Battling gastrointestinal stromal tumor

LIFE RAFT GROUP

November 2007 | In memory of Bob Caraway, Russ Jones, Michele Scheiperpeter & Paty Roig | Vol. 8, No. 11

# Compassionate use process is not so compassionate

**By Judi Lifton, GIST caregiver**
**Interviewed by Erin Kristoff**

Judi Lifton had no idea what she was getting into when she tried to get compassionate use of AMN107 for her partner, Leland Emerson. He could not qualify for the trial as he had already taken Gleevec, Sutent and AMG706. Being ever the vigilant caregiver, Judi began the process which would end up consuming all of her time and possibly her sanity.

There are four required parts to obtaining AMN107 via "compassionate use": the doctor application, the clinical trial agreement (CTA), the Institutional Review Board (IRB) approval and the patient and doctor signature.

The actual story is a long and complicated one that would make your head spin. However, here are the highlights of what was almost a tragedy.

The first problem Judi encountered was going to a satellite location of a downtown cancer center. They were unable to handle such a task and put the

See AMN107, Page 11

# 'Viva Carolina' team exceeds $10,000 goal for Life Rafter

**By Erin Kristoff**
Newsletter Editor

On Sunday, October 14, the self-named "Viva Carolina" marathon team finally accomplished what they had been preparing for: the Long Beach Marathon and reaching their fundraising goal.

Paul Montuori thought he had an ambitious goal when he set it at $10,000 but he wanted to "shoot high and think big and actually make a difference." Well, Paul exceeded even his own expectations; over 98 percent of those asked for a contribution helped out. The group managed to raise an astounding

Runners pause before the big event.

$14,053 for the Life Raft Group in honor of member, Carolina Ponce-Williams!

Chad, Kira, Paul and Hillary Montuori would like to thank everyone who participated that day including: Phillip and Eriko Archunde; Randy and Wendy Horn; Carlos and Beatriz Fernandez; Jean Paul and Bob Mercado; Gerald

See MARATHON, Page 5

# Thanksgiving for LRG research

**By Norman Scherzer**
LRG, Executive Director

*A woman sits at her Thanksgiving Day table with tears of joy in her eyes. This year she was thankful for a piece of paper and three letters. The paper was the results of her last CT scan and the three little letters were NED. After fighting so hard for so long, she was classified as "no evidence of disease".*

*But these tears were bittersweet. She could not forget all of her friends who might not be so thankful, those friends who still had a long way to go and perhaps might never make it there. She also remembered that her three little letters were not a guarantee of more Thanksgivings, at least not yet.*

More research is needed if we want to bring patients like this their peace. It is not enough to be stable or NED for an undetermined length of time. **One patient** is not enough, every life is important. To do that, we need your help.

Prior to 2000 there was no viable treatment for GIST. In that year, clinical trials discovered that GIST patients responded to a new targeted oral drug called Gleevec (imatinib). Gleevec produces an excellent initial response in eighty-five percent of GIST patients with metastatic or unresectable disease. While this response is relatively long-lasting, half of all patients fail Gleevec within two years and almost all patients can be expected to eventually fail Gleevec. Half of the patients responding to Sutent, a second-line therapy, will progress within six months.

**What this means is that unless we can discover new therapies to more effectively prevent treatment resis-**

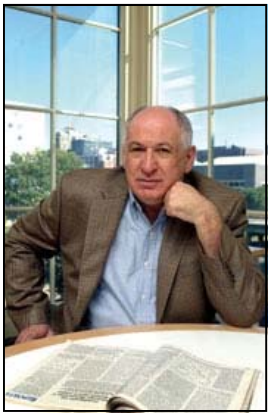
See RESEARCH, Page 6

# Looking for new ways to fight GIST

**By Jerry Call**
LRG Science Coordinator

In 1991, Joseph Schlessinger, PhD, and Axel Ullrich, PhD, co-founded a small bio-tech company. Taking the first letter of each of their names, they named the company Sugen. Sugen was considered to be one of the early pioneers in the signal transduction field.

Many Life Raft Group members will recognize the name Sugen. It is what GIST patients called the experimental drug SU11248 before it had a real name. SU11248 was a mouthful, so patients made up their own name, Sugen, named after the drug manufacturer.



**SCHLESSINGER**

Sugen was acquired by Pharmacia in 2003 and Pharmacia was later acquired by Pfizer. Pfizer completed development of SU11248 bringing the drug to trials for both Gleevec-resistant GIST and advanced kidney cancer. In early 2006 the drug became the first drug to be approved for two cancers at the same time. The drug is now known as Sutent; apparently in tribute to those early pioneers at Sugen.

On page six of this newsletter, we have published a Yale press release describing recent advances in understanding the mechanisms of KIT signaling. Joseph Schlessinger and his colleagues continue to try to find ways to improve the treatment of GIST.

The current generation of KIT inhibitors (drugs like Gleevec and Sutent) work on the inside of GIST cells by blocking the binding of ATP (needed for signaling to occur) in the kinase region of the receptor. This works very well as long as the receptor is not mutated so much that the drug is no longer able to reach the binding pocket in the kinase.

Primary (initial) mutations seldom prevent Gleevec (the drug used as front-line therapy) from reaching the drug/ATP binding pocket. Secondary mutations can develop over time (or they were initially present at low levels and become dominant over time under selective pressure) and change the shape of the receptor enough that Gleevec can no longer bind to the receptor. Sutent and some other drugs have activity against some of the secondary mutations that cause resistance to Gleevec but so far, no drug is known to have activity against all of the many secondary mutations that can occur. Newer drugs like HSP90 inhibitors are trying to overcome this limitation.

