Details inside: Life Raft Group's general meeting, April 30-May 2, Orlando, Fla., USA

Battling gastrointestinal stromal tumor

LIFE RAFT GROUP

February 2004 In memory of Donald Kew **Vol. 5, No. 2**

Gleevec vs. GIST trial at 3 years

About half of patients have left the trial due to growth, side effects

Gleevec works well for most GIST patients, either stopping the cancer in its tracks or shrinking tumors to a faction of their original side.

But how long will it work?

That's the question researchers intend to answer, and some preliminary indications were announced at the Gastrointestinal Cancers Symposium held Jan. 22-24 in San Francisco.

Dr. Charles Blanke, GIST expert at Oregon Health & Sciences University in Portland, spoke at the first day of the symposium. One of three researchers in the United States to lead the phase II clinical trial of Gleevec (imatinib mesylate) for GIST (gastrointestinal stromal tumor), Blanke related how trial patients are doing after three years on Gleevec.

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Deep in the heart of Texas

Thirty GIST patients and caregivers from the Life Raft Group gathered Saturday, Feb. 7 at Gilda's Club North Dallas for the first-ever Texas area meeting. Gathering coordinator Kerry Hammett (the blonde in black, on the right) is seen talking to the group. See story, more photos on Page 3

Test to predict Gleevec response expands

Testing includes virtually all possible mutations that drive GIST growth

PORTLAND, Ore. — As reported in the June 2003 issue of the Life Raft Group newsletter, an international team of researchers has developed testing that can forecast how well gastrointestinal stromal tumor patients will respond to Gleevec (imatinib mesylate).

Now the tests have been expanded to more accurately predict how GIST patients will fare on Gleevec (Glivec outside the U.S.A.)

"These results demonstrate that the most important predictor of tumor shrinkage during Gleevec therapy is not age or tumor size, but rather the specific type of mutation causing the tumor," said Dr. Michael Heinrich of the Oregon Health & Sciences University (OHSU), co-principal investigator of the study.

"The majority of GI stromal tumors have a mutant form of KIT that acts

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TESTING

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like a gas pedal stuck to the floor, providing a constant stimulus for GIST cells to grow," Heinrich said.

Among GIST tumors that lack a KIT mutation, some (5 to 7 percent) had a mutation in a different (but closely related) tyrosine kinase called PDGFRA. (platelet-derived growth factor receptor alpha). Gleevec is effective against some of these PDGFRA mutations.

Approximately 7 to 12 percent of GISTs have no detectable KIT or PDGFRA gene mutation. GISTs with no KIT or PDGFRA mutations are called "wild-type" (for KIT/PDGFRA). Found in about 7 percent of patients, it isn't clear what supports the growth of this subset of GISTs.

The information in genes is divided into different sections called exons and introns. Exons contain coding information and introns do not. Mutations in different exons in the gene cause changes in shape in different parts of the receptor. Mutations are known to occur in the c-KIT gene in exon 11, exon 9, exon 13, and exon 17. Mutations in the PDGFRA gene are known to occur in exon 12, and exon 18.

Clinical testing for mutations in exon 11 and exon 9 of the c-KIT gene has been available at OHSU for about a year. This mutation testing service has recently been expanded so that it adds exons 13 and 17 in KIT and exons 12 and 18 in PDGFRA.

"In effect, this expanded testing closes the loop on mutation testing," says Jerry Call, science coordinator for the Life Raft Group. "Before, they could say you had an exon 11 mutation (about 67 percent of patients) or exon 9 (about 18 percent), but if both of these were negative, you were left without knowing if you had wild-type or one of the other mutations. The expanded testing now accounts for the other 15 percent of people whose mutation was previously unknown."

"If they find that you don't have any of the four types of KIT mutation or the two types of PDGFRA mutations," added Call, "then you know (with less than a 1 percent chance for error) that you are 'wild-type' for KIT and PDGFRA."

Regardless of the test results, however, all patients eligible for treatment should undergo a therapeutic trial or treatment with the drug. Even among patients with tumors lacking a detectable KIT or PDGFRA gene mutation, the response to Gleevec is higher than the response to traditional chemotherapy (less than 5 percent).

