

# GDOL draws big turnout in Florida

By Tricia McAleer  
LRG Program Director

**L**ife Raft Group's first GIST Day of Learning event was a big success. Partnering with Jonathan C. Trent, M.D., PhD and University of Miami Sylvester Cancer Center, we were able to provide education and support for over 50 GISTers and caregivers.

GIST Day of Learning is a free one-day regional educational event. In addition to sharing hope and friendship, attendees can learn important facts about their cancer, prevailing treatment options, tests as indicators for treatment surveillance, and coping with dietary changes and side-effects of treatment. This event was sponsored by Amgen and in large part by Bayer.

Both old friends and new came from as far away as Texas and Illinois to attend our one day seminar. We had six wonderful speakers who gave extensive presentations on each of their areas of expertise.

Our host, Dr. Trent, is the Co-Director

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## Battling gastrointestinal stromal tumor



# LIFE RAFT GROUP

June 2012

In memory of Catherine Melillo, Cindy Dunigan,  
Francis Kariuki & Linda Brecko

Vol 13, No. 3

## Guidelines proposed for pediatric GIST

By Pete Knox

LRG Special Projects Coordinator

**G**IST is a disease with a number of subtypes, and is beginning to be increasingly viewed as a "family of diseases". One particular form of the disease is commonly known as "Pediatric GIST" - in this form the mutation occurs when the patient is either a child or



PAPPO

young adult (but may not be discovered until later). In their article, "Treatment Guidelines for Gastrointestinal Stromal Tumors in Children and Young Adults", published in the May 2012 edition of the *Journal of Pedi-*

*atric Hematology/Oncology*, Katherine A. Janeway, MD

(from Dana-Farber Cancer Institute in Boston, MA) and Alberto Pappo, MD (from St Jude Children's Research Hospital in Memphis, TN) performed a detailed review of the clinical presentation, natural history, diagnosis and staging of Pediatric GIST, and proposed specific treatment guidelines for patients with this form of GIST.



JANEWAY

### Clinical Presentation

Janeway and Pappo cited a study of 44 GIST patients diagnosed before the age of 21 that identified anemia secondary

See PEDIATRIC, Page 10

# Cesarini wins 2012 Courage Award

By Magda Sarnas

LRG Patient Registry Supervisor

**T**he Sarcoma Foundation of America held its Tenth Annual Gala on April 30 in New York, honoring doctors and survivors from many different corners of the sarcoma world.

One of our own longtime Life Raft Group members, Maura Cesarini, was honored, along with seven other recipients, with the 2012 Courage Award.

This year Maura will also be celebrat-



Maura accepting her award.

ing her 10-year Cancer-versary with GIST. Her daughters, Bella and Michaela, and husband, Mike, were in attendance to honor her.

When Maura first experienced symptoms while pregnant with her second daughter, Bella Danielle, it was thought to be a cyst. After giving birth she was readmitted to remove a tumor and other organs that had been affected by her tumor.

We are touched by the strength and perseverance in her story, as was first

See COURAGE, Page 11

# LRG Tissue Bank uncovers diagnosis issues in LatAm

By Roberto Pazmino  
LRG Administrative Director

**A** Life Raft Group meeting with Latin American representatives underscored barriers to the tissue bank program and proper diagnosis of GIST in the region while generating suggestions for ways of improving the system.

An informal survey found that many patients and doctors are not aware of the GIST Collaborative Tissue Bank (GCTB) program, or do not quite understand the logistics of the process.

Tissue samples are routinely taken during surgery for review and evaluation by pathologists. These tissues are fixed in formalin, processed to remove water, and then infused with molten paraffin wax. These samples are kept in the hospital; to participate in the GCTB, tissue must be sent to the LRG along with a pathology report. Many of these pathology reports are missing a key piece of information, DOB, which is used to identify the tumor and to correlate tests.

By having patients send their paraffin tissue blocks to LRG, who in turn sends them to Oregon Health & Science University (OHSU), and then to the research team, researchers have GIST tissue that is linked to patient clinical histories for the first time. Personal information is encoded to comply with research privacy regulations.

The package must include:

- Tissue embedded in paraffin block. Recent advances in research technology have resulted in new and exciting methods of using paraffin tissue blocks, making them an extremely valuable resource for researchers. Tissue in paraffin block is easier to ship and doesn't need to be kept frozen.

- Pathology report from the original tissue analysis.
- Consent form A. Authorization to

send the tissue from hospital to LRG.

- Consent form B. Authorization to share mutational test results with LRG.

Tests performed in OHSU have identified some misdiagnosed cases, others in which the sample received was not sufficient, and misinterpretations in the original reports.

Tissues confirmed with GIST by mutational screening are shared with the patients' doctors to review the treatment response based on the mutation found.

From here the tissue is sent to Stanford University for further research for a cure for GIST.

