

# Hoping for a miracle, preparing for death

Michael Matthews fought to survive the time gap between clinical trials

By Norman Scherzer

In the summer of 2001, Mike Matthews is dying. He becomes part of a small group of cancer patients whose gastrointestinal stromal tumors dramatically respond to an experimental drug, STI571. The tumors that were about to take his life begin to shrink until they are more than 80 percent gone. In early 2002, U.S. officials approve the drug, now called Gleevec, in record time.



MATTHEWS

What's left of Mike's tumors remain stable until December 2002 when a new tumor appears. Surgery in March 2003 appears to wipe the slate clean. But nine months later, new tumors are discovered, this time in his liver. It is January 2004.

March brings the promise of the new phase III clinical trial for Pfizer's SU11248. As fate would have it, Mike gets the real drug, not the placebo. But he doesn't respond. His tumors continue to grow and multiply at an aggressive rate. It is clear that SU11248 is not helping and he is taken off the trial. It is May 27.

What now? Mike is as determined a fighter as I have seen, and he begins to

See MIKE'S STORY, Page 4

Battling gastrointestinal stromal tumor



## LIFE RAFT GROUP

July 2004

In memory of Lupe Zertuche, Leif Arne Blixt, Wayne Berhaut and Mike Matthews

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## New drug holds promise for GIST's sister cancer

LOS ANGELES (Newswise) — An experimental therapy that may battle resistance to the drug Gleevec in patients with chronic myeloid leukemia (CML) has shown promising results in a study at UCLA's Jonsson Cancer center, increasing survival in animal models and perhaps paving the way for a second generation targeted therapy.

The results of the study appear in the July 16 issue of the peer-reviewed journal *Science*, published by the American Association for the Advancement of Science. Early phase human studies of BMS354825, being developed by Bristol-Myers Squibb, already are underway at the Jonsson Cancer Center in CML patients who developed a resistance to Gleevec.

See BMS DRUG, Page 7



The young women fund-raisers of St. John Villa Academy High School.

## School aids teen's fight vs. GIST

By Brian McBride

My daughter, Malorie McBride, began her battle against GIST when she was 12 years old.

When Malorie was a freshman in

high school, her classmates and teachers were not aware of her situation because her condition was stable. Since school records indicated her condition when she applied to high school, the

See TEEN'S FIGHT, Page 10

# Medicare lottery to cover Gleevec, for some

By Pamela Barckett

**W**hat is the Medicare lottery? It is the system devised to implement the Medicare Replacement Drug Demonstration that will cover some oral drugs before Medicare's comprehensive prescription drug program begins in 2006.

This lottery is a temporary bridge for qualified people until the comprehensive program begins. Seniors and people with disabilities could save substantial money by participating. For GIST patients, the oral cancer drug Gleevec will be covered.

The Medicare Replacement Drug Demonstration is limited to 50,000 people or \$500 million, whichever comes first. The selection into the lottery will be random from the pool of applicants, alternating between cancer patients and others with serious diseases. Applications for the lottery will be accepted until Sept. 30. Completed applications received by Aug. 16 will be in the first drawing, with coverage starting Sept. 1.

A second drawing will take place after the Sept. 30 deadline. Applicants not chosen in the first drawing will be included in the second drawing. For those picked in the second lottery, coverage takes effect Oct. 18. Trail Blazer, a subsidiary of Blue Cross and Blue Shield, will manage this demonstration. Caremark is the pharmacy that will be providing the drug benefit to patients.

The requirements to be eligible for the lottery (demonstration) are:

- Be able to obtain Medicare Part A and have Medicare Part B
- Have Medicare as primary insurance
- Live in one of the 50 states or the District of Columbia
- Have a signed document from your doctor explaining that you need

## Medicare coverage vs. patient costs for \$30,000 worth of Gleevec per year

	You pay	Medicare pays
The first \$250:	\$250 (100%)	0
The next \$2,000:	\$500 (25%)	\$1,500 (75%)
The next \$2,850: (the "doughnut hole")	\$2,850 (100%)	0
The next \$24,900:	\$1,245 (5%)	\$23,655 (95%)
Subtotal:	\$4,845 (16%)	\$25,155 (84%)
Your annual premium:	\$420	0
<b>Grand Total:</b>	<b>\$5,265 (18%)</b>	<b>\$25,155 (82%)</b>

one of the drugs covered under this demonstration for your health condition (you currently don't need to be taking one of the drugs to qualify)

