple more great new features thrown in.

## The Homepage

Besides the overall look of the LRG website, the homepage is where you'll find the most change. We've organized the menu differently, in the hopes that you will be able to find things like where to join and how to volunteer more easily. We've also added a slideshow to the top which will constantly be scrolling through upcoming events, a link to join, and patient support-, advocacy- and research-related content. This way the main services of the LRG are right there at your fingertips. Below you'll always find the most up-to-date news info as well as links to our social media sites.

## Managing GIST/Understanding GIST

One of our biggest goals was to make our GIST Education section more structured and easy to follow. We understand how hard it is to be faced with a cancer diagnosis or recurrence and how quickly you want the information you need. We've separated the content on this site into two categories: Managing GIST & Understanding GIST. By separating these areas, you can now more easily pick and choose the content you want to read at the pace you decide. Managing GIST is where you will find items related to the management of your GIST care, subjects like treatment types, side-

effects management and our clinical trials database. In Understanding GIST you will find information on the science of this disease, such as GIST types and survival statistics. Additionally, most pages include a small summary of the subject with a link to read more. This was done so that anyone, regardless of their GIST knowledge can understand the subject, but can very easily go more in-depth into each subject- in keeping with the quality information we have been giving you all these years.

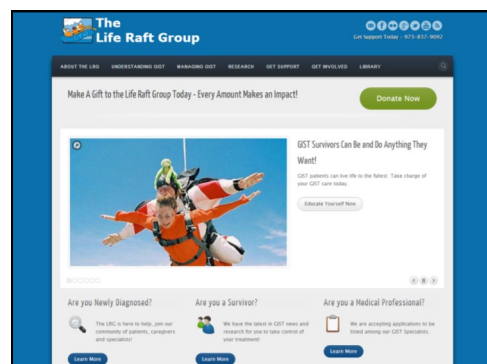
## Newly Diagnosed

When first diagnosed with GIST, people are often overwhelmed and desperate for information. We have added a feature to our homepage that allows these survivors to find what they need and find it quickly. This section offers a detailed slideshow, walking you through issues that you might find important, like an explanation of GIST types, survival rates and the importance of support groups. Throughout the year we will be making even more changes to improve this vital area.

## GIST Survivor & Medical Professional Areas

Also on the homepage, are two buttons asking whether you are a medical professional or GIST survivor. We wanted to include these areas so that anyone can find the information they are looking for, even if they don't know exactly where to look. On the Survivor page, you can find links to Coping content and information on managing your care, all in the same place. A medical professional at any stage of their career can find useful information on our Medical Professional page some items found there are information on our Research Team, Clinical Trials Database and how to order a Physician Outreach package.

There are many more new features to the new LRG site than we can list here. We encourage everyone to explore and let us know what you think. Comments, suggestions and words of encouragement



are always welcome. Email us at [life raft@liferaftgroup.org](mailto:liferaft@liferaftgroup.org) with your input.

## Online Store

We are also really excited to share our new online store. Run through Cafe Press, we have set up a store where you can get almost anything you can think of LRG-branded. Some of the items available for purchase are t-shirts, pajamas, sweatshirts, pillows, phone cases, mugs, and doggy clothes. But these are just a few of the hundreds of items to choose from and there is sure to be something for everyone. All of these items are great, fun, and creative ways to show off your LRG pride and to raise GIST awareness. You can find the store at <http://www.cafepress.com/liferaftgroup>

## Mobile App

The Life Raft Group is also proud to announce our brand new mobile app. From this app you can read the latest newsletter articles, check out what's happening on our Facebook and Twitter, and get all of our contact info. Two of the biggest features are the mobile Medical Update form and the ability to request your GISTory. This app will not only help everyone in the GIST community stay informed & connected but easily and quickly update or request their medical records from their own phone. It is available on the Apple app store, the Android play market, and the Windows Phone app store, just search "Life Raft Group".

## LRG welcomes Deputy Director and two new members to Board

Campbell brings Wall Street smarts and warrior spirit to deputy director position

By **Karen Kelley**, LRG Writer & **Gale Kenny**, Program Associate

**T**he Life Raft Group is pleased to welcome Roger C. Campbell as our new Deputy Director. Roger, an attorney, brings to the LRG over 25 years of experience in the global financial services sector. He served in various capacities, including General Counsel, Senior Vice President, and Chief Administrative Officer in several Wall Street firms. When he joined BCG Partners, a subsidiary of Cantor Fitzgerald, he managed the merger, integration and re-branding of a major financial company and its subsidiary divisions which more than doubled the size of BCG's business in New York.

While Roger has had a distinguished career on Wall Street, he is most recognized for his contributions to raising significant sums of money for charities. On behalf of BCG Partners in 2007, he initiated, orchestrated and implemented the production of BCG Charity Day which has grown to be a major event of remembrance and giving on September 11 of each year, honoring the 658 employees of Cantor Fitzgerald who lost

their lives in the World Trade Center attacks. Over \$13 million has been raised over the past six years for more than 75 charities. Roger also has been actively involved in supporting the Wounded Warrior Project (WWP) since 2007 and currently serves on its Board of Directors and is Chairman of its Investment Committee.



**CAMPBELL**

In addition to his legal expertise, Roger brings to the LRG a commitment to serving others and a wide-ranging skill set of program management, fundraising, and marketing experience. As he stated in his welcome message to staff, "The LRG provides me with a wonderful opportunity to contribute in the non-profit sector in a mission driven organization that works toward improving the lives of others."