The informal survey found that patients have had problems obtaining samples from hospitals. It was suggested that a webinar on the tissue bank process be created and shared with patients, doctors and hospitals in the region to clear up any misunderstandings. Easy to understand webpages on the topic on the LRG and Alianza GIST websites were also recommended.

Another suggestion was to partner with pharmaceutical companies to use their distribution channels for educational materials that would explain the program.

The LRG did a sampling of 21 GCTB participants from Latin America.

- The participants included 11 from Chile, two from Bolivia, two from Brazil, two from Colombia, one from the Dominican Republic, one from Honduras, one from Nicaragua and one from Venezuela.

- Of the 21 participants, only 13 had c-kit testing done.

- Of the 13 with c-kit testing, four (31%) were subsequently confirmed to be incurrant, meaning they did not have a GIST.

Clearly, there are service inadequacies in the diagnostic process. The LRG is working on developing an educational program to address

## 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/donate.html](http://www.liferaftgroup.org/donate.html) 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, the Newsletter Editor, at [ekristoff@liferaftgroup.org](mailto:ekristoff@liferaftgroup.org) of any errors.

this. Another recommendation generated by the informal survey was to design a model for a GIST center of excellence in Latin America, to improve diagnosis and treatment in the region.

Thank you to all of our members who sent in generous contributions in recognition of Mother's Day 2012. We've received over \$1,000 so far that will be applied to our educational programs and research!



# Alianza GIST launches new website!

By Sara Rothschild

LRG Global Relations Director

**A**lianza GIST is proud to present the official launch of its new website: <http://www.alianzagist.org>.

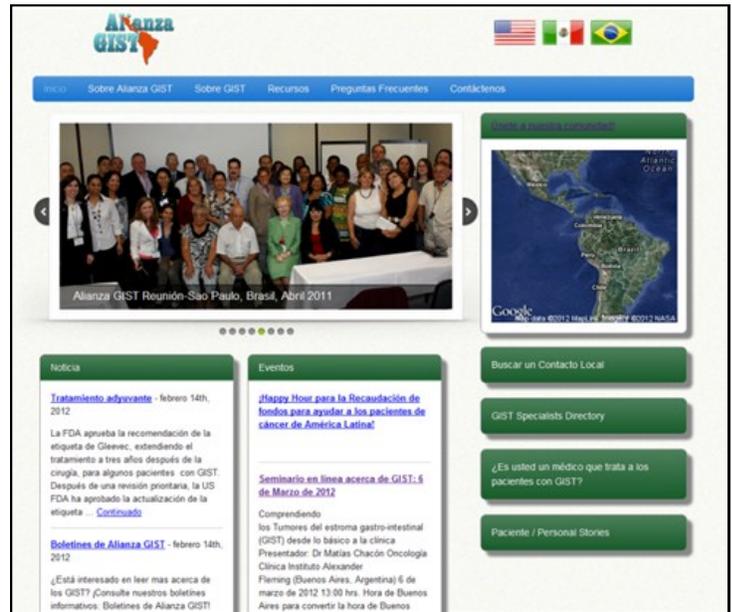
The website is available in three languages: English, Spanish, and Portuguese.

Visitors will be able to read more about Alianza GIST, including press releases, newsletters, reports and presentations. There will also be a great deal of information about GIST, its treatment, and side effects.

If visitors would like to contact a person in their country for support or more information, they have access to contact information for each country representative. In addition, the website regularly updates information about News and Events that are relevant to GISTers in

Latin America.

Alianza GIST also launched a new social networking community where GIST patients and caregivers can login to the Comunidad de Alianza GIST and chat with each other about relevant topics related to their cancer. There is also functionality where each country organization has a “Group” set up where they can communicate internally with their members. At the moment, this comunidad site is only available in Spanish.



If you would like more information about the website or if you have any questions, please contact us at [info@alianzagist.org](mailto:info@alianzagist.org).

# Alianza GIST's first webinar a success

By Sara Rothschild

LRG Global Relations Director

**A**lianza GIST thanks all those who participated in the online seminar in Spanish, “Comprendiendo Los Tumores Del Estroma Gastro Intestinal (GIST) Desde Lo Básico A La Clínica,” presented by Dr. Matías Chacón of the Instituto Alexander Fleming de Buenos Aires, Argentina.

We especially want to thank Dr. Chacón for his interesting presentation and his collaboration in spreading the word about this rare form of cancer; likewise, we are grateful to all the representatives of Alianza GIST for their efforts in helping to make this happen in their respective countries.

For Alianza GIST, this has been a big step in meeting its goals:

- Decreasing the gap between scientific knowledge and practices



**CHACÓN**

- Getting the most information about GIST out to doctors, patients and family members, and
- Helping patients to not feel alone in their

journeys with GIST.

We invite you to visit our webpage [www.alianzagist.org/](http://www.alianzagist.org/) webinar where we have placed the seminar and anyone can access the information. At the same time, we would like to remind you that you can send your comments or questions to [piga@alianzagist.org](mailto:piga@alianzagist.org).