— Not have any other insurance that has comprehensive drug coverage such as Medicaid, employer or union group health plan or Tricare

Patients will pay an annual premium and various co-pays. For the first full year 2005, patients will be responsible for the first \$250 in drug expenses, and will pay an average of 25 percent co-insurance until they reach the benefit limit of \$2,250. After reaching this limit, patients will face a gap in coverage (the so-called "doughnut hole") in which they will pay all of their drug costs up to \$5,100 in total drug spending. The total out-of-pocket cost at this point is \$3,600. Medicare will then cover 95 percent of drug costs above that amount. Some low-income patients

whose incomes are 135 to 150 percent of the federal poverty level will have a reduced deductible.

The table above shows what happens to a patient who takes \$30,000 worth of Gleevec per year.

For those currently enrolled in the Novartis Assistance Program who qualify for the lottery, Novartis will help with the out-of-pocket expenses. If you don't qualify, Novartis will continue your assistance as before.

To apply and or obtain any information regarding this lottery, call 866-563-5386 (Monday-Friday, 8 a.m.-7:30 p.m. EST) or go to [www.medicare.gov](http://www.medicare.gov) and click on "Medicare to Extend Access to Certain Drugs For Beneficiaries with Serious and Chronic Illness." You will be able to download the application from there. The doctor's portion of the application must be completed or your application will not be processed.

# Error reported adjuvant Gleevec trial

Computer error means 60 patients are off trial; results will be delayed

A randomization error has been discovered in the American College of Surgeon's Oncology Group (ASCOG) Z9001 clinical trial for GIST patients. The trial will determine if 400 mg. of Gleevec following surgery is successful in preventing a relapse. This is called adjuvant, or preventive, treatment.

The randomization error means 60 patients will be removed from the trial, and the trial study period will be lengthened. Although the patients dropped from the trial will continue to be monitored and should suffer no adverse results, they will know whether they were on a placebo.

Bottom line: the trial results about adjuvant treatment will be delayed several months, at least.

Dr. Samuel Wells, Oncology Group chairman of the American College of Surgeons, and Dr. Ron DeMato,



Life Raft Executive Director Norman Scherzer, left, discusses the error in the clinical trial study of adjuvant Gleevec following GIST surgery with Dr. Samuel A. Wells Jr., center, and Dr. Ron DeMatteo.

Hepatobiliary Service, Memorial Sloan-Kettering Cancer Center, visited the Life Raft Group offices July 23 to explain the error and to discuss future clinical trial initiatives, including a proposal by the Life Raft Group to create an adjuvant trial comparing differ-

ent doses of Gleevec.

The following letter was sent out July 14 by Wells:

"The American College of Surgeons Oncology Group (ACOSOG) has de-

**See ERROR, Page 8**

## Dr. Larry Baker addresses Michigan chapter

By Allan Tobes

The Michigan Life Raft Group met June 26 in Detroit for one of its most informative meetings, thanks to Dr. Larry Baker. His reputation as one of the world's leading GIST experts drew members from as far away as the Cleveland, Ohio area. It was clear that everybody felt the presentation was well worth the drive.

Baker spoke for an hour, starting with the basics of anatomy. He described the various types of cancer and explained the differences between

sarcoma and carcinoma.

From there Baker explained the uniqueness of GIST and how it differed from most other sarcomas. He updated the group on the treatments available and his views on the options facing a GIST patient.

He announced that the University of Michigan would be participating in the Amgen trial and that it would be conducted without the use of a placebo. He indicated that although there is a definite place for placebos in medical trials, he feels very strongly that it is not appropriate for GIST patients.

Following Dr. Baker's talk, GIST'ers had an opportunity to ask questions and found him to be quite direct in his advice. After the Q&A session, the chapter had an abbreviated regular meeting.

The next meeting of the Michigan area group will be Sept. 18, with David Josephy, Ph.D., giving his "GIST 101" presentation. Professor Josephy has offered to make the five-hour drive from the Toronto area. The group is looking forward to his presentation, which has been exceptionally well received at both of the Life Raft conferences.