"Mutational testing can be helpful in confirming the diagnosis of GIST and in defining the prognosis for patients who need Gleevec therapy," said Dr. Christopher Corless, associate professor of pathology in the OHSU School of Medicine and co-investigator in the GIST research.

"As Gleevec is used more and more in combination with surgery, we believe that testing for mutations in GIST will be important in deciding whether Gleevec therapy should be used before and/or after surgery for GIST," added Heinrich.

Follow-Up For Common GIST Subgroups	Frequency in Phase II Trial	Gleevec Partial Response	Gleevec Stable Disease	Gleevec Progressive Disease
KIT Exon 11 mutation (n=82)	67%	86.5%	8.5%	4.9%
KIT Exon 9 insertion (n=21)	18%	52.4%	28.6%	19%
No *KIT* or *PDGFRA* mutation (n=8)	7%	0%	37.5%	62.5%
Follow-Up For GISTs With Rare Mutations				
PDGFRA D842V (n=2)	2.3%	-	-	2 of 2 pts
PDGFRA other (n=3)	2.3%	2 of 3 pts	-	1 of 3 pts
KIT Exon 13 (n=2)	1.6%	2 of 2 pts	-	-
KIT Exon 17 (n=2)	1.6%	1 of 2 pts	-	1 of 2 pts

Quote:

"In effect, this expanded testing closes the loop on mutation testing. ... expanded testing now accounts for the other 15% people whose mutation was previously unknown."

— Jerry Call, Life Raft Science Coordinator

How to Order the Test

Requests for KIT and PDGFRA mutation screening must originate from a pathologist or treating physician. One paraffin block of the tumor (either biopsy or surgical specimen) or 15 unstained sections of the tumor should be sent to address listed below. A copy of the original pathology report as well as the patient's insurance information must be included.

Dr. Christopher Corless
OHSU Dept. of Pathology (mailcode L471)
3181 SW Sam Jackson Park Rd
Portland, OR 97239
Tel. 503-494-6776
Email: corlessc@ohsu.edu

Texas area gathering draws 30 Life Rafters

Thirty GISTers and caregivers gathered Saturday, Feb. 7 at Gilda's Club North Dallas for the firstever Texas area meeting and lunch. The event created a positive opportunity to share how the diagnosis and treatment of GIST impacts has our lives.

Attendees traveled from Oklahoma, Louisiana, and all parts of Texas, reaching out to one another to share their strengths, knowledge and questions. A gracious and welcoming atmosphere, Gilda's Club of North Texas is a new $4.5 million facility dedicated to creating a community of support for all who are affected by cancer.

The meeting included of an overview of the history of the Life Raft Group, well presented by our own John Poss, chief financial officer of the LRG. Each participant told his/her story of diagnosis, journey through treatment and current status, inspiring many personal connections.

Plans to keep in touch with one another and to hold future quarterly meetings were made. The next meeting will be Saturday, June 12, at Gilda's Club North Texas. All area GISTers are invited to come together and spend the weekend as a block of affordable hotel rooms are being reserved just for our group. The meeting will feature separate sessions for patients and caregivers facilitated by small group leaders who will also offer a coping skills counseling model.

To make a reservation for June 12 or for more information, contact Kerry Hammett at yaloo@gvtc.com or (830) 935-3420.

Patients and caregivers, above, pose for a group portrait at Giilda's Club in North Dallas, Texas. At left, Life Rafters at the meeting get to know each other by sharing their individual stories of how they were diagnosed, the treatments they've undergone and current status. The group is already putting together another meeting at Gilda's Club, this one set for June 12.

THREE YEARS

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The phase II trial involved 147 patients who were randomly given either 400 mg. or 600 mg of Gleevec a day. Seventy-three patients ended up taking 400 mg., while 74 received 600 mg.

How well did Gleevec work?

For most, it works very well — and quickly. Blanke said the median time to measurable response was 13 weeks, regardless of whether patients were given 400 mg. or 600 mg.