Thank you again for participating in this seminar.



# Adieu, Adios, Ciao, Auf Wiedersehen, Shalom, Sayonara, Xaipete Tricia McAleer

**By Gale Kenny**  
LRG Accounts Manager

All of you who have been a part of The Life Raft Group have had the pleasure of knowing Tricia McAleer, our Program Director. After nearly 10 years of advocacy and accomplishment, she has decided to leave the LRG and pursue other opportunities.

Trish was recruited by Norman Scherzer to be an Administrative Assistant, just a handful of months following LRG's incorporation in 2002. Over the years, she graduated from college, held



several titles, and wore many 'hats'. Trish has seen this group expand its reach far beyond the states, spreading our mission into over 50 countries.

She was part of the inception of the LRG GIST Research Project; which brought on an esteemed team of physicians who continue to collaborate and work tirelessly for a cure. She has spearheaded the first official Life Fest, the first Poker Tournament, and the NIH

Pediatric Clinic just to name a few.

Tricia has always shown unlimited selfless dedication to the LRG, the patient and caregiver community, as well as the many facets that make this organization ever thriving and successful. With intuitive sensitivity and boundless integrity, Trish has taken on the collected challenges presented by our rapidly growing organization, and handled them with pride and commitment. We will miss her purpose-driven work ethic and her friendship. All of us here at the Life Raft Group extend heartfelt good wishes to her and all her future endeavors. Trish, you will be greatly missed.

## New Horizons GIST: Patient Advocates meet in Paris

**By Norman Scherzer**  
LRG Executive Director

Patient advocates from 19 countries convened in Paris on April 27 to discuss GIST issues. Prior annual meetings had brought together patient representatives from the CML (Chronic Myelogenous Leukemia) and GIST disease communities. This was the first time that the GIST community met on its own.

Leading the agenda were research presentations by Dr. Maria Debiec-Rychter from Belgium, who discussed the LRG research team progress, and Dr. Frederic Chibon from France, who discussed his work helping to define recurrence risk factors. Markus Wartenberg then discussed the work

of Sarcoma Patients Euronet (SPAEN), one of the regional groups providing representatives to this global forum.

Workshops then followed, one focusing upon treatment access and one on therapy management, particularly side effects. It was quite clear that both access to treatment and to quality management of that treatment have a long way to go and in



**DEBIEC-RYCHTER**

some parts of the world a very long way.

The final part of the meeting consisted of sessions with representatives from three pharmaceutical companies, Novartis, Bayer and Pfizer Pharmaceuticals. This provided a unique opportunity to look inside each other's worlds and find common cause on a number of issues.

The meeting also produced an updated declaration, which we have included below.

### "THE BAD NAUHEIM DECLARATION"

(April 2012 update)

*An international declaration affirmed by the "Global GIST Patient Community" to promote access to "state-of-the-art GIST treatment and care" for all patients, wherever they may live. This update was developed, agreed and signed by representatives of worldwide "GIST Patient Advocacy Groups" dur-*



Advocates from 19 countries met at New Horizons in Paris.

# Ninth Swiss meeting covers uplifting topics

By **Helga Meier Schnorf**  
GIST-Gruppe Schweiz

About 80 GIST patients, relatives, GIST experts, interested professionals, physicians and representatives of the pharmaceutical industry gathered at the Restaurant *Au Premier* at Zurich Main Station in Zurich on April 8 for the ninth meeting of the Swiss GIST group. A warm welcome was extended to special

guests Candy Heberlein, President of the Swiss Foundation for the Promotion of Bone Marrow Transplantation, and Christel Jäger-Freysoldt, chief executive of Das Lebenshaus in Germany.

Representatives from Bayer, Bristol-Myers Squibb, Novartis and Pfizer Pharmaceuticals were also present. As always, it was a unique opportunity for the participants to learn about the latest insight into GIST and its treatment, as well as to meet experts in this field. Markus

Wartenberg from Das Lebenshaus moderated the meeting.

The meeting was opened by the president of the Swiss GIST group, Martin Wettstein. Firstly, our two deceased regional managers, Matthias Merki of the Northwest Switzerland regional support group, and Herbert Blatter of the Romandie regional support group, were remembered by a minute's silence. Matthias Merki passed away on

August 6, 2011, in the Hospital St Clara in Basel. Herbert Blatter lost his fight against GIST on March 6, 2012, in the Center Hospitalier Universitaire Vaudois (CHUV), Lausanne. Matthias Merki's tasks will be taken over by Martina Kuoni, and Gisèle Mōri and Audrey Mathot will be responsible for West Switzerland. Martin Wettstein thanked the new regional managers for their willingness in taking over these tasks.

The topic of GIST was handled in two parts with cutting-edge presentations from two international GIST specialists, Dr. Michael Montemurro and Dr. Sebastian Bauer.