## MIKE'S STORY

From Page 1

explore other treatment options. He and his wife, Valerie, traveled in April to the Life Raft Group meeting in Orlando, Fla., and had spoken to a number of GIST specialists there. He'd heard that the new Amgen drug, about to go into phase II clinical trials for GIST patients, is too similar to the Pfizer drug that had failed him and he is therefore not eligible for the Amgen trial.

(We have since learned that although failure on the Sugem drug will preclude entry into the phase II Amgen trial, the two drugs may not be that similar and Amgen should not be precluded from helping Sugem-resistant GIST patients in future.)

A panel of the world's GIST experts reviews the available options for patients in Mike's situation and concludes that only traditional disease-holding actions are available, including higher doses of Gleevec, surgery, and chemo embolization -- none of which are applicable to Mike's situation. Finally, as the meeting draws to close, he hears about a new Bristol Myers Squibb (BMS) drug about to enter phase I trials at Dana-Farber.

By early June, Mike is back at home in Nova Scotia trying higher doses of Gleevec — 800 mg., 1,000 mg., 1,200 mg. Side effects get worse and his tumors continue to grow. At the June 5-8 meeting of the American Society of Clinical Oncology, Dr. George Demetri of Dana-Farber Cancer Institute and I discuss Mike's options, particularly whether he can get into the coming BMS trial. The trial has not yet started and patients are lining up for the few slots. Mike is not a Dana-Farber patient and is not a U.S. citizen, so he will have to pay more than \$100,000 just to get into the door, in addition to travel and lodging. I call Mike and we discuss the situation. Mike decides to try the higher doses of Gleevec a while longer, and in the

meantime make an appointment at Dana-Farber. It is June 6.

Weeks go by. Mike's tumors continue to grow. I call Demetri about the BMS trial. There are far too many patients for the handful of slots at Dana-Farber, and Demetri suggests trying Scotland. I track down the principal investigator in Glasgow and contact Mike. Numerous phone calls and e-mails follow. There is an

obstacle: Mike is not a U.K. citizen. More exchanges. Mike's local oncologist gets involved and finally Mike gets a July 27 appointment to see Dr. Jeffrey Evans in Glasgow for a pre-trial consult. He has missed the July trial slot by a few days but there is a potential slot open on Aug. 19.

Mike then flies to Dana-Farber to see if there is something, anything they can do for him. There isn't. So he continues making plans for Scotland. It is July 17.

Mike's tumors continue to grow. He is getting weaker. His liver enzymes are rising to the point where they could exclude him from the Scotland trial. He sees his local oncologist, Dr. Lori Wood. She schedules a blood test to check his liver enzymes.

Mike also meets with his lawyer, and writes to the Life Raft Group: "... I will be meeting with palliative care as well to discuss pain management and other end-of-life issues. I am so close. I just need to get through the next few weeks. I am doing everything I can; your thoughts and prayers will be greatly appreciated."

**July 17, Mike writes:**  
**"I am so close. I just need to get through the next few weeks. I am doing everything I can; your thoughts and prayers will be greatly appreciated."**

**July 23, Valerie calls:**  
**Mike's liver enzymes are too high for him to qualify for the trial. Mike asks if Norman Scherzer can come to his going-away party.**

It is July 23. Valerie calls. Mike's liver enzymes are too high. Mike asks if I can come to his going-away party. Saturday, July 24: Mike's friends have arranged a flight to Halifax, Nova Scotia. Halfway to La Guardia airport the radio tells me the expressway ahead is closed. A secondary route gets me to the airport. Unfortunately, I have no passport (sent it off to be renewed) and I've mis-

takenly taken a copy of my birth certificate rather than the original. My ticket, booked less than 24 hours ago, my 14-hour stay in Nova Scotia, and the buzzing alarm clock in my overnight bag earns me the attention of various security officials.

A fellow with my name on a sign greets me at Halifax airport. Neither of us knows where we're going, but a few phone calls fixes that.

We arrive at Purcell's Cove Club outside of Halifax. Valerie greets me and we go inside. Must be over 150 people here. After dozens of hello hugs I am led to where Mike is sitting.