In their paper published in the journal, *Cell*, Dr. Schlessinger and his colleagues describe how two KIT receptors join together to become activated. This process, called dimerization, occurs outside of the cell. Since dimerization and binding of ATP are both required for KIT signaling to occur, both represent drug targets. Blocking ATP binding with Gleevec is a proven approach, but subject to derailment by secondary mutations. Blocking dimerization with a different type of drug that "can form wedge in the KIT molecule", as noted by Schlessinger, provides a potential approach that could conceivable bypass the secondary mutations that occur in the kinase domain. Since the dimerization area is at or near the exon 9 region of KIT, it will be interesting to see if Schlessinger's work translates into increased understanding of exon 9 mutations.

According to Schlessinger, "This work provides a roadmap for how to develop new drugs that will overcome the resistance to Gleevec and Sutent that develops in GIST patients and in other diseases driven by activated form of KIT. We are now starting to pursue this idea and raise the necessary funds for this to be accomplished. Although it will take time and quite a lot of funds I am very optimistic that this goal could be accomplished."

## The Life Raft Group

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

The Life Raft Group is an international, Internet-based, non-profit organization offering support through education and research to patients with a rare cancer called GIST (gastrointestinal stromal tumor). The Association of Cancer Online Resources provides the group with several listservs that permit members to communicate via secure e-mail. Many members are being successfully treated with an oral cancer drug Gleevec (Glivec outside the U.S.A.). This molecularly targeted therapy represents a new category of drugs known as signal transduction inhibitors and has been described by the scientific community as the medical model for the treatment of cancer. Several new drugs are now in clinical trials.

### How to join

GIST patients and their caregivers may apply for membership free of charge at the Life Raft Group's Web site, www.liferaftgroup.org or by contacting our office directly.

### Privacy

Privacy is of paramount concern, and we try to err on the side of privacy. We do not send information that might be considered private to anyone outside the group, including medical professionals. However, this newsletter serves as an outreach and is widely distributed. Hence, all articles are edited to maintain the anonymity of members unless they have granted publication of more information.

### How to help

Donations to The Life Raft Group, incorporated in New Jersey, U.S.A., as a 501(c)(3) nonprofit organization, are tax deductible in the United States.

Donations, payable to The Life Raft Group, should be mailed to:

The Life Raft Group
40 Galesi Dr., Suite 19
Wayne, NJ 07470

### Disclaimer

We are patients and caregivers, not doctors. Information shared is not a substitute for discussion with your doctor. As for the newsletter, every effort to achieve accuracy is made but we are human and errors occur. Please advise the newsletter editor of any errors.

# November 2007 clinical trial update

**By Jim Hughes**
LRG Science Team

**AMN107:** Phase III continues to enroll patients at a rapid rate, according to Novartis. Sites listed in the clinicaltrials.gov database are open with the following exceptions as of October 12: Lee Moffitt in Tampa, FL, MD Anderson in Houston, TX, and New York University, New York, NY. These sites were not yet open. Over 30 other sites are open outside the United States, in Canada, Europe, Australia and Taiwan.

**FR901228:** Phase I is suspended.

**Oblimersen (Genasense) + Imatinib:** Phase II is no longer accruing patients.

**BEZ235:** Phase I for Advanced Solid Tumors is open at the Nevada Cancer Institute in Las Vegas, NV. BEZ235 is a Novartis drug that targets the PI3K tyrosine kinase and indirectly inhibits the downstream targets AKT and mTOR.

**Imatinib + Pegylated Interferon-a 2B:** This Phase II trial for GIST is a whole different approach. It is primarily for patients who are newly diagnosed and who have not had Gleevec (or have been off Gleevec for six months or longer). Dr. Lei L. Chen is the primary investigator at the Huntsman Cancer Institute in Salt Lake City, Utah. Dr. Chen theorizes that Gleevec causes GIST cell death which in turn marks GIST cells for immune system response. Interferon will help stimulate that response and aid the immune system in identifying and destroying the remaining GIST stem cells that can cause relapse after Gleevec has shrunk tumors. This trial is planned to have several other innovative aspects that are explained in detail in the May 2007 Newsletter article "Immunotherapy trial strives to improve Gleevec response" by Jerry Call, also available on the LRG website.

**Note:** Dana-Farber— Travis Quigley, RN, Research Nurse, has indicated that both the Sorafenib (BAY 43-9006) Phase II and the Phase III Sunitinib or Imatinib trial are available there.

## AMN107 (nilotinib, Tasigna®)

*Efficacy and safety of AMN107 compared to current treatment options in GIST patients who failed imatinib and sunitinib*

Phase: III
Conditions: GIST
Strategy: Inihibit KIT
NCT#: NCT00471328
US Contact: Novartis
Telephone: 800-340-6843, Trial# CAM-N107A2201
Sites: **UCLA**, Los Angeles, CA
Myung Lee, 310-825-4494
**Wash. Cancer Inst.**, Wash. DC
Jake Paterson, 202-877-5371
**Univ. of Chicago**, Chicago, IL
Patient Coordinator:773-834-7424
**Dana Farber**, Boston, MA
Travis Quigley, RN, 617-632-5117
**Karmanos Cancer Institute.**, Detroit, MI
Anne Marie Ferris, 313-576-9373
**Wash. Univ**., St. Louis, MO
Nick Fisher, 314-354-5102
**Wake Forest**, Winston-Salem, NC
Scarlet Hutchins, RN, 336-713-6915
**Fox Chase**, Philadelphia, PA
1-800-FOX-CHASE

## Up to the minute...

In last month's newsletter we reported that 2 patients who were resistant to Gleevec and other drugs appeared to be responding to Nexavar. We have subsequently learned that the patient with symptomatic improvement, that was able to leave hospice, demonstrated progression upon further examination.