Dr. Montemurro, consultant at the CHUV in Lausanne and medical adviser to the Swiss GIST group, introduced the first part. Dr. Montemurro provided all participants – and notably those who were attending the meeting for the first time – with basic knowledge of GIST. He also talked about “GIST 2012: State of the Art in the Research and Treatment for GIST.”

Dr. Sebastian Bauer of the University



President Martin Wettstein & his wife, Claudia.

See **SWISS**, Page 9

## I found a friend in the LRG

By **Jeff Bernstein**  
LRG Western Penn. Rep

I was caught up in a whirlwind when I found out that I had cancer on a Monday morning in October of 2011. I was admitted to the hospital that same day in preparation to have emergency surgery the next morning to remove the tumors and then found out I had GIST - all in a 24 hour period! I felt like my world had turned upside down and shortly after that during my recovery, I was lucky enough to find Life Raft.

In that discovery, I have found people willing to take the time to answer my questions; I have made good friends and



**BERNSTEIN**

feel so welcomed. I am a retired steel worker and live in a little town, East Brady, Pennsylvania. I have been happily married to my wife, Chris, for 43 years. We have two daughters and four beautiful grandchildren. I know what a caring community can do to offer support in times of need! I know how invaluable the support of family and friends can be. Life Raft Group is a community of caring, and I am so honored to take on my new role as LRG's western Pennsylvania representative. I hope to be able to give others opportunities to share, seek answers, and find support just as I have done.

**Join us at ASCO!**

**THE LIFE RAFT STAFF WILL BE MAKING THEIR WAY TO CHICAGO ON JUNE 1 TO ATTEND AND EXHIBIT**



**AT THE ANNUAL AMERICAN SOCIETY OF CLINICAL ONCOLOGY (ASCO) CONFERENCE. IF YOU'RE ATTENDING TOO, COME VISIT US AT THE PATIENT ADVOCATES BOOTH! OR YOU CAN CATCH UP WITH EXECUTIVE DIRECTOR NORMAN SCHERZER AND BOARD MEMBER AND CLINICAL TRIALS COORDINATOR JIM HUGHES BETWEEN SESSIONS.**

# THE LRG TURNS 10!

## Our favorite moments...

"My first and one of my most endearing memories of the Life Raft Group was back in September 2004. I had just moved down to New Jersey and said "WOW Hot-Diggity, I am going to be closer to the LRG! I called them up and they said they would be delighted if I dropped in. I was greeted with hugs and the warmth likened to that of old friends whom you haven't seen in years! It was wonderful!

-Anita Getler

I recall at our first ever Life Fest (only it wasn't called Life Fest back then) in Boston 2002. Our nascent little group (not even incorporated) gave an award to Dr. Demetri. He came in person to accept and brought his family to show that it really meant something to him. I was very impressed by that.

-Ellen Rosenthal

My favorite memory is the first time I met Norman and Jerry at a CTOS meeting in Montreal about 10 years ago and I was standing alone at the welcome reception when up comes Norman & Jerry. They said "Hi!" and I said, "Do I know you?" They said "No, but we know you". Thus was the beginning of a long friendship and fruitful research collaboration.

-Dr. Brian Rubin  
LRG Research Team

"Hope Floats in the Life Raft" being played at the 2010 Life Fest Gala. Esteemed lyricist & songwriter George Teren authored, sang and professionally produced this song for us, It embodies the spirit as well as the mission of our organization. "We keep fighting till we beat it, no matter what the cost, searching for new treatments, working for a cure, but we have to raise awareness and spread the word..."

-Gale Kenny  
LRG Staff

I only had the pleasure of attending one Life Fest – the one in Chicago a few years ago, what I remember most about the weekend was the announcement that if some of the guests didn't move their cars they were going to float away!

-Elaine Rys

On a rainy day in May, near our office, a turtle found itself stranded on the road. Matthew came to the rescue & carried the turtle to safety, capturing a photo of the creature before he let it go. For the next few days, everything seemed to go right for the Life Raft Group. I think that turtle had something to do with it.

-Norman Scherzer  
LRG Executive Director



## Ten ways to help us celebrate

1. **Join the Patient Registry**
2. **Make a contribution**
3. **Donate your tissue to research**
4. **Tell somebody about the LRG**
5. **Sign our Safe Medicines Save Lives petition**
6. **Give this newsletter to your doctor**
7. **Say Happy Birthday on Facebook or Twitter**
8. **Post your birthday pictures on our FB wall**
9. **Ask others to support the LRG in honor of your birthday**
10. **Wear your LRG gear with pride!**



My favorite memory was arriving at the first NIH Clinic. It was great to see the expert care, hope & support given to young adults with GIST.

-Tricia McAleer  
LRG Staff

My favorite moment was at CTOS 2011. People often noticed my LRG nametag and praised the organization. As the day wore on I began appreciate what a special place the LRG is, and how lucky we are to represent such a wonderful group of patients & caregivers.

-Pete Knox  
LRG Staff

I guess one of my best moments was at Life Fest 2010 in New Jersey with a roomful of healthy GISTers.