He has no idea that I was coming and the look on his face is one of complete surprise. Mike gets up from the living room chair that his friends brought from home to make him comfortable, and we hold one another tight, the tears rolling down my face.

The party is really something, complete with a rock band playing my kind of music from the '60s. I am part of the Matthews' extended family and

**See MIKE'S PARTY, Page 6**

# Update on new drugs with GIST potential

Pfizer's SU11248 is still the only one with a track record of success

By Jerry Call and Norman Scherzer

Recent newsletters have reported on the growing list of drugs that Gleevec-resistant GIST patients may turn to. This is an update.

For the latest information, GIST patients should consider joining the Life Raft Group, and both patients and GIST physicians should regularly check the Life Raft Web site at [www.liferaftgroup.org](http://www.liferaftgroup.org)

**SU11248:** The phase III trial for Pfizer's Sugem drug (SU11248) continues to enroll patients around the United States and abroad. This is the only drug that has a track record of success for Gleevec-resistant GIST patients. About 60 percent of patients enrolled so far show some benefit, mostly stability. The trial has generated considerable controversy because patients must discontinue Gleevec for at least two weeks prior to starting the trial, and because a third of the patients receive a placebo.

SU11248: Inhibits KIT, PDGFRA, VEGFR, FLT3; simultaneously inhibition of multiple signaling pathways may be important for controlling resistant GIST; could interact differently with structural variants of new kinase mutants in GIST clones resistant to Gleevec.

**AMG706:** Amgen's AMG706 is projected to begin phase II clinical trials, starting in the United States, in about a month. It is expected that there will be about 20 sites in the United States and 10 sites in other countries. Three known U.S. sites are M.D. Anderson Cancer Center in Houston,

University of Michigan in Detroit and Northwestern University in Chicago.

In the interim, the Life Raft Group has been advised that there are still some slots for the phase I AMG706 trial at M.D. Anderson. This phase I trial was recently amended to add four to eight weeks. Sugem-resistant GIST patients are eligible for the phase I trial.

Note: Although Sugem-resistant GIST patients will not be eligible for the phase II Amgen trial, there is no data to support the assumption that pa-



tients won't respond to AMG706 because of similarities between the two drugs.

SU11248 is administered in a cycle of four weeks on and two weeks off. AMG706 is administered daily. While the targets of AMG706 may be similar to SU11248, the drugs may have entirely different activity profiles. One drug may work better on one type of mutation while the other might work better on another type. At this point, which type AMG706 might work on is unknown.

**PKC412:** Novartis' phase I trial for PKC412 plus Gleevec has begun at Oregon Health & Sciences University and in Berlin.

PKC412: Inhibits more targets than Gleevec; inhibits PKC (which isoforms of PKC is unknown), KIT, VEGFR, FLT3; requires a higher dose of Gleevec due to interactions between Gleevec and PKC412; may be a choice for some PDGFRA mutations.

**BMS354825:** A phase I trial for Bristol-Myers Squibb's BMS354825 has begun at Dana-Farber Cancer In-

stitute in Boston and at Glasgow University in Glasgow, Scotland. Very few slots are available for GIST patients. Incredible results have been reported for Gleevec-resistant chronic myelogenous leukemia patients (see Page 1) and this drug is very high on the Life Raft's radar. This drug inhibits KIT, PDGFRA, Src, and Bcr-Abl.

**AP23573:** ARIAD's AP23573 continues in phase I clinical trials at University of Texas in San Antonio and University of Chicago Medical Center. Life Raft representatives will meet with ARIAD officials in a few days to discuss expanded trials for GIST. Also of interest is a second ARIAD drug, AP23464.

**17DMAG:** In this newsletter the Life Raft adds a new drug to the list. It is 17DMAG, made by InvivoGen. A phase I trial for prostate cancer patients at Memorial Sloan-Kettering is expected, but the Life Raft is seeing if GIST patients can be included.

17DMAG: Is an improved version of 17AAG; has improved bio-availability; inhibits HSP90 and multiple proteins that might be involved in cancer including AKT, and possibly some types of KIT mutations that are resistant to Gleevec.

**G3139:** Genta's long-awaited phase II trial of G3139 (Gentasense) plus Gleevec has been put on hold. It is expected to take place at M.D. Anderson, Dana-Farber, University of Michigan, Memorial Sloan-Kettering, and the Mayo Clinic.