He is married to Kathy McNamee, PhD., a Psychologist at the VA Vet Center in White Plains, New York. They are the parents of four children and live in Ridge-wood, New Jersey.

### Miller & Glasser use LRG devotion in new board roles

Effective January 1, 2013, The Life Raft Group Board of Directors announced with pleasure the addition of two new members, Gary Glasser and Dan Miller. We wish to extend a warm welcome to these valued team members.

Dan comes to the LRG with the shared dedication to find a cure. After learning that his wife Shawna was diagnosed with GIST in 2011, he became involved in the GIST research currently underway at Oregon Health & Science University (OHSU) in Portland, dedicating both resources and commitment. Dan has worked in the telecom industry for over 35 years. He and Shawna have six children and nine grandchildren, and currently live in Brush Prairie, Wash. They delight in family time outdoors, whether playing in the winter snow or boating on the water. They also love traveling.



**MILLER**

Gary heads research at Lucidus Capital Partners, which focuses on the global macro-economy, financial institutions, and real estate. He was previously Head of Research at Caxton Associates following 15 years of investment banking primarily with Goldman Sachs and JPMorgan. Gary joined the Life Raft Group shortly after his father, Lawrence, was diagnosed. Sadly, his dad lost his battle with GIST on October 10, 2011. Gary's mother, Eileen, is a welcomed and hardworking volunteer who donates time weekly for the LRG. Gary, a graduate of Yale, resides in Fort Lee, N.J. with his wife and four children.



**GLASSER**

## LRG sets GDOL Miami date for March

**T**he second annual GIST Day of Learning (GDOL) will be held on March 23, 2013. This year we are privileged once again that Dr. Jonathan Trent of Sylvester Cancer Center in Miami has agreed to host our gathering and we are honored to hear what he has to say. This is an important opportunity for continuing our ongoing education so we can all learn more and be empowered by the knowledge we receive. It is also an opportunity to meet others facing the same issues as well as connect with local experts in GIST treatment. Go to: [liferaftgroup.org/events/](http://liferaftgroup.org/events/) for more info.



Dr. Trent (far right) with other GDOL 2012 presenters.



# LRG's annual holiday fundraiser going strong!

**By Gale Kenny**  
LRG Program Associate

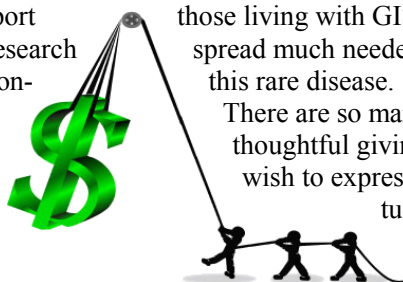
**O**ur Holiday Fundraiser is still going strong, and has thus far has brought in \$45,000. The Life Raft Group remains beholden to all who have donated both large and small, as it is the collective generosity of those who support us that make The Life Raft Group the dynamic organization it is. As you are aware, our

patient support, education, and advocacy knows no boundaries, as we continually strive to spread awareness and survivorship into all corners of the world. Of equal importance is the support these funds provide to our research team who share in our passionate commitment to find a cure for GIST.

If you have been mailed a fundraising packet and still have donor cards to

send out, *it is not too late*. Consider passing one on to a neighbour, co-worker, friend or family member. This gesture will not only raise funds to help those living with GIST, but will also spread much needed awareness about this rare disease.

There are so many relying upon the thoughtful giving of others. We wish to express our warm gratitude that we are the recipient of yours.



## Life Fest 2012 hits big in Las Vegas!

**By Janeen Ryan**  
LRG Outreach Coordinator

**L**ife Fest 2012 was not only the premier gathering of the Life Raft Group community but a celebration of our ten year anniversary as well! And, what better venue to celebrate than Las Vegas! The glitz and glamour of this beautiful, legendary city could only be eclipsed by our immense joy of reconnecting with old friends, giving and receiving hugs and of course, meeting so many new faces. People from around the country and across the globe came together for three spectacular days of learning and fellowship.

### Special Thanks

The LRG would like to thank everyone who helped put this event together: Melissa Tribus from Red Rock Resort and Cheri Martinez from Suncoast Ho-

tel. Thank you to Neill Miller of PM Digital Group for videotaping the event. Big thanks to our Presenting Sponsor, Bayer Pharmaceuticals; Gold Sponsor, Pfizer Pharmaceuticals; Silver Sponsor, Novartis Pharmaceuticals; Sapphire Sponsor, LRG Board President Jerry Cudzil; Ruby Sponsors, Sanofi, Genentech and Michael & Maggie Marcantonio; and Emerald Sponsor, Sylvester Cancer Center. We would also like to extend special thanks to Nadine Sta. Brigida, Shun Otsubo and Matt Consoli for volunteering to photograph and take videos of the event.

Thank you to all those who volunteered to be mentors during the event as well as all of the LRG members who generously gave of their time to help us coordinate this event.