-Norma Siegell

A favorite "memory" is one of TomO's writings that has meant a lot to me and that graces the first page of my 2012 journal. It starts with, "Each day is truly a gift and you are the master of it. Pay attention to your bucket list and move the "people" parts of your list to the top."

-Barbara Alesandrini

My memories are probably the same as many others. The day I found Life Raft ! I talked to Jerry Call, & found out I wasn't alone.

-Sandra West

On my first day, Norm, who has many stories, told one about a patient named Mike Matthews and being able to witness Mike's final dance with his wife. This drove home to me how much this man, and the rest of the LRG staff, cares about each individual touched through our programs. It made me proud to be a part of LRG.

-Phil Avila,  
LRG staff

Within 6 months of being diagnosed with GIST in Jan 2010, I was able to meet and shake the hands of the pioneers who are the reason I am alive today. Dr. Brian Druker at OHSU, Dr. Dan Vasella of Novartis in the picture below, and Norman Scherzer who steers the "Raft".

I am eternally thankful for the LRG and the community we have to support each other. We are not alone.

-Dave Safford



February 12, 2012 I received my first email from Janeen Ryan welcoming me to The Life Raft Group. Janeen gave me support and knowledge that has made GIST easier for me to understand. LRG means hope and knowledge to me knowing I have the support of many others who are walking the same path as I am.

-Jeff Bernstein

There was one other memorable event that we all shared and that was when the Gleevec was authorized by the FDA for GIST patient use in February of 2002. It changed the whole course of events for all of us.

-Dick Kinzig

My fondest memory was talking with Butch Eller. That man's voice just put a smile on my face every time I would talk with him. I miss that man.

-Magda Sarnas  
LRG Staff

Wading to the office in the middle of a flood!

-Roberto Pazmino  
LRG Staff

This may not be my favorite memory from the past decade, but it's a milestone in my book! I turned 50 this year! I didn't think I'd live to see 50 after receiving the GIST diagnosis 7 years ago this August. It has been rough at times, but I'm a survivor and glad to be here at 50!

-Jodi Merry

Happy birthday to LRG from Switzerland and Italy: Helga Meier and I wish to thank all of you for your invaluable work in favor of the global GIST community.

Our favorite memories are from Life Fest 2010. Check out our picture!

-Anna Costato



"Once, when I was down, scared and unsure of what to do, I received an e-mail from Norman who had never written me before. It said simply, 'You are not alone'. It gave me strength at a moment when I had none left."

-Janeen Ryan

## PARIS

From Page 4

ing an international conference on the 28th of April 2012 in Paris.

### Background & Purpose

(1) Gastrointestinal stromal tumor (GIST) is a rare cancer of the mesenchymal (connective) tissues of the stomach, gastrointestinal tract and related organs. The incidence of this cancer is approximately 12 -15 per million of population, and it accounts for less than one half of one percent of all cancers diagnosed.

(2) A decade after the introduction of the first tyrosine kinase inhibitor (TKI), this novel class of medications has transformed the experience of GIST. Prior to this time, a patient diagnosed with advanced GIST had a life expectancy of less than two years. While imatinib mesylate remains a recognized front-line therapy, several second-generation TKIs are in development. More than 85% of patients respond to these drugs and benefit for many years. For those who are nonresponsive, however, new technologies must be developed and made available.

(3) The Bad Nauheim Declaration is a key document of the “Global GIST Patient Community”. The initial version was adopted on 1 July 2007. This document reflects the first revision of this original statement. The current version (2012) version is the only official one; all previous versions have been replaced and should not be used or cited except for historical purposes. The “Global GIST Patient Community” will promote utilization of this document at a global level and in national situations as appropriate as a tool to foster favourable changes in health policy and practice.

### The Declaration

The undersigned “GIST Patient Advocacy Groups” collectively call on all those responsible for the treatment and care of patients with GIST to:

1) Ensure that no patient with GIST is denied access to approved therapies or suffers through failure to treat, or through stopping treatment recommended by medical specialists, regardless of race, nationality, faith, age, sex or economic

status.

2) Ensure that patients are diagnosed promptly and accurately, supported by formal classification of GIST as a specific disease state incorporating accepted diagnostic criteria, documented pathology and appropriate confirmatory testing.

3) Ensure that patients are provided accurate and timely information, including the resources to obtain second opinions from specialist, and psychological support at each step along their treatment journey.

4) Provide treatment and care to GIST patients through the hands of specialist multidisciplinary teams which conform to standards for a center of expertise in rare diseases (*see note*). All members of these teams should have specialist knowledge, continuing experience of treating GIST, and participate in national and/or international networking with other centers of expertise.

5) Provide access to expert pathology and mutation analysis services which give doctors and patients the information they need to make an informed clinical decision.

6) Adopt an internationally accepted treatment guideline (see 7 below) and ensure that the resources required to deliver it are available to doctors and patients.