Gentasense: Is an antisense drug that targets mRNA for bcl-2. This reduces the production of bcl-2 protein, which acts to prevent apoptosis (cell death).

The Life Raft aims to monitor other potential drugs, including Novartis' RAD001 plus Gleevec, Gleevec plus Avastin (Bevacizumab), Abbot's ABT869, Bayer's BAY439006, AB Science's new drug in France, and Kirin's KRN951.

## MIKE'S PARTY

From Page 4

spent the next few hours being passed from one tearful hug to another. Everyone had heard about Mike's Life Raft Group and I am asked to say a few words. Then Mike gives instructions to his friends about the fundraiser he wants them to throw for us.

Two moments will forever remain with me. The first is when Mike got up from his chair and danced with Valerie. Only music and heartbeats could be heard as we watched, each of us with the same thoughts and feelings. The second was when Mike had to sit

down and take off his shoes. His eldest daughter, Ashley, kneeled by his side and gently massaged his feet.

After the party, it's off to Mike and Valerie's home for a very late meal with a few close friends and their daughters, Ashley and Laura, two of the sweetest girls I have ever met. We talk and I have a chance to spend some private time with Mike.

The setting is peaceful and surreal. Their home is on a cove with the ocean off to the right and tall ships passing by in front.

Many goodbyes and hugs later, and I head to a friend's home for a few hours' sleep before rising with the sun to fly back home. I pack many bitter-sweet memories, honored to have been invited and overwhelmed by the experience.

Tuesday, July 27: It is the day Mike was supposed to be in Scotland, seeing a doctor, getting on a trial. But at 6:45 a.m., as the sun rises over the Atlantic and tall ships pass by, Mike Matthews' fight ends. The lethal time gap between clinical trials was too much.

## Chicago LRG chapter learns of Amgen trial

By Richard Kinzig

**A** new drug for GIST patients for whom Gleevec no longer works was one of the topics covered at the July 18 Chicago chapter meeting of Life Rafter, held at The Wellness Place in Palatine, Ill.

The meeting drew 19 people from Illinois, Indiana and Wisconsin, including Rafter and spouses/family and guest.

Dr. Andrew Evens of Northwestern University talked about surgery, Gleevec and new drugs. Most interesting for most were the new drugs. Evens said the drugs being developed are more potent, yet often without any increase in side effects. The new drugs are focusing on good quality of life end points, which are as important as tumor shrinkage.

He was pleased to inform us that a new phase II trial for Amgen's new drug, AMG706 will be initiated in approximately six weeks at Northwestern University & Evanston Northwestern Health Care locations, which will be among 20 centers in the nation. Northwestern is well into the process to



EVENS

launching the program. This trial is directed at patients that no longer benefit from Gleevec. He also pointed out that, if you failed to meet the Sugem trial criteria, to apply for the Amgen trial.

Patients already on Sugem are not eligible for AMG706 at this time.

Life Rafter Jim Hughes gave an outstanding summary of the knowledge he gained at the recent convention of the American Society of Clinical Oncology, which he and Norman Scherzer attended. He covered comments from Dr. Charles Blanke of Oregon Health & Sciences University, talked of a new drug that shows promise, ARIAD, a new rapamycin analog that is heading for a phase II trial, and talked of the many poster presentations at the convention.

One thing that we found interesting was a printout dealing with guidelines a patient can use in discussions with his/her doctor about GIST treatment options. Hughes directed patients to

Web site, [www.NCCN.org](http://www.NCCN.org), which has a blueprint for all cancers. Select the Gastrointestinal Stromal Tumors section, which contains five pages of progressive treatment from initial diagnostic evaluation to therapy for progressive disease.

Chapter member Bob Book, who also serves on the Life Raft Group Board of Directors, talked of the discussions at the recent board meeting in Orlando, Fla., and explained the organization's opposition on the use of placebos in clinical trials for patients who have no other options for disease containment.

There will be a fund-raising program in November (Thanksgiving in the U.S.) again this year. One goal is to raise enough money to add Jerry Call to the staff for research, a much needed effort if we are going to influence the medical profession to focus on GIST.

The next Chicago area meeting will be Sunday, Nov. 14.