## Sorafenib (BAY 43-9006, Nexavar®)

*Sorafenib in treating patients with malignant GIST that progressed during or after treatment with imatinib and sunitinib*

Phase: II
Conditions: GIST
Strategy: Multiple Targets
NCT#: NCT00265798
US Contact: **Univ. Of Chicago Cancer Research Center**, Chicago, IL
Telephone: 773-834-7424
Sites: **City of Hope,** Duarte, CA
Warren Chow, MD, 866-434-4673 xt 64215
**Cancer Care Specialists,** Decatur, IL
James Wade III, MD, 217-876-6617
**Oncol./Hematology Assoc. of Cent. Il**, Peoria, IL
John Kugler, MD, 309-243-3605
**Dana Farber**, Boston, MA
Travis Quigley, RN: 617-632-5117
**Memorial Sloan-Kettering,** New York, NY
David D'Adamo, MD, 212-639-7573

## Imatinib + Pegylated Interferon-a 2B

*Combines targeted therapy with immunotherapy using Imatinib + Pegylated Interferon-a 2B in imatinib-naïve GIST patient*

Phase: II
Conditions: GIST
Strategy: Kill GIST cells
Study #: HCI 22172
US Contact: **Huntsman Cancer Institute**
Candace, 801-581-4477

## Sunitinib or Imatinib (Sutent®, Gleevec®)

*Safety and effectiveness of daily dosing with sunitinib or imatinib in GIST patients*

Phase: III
Conditions: GIST
Strategy: Multiple Targets
NCT#: NCT00372567
US Contact: Pfizer Clinical Trial Information
pfizercancertrials@emergingmed.com
Telephone: 877-369-9753
Sites: Contact Pfizer
**Dana-Farber**, Boston, MA
Travis Quigley, RN, 617-632-5117

## Perifosine+Gleevec (imatinib)

*Phase II study of Perifosine plus Gleevec for GIST patients*

Phase: II
Conditions: GIST
Strategy: Multiple Targets
NCT#: NCT00455559
US Contact: Online Collaborative Oncology Group
ocogtrials@ocog.net
Telephone: 415-946-2410
Sites: **Cancer Center at Century City,** Los Angeles, CA
Sant Chawla, MD
Coeur D'Alene, ID
**Oncology Specialists**, Park Ridge, IL
Kathy Tolzein, RN, 847-268-8200
Grand Rapids, MI
Sayre, PA
**MD Anderson**, Houston, TX
800-392-1611

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# Fourth annual Contactgroup GIST meeting held on September 29

**By Anja Long**
Contactgroep GIST

On September 29, the fourth annual Contactday for the Contactgroup GIST/Life Raft Group The Netherlands-/Belgium was held. This year the weather was not sunny and balmy like on previous occasions but that really made no difference at all compared to the warmth of the event. The group photograph still had to be taken indoors though (See above)!

About 100 patients, caregivers and people involved in the GIST world gathered in the small town of Lunteren, in the center of the Netherlands. The hotel and conference center was located in the woods, a very pretty setting but one which for some of us proved to be a bit of a challenge on the way out!

We started off by remembering those who have lost the struggle with GIST over the last 12 months; this time with particular reference to Peter van der Meer, a co-founding member of the group and public relations specialist of the current committee. Peter was *the face* of the Group for a lot of the members because he hosted all the previous contact meetings.

*"No one has to face GIST alone"*, this is the slogan for all the annual meetings and that was nicely reflected on that day. The German GIST group, Das Lebenshaus, was having their annual meeting the same weekend, which meant that about 300 GIST patients and caregivers were meeting in Europe at the same time. Group photographs and best wishes were exchanged on Saturday.

Three speakers made their presentations .The first speaker was Dr. Stefan Sleijfer, M.D., Ph.D., medical oncologist at the Erasmus University Medical Center, Daniel den Hoed Cancer Center in Rotterdam. Dr. Sleijfer is a recognized specialist in, amongst others, soft tissue sarcomas, including GIST. He gave an overview of the developments in GIST management, past, present and future. His presentation was lucid and informative, no mean feat when dealing with complex issues such as developments in mutational testing.

**SLEIJFER**

**TUMMERS**

After lunch, Roos-Marie Tummers, psychologist and advisor of psycho-social care with the IKO (Integral Cancer Center East), spoke to the group. There are nine such centers in the Netherlands and the purpose of these comprehensive cancer centers (CCCs) is to provide cancer patients and their families access to comprehensive and high-quality care as close to home as possible. The CCCs were set up to improve treatment, patient care and clinical research within the field of oncology in a partnership between health care professionals and cancer and palliative care institutions. CCCs are centers of knowledge and quality control that maintain an extensive network and fulfill a coordinating function within the field of oncology. Today, cancer is increasingly considered a chronic disease and survivors need appropriate support for their rehabilitation. The CCCs recognized this need and developed and implemented a health-oriented rehabilitation program "Herstel & Balans" (recovery & stability), including physical training and psycho-education over a three month period. Roos-Marie Tummers gave her presentation regarding this cancer rehabilitation program. The program is available at various centers in the Netherlands, though some patients are still having difficulty in receiving funding from their insurers, or even in getting a referral to the program by their medical specialists. This and other support programs were the mainstay of the presentation, which was started off by showing a film called, "Today a day, tomorrow a life" about the way in which several cancer patients had found a way to live their lives again after a cancer diagnosis. This was a premiere as the film had not been shown to outsiders before.