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**Read the whole article at:**  
<http://tinyurl.com/Lifest2012>

# Swiss GIST Group awards 2012 GIST Prize

By Helga Meier-Schnorf  
GIST Gruppe-Schweiz

*The GIST Group of Switzerland awarded its science prize for the third time on November 22, 2012. It has been awarded ex aequo to Dr. Daniel Perez, Memorial Sloan-Kettering Cancer Center (MSKCC), New York, USA, and Dr. Michael Montemurro, CHUV Lausanne/ University Hospital Zurich.*

*The presentation was given by Dr. Urs Metzger, Chairman of the Awards Committee of the GIST Group.*

**T**he association for the support of people affected by gastrointestinal stromal tumors (GIST), the GIST Group of Switzerland has awarded its science prize for the third time. The 2012 GIST Prize, worth 10,000 francs, is shared equally by Dr. Daniel Perez for his study "Blood Neutrophil-to-Lymphocyte Ratio is Prognostic in Gastrointestinal Stromal Tumor", published in the *Annals of Surgical Oncology* and Dr. Michael Montemurro for his study "Sorafenib as third or fourth-line treatment of advanced gastrointestinal stromal tumor and pre-treatment including both imatinib and sunitinib, and nilotinib. A retrospective analysis", published in the *European Journal of Cancer*. The award ceremony took place on November 22 at the semi-annual meeting of the Swiss Group for Clinical Cancer Research (SAKK) in

Basel.

The jury reached this unanimous decision under the chairmanship of Dr. Urs Metzger.

The study by **Dr. Perez** was designed to establish whether the ratio of neutrophil granulocytes and lymphocytes in the blood circulation (NLR) is prognostic for GIST. It is a readily available method for measuring the degree of immunological activity in the blood.

To this end, a prospective analysis was conducted on the blood values of 339 patients with primary, localized GIST, who were treated at MSKCC from 1995 to 2010. Patients following adjuvant imatinib therapy (n=64) were excluded from the study. The NLR was correlated with the clinical course.

Progression-free survival was significantly longer in GIST patients with a low NLR. A correlation was likewise observed between NLR and the number of mitoses and between NLR and tumor size. Only a small number of neutrophil granulocytes were detected in the tumor tissue.

In summary, it can be said that high-risk GIST patients have a high NLR. The elevated NLR values are a parameter for systemic tumor-induced inflammation.

**Dr. Montemurro** and colleagues undertook a retrospective analysis of the efficacy of sorafenib (Nexavar) in the treatment of patients with a previously treated gastrointestinal stromal tumor based on data from 13 cancer centers. All 124 patients in this largest series worldwide had previously been treated with imatinib (Gleevec) and sunitinib (Sutent), and 68 patients had also been treated with nilotinib (Tasigna). The prior treatments were ended in most cases because of tumor progression and there was therefore an urgent need for treatment in these patients.

Sorafenib was started at twice daily doses of 400 mg. This dose regimen led to mild side-effects in most cases. Frequent side-effects were skin reactions



From left to right: Dr. Michael Montemurro, Helga Meier Schnorf, Dr. Daniel Perez, Dr. Urs Metzger.

(38%), tiredness and diarrhea.

A response to sorafenib was seen in 10 percent of patients and a stabilization of the disease in 57 percent. The median duration of treatment was 4.5 months, median progression-free survival (PFS) 6.4 months and overall survival 13.5 months. The survival data for sorafenib were identical for the third and fourth line of therapy. An interesting observation was the trend towards a longer PFS in patients in whom the sorafenib dose was reduced or in whom a skin reaction occurred.

Sorafenib appears very effective in the treatment of previously treated GIST patients.

Besides the indisputable quality of the work submitted, the jury was also impressed by the engagement of Dr. Montemurro in this rare disease. In the last five years he has given over 40 lectures on the subject of GIST and published nine studies listed in Medline on the subject, either as author or co-author, five of which are original studies.

## SAKK

*The Swiss Group for Clinical Cancer Research (SAKK) is a non-profit organization that has carried out clinical cancer studies as a cooperative group since 1965. The aim of SAKK is to develop existing cancer treatments further and to investigate the efficacy and tolerability of new treatments.*

Further information: [www.sakk.ch](http://www.sakk.ch), [info@sakk.ch](mailto:info@sakk.ch)

## HELP GIST RESEARCHERS LEARN MORE ABOUT NF1!

If you have been diagnosed with GIST and your tumor was classified as Neurofibromatosis 1 (NF1), please play a valuable part in GIST research by helping researchers learn more about NF1.

Contact Magdalena Sarnas at the LRG GIST Collaborative Tissue Bank by phone: 973-837-9092 X 114 or email: [msarnas@liferaftgroup.org](mailto:msarnas@liferaftgroup.org)



# LRG closes out 2012 in the spotlight: advocacy, support & research highlighted

**T**he Life Raft Group is dedicated to the welfare and survival of GIST patients worldwide and we carry out our mission through research, patient support & education and advocacy. The LRG was highlighted in all three areas at the end of 2012 and we wanted to share the important work we're doing with all of you.

## LRG Research Article Grows in Popularity

In 2012, the LRG published an article in the Journal BMC Cancer called "Survival of gastrointestinal stromal tumor patients in the imatinib era: life raft group observational registry." The authors were LRG Science Director Jerry Call, Christopher D. Walentas, Jens C. Eickhoff, and LRG Executive Director Norman Scherzer.

We are happy to announce that the article has received over 2000 views including 317 within December. If you would like to view the article, go to [www.biomedcentral.com/1471-2407/12/90](http://www.biomedcentral.com/1471-2407/12/90)

## E-patients.net post channels Norman

Susannah Fox, Associate Director of the Pew Internet & American Life Project, wrote an interesting post on e-patients.net called As She Lay Dying - a son calls on the health system to involve patients and families in improving safety.

She highlighted what her strategy would be when faced with a sudden hos-

pitalization of a loved one and stated that her plan would include calling upon Norman Scherzer whose survival advice would be "Get angry. Get smart. Get organized."

If you want to read the article, go to e-patients.net and search "As She Lay Dying"

## Talking new clinical trials models at Sanofi

Norman participated in a workshop at Sanofi's site in Bridgewater, New Jersey, along with a number of other patient advocacy groups.