Only one patient (600 mg.) had "complete response," that is, the disease can no longer be detected.

Some 67 percent of patients had "partial response," which means their tumors shrank in size. The dose made no difference, with 49 patients on 400 mg. and 49 patients on 600 mg. falling into this category.

Eleven patients on 400 mg. had stable disease, as did 13 patients on 600 mg., bringing the overall rate of patients for whom Gleevec worked to 84 percent.

BLANKE

For 11 patients on 400 mg., their tumors continued to grow. Just six patients on 600 mg. had disease progression. Six patients total could not be evaluated.

And how long does Gleevec work?

That hasn't been definitively answered, Blanke said. The good news is that the "median survival" time for GIST patients on Gleevec hasn't been reached. The bad news is that Gleevec has stopped working in slightly more than half of GIST patients.

At a median time of 34 months on Gleevec (the range was 21 to 43 months), Blanke said nearly half of the phase II patients are still being treated.

In the 400 mg. group, 30 of 73 patients continue on the trial. Forty-three are no longer on the trial, either because their tumors grew (30), they had a change of heart and dropped out (4), or side effects were too severe (3).

In the 600 mg. group, 37 of the 74 patients are still being treated. Thirty-seven are no longer on the trial, either because their tumors grew (23), they had a change of heart and dropped out (4), or side effects were too severe (4).

Sadly, "29 percent of the overall study population have died," he said, with essentially the same number of deaths from the 400 mg. and 600 mg. groups. The median time to treatment failure, Blanke said, is 84 weeks.

"Survival did appear strongly correlated with response," Blanke said. "Survival rates at 83 weeks were 95

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In Memoriam

There have been 30 deaths in the Life Raft Group to date:

Debbie Nance, 38, Oct. 2, 2000, wife to Eddie, mother of Chris.

Jim Ackerman, 49, Jan. 16, 2001, husband to Betsye, father of Jill and Tom.

Jim Perham, 63, May 2001, husband to Karen, father of Craig, Kathy, Jennifer.

Amy Barney, 25, June 10, 2001, wife to Reed, mother of Joshua.

Jeff Prichard, 52, July 11, 2001, husband to Joyce, father of Gregory and Scott.

Ron Martinez, 60, July 25, 2001, husband to Jo Ann, father of Ron, Wendy, Natalie.

Ehud Nehemya, Aug. 7, 2001, father to Einat Zelinger, father-in-law of Ophir Zelinger, Hadar Nir.

Bruce Gunn, 43, Nov. 8, 2001, husband to Roisin, father of Seamus, Liam, Brendan and Aislinn.

Robert Carr, Dec. 30, 2001, father of Robert, Steven, Scott and Melissa.

Jonathan Montague, 23, Jan. 19, 2002, son of Ray and Sheila Montague, brother to Jamie, Adam, Meghan.

Robert Lecca, 49, Jan. 28, 2002, husband to Diane.

Jacob Winfield Waller III, 67, March 31, 2002, husband to Jerry, father to Rita, Richard.

Mary Golnik, 50, April 18, 2002, wife to Gary, mother to Timothy.

Ana Maria Baldor-Bunn, 30, April 19, 2002, wife to Stan, mother to William.

Stewart "George" Wolf, 51, April 19, 2002, husband to Maggy, father to Thomas.

Michael Cornwell, April 19, 2002, husband to Cathy.

Jerry Pat Rylant, 61, May 5, 2002, husband to Pamela, father of four, grandfather to 10.

Jill B. Meyer, 53, June 9, 2002, mother of Aliza.

Todd Hendrickson, 44, June 29, 2002, husband to Janet, father to Max, Tyler and T.J.

Chet Duszak, 79, Oct. 5, 2002, husband to Kay, father to Lori.

Nora Shaulis, 42, Nov. 4, 2002, wife to David, mother to Griffin.

Howard Delapenha, 41, Dec. 14, 2002, husband to Sandra, father to Joshua and Hannah.

Kathy Colwell, 45, Jan. 5, 2003, wife to Tom, mother of Katherine, Mary and Tom.