Afterwards, Carolien Verhoogt, on

See CONTACTDAY, Page 8

# GCRF 2007 'Walk for a Cure' is resounding success

**By Erin Kristoff**
Newsletter Editor

The air was chilly but the sun was shining when the GIST Cancer Research Fund (GCRF) held their annual "Walk for a Cure" on Sunday, October 14 at Rockland Lake State Park in Congers, New York. Once again, The GCRF has outdone itself. An announcement on New York radio station 95.5 WPLJ set the tone for a wonderful day for the GIST community.

In addition to the scores of GIST patients, caregivers and supporters, Paulo Costa, President and Chief Executive Officer (CEO) of Novartis Corporation attended the event, accepting an award for Humanitarian of the Year on behalf of the CEO of Novartis AG, Dr. Dan Vasella.

Dr. Marvel Scott from ABC's Channel 7 Eyewitness News was the Master of Ceremonies of the walk and did a spectacular job describing Tania and Robert Stutman's (Co-founders of GCRF) dedication and perseverance over the years.

Camera crews from Novartis and News Channel 12 were also there to film the proceedings.

Paulo Costa accepting an award on behalf of Novartis CEO, Dan Vasella with Tania Stutman, co-founder of GCRF and Dr. Marvel Scott of Eyewitness News looking on at the "Walk for a Cure" on October 14 .

Once again, basketball Hall-of-Famer, Walt "Clyde" Frazier, formerly of the NY Knicks, made an appearance signing autographs and answering questions from fans of all ages.

The research world also came out for this year's walk. Dr. Anette and Stefan Deunsing of the University of Pittsburgh Cancer Institute; Dr. Cristina Antonescu and Dr. Ephraim Casper of Memorial Sloan-Kettering Cancer Center; Dr. Margaret von Mehren of Fox Chase Cancer Center and researchers from various institutions, including Dr. Jonathan Fletcher's lab at Dana Farber Cancer Institute, were all in attendance.

Staff members from the Life Raft Group again came out to show their support for the GCRF and delighted in talking to members of the GIST community.

Those who could not make it this year were able to watch a live streaming video of the event for the first time right at their own computers.

Please visit the GCRF's site, gistinfo.org for more updated information on the "2007 Walk for a Cure".

## MARATHON

From Page 1

Loughran and Ivonne Rudnitsky; Vicky and Pety Ossio; Carolina and Terrance Williams; Telma Aquilar; Elizabeth Palenque and Luis, Gabriel, Samuel, and Elvira Levy.

Please see the article on how this all came about on page four of the LRG's August 2007 issue.

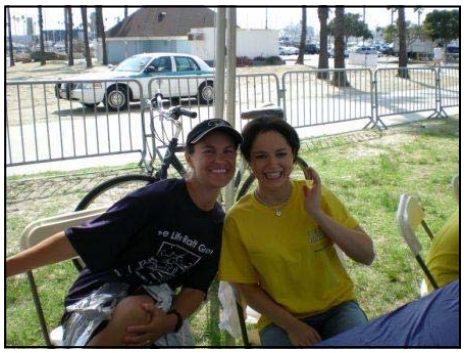
Here are just a few photos that express just how much fun the team had.


Eriko & Phillip express their joy for running.


Vicky & Paul sport LRG T-shirts to show their support.


Chad & Kira are pumped up!


Paul and Hillary, glad to be there


Kira & Carolina smile for the camera.

# New views of receptor molecule may open doors

*The following is excerpted from a Yale University press release entitled, "First Detailed View of Molecular Structure May Usher in New Class of Cancer Drugs".*

**New Haven, Conn., July 26, 2007** — High resolution views of a receptor molecule that is implicated in cancer offer a clear target for the development of a new class of cancer drugs, Yale School of Medicine researchers report July 27 in *Cell*.

It is also anticipated that the new family of drugs may be applied for the treatment of gastrointestinal stromal tumor (GIST) cancers that are resistant to Gleevec and Sutent. Although these drugs would target the same receptors as Gleevec and Sutent, they would do so by a different mechanism and may therefore be useful for patients who are resistant to these drugs.

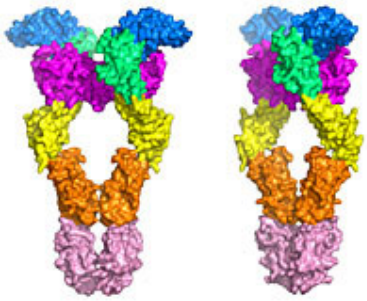
"I was surprised to see what the molecules did when they were activated," said Joseph Schlessinger, professor and chair of pharmacology and senior author of the study. "The arrangement is much simpler and more elegant than I thought."

The paper...provides the first detailed, atomic level view of the receptor tyrosine kinase (RTK) Kit before and after it is activated.

Schlessinger said the newly-revealed mechanism for RTK activation is utilized by many RTKs." Moreover, these structures offer unexpected new opportunities for drug discovery for the treatment of cancers and other diseases driven by activated RTKs," he said.

The finding is the result of many years of work involving protein expression, biochemical and biophysical studies and X-ray crystallography, a method used for viewing molecules at a resolution high enough to see atomic detail.

The receptor tyrosine kinase Kit and its natural ligand stem cell factor play an important role in normal blood cell production, gut function, pigmentation, and reproduction. Mutations which cause overactivation of Kit are found in a variety of human cancers, among them gastrointestinal stromal tumors (GIST) and several forms of leukemia.