They brainstormed ways of putting the patient first and how patient advocacy groups are important to the clinical trial process. They discussed how the pharmaceutical industry can better deliver patient value as well as defining opportunities to collaborate effectively.



# Dave Safford shares his list of happiest moments since GIST and those still to come

**R**ecently, LRG Board Member Dave Safford decided to sit down and think about all of the good fortune that has come into his life since being diagnosed with cancer. We liked his list so much we decided to put it in the newsletter so that everyone in the GIST community can see a story of positivity and hope.

*"Cool, Fun things I've done since I've had cancer:*

*Topping the list, two-week family vacation in Maui; Went to game seven of the NLCS to send Giants to World Series; played in the Peter Jacobsen Pro-Am golf tournament; helped "invent and launch" several new products at work; saw multiple Broadway shows in N.Y., Cirque de Soleil in Vegas; Sunriver Zebra club weekends; watched Natalie,*

*Kiana, and Alani excel in everything they do; tell Cherry and the kids I love them every day.*

*"Cool Fun things I have planned or hope to do: Superbowl party weekend in Vegas with friends and helicopter tour of Grand Canyon (coming up soon and would love if Seahawks are in it!); play golf at midnight in Alaska again; have a "Darjeeling Limited-ish" roadtrip with my brothers Ron and Scott; Play in the World Series of Poker (not the main event - too rich for me); gaze at and ponder the Milky Way on a clear night (who knows the best place in the world to do this?); be a contestant on Jeopardy, Wheel of Fortune or Who Wants to be a Millionaire; visit Cooperstown Baseball Hall of*



Dave and his wife, Cherry.

*Fame; spend as much time as possible with my family.*

Do you have any fun ideas you'd like to share with Dave or fellow members? Email us at [liferaft@liferaftgroup.org](mailto:liferaft@liferaftgroup.org) and let us know!



# GIST

## Day of Learning

SATURDAY, MARCH 23RD

UNIVERSITY OF MIAMI  
LIFE SCIENCE & TECHNOLOGY PARK

1951 NW 7TH AVENUE  
MIAMI, FLORIDA 33136



### THE MEETING

This meeting will focus on learning about Gastrointestinal Stromal Tumor (GIST). If you or someone you know has GIST and would like to learn more about this cancer, available treatments, and managing care, please join us to hear from experts.

IN COLLABORATION WITH



## Pediatric GISTer ties the knot!



Jason DeLorenzo, LRG member and pediatric GISTer, got married on November 17, 2012 to Jill Medhus. The ceremony was held at Corpus Christi in Hasbrouck Heights, N.J., the reception followed at Tides in North Haledon, N.J.

**The LRG is excited to hear of another GISTer wedding and we give our deepest congratulations to the happy couple!**

## LRG researchers talk microGISTs: how common?



Did you check out a recent article we sent by LRG Research Team members, Dr. Sebastian Bauer & Jonathan Fletcher.

Here's an excerpt to he great article coming:

**FLETCHER** "Most of us have moles somewhere on our bodies. Yet, although many moles are akin to benign neoplasms, and are exceptionally common in the general population, we all know they very rarely transform into malignant skin cancers. What few people know is that one could describe GISTs as the "moles" of the stomach.



**BAUER**

Every third person has a tiny GIST in the stomach, so the incidence of GIST in the readers of this LRG newsletter is probably not substantially different from the incidence of GIST in the readers of 'Sports Illustrated'."

If you missed it, you can find it on our website at [liferaftgroup.org](http://liferaftgroup.org).

## 2013

### From Page 1

- Helping to restore \$1 million in cancer research funding to New Jersey's budget
- Our Research Team making several breakthroughs in the search for a cure
- Debuting features like a new and improved website and mobile application

Now I would like to focus on our future, so I am going to direct your attention on personalized medicine—a myth or reality—and begin a dialogue about its impact upon GIST patient survival.

## Personalized Medicine

**Question:** Myth or Reality?

**Answer:** Unfortunately, myth.

### Let me explain:

We have the mutational testing tools to categorize many different subtypes of GIST.

We have a growing body of evidence to optimize treatments based upon knowing such subtypes.

Despite this, few GIST patients in the United States receive mutational testing.

The LRG is determined to make personalized treatment for GIST a reality.

Throughout time there have been enormous time gaps between the discovery of new ideas and the systematic implementation of these ideas. We have seen some fairly extraordinary examples of

this in the world of medicine.

We've seen this in breast cancer. How long after the introduction of the lumpectomy did it take for this option to be considered instead of radical mastectomy? Too long.

We find the same issue in lung cancer. How long did the public wait for the admission that smoking damages your health? How many decades went by before the statement "Smoking causes lung cancer" was legally required on every pack of cigarettes? Again, too long.

While the world waited for these discoveries to become common knowledge, patients suffered, some even died.

The past decade has seen enormous breakthroughs in the molecular characterization of different cancers with GIST leading the way.

**Here is what we know:** GIST patients can be categorized by a range of mutations like exon 11 or 9. These subtypes can have different response rates to different dosage levels or treatments.

For example, approximately 10 percent of GIST patients have a KIT exon 9 mutation. This type of GIST has been found to respond better to higher doses of Gleevec.

Even more telling, approximately five percent of GIST patients have a D842V PDGFRA mutation which is totally resistant to Gleevec. Following surgery, these patients are routinely prescribed three years adjuvant Gleevec (standard care) because they have not had a muta-

tional test. Further, without mutational testing, these patients are unaware of mutation-specific trials like AROG's crenolanib trial.