7) Treat patients in line with an internationally accepted treatment guideline (*see note*) informed by the published evidence from scientific and clinical research.

8) Provide a voice to the GIST patient community, through representation and consultation on decisions regarding approval of new treatments, health technology assessments (HTA) and treatment reimbursement.

9) Provide patients with access to clinical trials regardless of race, nationality, faith, age, sex or economic status.

10) Provide GIST patients with access to existing, approved treatments that do not have an indication for GIST but have clinical data supportive

of a role in GIST therapy.

*Note to 4.* An example of standards for a Centre of Excellence is given by the EU in its report on Networks of Reference for Rare Diseases which can be found at [http://ec.europa.eu/health/rare\\_diseases/european\\_reference\\_networks/erf/index\\_en.htm](http://ec.europa.eu/health/rare_diseases/european_reference_networks/erf/index_en.htm)

*Note to 7.* An example guideline is offered by the National Comprehensive Cancer Network (NCCN). You will find the NCCN Guidelines for GIST at <https://subscriptions.nccn.org> The new European GIST-Guidelines are provided by ESMO very soon.

## Phase III Regorafenib Trial Meets Primary Endpoint

By Pete Knox

LRG Special Projects Coordinator

**B**ayer recently announced the results of its Phase III trial for regorafenib (BAY 73-4506), which is designed for patients with metastatic and/or unresectable GIST that have failed both imatinib and sunitinib.

Results from the trial showed a statistically significant improvement in progression-free survival, and the safety and tolerability of regorafenib were consistent with what had been seen in earlier studies.

Data from this study will be presented at an upcoming scientific meeting, and Bayer has plans to file for U.S. approval of regorafenib for the treatment of metastatic GIST.

This is of course a potentially exciting development as effective third-line drugs are sorely needed, and we will be sure to report any further information as soon as it becomes available.



## GDOL

From Page 1

of the Musculoskeletal Center, Director of the Sarcoma Medical Research Program and professor of medicine at the University of Miami Sylvester Comprehensive Cancer Center. In addition to facilitating the meeting, Trent presented “GIST: The Basics, Overview of History, Biology and Treatments”.

Jerry Call, Science Director at the Life Raft Group and caregiver to his wife and GIST survivor Stephanie, presented on “Clinical Trial Explained and Explored”. Jerry developed a GIST-specific clinical trials database, and also oversees the Science Team and Patient Registry. Most importantly, Jerry helps GISTers navigate their care at crucial points in their GIST journey.

Elizabeth Fontao, M.S., P.A.-C., who works closely with Dr. Trent at the University of Miami Sylvester Cancer Center to support sarcoma patients, presented for the “Managing Side-Effects” session.

Andrew E. Rosenberg, M.D., Professor of Pathology, Director of Bone and Soft Tissue Pathology and Director of Surgical Pathology at the University of Miami gave a talk on “Understanding Pathology and the Role of Mutations in GIST Treatment”. During his 27 years working in the pathology field, Dr. Rosenberg

## SWISS

From Page 5

of Essen in Germany, as well as the LRG Research Team, then gave a report on “Future GIST Treatment.” These two presentations conveyed a great deal of hope, after all, several new and effective substances are at the testing stage or will be within reach for GIST patients. The result of the 2012 American Society of Clinical Oncology (ASCO) convention in Chicago at the beginning of June is keenly awaited because data related to regorafenib in GIST will likely be available then.

The third presentation was by Dr. Gilles Allenbach, consultant at the

has published numerous research papers and has held the prestigious honor of serving as a professor at Harvard Medical School, Boston, Mass.

Alice Sulkowski, R.D., C.D.E., a member of the Life Raft Group and a GIST survivor since 2002, presented on “Nutrition”. She has practiced as a registered dietician for over 30 years. In addition to her hospital-based outpatient nutrition education, she has worked in medical oncology as a consultant. She currently works at Mountain States Health Alliance in Johnson City, Tennessee.

We would like to give a special thank you to Dr. Steven Rodgers, M.D., PhD for volunteering his time at the last minute to step in for Alan S. Livingstone, M.D. for the “Surgery and Other Options” session. Dr. Rodgers completed his fellowship at M.D. Anderson Cancer Center and has served as an Assistant Professor of Surgery at the University of Miami Sylvester Cancer Center since 2006. Although Dr. Livingstone regrettably couldn’t join us, we would like to

CHUV. His topic was “How does PET (Positron Emission Tomography) function?” Dr. Allenbach not only explained the functioning of PET scans, but also all the related important preparatory operations. Dr. Francis Verdun, CHUV, then spoke on the topic of radiation exposure resulting from various types of imaging. This provided a profound insight into the world of measurement of radiation to the participants.