Side view of Kit in complex with stem cell factor (in magenta). *Courtesy of Yale University*

The structural analysis clearly shows a conformational change as stem cell factor brings two Kit molecules together, resulting in a large rearrangement of and associations between small parts of the molecules in each pair. Schlessinger said the next step is to identify drugs that can form a wedge in the Kit molecule, making it impossible for them to join together and be activated.

## RESEARCH

From Page 1

**tance and to overcome such resistance if it were to occur, patients with GIST will continue to die.**

In 2006, the LRG initiated a unique research program to find the answers to GIST treatment resistance and to embark upon a pathway to find a cure for GIST that will serve as a model for other cancers.

Starting with the creation of a comprehensive five year strategic plan to identify the priority projects needed to overcome GIST treatment resistance, we brought together a core group of the world's best GIST researchers and introduced cooperation, coordination and accountability as key building blocks of this historic effort.

We created a grants structure designed to give maximum support to this research effort, including a 10 percent cap on the administrative overhead that could be charged by the researcher's institutions. What is notable about this is the fact that typical administrative overhead rates at cancer research institutions range from 55 percent to 75 percent. The LRG cap means that 90 percent of the funds we grant to our research institutions will actually be used for research.

The initial funding for this research program covered the first two years and will run out in a few months. Its ultimate success in finding a cure for GIST will depend upon our raising additional funds.

Thus far, our research team has reported substantial progress. We are approaching a critical intersection on the pathway to finding a cure for a cancer. We have the right scientific tools and the right researchers at the perfect time and place to demonstrate how to treat and cure this cancer. We have achieved a historic understanding of the fundamental genetics driving GIST and the know how to identify and overcome the re-

See RESEARCH, Page 10

## Canada LRG meets

Life Raft Group members gathered at David Josephy's house in Guelph, Ontario, Canada on Sunday afternoon, Oct. 14. Seven people attended, driving in from east and west. Dr. Jawaid Younus, of the London Regional Cancer Center in London, Ontario, gave an excellent presentation, providing a general introduction to GIST and its therapy, as well as summarizing results of recent international clinical trials. He also answered questions from the group. David displayed materials from the Germany Patient Summit in July. The group also discussed local challenges like drug coverage and benefits plans in Canada, treatment possibilities, and the need for additional organizational support for patients in Canada. Thanks to Jennifer Burton, Medical Liaison Manager-Ontario Region, of Novartis Oncology, for helping to make the gathering a success.

# Dana Farber holding GIST Patient Support Forum on November 12

Dana Farber Cancer Institute and Brigham and Women's Hospital will be holding a "GIST Patient Support Forum" on Monday, November 12 at Dana Farber. The event will take place from 3:00 to 5:00 P.M. at the Smith Building, Room 764.

This is a chance to meet others who share the same diagnosis and understand its challenges. The first hour will be a facilitated support group; the second, an opportunity for patients and families to talk to each other.

Group facilitator Sarah Murphy, LICSW and Travis Quigley, R.N. felt that the first forum was a great success

and "an incredible building block for future groups".

Light refreshments will be served. Parking will be validated in the Smith garage at Dana Farber. For more information, please contact Sara Murphy at (617) 632-6463.

# FDA approves Tasigna in CML

The Food and Drug Administration approved a new drug from Novartis to treat patients with chronic myeloid leukemia (CML), who are resistant or intolerant to Gleevec.

"Tasigna (also referred to as AMN107 or nilotinib) represents an important advance for the small number of patients who are resistant or intolerant to prior therapy," said David Epstein, president and chief executive of Novartis Oncology. "This approval means we can offer physicians a comprehensive treatment approach with effective medicines to treat their Ph+ CML patients."

NOVARTIS

# CMS changes proposed clinical trials policy

## Patient advocacy groups appeal to Senate, 'Stop CMS changes for cancer patient community'

*On October 17, the Centers for Medicaid and Medicare Services (CMS) had planned to issue a Decision memo for Clinical Trial Policy. The proposal represented a significant reversal of the standards for Medicare coverage of clinical trials that has been in effect since 2000. It posed a threat to the ability of Medicare beneficiaries to receive care in clinical trials.*

*Senators Cardin and Brownback circulated a letter, asking their Senate colleagues to join them in signing a letter to CMS objecting to this proposed policy.*

*The Life Raft Group joined other advocacy groups in opposing the policy. It reached out to senators and patients, asking for more voices in the struggle.*

*The following was sent by LegisLink Action Center to the Life Raft Group.*

After a comprehensive review of comments from the clinical research community, the Centers for Medicare and Medicaid Services (CMS) has announced that it will not move forward with a Proposed Clinical Research Policy that would have required trial sponsors to secure CMS approval that trials meet 13 technical and scientific standards as a prerequisite for Medicare coverage of the routine costs of trial participation.

In comments submitted to CMS in August by Dr. Atul Dhir and Dr. Nicholas Robert, the US Oncology Research Network expressed concern that the new proposed Clinical Research Policy (CRP) would have frustrated the intent of the agency's original policy on Medicare coverage of clinical trials issued in 2000. The Network believed that the Proposed CRP would have significantly reduced the number of trials that would qualify for coverage under the CRP and that finalizing the Proposed CRP as published would have had a significant negative effect on participation of Medicare beneficiaries in clinical trials and it reduced the amount of information available to physicians when they must make clinical decisions about patient care. The Network requested that CMS withdraw its proposal and maintain its current clinical trials policy until such time as a workable coverage policy could be developed with the benefit of public comment.