Nearly every type of GIST has potential for optimizing treatments based on mutation type.

Mutational testing has been readily available for several years. The process of obtaining tissue for mutational testing is a relatively simple one. Several laboratories perform the test on routinely collected tissue following surgery. In fact this testing is free of charge through the LRG Tissue Bank or directly shipped to Oregon Health & Science University.

**Let me recap:** We know that several GIST subtypes require non-standard treatment. We know that this testing is fairly easy to have done and is often free of charge.

**But we also know that, according to a recent report, the total number of GIST patients receiving such mutational tests is six percent<sup>1</sup>.**

The LRG thinks this is unacceptable and we hope you do too.

We will bring you more information about what is going on, how it impacts the survival of GIST patients and what we can do about it. In the meantime, you can take a stand with us and join the LRG Patient Registry & Tissue Bank to get mutational testing free of charge.

*1. Pisters PWT, Blanke CD, Von Mehren M, Picus J, Sirulnik A, Stealey E, Trent JC: A USA registry of gastrointestinal stromal tumor patients: changes in practice over time and differences between community and academic practices. Ann Oncol 2011.*

## Life Raft Group adds new local group leaders & international partners

### Newest local group leaders:

Debbie Farmer - Indiana  
farmer\_debbie@yahoo.com  
Jill Heinrich - Alaska  
heinrichfamily@hotmail.com  
Joan Smith - Nevada  
benjoanfitz@yahoo.com  
Paula Stover - South Carolina  
pjstover.1121@gmail.com  
Maryanne Snowbrick - Ohio  
meleanawons@hotmail.com



Ananth Pai- Minnesota  
ananthpaius@gmail.com

### New international group leaders:

Roy Neil - Australia  
uncknuckle@gmail.com  
Piotr Fonrobert- Poland  
pfonrobert@gmail.com  
Marie Lagaluga- New Zealand  
marielagaluga@xtra.co.nz

Janeen Ryan, LRG Outreach Coordinator is currently filling positions in Arkansas, Hawaii, Kansas, Kentucky, Mississippi, Montana, New Mexico, North and South Dakota, Vermont, W. Virginia, Wyoming and Washington DC. If you are interesting in being a local group leader, please contact her at 973-837-9092 ext 113 or jryan@liferaftgroup.org.

## GARBER

From Page 1

Up until then, the word ‘gist’ meant ‘the essential part of a matter;’ but now the essential part of my GIST became a tiny orange bullet - Gleevec. I had braced myself for the horrors of chemotherapy and the oncologist replaced my imagined nightmare with a once-a-day pill that amazingly came with almost no side-effects. Instead of chemotherapy, I prescribed baseball therapy for myself. I took my pill in the morning and went to Colorado Rockies baseball games in the afternoons and evenings while waiting for Gleevec to do its job. My nightmare became a dream come true. Since my son-in-law works for the Rockies, my therapy was free.

### Oh No, Anything But That

The only problem on my medical horizon was the specter of a permanent colostomy. My family physician assured me a colostomy would be the certain aftermath of any surgery to remove Golum (more on that later). The required surgical margins would severely compromise the surrounding sphincter muscles, thereby requiring that my closest friend be an unmentionable bag.

My daughter decided my tumor needed a name and what better name than Gollum, the gray and slimy, mysterious creature who kept popping up unwanted in the Lord of the Rings trilogy. Gleevec quickly shrunk Gollum to 6.5 cm, but no more. My surgeon assured me that the only way to avoid a colostomy would be for Gleevec to reduce Gollum to three centimeters or less. It wasn’t happening, but my surgeon wanted to cut anyway.

In the meantime, I’d heard about doctors using DaVinci robotics to perform rectal surgery in a way that preserved sphincter abilities, thus avoiding the permanent installation of the nasty bag. I mentioned it to my surgeon and he frowned. He grudgingly referred me to

both a colo-rectal surgeon and the only surgeon in their surgical group who performed robotic surgery. The colo-rectal surgeon assured me that it was impossible to avoid a permanent colostomy and openly mocked the idea of robotic surgery *and* he surprisingly mocked his

**“Up until then, the word ‘gist’ meant ‘the essential part of a matter;’ but now the essential part of my GIST became a tiny orange bullet - Gleevec.”**

own colleague who had embraced this new technology. I left his office totally discouraged, but convinced I would never let such a man touch my body with a scalpel.

### R2D2 to the Rescue

My next and last surgical consultation was with Dr. Warren Kortz whose office suite adjoined Porter Adventist Hospital in Denver. He was as optimistic as the prior surgeons had been discouraging. So on February 26, 2010, they carted me into the sterile operating room where the scary looking robot waited patiently in the corner. For the next 11 hours, without ever touching my body with his own hands, Dr. Kortz painstakingly and meticulously excised Golum while sitting at a

computer console on the opposite side of the room. The DaVinci

robot has three surgical arms and one camera arm that move around your body with a minimum of disruption.

The camera shows the surgeon a view of the surgical site that he could never see with his own eyes alone.

Just a few hours after waking up, I spent most of the day out of bed, sitting in the guest chair in my room. I was walking the ward the next day. And only three days after surgery, I walked out of the hospital under my own power. I love robots and I’m absolutely in awe of Dr. Kortz’s care, compassion and capability as a surgeon.