A thank you also goes to members who helped with the snacks and beverages, and to the Wellness Place for allowing us the use of their facility on a weekend when they are not normally open.



## Life Raft T-shirts, pins now available

Life Raft Group staffers Pam Barckett, left, and Trish McAleer model the new Life Raft Group T-shirts. The \$15 shirts come in sizes youth-large, small, medium, large, X-large and 2XL. Also available are Life Raft Group pins, seen below, for \$10 each or three 3 for \$25. All prices include shipping and handling. Send your check or money order to the Life Raft Group, 40 Galesi Drive, Wayne, N.J. 07470



## BMS DRUG

### From Page 1

About 15 to 20 percent of CML patients who take Gleevec become resistant to the drug and suffer a relapse, leaving them with few effective treatment options, said Dr. Neil Shah, an oncologist and researcher, an assistant professor of hematology/oncology and the study's first author. In patients with resistant disease, secondary mutations in the gene linked to CML allow the cancer to evade therapy. It is those mutations that are targeted by the BMS354825, which is taken in pill form like Gleevec.

"Learning from what happens when a drug fails in some patients can lead

to a new treatment paradigm," Shah said. "In the future, we may be combining therapies that can, amongst them, override all the resistance mechanisms that allow cancer to evade individual therapies. In the future, cancer may be treated similarly to HIV, with a cocktail of drugs."

Shah said the drug also may be useful for treating other diseases that respond to Gleevec initially, such as gastrointestinal stromal tumors.

Gleevec is a tyrosine kinase inhibitor, a new class of drugs that can interfere with cell signaling pathways implicated in tumor development.

Dr. Charles Sawyers, a professor of hematology/oncology, an investigator with the Howard Hughes Medical Institute and the senior author of the

study, said this work could represent a major advance for the patients who suffer a relapse on Gleevec.

"Gleevec remains a spectacular step forward in the application of targeted therapy to CML specifically, and serves as a model for how to do this more generally in cancer," Sawyers said. His paper reports the effectiveness of the BMS inhibitor in Gleevec-resistant cells in tissue culture and in mice. If the experimental inhibitor proves effective in human patients as well, future studies may eventually pair Gleevec with BMS354825.

"We hope this represents another viable treatment option for patients with this disease," Shah said. "There may now be hope beyond Gleevec should their disease relapse."

## ERROR

From Page 3

tected a computer error that affected the randomization of some patients on the Z9001 clinical trial: 'A Phase III randomized double-blind study of 400 mg of STI571 (Gleevec) versus placebo in patients following resection of Gastrointestinal Stromal Tumor (GIST).' The error occurred between November 2003 and May 2004. As a result, sixty patients who entered the trial during that time were placed non-randomly into the placebo arm, when approximately half of them should have been randomized to the treatment arm. Another 11 patients were randomized such that they had an 80 percent chance of receiving Gleevec, when approximately half of them should have been randomized to the treatment arm.

"It is important to note that not all patients who were registered to the Z9001 trial during the November 2003 to May 2004 time period were affected by the randomization error. If you are one of the patients, who was affected by the randomization error, your doctor will be notifying you. Errors in randomization during clinical trials are rare and the present event involved only the ACOSOG Z9001 study. Other clinical trials in GIST have completely separate mechanisms of randomization

and were not affected.

"Once the error was identified, the ACOSOG Executive Committee developed a plan of action, which was based on discussions with the ACOSOG Data and Safety Monitoring Committee, the ACOSOG Ethics Committee and the Cancer Therapy Evaluation Program of the National Cancer Institute.

"The physicians caring for the 60 patients assigned to placebo have been notified, personally and in writing. The 60 patients will be taken off the study because their assignment to the placebo arm became unblinded during the investigation of the randomization error. The 60 patients will meet individually with their physicians, who enrolled them on the study, to discuss available treatment options. It is important to understand that we currently do not know whether Gleevec is beneficial in patients who have undergone complete removal of a primary GIST. Indeed, this is why this clinical study is being performed. Therefore, patients who were placed in the placebo arm and did not receive Gleevec, did receive the current standard of care, which is surgery plus observation.