As CMS decided not to finalize its July proposed decision, clinical trials will continue to be covered under the previous policy, a Final Decision Memorandum released July 9, 2007. Details of the current Medicare Clinical Trial Policy are available online at www.cms.hhs.gov/mcd/viewtrackingsheet.asp?id=210.

While it is expected that CMS will reopen the Clinical Trials NCD (National Council on Disability) soon, US Oncology applauds CMS for recognizing the concerns expressed by leaders in the clinical research community and looks forward to working with CMS to improve Medicare beneficiary participation in cancer clinical trials.

# Peter Thomas Memorial Ride has raised over $22,000

On August 5, riders for the 2007 Peter Thomas Memorial Ride began a bike ride of 1000 miles in 10 days to find 1 cure for GIST. Five riders started at Bremerton, Washington for a trip to San Francisco, California.

The ride is in memory of Peter Thomas, who conceived the idea in 2006. Because of GIST, he was prevented from riding in the 2006 event but friends and family did, riding the length of England. Peter passed away in August 2006.

This year, all funds were raised to support research at Oregon Health and Science University.

As of September 30, the ride has raised over $22,000 for this research.

Dr. Michael Heinrich; riders Mike Prozan, Deanne Johnson, Erica Johnson and Andy Johnson and Dr. Chris Corless at the OHSU reception in August.

# CONTACTDAY

From Page 4

behalf of the Committee, presented an overview of the activitities and objectives of the Contactgroep GIST. One of the developments she described was the closer relationship and cooperation between the various European sarcoma groups via Conticanet as well as the involvement in the New Horizons Conference in Germany which resulted in the "Bad Nauheim Declaration".

Now that the group is a member of the Dutch Federation of Cancer Patient groups (NFK) and receives financial support from the Dutch Cancer Society (KWF), the group has a higher profile. In turn, it also means more work, as the group is being asked to take part in various platforms and discussion groups. In particular, the KWF is encouraging patient organizations to become more professional and to cooperate in various ways. At times, this puts a strain on our essentially small patient group.

Another highlight has been the founding of the Belgian GIST sub-group to encourage more Flemish-speaking patients and caregivers in Belgium to join the group. This is part of a strategy by the committee to raise the profile of the group amongst GIST patients and caregivers. The group is planning to take part in more large events such as the Annual Conference of Oncology Nurses (in November). The group has already begun such plans earlier in the year by taking part in the prestigious Cancer Genomics Plaza meeting in March, as well as the big annual cancer happening of the Flemish Cancer League (VLK) in Antwerp, Belgium.

Other major developments include the creation of a virtual office, an intranet which helps committee members as well as working groups such as the editorial board of the newsletter to cooperate through the exchange of documents and the holding of virtual discussions. Another project is the renewal of the website by using a management content system, which will enable, amongst others, the development of a members-only section, in which members can exchange photographs and personal information if they so wish. Carolien also referred to the work carried out by different working groups who liaise with the Committee, such as the organising committee of the Contactday, public relations and the editorial board of the newsletter. All these activities mean that new volunteers are badly needed, so she made a plea to the audience to come forward.

Carolien Verhoogt presents Ton de Keijser with the Life Raft Group's Humanitarian Award during the October "Contactday".

Last but not least, it was announced that Ton de Keijser, the inspirational chairman of the group, was stepping down on October 1, 2007. Ton has been with the group five years, first founding it and then turning it into what it is today. He felt that the time had come for someone else to take on his duties. Ton received a golden chairman's hammer, a digital photograph holder which showed the photos taken on previous occasions. And of course, a Humanitarian Award, given by the International Life Raft Group for services rendered.

A function description for a new chairman has been drawn up and the committee is now actively recruiting a new chairman.

The rest of the day was spent as a social get-together, with a mix of people who see each other once a year but know one another via the mail, new members and those that have become friends via the group. As ever, it proved to be a warm, social gathering, which towards the end always seems to have been too short, but leaves you with a content feeling of belonging to a like-minded group of people.

Next year will be the fifth anniversary meeting! This will be held on Saturday, September 27th, 2008. I, for one am already looking forward to it!

# TRIALS 2007

From Page 3

## Doxorubicin & Flavopiridol

*Doxorubicin and Flavopiridol in Treating Patients With Metastatic or Recurrent Sarcoma That Cannot Be Removed By Surgery*

Phase: I
Conditions: GIST/Sarcoma
Strategy: Inhibits Production of KIT
NCT#: NCT00098579
US Contact: **Memorial Sloan-Kettering**, NY, NY
David R. D'Adamo, MD, Phd
Telephone: 212-639-7573

## IPI-504

*Safety study for GIST or soft tissue sarcoma*

Phase: I
Conditions: GIST or Soft Tissue Sarcoma
Strategy: Destroy KIT
NCT#: NCT00276302
US Contact: **Premiere Oncology**, Scottsdale, AZ
Michael S. Gordon, MD
Telephone: 480-860-5000
**Premiere Onc.**, Santa Monica, CA
Courtney Carmichael, RN, 310-633-8400
**Dana-Farber**, Boston, MA
Travis Quigley, RN, 617-632-5117
**Univ. of Michigan**, Ann Arbor, MI
Rashmi Chugh, MD, 734-936-0453

## Editor's Note

In the September 2007 issue we printed a picture of the NYC GISTers meeting without identifying the people in the photo. Michael Ting, who organized the event, was kind enough to give us those names: Mark Landesman; Pamela Fedowich and husband, Hitesh; Myron & Ora Gelberg; Norman & Anita Scherzer; Anita Getler and sister, Julie; Craig Seebach; not pictured was Ken Schou.