### My Worst Friend – P.T.

That’s the good news. Moderating that good news only slightly is the fact that

my GIST was attached to my rectum for its entire length of 6.5 cm. With that amount of surgical disruption, Dr. Kortz felt it necessary give me a temporary diverting ileostomy in order to let the rectum heal for three months before further use. He had warned me before surgery that a lengthy tumor-to-rectum connection would necessitate this procedure. In keeping with the family naming tradition, I named my bag P.T. (an abbreviated form of “Poop Tank”).

True to his assurance, three months later in June 2010, Dr. Kortz was ready to reverse the ‘ostomy. Before that procedure, he performed a proctoscopy to make sure the surgical site was completely healed. I thought the hospital receptionist would split a gut laughing the morning I walked up to her desk and said, “Hi, I’m here to play a starring role in your ‘bend-over’ movies.” That was the last fun I had that morning. When I asked Dr. Kortz if I would be under anesthesia for the procedure, he laughed

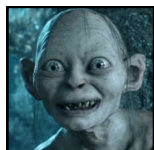
and said, “No; instead you’ll get to watch the whole thing on live TV.” So for an hour he took me on a guided tour of a part of my body I’d never seen before and hope to never see again; down where the sun don’t shine, but the camera

lights do. Immediately following my movie debut, I had the privilege of enduring a barium enema. Oh boy! I passed both tests with flying colors and believe me, with the enema there were a lot of flying colors. Next stop – ileostomy reversal.

So I celebrated my 60th birthday on the operating table, while Dr. Kortz ripped me a new you know what. Later his surgical assistant told me that while he was stuffing my intestines back into my body, he sang, “Back in the bus, Guts” to the tune of Paul Simon’s “50 Ways to Leave your Lover.” A few days later he came into my room and said, “I’m releasing you, but before you go, I want you to roll on your side and wink



The DaVinci robot...or R2D2?



GOLLUM



## Cesarini's ten year GIST battle ends with family by her side

**M**aura Cesarini passed away on the fifth of January after a 10 year battle with cancer. She died peacefully surrounded by her husband and siblings. Maura was the daughter of Irish immigrants, Humphrey Moynihan from Gneeveguila, County Kerry, Ireland and Maura Derwin from Athlone, County Westmeath, Ireland. She leaves behind her husband Michael, daughters Michaela and Bella, sisters Mary Conneely (Galway, Ireland) and Sharon Moynihan (Duxbury), brother Frank Moynihan (Randolph), nephew Rory Conneely (Galway, Ireland), niece Jade Moynihan (Randolph) brother-in-law Kevin Conneely (Galway, Ireland) sister-in-law Judy Woods (Randolph), mother-in-law Nancy Cesarini (Brockton) and father-in-law Joseph Cesarini (Brockton).

Although only 43 at the time of her death, Maura accomplished a great deal in her life. Maura was a graduate of Emerson College where she received both a bachelor's and Master's degree in Communications. Maura worked for a time in the telecommunications field both here in Massachusetts and in London, England where she lived for two years. In 1995, Maura decided to change careers and went to the police academy. It was here that she met her future husband Brockton Police Detective Michael Cesarini. Maura graduated from the police academy in 1996. She became a police

officer with the Town of Braintree and was later promoted to detective. On December 26, 1998, Maura married Michael. They had two daughters Michaela and Bella. Shortly after Bella's birth, Maura was diagnosed with a rare and incurable cancer, called GIST. This diagnosis made Maura and her family determined to appreciate every moment of life and to live their lives to the fullest.

Maura loved spending time with her family. She was very close to her sisters Sharon Moynihan and Mary Conneely and her brother Frank Moynihan. It was very important to her that her family remain close. Despite her cancer diagnosis, Maura and her family travelled to many places including, Ireland, France, Canada, the Caribbean and numerous National Parks. Maura was very compassionate and empathetic. She belonged to several on-line support groups for people battling GIST offering guidance and encouraging words to other patients. She also set up a support group in Duxbury for other women battling cancer. Through her active participation in



Maura, with husband Michael, and daughter Michaela and Bella.

these groups, Maura made friends all over the world. Maura was dedicated to trying to find a cure for this disease and did extensive fundraising for research with her husband Michael.

Maura's determination to beat this disease was recognized by the Sarcoma Foundation of America whom presented her with a Courage Award at a ceremony in New York City in April 2012.

In lieu of flowers, donations can be made to the Cesarini G.I.S.T. Research Fund at The Dana Farber Cancer Institute, PO Box 849168, Boston, MA 02284-9168 and/or Cesarini Education Fund at Michael J. Cesarini, FBO The Cesarini Children, Acct XX0739 c/o Eastern Bank, PO Box 1716, Duxbury, MA 02331.

## LRG Canada holds first GIST Day of Learning in Toronto



From left to right: Presenters Ko, Josephy, Koch & Law

**L**RG Canada held it's first GIST Day of Learning in November and Dr. David Josephy had nothing but great things to say the event, "Our Day of Learning was a 100% success—rave reviews all around!

"The two doctors from Sunnybrook Hospital, Dr. Ko (medical oncologist and Dr. Law (surgeon) were just outstanding - very knowledgeable, approachable, and generous with their time."

## PI3K

From Page 1

additional measurement taken four weeks after treatment was discontinued to see if there was any further tumor growth. Tumor response was assessed via volume measurements, micro-PET imaging, histopathology and immunoblotting. Evaluations of genomic alterations in the PTEN/PI3K/AKT pathway were also performed.