"The physicians caring for the 11 patients who had an 80 percent chance of receiving Gleevec have similarly been notified. Since the physicians do not know whether their patients are receiv-

ing placebo or Gleevec, the 11 patients will be offered the option of staying on the study or withdrawing from the study. A patient who chooses to remain on the study will be required to sign a new consent form.

"The U.S. Food and Drug Administration, Novartis (the company that manufactures Gleevec and provides the drug for the Z9001 trial) and the Office of Human Research Protection have been notified of the randomization error.

"We have visited Mr. Norman Scherzer, the Executive Director of the Life Raft Group, and Dr. Mark Landesman, DVM, a member of the Science Committee of GIST Support International. They have been fully informed of the situation and our discussions have led to considerations of how ACOSOG can work with each of these support groups in the development and operation of future clinical studies involving patients with GIST.

"The ACOSOG deeply regrets that this error has occurred. The Z9001 trial is a very important study. Appropriate randomization was restored, shortly after the randomization error was detected and equal assignment to placebo or Gleevec has occurred. The length of the study will need to be extended by approximately three months. The ACOSOG has instituted measures to prevent the occurrence of such randomization errors in the future."

## Life Raft is getting the word out about GIST

The Life Raft Group has been making headlines and hitting the airwaves in the United States and Britain. Here is a quick roundup:

The Boston Globe, July 2004: *Dying Cancer Patients May Get Drug Placebos in Some Clinical Trials*

The Wall Street Journal, June 2004: *Fighting Cancer with a Sugar Pill — Move to Give Some Patients Placebos Instead of Drugs Causes Rift*

### *Among Doctors*

Michael Smerconish Radio Show WPHT, Philadelphia, June 2004: *Discussion Concerning Placebo and Clinical Trials*

The Wall Street Journal, June 2004: *Doctors argue over use of placebos in cancer trials*

WTMJ 10 News, Altoona, Penn., June 2004: *New Treatment for Stomach Tumors — Treating GIST*

Staten Island Register, June 2004:

### *Making a difference*

British Medical Journal, May 2004: *The First Generation of e-Patients: These New Medical Colleagues Could Provide Sustainable Healthcare Solutions*

New York Stock Exchange Magazine, May 2004: *From Pipeline to Lifeline*

CBS News, March 2004: *Cancer Sufferer's Medicare Doubts — Anita & Norman Scherzer*



## EDITORIAL

# You can bet legislators will never play the Medicare lottery

**A**s Pam Barckett's article on Page 2 notes, the United States is about to initiate a lottery to determine which Medicare patients will have access to oral cancer drugs like Gleevec. The Life Raft Group has been part of a nationwide, ad hoc group of cancer support groups and pharmaceutical companies that have been working to create Medicare coverage for oral cancer drugs (and other serious diseases) during the period leading up to comprehensive prescription drug coverage for Medicare beneficiaries. The comprehensive program is scheduled to begin Jan. 1, 2006.

Although we are pleased the U.S. Congress passed a pilot project to address oral cancer drug coverage during the period leading up to 2006, it was left to the Center for Medicare Services (CMS) to figure out how to implement a project that was capped. In

essence, Congress allocated money for fewer lifeboats than needed, and left it to this branch of the U.S. Department of Health and Human Services to figure out who would get a seat.

After a considerable amount of planning, CMS has developed a scheme that, after determining more clearly what drugs will be covered, will decide who will have access to drugs.

The solution was a lottery. Eligible participants will submit their applications and wait to see if their name is pulled from the lottery hat, or some sort of equivalent device. Millions will be spent administering this project -- but thus far we are not aware of any plans to evaluate what happens to those who do not win this lottery. Do they manage to gain access to their lifesaving drugs on their own? If not, do they die?

Are we ungrateful for having raised this issue? After all, 50,000 winners

will be chosen and that is 50,000 more than before this demonstration project. But I keep thinking about the losers. If you lose your state lottery, you lose a few bucks out of pocket and you're deprived of a little fantasy. If you lose this lottery, you may die.

One thing that this demonstration project will do is to permit us to understand how the comprehensive prescription drug program will really work, because the financial benefits are based upon the comprehensive program. After you sort through the premium and co-pays — including something called a “doughnut hole” — the reality is patients facing catastrophic drug bills may be out-of-pocket more than \$5,000 per year.