Cynthia G. Whitson, 64, Jan. 19, 2003, wife to Jerry, mother to Steve, Jill, Randy and Donna.

Abdul Hai, 76, Feb. 26, 2003.

William Lawson, 56, July 3, 2003, husband to Gwen, father of Cory, Jennifer and Rhonda.

Laura Blanchette, 47, Aug. 4, 2003; wife of Mitch, mother of Sarah and Curtis.

Maryann Klein, 56, Sept. 4, 2003; wife of Gary, mother of Michelle, grandmother to Brandon.

Pat Ford, 48, Sept. 6, 2003; wife to Brad, mother to David and Laura.

Frank Weigand, 66, Oct. 24, 2003; husband to Ruth, father to Susan and Drew.

Tuomas Hemminki, 41, Jan. 2, 2004; husband to Leena, father to Heidi and Ilkka.

Donald Kew, 43, Jan. 31, 2004; husband to Jocelyn, father to Erin and Christine.

Artist, musician, filmmaker Donald Kew was 43

Donald George Kew of Okotoks, Alberta, Canada, died Jan. 31, 2004, at Carewest Sarcee Hospice in Calgary. He was 43.

Donald was born in Weyburn, Saskatchewan, but spent his formative years in Vauxhall, Alberta. After graduating from Vauxhall High in 1978, he moved to Edmonton to attend Grant MacEwan College and get his diploma in commercial art.

Donald worked in the Alberta oilfield until 1985 before deciding to dedicate himself to his passion creating political and editorial cartoons. Donald contributed as a freelance editorial cartoonist to the Edmonton Sun for 18 years, and self-syndicated his work throughout Alberta. Hundreds of Donald's cartoons are part of the Archives of Canada and Alberta provincial historical collections.

He was also the founding publisher of the Nisku Trader, now known as The Wildcatter News and currently managed by his wife.

Once settled in Okotoks and inspired by a trip to Paris, France, Donald found that he had other artistic avenues yet to explore and started painting and writing songs. His CD, "Lost and Found in Okotoks," was released in December 2002. He also created several paintings for his first art show at the Picture Hook Gallery in Okotoks.

Donald Kew is shown at the Canmore Folk Music Festival where he gave a stunning main stage performance last August.

Intrigued by the independent film industry, he worked as a background actor before he took on his first assignment as chief cameraman for a local production. He was so inspired by the experience he co-wrote a script, filmed and edited a short — "East Meets West in 2003.

His last major accomplishment was a stunning main stage performance on Aug. 3, 2003 at the Canmore Folk Music Festival.

Services were held Feb. 5 in Okotoks.

He is survived by his wife of 19 years, Jocelyn; daughters, Erin, 15, and Christine, 12; his mother, Joan Kew of Vauxhall; sisters, Christine Wing of Edmonton, Joanne Helmer of Yellowgrass; and his brother James Kew of Sherwood Park; brothers-in-law Ron Wing and Ken Miller; sisters-in-law Connie Kew, Joan Miller and Judy White; and mother-in-law Shirley Elliot; Uncle Donald Mclean; cousin Margaret McCart and husband from North Queensferry, Scotland; nieces and nephews, Rod Wing, Larissa Wing, Mason Wing, Angela and Jeff Jones, Mclean Kew, Scott Kew, Jennifer Kew, Cole Miller, Logan Miller.

Roy, Margolis team to improve LRG Web site

The Life Raft Group recently unveiled a new face to the world, via a complete Web site design.

The Life Raft Group Web site was first created as a labor of love by board member Gary Golnik and dedicated in perpetuity in honor of his beloved wife, Mary, by the LRG Board.

After many months of hard work, Gary decided to move on and stepped down as webmaster.

Stepping in to fill his shoes is a new team. Jim Roy, LRG information technology director, will serve as webmaster and Tami Margolis, LRG member, as Web site designer. Tami has creatively redesigned the Web site and will continue to serve as a key Web team member.

The Web site features the customary links to an explanation of what GIST is, treatments, methods of coping, news, FAQs that answer common questions (in English and German), and links to a host of groups based in the United States, United Kingdom, the Netherlands and Germany.