The results of the study showed that in all models GDC-0941 alone did cause stabilization of tumor growth and inhibit tumor cell proliferation, but apoptosis

(cell death) was not observed. However, when GDC-0941 was combined with imatinib, profound tumor regression (as opposed to stability) was observed, as was a nearly complete stopping of tumor cell proliferation and an increase in apoptosis, making the combined treatment superior. This was also confirmed when looking for tumor regrowth. In three out of the six models, the tumor size remained reduced and stable even after the treatment was discontinued for the combined treatment group. This was not observed in the imatinib-only group. In addition, a positive correlation was demonstrated between response to the combined GDC-0941/imatinib treatment

and loss of PTEN both on the gene and protein levels.

In conclusion, this study showed that the combination of GDC-0941 and imatinib is more effective than either alone in terms of antitumor efficacy, and had a sustained effect even after withdrawal of treatment. In addition, assessment of PTEN status may represent a useful biomarker for patient selection. Combining drugs to enhance efficacy is an interesting approach to research, and perhaps we will see more studies like this going forward.

*Read more about the LRG Research Team on our website: [liferaftgroup.org/the-lrg-research-team](http://liferaftgroup.org/the-lrg-research-team)*

## TREATMENT

From Page 2

cialists like Dr. Trent rather than relying on just clinical trials for drug approvals. “A big factor is modernizing clinical trials,” he says, particularly doing away with studies where cancer patients who are fighting to survive are given placebos.

“I’d much rather have my patients on Votrient than taking a placebo in a clinical trial,” Dr. Trent says.

In the meantime, insurance companies have been cutting back on their coverage of off-label drugs for cancer treatment,



according to some industry observers.

Susan Pisano, a spokesperson for America’s Health Insurance Plans, an industry association, says insurance companies take three factors into

consideration in coverage of off-label drugs: FDA approval, support from compendiums such as the National Comprehensive Cancer Network (NCCN), and published studies on the efficacy of the drugs. Pisano says she

doesn’t know of any trends limiting coverage of off-label cancer drugs. “I’m not sure if some kind of early approval of drugs by the FDA would make a difference,” she says, since the other factors are also taken into consideration. “It’s just one of the things they look at.”

Pisano says she’s not aware of any provisions in the Affordable Care Act (ACA) that might affect how off-label drugs are covered in the future, but some fear that insurance companies, facing a squeeze, will cut back more.

“The jury is still out on that,” the Abigail Alliance’s Burroughs says, noting that there are significant benefits from

the ACA, such as coverage for pre-existing conditions, which will help cancer patients.

The issue of access to off-label drugs is only going to become more important as targeted treatments become more available and personalized medicine grows.

“As we get more data, we’ll figure out which patients respond better to which drug. We’re not quite there yet,” Dr.

Trent says.

As for Linda Martin, she is continuing the fundraising she started to help pay for Jessica’s Votrient. Jessica loved learning,

and completed her master’s degree in May 2011. In her honor, Linda has raised money to start a college fund for people with life-changing diagnoses.



### Mark your calendars!

- The US & Canadian Academy of Pathology conference (USCAP) will be held **March 2-8** in Baltimore.
- The next GIST Day of Learning will be held in Miami on **March 23** at the Sylvester Cancer Center. More details coming soon.
- The LRG researchers will be meeting **March 24-26** at the LRG office in N.J.



The LRG will be bringing you more and more of these investigative articles in the future. If you know of an issue that should be addressed by the LRG, please email us at [liferaft@liferaftgroup.org](mailto:liferaft@liferaftgroup.org)



## Beloved husband, father, grandfather and trusted LRG Board Member, Bob Book passes away at 82

**B**ob Book, 82, of Carmel, Ind. passed away the morning of December 9, 2012. He was born on December 31, 1929 in Indianapolis, IN to Wm. H. and Margaret V. Book. A 1948 graduate of Arsenal Technical High School, Bob enlisted in the United States Marine Corps Reserve that year, and spent 31 years in both US Marine Corps and US Marine Corps Reserve, retiring in 1983 with the rank of Colonel.

After earning a BA in animal science from Purdue University in 1952, Bob earned an MBA from Indiana University and completed the Senior Executive Program at Columbia University. He became a driving force behind Elanco Products Company, a division of Eli Lilly Company, when he joined as a sales representative in 1955. After retiring in 1983 as Group Vice President of Agricultural Marketing, Bob became the first president of The Indiana Institute of Agriculture, Food and Nutrition, Inc.,



**SEMPER FI**

serving until 1992. Bob and Dr. Bud Harmon established the Purdue University Book Harmon Leadership Program in Animal Sciences in 2005. Board memberships included: The Marine Corps League, the Indiana Military Coalition, Military Officers of America Assn., the Farm Foundation, Neogen Corporation, Ag Alumni Seed, U.S.

Feed Grains Council, National FFA and The Life Raft Group. For 29 years he was both an elected representative on the Clay Township Board in Hamilton County, Indiana, and President of Clay Township Regional Waste District. He was Precinct Committeeman for Clay Center 2, for over 30 years and served St. Vincent's Carmel Hospital from ground breaking until he was named Chairman of the Board, that board membership continuing until June 2012.

Bob is survived by his wife of 47 years, Jeanne Book; their children, Mary M. Klunder (Robert), Nancy Joanne Fletcher, William H. Book (Dede), and

Bart J. Book (Sherri). Grandchildren include: Benjamin R. Klunder, Lauren E. Book Linos (Constantine), Hilary R. Book, Christine Hatfield Waldrup (Brad), Michael M. Hatfield, and great-granddaughter, Isabella Waldrup. Others include cousins Martha Powell and family, and Kathryn Ann Hammons, and daughter Kathy Hammons, as well as many good friends and associates. The family wishes to thank the St. Vincent New Hope Foundation staff for excellence in care and kindness toward Colonel Book and his family and many friends.