One could only imagine such a health plan being proposed for our congressional representatives.

— **Norman J. Scherzer**,  
Life Raft Group Executive Director

## GIST'ers join for artist Ellen Mayer's solo exhibition in NYC



The works of artist and GIST survivor Ellen Mayer are being displayed at a solo exhibition this summer at Midoma Gallery in New York City. Several GIST'ers attended the July 11 reception for Ellen's show. Pictured, from left, are Pam Fedowich, Ellen Mayer, Mark Landesman, Ken Schou and Donna Geraghty. Midoma Gallery is at 439 E. 75th St., New York; Ellen's show runs through Aug. 28

# Lupe Zertuche battled GIST four years

**L**upe Zertuche, 65, of Payson, Ariz., died June 20, 2004. He was born Dec. 12, 1938. Mr. Zertuche and his family moved to Payson in February of 1978. He first went to work as a painter for Billy Ray Harris. Later, he started his own business, Payson Painters.

He served the Payson area with his high-quality painting for 26 years until his long struggle with cancer began in the fall of 2000. His son, Dan, gradually took over the business under the new name of A to Z Painting.



ZERTUCHE

He is survived by his wife of 44 years, Judy; two sons, Dan (Kim) Zertuche and Toby Zertuche; four grandchildren, Mitchell, Maureen, Aaron and Jacob; brothers and sisters, Victor Jr., John and Catalina Zertuche, Lila Harcharik, Esther Gomez, Jeanne Ortiz and Maryann Enberg; and 25 nephews

and nieces.

A memorial service was held at the Kingdom Hall of Jehovah's Witness in Payson, followed by a gathering at Rumsey Park.

"Gleevec gave Lupe three extra years of life, for which we will be eternally grateful," said Judy. "To Norman, Mia, and all the others who make the Life Raft possible, I would like to express my deepest thanks for the help and support it provided us when we felt so alone and without knowledge of the enemy we faced."

## TEEN'S FIGHT

From Page 1

administration knew there were days when she would miss school for various tests and doctor appointments but they really had no idea of the seriousness of the illness.

When Malorie had a recurrence last September, I and her mother, Dorothy, started communicating more with her principal since she had to go through a battery of tests between October and December which caused her to miss several days of school. It was during these numerous discussions that we began to explain GIST and how rare the disease is, especially the pediatric form. We were able to tell her about the great strides made with Gleevec and more recently with Sugem in the adult cases. We mentioned that much more research and investigation is needed for the pediatric form of GIST, and that we were committed to help with any type of fund-raising needed to help the cause.

Each year Malorie's high school sponsors a "Super Dance" which raises money for various causes and charities. In December 2003, Sister Antonio called and asked us how we felt about having the 2004 Super Dance raise

**"The school administration tried to keep Malorie's name anonymous but it soon leaked out. Once the students were able to associate a person they knew with the cause, they were energized in their fund-raising quest."**

money for GIST. We thought it was a great idea but we had to see how Malorie felt about the exposure to all of her schoolmates and having to share a situation with people who, until that point, had no idea.

This was a tough sell. Malorie has fought a fierce, three-year battle against GIST with quiet dignity. She was comfortable with the fact that she was treated as a normal teenager at school because no one knew her situation. But Malorie agreed with the idea because she understands the dire need for the research and the fact that money is needed to expand the investigation of pediatric GIST.

So, we gave the OK to Sister Antonio and, as I said earlier, we directed them to different Web sites where they could find information about GIST. Dorothy and I had pediatric GIST banners and buttons made that the kids used when chasing people for donations.

The Super Dance had a "carnival" theme. The teens sold cotton candy, jelly apples, root beer floats and more during school on Fridays. There were actually two schools involved, Malorie's St. John Villa Academy High School and their partner school, St Peter's High School for Boys of Staten Island, New York.

The school administration tried to keep Malorie's name anonymous but it soon leaked out. Once the students were able to associate a person they knew with the cause, they were energized in their fund-raising quest.

The Super Dance was held in March and raised more than \$20,000. The students had decided to split the donations between pediatric GIST and the Leukemia and Lymphoma Society, as a former St John Villa student is battling leukemia.

# THE LIFE RAFT GROUP

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## Who are we, 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](http://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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