The Web site has its own search function that includes the index of newsletter articles.

Few of our issues are theoretical

GIST patients are living longer and doing better

By Norman Scherzer
Executive director, Life Raft Group

No member of the Life Raft Group can detach themselves for long from the terror and exaltation that accompanies each battle for survival. Momentary periods of escape into the fantasy of denial or the reality of a recent good checkup are interrupted by the loss of innocence that each report of relapse or death brings.

We have few theoretical issues. Within days of debating the issue of a placebo in the SU11248 clinical trial, one of our key members found himself part of the very trial he has been addressing — and then part of the very placebo group he'd been so concerned about.


SCHERZER

Each month we share our triumphs and mourn our losses. Some turn to prayer. Some turn to science. A few turn to anger and lash out indiscriminately. Most turn to one another for the unique support that only those who walk in our shoes can provide.

Such is the ultimate difference between those who produce the drugs and do the research that is desperately needed, and those who live each day shadowed by death.

We are so teasingly close to the rescue we seek. Extraordinary breakthroughs in laboratory testing have peeled away many layers of genetic mystery. Equally extraordinary breakthroughs in developing targeted drugs like Gleevec have replaced hospital and hospice with the fruits of daily living. New targeted drugs lie just over the horizon.

GIST patients are living longer and doing better!

Membership meeting

And so for the second time in our history we will convene a general membership meeting, this time in Orlando on the first weekend of May, to share the unique bond of those who live with a rare and deadly disease.

Hundreds of GIST patients and family are expected to come together for a weekend of support and information. Good friends who've never met except through the Internet will hear speakers offer cutting-edge medical and scientific information. There will be breakout sessions for patients and for care-

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percent for patients achieving partial response, 92 percent for patients achieving stable disease, and only 24 percent for patients achieving a best response of progressive disease."

Also, response and survival correspond greatly to the particular mutation driving the cancer.

Most GISTs (93 percent) are caused by cell mutations of KIT or PDGFRA tyrosine kinase proteins. These mutant forms of KIT act like a gas pedal stuck to the floor, providing a constant stimulus for GIST cells to grow.

Blanke reported that 84 percent of patients with an exon 11 mutation in KIT achieved partial response; while 8 percent had stable disease and just 5 percent had tumor growth.

Just 48 percent of patients with an exon 9 KIT mutation had partial response, while 26 percent had stable disease, and 17 percent experienced disease progression.

Patients without any detectable mutation in KIT or PDGFR never responded to Gleevec, but a third of patients treated with Gleevec did have prolonged, stable disease.

Because of this, "even those with no detectable mutation in KIT or PDGFRA should be offered imatinib, as a significant fraction will achieve prolonged, stable disease," Blanke said.

So what's a patient to do if his or her tumor(s) grow while on Gleevec? Life Rafter Martha Zielinski was at the symposium, and says Blanke was clear that patients should not stop taking Gleevec, since it's likely slowing the disease or still working on some tumors. Second, patients on 400 mg or 600 mg. should increase their dose to 800 mg. If that doesn't stop the tumors, patients should look into the SU11248 clinical trial.

In summary:

- There are about 5,000 cases of GIST in the U.S. each year.
- GISTs rarely respond to standard chemotherapy.
- 93 percent of GISTs are activated by mutations of KIT or PDGFRA.
- "Imatinib appears to be effective long-term therapy for advanced GIST."
- Survival strongly correlates with response or achievement of stable disease.
- Drug efficacy is related to kinase genotype.

Loyola utilizes team approach to GIST

This is the third article on "centers of excellence" for GIST treatment in the Chicago area, profiling Loyola Medical Center. It comes from Dr. Margo Shoup via the Chicago chapter of the Life Raft Group. Prior newsletter articles profiled the University of Chicago (October 2003) and Evanston Northwestern Healthcare (January 2004).

Loyola University Medical Center and the Cardinal Bernardin Cancer Center group of physicians have treated approximately 35 GIST patients over the past three years. Many of these patients had their primary surgery done elsewhere and were referred to the Cardinal Bernardin Cancer Center for further care and recommendations.