Ting intends to make the meetings bi-yearly, with the next one hopefully occurring in February.

## Flu Shots

Cancer patients are considered high priority candidates for annual influenza immunizations and this certainly includes GIST patients. For those patients living in countries approaching winter (like the United States) this is the time to get your flu shot. In addition, you should talk to your doctor about getting pneumococcal vaccine.

## BEZ235

*A Phase I/II, multi-center, open-label study of BEZ235, administered orally on a continuous daily dosing schedule in adult patients with advanced solid malignancies including patients with advanced breast cancer*

Phase: I/II
Conditions: Advanced Solid Malignancies/Adv. Breast Cancer
Strategy: Target KIT Downstream Signaling
US Contact: **Nevada Cancer Inst.**, Las Vegas, NV
Donna Adkins, RN
Telephone: 702-822-5173

## Perifosine + Sunitinib

(Sutent®)

*Perifosine + Sunitinib Malate for Patients With Advanced Cancers*

Phase: I
Conditions: GIST/ Renal Cancer
Strategy: Multiple Targets
NCT#: NCT00399152
US Contact: Online Collaborative Oncology Group
Telephone: ocogtrials@ocog.net
US Sites: 415-946-2410
Huntsville, AL
**Tower Hem. & Onc.**, Beverly Hills, CA
Pomona, CA
Santa Monica, CA
**Onc. Specialists**, Park Ridge, IL
Kathy Tolzien, RN, 847-268-8200
Kalamazoo, MI

## CNF2024

*Study of oral CNF2024 in advanced solid*

Phase: I
Conditions: Tumors/Lymphoma
Strategy: Destroy KIT, (HSP90)
NCT#: NCT00345189
US Contact: Biogen Idec
oncologyclinicaltrials@biogenidec.com
Scottsdale, AZ
New Haven, CT
**Cancer Therapy and Res. Center,** San Antonio, TX
Pat O'Rourke, RN, 210-616-5976

## MP470

*In treating patients with unresectable or metastatic solid tumor or lymphoma*

Phase: I
Conditions: Solid Tumor/Lymphoma
Strategy: Multiple Targets
NCT#: NCT00504205
US Sites: **Virginia Piper Cancer Center**, Scottsdale, AZ
Raoul Tibes, MD, 480-323-1350
**S. Texas Accelerated Res. Therapeutics**, San Antonio, TX
Anthony Tolcher, MD, 210-593-5255

## KOS-1022

*Study of Oral KOS-1022in Patients With Advanced Solid Tumors*

Phase: I
Conditions: Advanced Solid Tumors
Strategy: Destroy KIT (HSP90)
Study #: COMIRB 05-0627
US Contact: **Univ. of Colorado Cancer Center**
Anschutz Cancer Pavilion, Aurora, CO
Sarah Eppers, sarah.eppers@uchsc.edu
Telephone: 720-848-0052

## LBH589

*A Phase IA, two-arm, multicenter, dose-escalating study of LBH589 administered by IV on two dose schedules in adult patients with advanced solid tumors and non-Hodgkin's lymphoma*

Phase: I
Conditions: Advanced Solid Tumors / Lymphoma
Strategy: Destroy KIT, Inhibit Cell Cycle, Induce Apoptosis
US Contact: **Nevada Cancer Institute**, Las Vegas, NV
Donna Adkins, RN
Telephone: 702-822-5173

## XL820

*Given orally to subjects with solid tumors*

Phase: I
Conditions: Cancer/Solid Tumors
Strategy: Multiple Targets
NCT#: NCT00350831
US Sites: **Cancer Inst. of New Jersey**, New Brunswick, NJ
Pamela Scott, 732-235-7459
**Cancer Therapy and Res. Center**, San Antonio, TX
Pat O'Rourke, 210-616-5976

## OSI-930

*Phase I dose escalation study of daily oral OSI-930 in patients with advanced solid tumors*

Phase: I
Conditions: Solid Tumors
Strategy: Multiple targets including inhibit KIT
NCT#: NCT00513851
US Contact: OSIP Medical Information
medical-information@osip.com
Telephone: 800.572.1932, x7821
US Sites: **Univ. of Colorado Cancer Center**, Aurora, CO
Mary Kay Schultz, 303-266-1740
**Dana-Farber**, Boston, MA
Travis Quigley, RN, 617-632-5117

# RESEARCH

From Page 6

maining downstream pathways of resistance. We need to keep our core research team in place and we need to raise the remaining three years of funding.

We expect this pathway to a cure for GIST to proceed in two phases. The first is to identify a combination of therapies that will convert GIST into a chronic disease for most patients. This would be analogous to what medicine has done for other chronic diseases such as hypertension and diabetes. The second and ultimate goal would be to discover a therapy, most likely also based upon a combination of drugs, that would eliminate the GIST cancer cells from the body altogether.

In the past, our members have faithfully raised money for the Life Raft Group's programs, this Thanksgiving campaign is dedicated to finding a cure for GIST. One hundred percent of donations will go directly to support our research.