A particular approach in the sector of surgery was taken by Dr. Christoph Kettelhack from the University Hospital in Basel in his presentation on “Selecting the Right Time to Operate.” By means of a patient case study coupled with impressive pictures, he explained when how neoadjuvant therapy (given before a



From left: Andrew Rosenberg, MD, LRG Science Director Jerry Call, Life Rafter Alice Sulkowski, RD, CDE, Steven Rodgers, MD, PhD, Elizabeth Fontao, PA-C, Jonathan Trent, MD, PhD.

thank him for all his preparation for this meeting and for volunteering to speak as well.

We also want to acknowledge our sponsors Amgen and Bayer Pharmaceuticals for their generous support of the meeting. A special thank you must go to Rich Proctor of Bayer and Leslie Fields of Novartis for attending the meeting and showing support for our educational events.

We would also like to thank Udderly Smooth and Shoprite for their product donations.

Pictures from the event can be found on Life Raft Group’s Facebook page courtesy of our volunteer photographer Don Edgar of dl photography based in Miami, Fla. Don spent the whole day with us and his expertise is truly appreciated.

GIST operation) can greatly reduce surgical risks.

A very current topic – though not yet so familiar in Switzerland or in the GIST community – is the role and task of the Oncological Nursing organization in Switzerland (Onkologiepflege Schweiz/ Soins en Oncologie Suisse). Ms. Anita Margulies gave an understanding of this in her presentation. Unlike in other countries, the topic of the task and/or involvement of nursing staff in oncology is not yet established in Switzerland. The final presentation was made by Martina Kuoni, regional manager of Northwest Switzerland, literary scholar and GIST patient. In her uplifting talk on “sickness in literature” with the aid of amusing literary extracts.

# PEDIATRIC

From Page 1

to chronic GI blood loss as the most frequent presentation, and thus pointed out that when pediatric patients present with this symptom, GIST should be considered as a possible diagnosis. They also pointed out some clinical and pathologic features that help distinguish the pediatric form of GIST, such as a higher proportion of female patients, multifocal gastric tumors and lymph node metastases being more common, and a higher likelihood of epithelioid histopathology.

## Natural History

Because Pediatric GIST is so rare (estimated as only about 1.4% of all GIST cases) developing precise estimations of progression-free survival and overall survival is challenging. It does seem, however, that the pediatric form of GIST is more indolent (slower growing).

## Diagnosis/Staging

The authors recommend that pediatric GIST should be diagnosed by a multidisciplinary team with expertise in managing sarcomas or tumors of the GI tract. In particular, a number of pieces of the patients medical history should receive specific attention, including family history, prior history of malignancy, symptoms of anemia and catecholamine excess, abdominal pain and/or distention, and the presence of skin findings associated with NF-1 and hyperpigmentation (these skin findings may be associated with familial, or hereditary, GIST, which can be passed on genetically). In terms of procedures, they recommend the following: a complete blood count, reticulocyte count, serum chemistries, and liver tests. In terms of imaging, at least a chest x-ray and abdominal and pelvic CT scan should be included, with an MRI being an acceptable alternative to a CT in some cases. PET scans may also

Patient has:	Recommendation
Initially diagnosed nonmetastatic resectable tumors	Surgery is the mainstay of therapy. Because pediatric GIST tends to have increased nodal involvement, lymph node sampling may be considered. Adjuvant imatinib is not recommended for wildtype patients as it might have less efficacy and has not been adequately studied.
Unresectable or metastatic disease and doesn't show any symptoms	Can remain off therapy and follow-up with frequent exams and imaging studies, as long as the patient remains clinically stable and the tumor doesn't increase in size.
Unresectable or metastatic disease and has significant symptoms or shows progression	Surgical resection (if feasible). If not feasible, treat with tyrosine kinase inhibitors
Multiple recurrent tumors, and doesn't show any symptoms	Treat with tyrosine kinase inhibitors

be helpful, particularly where there is peritoneal disease. As there are no prognostic factors that have been validated for pediatric GIST and no specific staging system (as opposed to the adult form of GIST), it is critical that tumors be examined for mutations in KIT/PDGFR $\alpha$ , and due to the possibility of familial GIST, all patients who are diagnosed with a wildtype GIST (no known mutation) be referred to a genetic counselor.

## Management Recommendations

In the event a Pediatric GIST patient also has a KIT or PDGFR $\alpha$  mutation, treatment should be given in the same manner as when treating the adult form of GIST. However, when there is no KIT or PDGFR $\alpha$  mutation present, the

authors recommend different courses of action (See Table ).

As far as drugs to treat Pediatric GIST, the authors note that while Gleevec and Sutent are established first and second line treatments for the adult form of GIST, it is not clear what the first-line treatment for Pediatric GIST patients should be. There have been studies for both drugs with pediatric patients, but they were with small numbers of patients, and in some cases are ongoing. As no study has been done that compares the two drugs, it is unclear which one is optimal for first-line treatment.

While there is increasing understanding of the pediatric form of GIST, there is still much more to be learned, in terms of both the science behind the disease and recommendations for its treatment. For more information about Pediatric GIST visit [www.pediatricgist.gov](http://www.pediatricgist.gov).