Patients with GIST are seen in the Multidisciplinary Gastrointestinal Oncology Center. This center has a unique approach to treating patients with GI malignancies. Patients have the opportunity to meet with a surgical oncologist, medical oncologists and a radiation oncologist all in one visit. This team approach includes discussion regarding individual patient care.

Physicians treating GIST include the center's directors, Dr. Margo Shoup and Dr. Kenneth Micetich, as well as Dr. Alex Hantel and Dr. Ketty Badrinath. Shoup is a surgical oncologist and is chief of GI surgical oncology at Loyola University, and Drs. Micetich, Hantel and Badrinath are medical oncologists with primary interests in GI oncology.

SHOUP

Through this center and the university, with Shoup as principal investigator, two trials for Gleevec have been opened; a Phase II trial for high-risk patients (ACOSOG Z9000, now closed), and a Phase III trial of Gleevec vs. placebo for intermediate risk patients (ACOSOG Z9001) which is actively accruing patients.

Many patients with metastatic disease, including those who have been treated to maximal response with Gleevec, are evaluated in the GI Oncology Center. The team's approach to such patients is individualized, based on response to therapy, progression of disease and other variables. The GIST team at Loyola has had an aggressive approach to patients who begin to progress on Gleevec. Those who may be completely resected are considered for an aggressive surgical approach.

Shoup, who trained in surgical oncology at Memorial Sloan-Kettering in New York City, has rendered patients disease-free after treatment with Gleevec followed by surgery. The surgery often includes liver resection and/or radiofrequency ablation. RFA is a tool to burn the tumors in the liver. It is preferred when treating deep-liver metastasis that would otherwise require removing a large portion of liver. Preliminary data from this approach is encouraging, although long-term follow-up is needed.

Many patients are not candidates for complete surgical resection due to the extent of disease. Those patients may be referred to the Sugen trial and, if severely symptomatic, may be candidates for debulking prior to referral.

Appointments for the GI Oncology Center may be made by calling the CAN-HELP line at the Cardinal Bernardin Cancer Center, (708) 226-4357. The hospital is located on the west side of the Chicago metropolitan area in Maywood.

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givers, for those seeking basic and advanced information. Forums will honor those who help us in our struggle. As we go to press, we've learned that we will be joined by a colleague from a sister organization in Germany, Das Lebenshaus.

Meeting details

All GIST patients, family and friends are welcome! The meeting will be held Friday, April 30 through Sunday, May 2 at the Embassy Suites, Lake Buena Vista in Orlando, Florida. Plan to join us for a welcoming reception Friday at 5:30 p.m., followed by dinner at 7:30 p.m. The general meeting and breakout sessions will run from 9 a.m. to 5 p.m. Saturday and again Sunday morning. The conference will end by 1 p.m. Sunday.

To make hotel reservations, please call (800) 257-8483 from 9 a.m. to 5 p. m. EST. Ask for the Life Raft Group rate of $129 (+ tax) per night. This rate is also available for three days prior and three days after the meeting. Also, please e-mail Tricia McAleer at tmcaleer@liferaftgroup.org to let her know you are coming. Include the names of all who are attending and indicate if you have any special needs.

Plan to pay a conference fee of $90 per person when you arrive. This fee will cover cocktail reception, hors d'oeuvres and dinner Friday night; cooked-to-order breakfasts (provided

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gratis by hotel) both Saturday and Sunday, lunch on Saturday, a cocktail hour from 5:30 to 7:30 p.m. (provided gratis by hotel), Saturday and beverage breaks at all meetings. Bring your conference fee, in the form of cash or check, to the registration desk.

Please make your hotel reservations and confirm attendance no later than March 20.

Outreach to Hungary

Discussions have begun with another sister organization, Gyogulas az STI-vel (Recovering with STI) in Hungary, to develop collaborative ties. As is the case with other organizations around the world, the Life Raft Group will provide information and other support to our new friends in Hungary.