Memorial contributions may be sent to:

Purdue Foundation; Book-Harmon Leadership Forum; St. Vincent Health Foundation, the Life Raft Group, Arsenal Technical High School Alumni Association, or Marine Corps University at Quantico, VA.



## Chilean GISTers & CML patients meet!



On Saturday December 15, 2012, a group of CML and GIST patients got together to summarize activities done during 2012, and celebrate the end of the year. Thanks to Piga Fernandez, LRG Global Relations Coordinator and head of Corporacion Nacional Maxi Vida Chile for sending a picture.



## CTOS

From Page 3

ETV1 in Gastrointestinal Stromal Tumors (GIST)”, looked at microRNA (miRNA), a small RNA molecule that helps regulate gene expression, and “aimed to discover any GIST specific miRNA expression patterns and identify miRNAs involved in GIST tumorigenesis.” The study involved 50 primary GIST tumors and 10 leiomyosarcomas of the GI tract. The results identified a number of miRNAs that may be implicated in GIST. A decreased level of three of these, MIR-17, 20A, and 222, may contribute to the overexpression of KIT as well as ETV1, genes that play a key role in GIST. When expression of these miRNAs was increased, KIT and ETV1 protein levels were decreased, thus inhibiting cell proliferation and causing cell death (apoptosis). This points to the possibility of these specific microRNAs as targets for future therapeutic treatments.

Another study, led by Dr. Piotr Rutkowski and also involving Dr. Maria Debiec-Rychter, was a long-term analysis of GIST survivors. The study, entitled “How Did the Results of Advanced GIST Treatment Improve Over 10 Years: Analysis of Inoperable/ Metastatic/Recurrent GIST Treated With Imatinib in 2001-2009. Who are Long Term Survivors?” was a retrospective analysis of 370 inopera-

ble/metastatic/recurrent GIST patients, and looked at a number of different prognostic factors. The results determined that long-term survivors (five years or greater) were characterized by having smaller tumors at start of imatinib treatment, higher neutrophil or albumin levels, better World Health Organization (WHO) performance status, and surgery that removed all residual disease. Perhaps the most striking aspect was that the median progression-free survival (PFS) of these patients on first-line imatinib was 35.6 months, a figure that is greater than the 24 months that is generally reported.

Another presentation, led by the aforementioned Dr. Michael Eriksson but also involving Drs. Peter Reichardt, Heikki Joensuu, Peter Hohenberger, and LRG Research Team member Dr. Sebastian Bauer, was entitled “Needle Biopsy Through the Abdominal Wall for the Diagnosis of GIST – Does it Pose any Risk for Tumor Cell Seeding” and looked to see if performing this particular type of biopsy was associated with negative outcomes for patients in terms of PFS or overall survival (OS). The results suggest that this is not the case, but the authors suggest they should be viewed with caution due to the retrospective nature of the study and the relatively small number of cases studied (393).

An additional study, entitled “A Medium Throughput Compound Screen Identifies Novel Treatment Options for Gas-

trointestinal Stromal Tumor (GIST)” was led by Life Raft Group Research Team member Dr. Anette Duensing. The study re-evaluated some chemotherapy drugs for their sensitivity to GIST. A total of 89 FDA-approved compounds were studied and tested against five GIST (imatinib-sensitive and imatinib-resistant) and two leiomyosarcoma cell lines. GISTs were found to be highly sensitive to several drug classes, most notably inhibitors of transcription and topoisomerase II – in some cases more so than imatinib or sunitinib. In the words of the study authors, “these drug classes should be considered as viable treatment options for GIST patients, and the initiation of clinical trials is warranted.”

Finally, Dr. Peter Reichardt and his colleagues gave updated results on the GRID trial for regorafenib, which included 199 patients. Their results showed that the study met its primary endpoint, significantly improving median PFS both per central review and investigator assessment. The median PFS for central review was 4.8 months (vs. 0.9 months for placebo) and for investigator assessment 7.4 months (vs. 1.7 months for placebo).

All in all, the CTOS meeting delivered a number of studies that showed particular promise for GIST, many involving esteemed members of the LRG Research Team, and the Life Raft Group was honored to both participate and present their poster at the event.

## GARBER

From Page 11

at me.” So I obediently rolled away from him and “winked” in a way I’d never winked at anyone before, all through the breezy side of my hospital gown. She was sufficiently satisfied that I could squeeze the right muscles. Dr. Kortz is an



Garber's team recently built this community training center.

excellent surgeon and a really funny guy. His father was a surgeon; his brother is also a surgeon. When the rest of the neighborhood kids were playing doctor, he and his brother were operating on the cat.

### Back to Work

Two months later I was back in Namibia continuing my missionary duties. It took a full year for my plumbing to return to pre-operative effectiveness, but I’m now completely back to normal. I remain cancer-free and I’m living each day to its fullest

**“I remain cancer-free and I’m living each day to its fullest with joy in my heart for another day to make a difference in someone else’s life.”**

with joy in my heart for another day to make a difference in someone else’s life. Our team just completed building a community training center in a very poor squatter’s camp (see photo).

Life is beautiful, even in a life raft. Carpe Diem.

*For an introduction to the DaVinci robotic surgical technique:*  
[www.davincisurgery.com](http://www.davincisurgery.com)

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\*in loving memory



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## Life Raft regional chapters: Find your reps info at [liferaftgroup.org/find-a-support-group/](http://liferaftgroup.org/find-a-support-group/)

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