## DC-area GISTers meet!



"The luncheon and meeting were very positive experiences. There was a great deal of interaction and enthusiasm. There were some serious moments but even more moments of shared laughter. The entire group encouraged each other to be positive knowing that the LRG is behind all of us and is dedicated to finding a cure for GIST. We plan to meet in the future and hope other MD/DC/VA members who weren't able to attend this meeting will come next time."

-Bonnie Emerson

## COURAGE

From Page 1

reported in the February 2003 edition of this newsletter in an article titled “True love, the ‘bomb squad baby’ and cancer.”

“I don’t know why I have cancer. I learned the mortality lesson a long time ago. I do know that if I dwell on the why, it will not change my diagnosis. I try to keep on living each day, enjoying my family and friends and appreciating all that life has to offer. That’s all any of us can do ...”

Other honorees at the gala included Dr. Kurt R. Weiss, M.D., Assistant Professor, Department of Orthopedic Surgery, and an Osteosarcoma survivor, from University of Pittsburgh Medical Center; Marc Ladanyi, M.D., The William Ruane Chair in Molecular Oncology, attending pathologist, Molecular Diagnostic Service at Memorial Sloan-Kettering Cancer Center, was honored with the *Nobility in Science Award*. Both doctors were members of an expert doctor panel, held the day before at the SFA’s Patient Educational Conference.

Dr. Weiss gave an inspiring and informative presentation on *Advances in Sarcoma Surgery*, speaking about advances in Osteosarcoma surgery and harsh realities faced in treating Osteosarcoma. He discussed the procedure to remove a sarcoma tumor and the process to replace bones and limbs with prosthesis. Dr. Weiss summarized that with the exception of GIST, progress in sarcoma has been poor. While sarcoma operations will get better and better, it is however a harsh reality to know that patient survival will not improve until novel biologically intelligent solutions are found!

Dr. Ladanyi gave an eye-opening presentation on *How Funding Basic Science Can Lead to New Targets and Treatments*. Dr. Ladanyi said that studying the genetic abnormalities of each cancer type generates advances in diagnosis and targeted therapies. As research becomes more cost effective, technological advances allow us to do more, get more data and work faster. New technol-

ogies will lead to more comprehensive studies. The funding available for this research must come from a combination of sources of federal grants and philanthropic/foundation support.

Other members of the panel included Crystal L. Mackall, MD, Chief, Pediatric Oncology Branch and Head Immunology Section of the National Cancer Institute, and George D. Demetri, MD, Director, Ludwig Center at Dana-Farber/Harvard and Sarcoma Center, Dana-Farber Cancer Institute.

Dr. Mackall presented on *Advances in Sarcoma Immunotherapy*, reviewing the role of Immunotherapy in cancer treatment. She educated the audience on the history of cancer treatment, particularly about Dr. James Ewing, father of radiation treatment, and Dr. William B. Coley, the father of cancer immunotherapy. While radiation and immunotherapy are different, it is possible that combining these two treatments can help as an effective treatment in some sarcomas. She cited that in the case of Ewing’s Sarcoma a dramatic response is shown if immunotherapy is administered in a certain window of time. She discussed the process of how a vaccine the NCI First Generation Consolidation Immunotherapy trial started, and how the process of the NCI Second Generation trial was done for High Risk Pediatric Solid Tumors.

Dr. Demetri, one of the pioneer trial investigators for Gleevec, gave an in-depth presentation regarding GIST and drug development for treatment. He spoke about how all cancers will be broken down and eventually will be moving to more and more specialized and custom treatments for individuals. He noted that the recent Regorafenib trial was able to be fast tracked for FDA approval. He discussed how drug development is inefficient and that mouse models aren’t replicating results in humans. He highlighted that the cost to produce one drug is one billion dollars and can take seven to 12 years to produce. While an effective treatment is available for GIST, better treatments are sorely needed.

As we celebrate and congratulate her on the honor given to Maura, it is amaz-



Maura poses for pictures with her family at the SFA’s tenth annual gala.

ing and eye opening to realize that within the branch of sarcoma, there are vast number of these rare cancers that do not have a magic bullet treatment like Gleevec. There is hope on the horizon, with improvements in surgery, research in genetic testing, new developments in immunotherapy and the drive to continue to seek faster methods and custom treatments for these cancers. We can hope that one day we will celebrate a 20-year survivorship and even more so to one day find a cure.

### Mark your calendars!

- The American Society of Clinical Oncology Conference (ASCO) is being held in Chicago

**June 1-4.**

- **June 10** is the LRG’s 10th Anniversary! See page 8 for ways to help us celebrate.

- The next NIH clinic is scheduled for **June 13-15** in Bethesda. Go to [www.pediatricgist.gov](http://www.pediatricgist.gov) for details.

- **June 18** kicks off Sarcoma Awareness week. How will you raise awareness for GIST?

**Life Fest 2012 in Las Vegas will be held November 9-11. More details to come!**



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