Medicare reform struggles on

Most U.S. patients know that Congress added prescription drug coverage to Medicare, to take effect at the onset of 2006. That legislation will help Medicare cancer patients pay for oral cancer drugs (like Gleevec) and for self-injectable drugs (like Procrit). Although this is a great step forward, patients will still have to pay significant out-of-pocket costs for high-priced drugs because of gaps in the coverage.

Behind the scenes, the battle to refine this legislation goes on.

In the rush to put the prescription drug legislation in place, Congress added a provision to address the cost of oral cancer drugs and self-injectable drugs in the interim two-year period before the legislation takes effect. But, as is often the case, Congress limited the cost by limiting the number of participants and the total amount of money that could be spent.

In addition, they created a demonstration project to further help cap the cost of these drugs. First, Congress put in place a provision that would have limited oral cancer drug coverage to about five states. Then they replaced that with a provision that would limit coverage of such oral cancer drugs to those that replaced prior I.V. drugs for that same cancer. So far so good, but as the government agency administering this provision, (the Centers for Medicare & Medicaid Services (CMS) attempts to understand and implement the Congressional mandate, we have learned that GIST patients are in danger of not being covered due to a misinterpretation that there was no prior treatments for this disease.

The Life Raft Group testified before the CMS that the oral drug Gleevec replaces two front-line chemotherapy drugs, Adriamycin (doxorubicin) and Ifosfamide, both of which were traditionally administered in an oncologist's office for soft tissue sarcomas, including GIST. These traditional chemotherapy drugs were effective less than 1 percent of the time. Gleevec in comparison has been effective about 85 percent of the time.

It is very important to note that GIST was more of a descriptive term ("gastrointestinal stromal tumor") than a functional diagnosis prior to the application of Gleevec to GIST, and the almost simultaneous introduction of a diagnostic test (c-kit), a little over three years ago. GIST patients were almost universally diagnosed with some other soft tissue sarcoma, including leiomyoma, leiomyosarcoma, and leiomyoblastoma. Thus it is not appropriate to compare Gleevec for GIST today with Adriamycin or Ifosfomide for GIST yesterday. Instead, thoughtful scientists understand that they need to substitute GIST for the alternative diagnoses that were used only a few years ago (leiomyoma, leiomyosarcoma, and leiomyoblastoma).

GIST is a perfect paradigm for this demonstration: Gleevec elegantly and effectively targets GIST cancer cells and replaces broad brushed and relatively ineffective traditional chemotherapy drugs.

The reality of this "demonstration project" is in fact a sham to artificially limit costs. As the end of the demonstration project is predetermined to be the implementation of the comprehensive prescription drug legislation, we are in effect demonstrating nothing.

The result, however, is to place patients with life-threatening or disabling diseases into a competition for limited resources. It is one thing to find oneself in a lifeboat with water for only some of the passengers. It is quite another thing to discover that the water supply was intentionally limited — in this case by Congress.

On another front we have learned that CMS is also considering changes to Medicare drug coverage that would prohibit such coverage for off-label therapies. What would this mean to GIST patients? Say that a new oral drug is approved by the FDA for another type of cancer that shows great promise for GIST patients. It would not be covered for GIST patients, even those for whom no other treatment was available.

PKC412 trial update

Jerry Call, Life Raft Group science coordinator, has learned that there may be a slight delay in the start of the phase II trial combining Gleevec and PKC412. In our last newsletter we reported that this trial would begin in Germany and in Portland, Oregon. We speculate that the delay may be due to a discovery in the phase I trial that PKC412 may be causing the Gleevec to be metabolized too quickly, which means that the amount of Gleevec (600 mg. per day) may be effectively decreased. One possible solution (and again we speculate) may be to increase the dosage of Gleevec).

Note that in the Gleevec plus RAD trials, the opposite effect may be happening. Gleevec may be causing RAD to be metabolized more slowly, which we speculate may effectively increase the effective amount of RAD.

The Life Raft Group

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Who are we and what do we do?

The Life Raft Group is an international, Internet-based, non-profit organization providing 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 inhibits the growth of cancer cells in a majority of patients. It 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:

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