## Donating Money to Cancer Research

| FREQUENTLY ASKED QUESTIONS | ABOUT THE LIFE RAFT GROUP |
|---|---|
| **Are you seeking a tax donation?** | |
| **Is the organization you are donating to recognized as a charity by the IRS (usually a 501(c)(3) designation)?** | The LRG is a 501(c)(3) organization. |
| **Do they provide a formal acknowledgment letter that will support a tax deduction?** | All contributions are acknowledged by a formal letter acceptable to the IRS. |
| **Is the organization you are thinking of donating to fiscally responsible?** | |
| **Does that organization account for its financial operations by making its tax returns available (form 990)?** | The LRG posts copies of its 990 on its website: www.liferaftgroup.org. |
| **Does that organization have an annual independent financial audit and does it make copies of that audit available?** | The LRG has an independent financial audit performed by an outside CPA firm on an annual basis and makes copies available to anyone who requests it. |
| **How much will actually go to research?** | |
| **Will a recognized cancer research center be the recipient of the donation?** | The LRG has awarded research grants to Stanford, Memorial Sloan Kettering, Brigham and Women's Hospital, Oregon Health & Science University, Cleveland Clinic, Catholic University in Belgium and the University of Seattle. |
| **How much does that center charge for administrative overhead (indirect costs)? Note that these can typically range from 55% to 75%.** | The LRG caps all indirect cost rates at 10%. When compared to a not-uncommon 75% rate this is what is needed to get $100,000 to a researcher:<br>A 75% indirect cost rate requires a donation of $400,000<br>The LRG rate requires a donation of $111,000. Thus every LRG research dollar is worth 3.6 typical research dollars. |
| **What accountability exists for the research?** | |
| **Is the research part of a strategic plan??** | All LRG research is part of a strategic plan consisting of clear priority projects and requiring the cooperation and collaboration of all participating researchers. A copy is posted to the LRG website. |
| **Is there a clear and objective financial report and a progress report?** | The LRG requires formal six month progress and budget reports. |

# NYC Poker Tournament has another great year

**By Gale Kenny**
LRG Staff

Amidst the stylishly appointed ambience of Slate/Plus, an exciting New York City venue, over a hundred players bought-in for a chance to win a $10,000 ticket to the World Series of Poker. The event, which was held on October 17, marked LRG's fourth annual Poker Tournament.

The evening began with friends greeting and shoulders squeezing sideways through the crowd. Some noshed on hot savories that floated through the crowd on silver trays and others lounged on the plush sofas that lined the perimeter of the room. The chatter and laughter subsided as the participants were seated and the games began.


Players gear up for a grueling night of poker.

The excitement and suspense grew as the tournament players were whittled down to the final table. The last three lucky players were Nicholas Chiara, a prior winner who took home third place this year, Matt Knopman, second place, and the winner, for the first time in LRG history, was female, Jan Hofstetter. Prizes for the second and third place are flat screen TVs. This event would not be possible if not for the dedicated work of board member, Jerry Cudzil, who organized another successful event.


Nick, Jan and Matt wear their "bling" in the winners circle.

Jerry's motivation is his father-in-law, Bill Roth, who was diagnosed with GIST in 2003.

We are grateful for all of those who attended, and to the Long Island Poker and Casino for providing their poker services. We will have to follow up on Jan after she returns from her exciting trip to the World Series of Poker in Las Vegas. See you all again next year!

## AMN107

From Page 1

burden on Judi. The downtown center was used to dealing with standard clinical trials. In this case two IRBs were needed (for both the downtown and satellite offices), once again the burden was on Judi.

The CTA is sent out to the hospital at the same time as the doctor applies but in this unfortunate case, the CTA fell through the cracks. No one can be sure how this happened, but it did. The downtown office should have been aware of the procedure.

When the CTA was finally addressed by the center properly, the satellite center refused to do it; Judi and Lee were forced to go to the downtown center. More roadblocks would soon emerge when the cancer center's lawyers began objecting unnecessarily to parts of the contract.

It is at this point that Judi pulls out all of the stops and writes a very frustrated email (below) to her personal contacts within the cancer center (who she had kept apprised of the situation), asking for their help.

*Dear Team,*

*Have continued to work on Lee's predicament in obtaining AMN 107.*

*Called Novartis late Friday for an explanation of why this is taking so long. Their answer was that the contract committee couldn't respond to the changes we made on the agreement. (They handle most changes.) So they called in their attorneys.*

*They still haven't resolved it.*

*What a quandary....*

*My mind can only recall 3 other members in the cancer group who have died waiting for this drug because of the red tape with their institutions. The one person who received the drug quickly (in a few weeks) was someone from a small town. Novartis sent a rep out. Everybody immediately signed everything. Obviously they were naive. Obviously, we are not.*

*I was hoping there wouldn't be a stalemate.*

*At some point I would like to be on a committee that develops a system for emergency expeditiousness. Apparently there is not one in place. It saddens me.*

*It has been almost two months. It is one patient who will sign documents saying the hospital is not liable. It is not 50 patients in a trial.*

*Now there is money involved...how much?*

*Could we plan future fundraising to expedite this? Call the news media and our families to raise money? Have a marathon?*

*There must be patrons that have donated money for patient advocacy. One of Lee's previous employers has grants for things like this but it will take time: we don't have it.*

*Can this be solved in this week?*

*I leave it to you.*

*My hands are tied...*

*Judi*

Thankfully, Judi's contacts breathed down the necks of those stalling the process and Lee was finally able to get AMN107 in October, after a long and grueling struggle.

"You need an advocate, a contact at the hospital and a contact with Novartis that's willing to work with you." Judi says, "I would not have been able to do it without the help of Novartis."

## Mark your calendars!

**Final reminder:** Floridians meet on November 10. Dr. Gina D'Amato from Moffitt Cancer Center will be a guest. Skip Ryan is coordinating the meeting & can be reached at skipryan@tampabay.rr.com

The Connective Tissue Oncology Society (CTOS) meeting is being held from November 1 through November 4 in Seattle, Wash.

# The Life Raft Group

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