

Photo by Linda Palmer

Richard Palmer and Roberta Gibson got to meet on the Big Island of Hawaii earlier this month, and used this sunset on the Kona coast as a backdrop.

From the editor

## Silver lining in the cloud of an orphan cancer

By Richard Palmer

**E**arlier this month, Linda and I got to see good friends we've never met before.

That's exactly what I said when I asked my boss for an extra day off. He looked at me funny (he does that a lot), then asked, "How can they be friends if you never met them?"

I explained they're part of the e-mail group of cancer patients on the same clinical trial I'm on.

"Roberta and Doug Gibson of Edmond, Oklahoma," I told him. "She's on the trial. They're visiting the other side of the island for a week; Linda and I figure we'll drive over and meet them."

David, my boss, had no problem giv-

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## Battling GIST with Gleevec (STI571)



## LIFE RAFT GROUP

November 2001

In memory of Bruce Gunn

Vol. 2, No. 10

# Novartis seeks U.S. OK to use Gleevec on GIST

### Approval would mean first treatment option beyond surgery for GIST

**B**ASEL, Switzerland — Novartis announced Oct. 19 that it has asked the U.S. Food and Drug Administration for approval to market Gleevec for the treatment of patients with unresectable (inoperable) and/or metastatic gastrointestinal stromal tumor.

GISTs are the most common malignant form of sarcoma arising in the gastrointestinal tract. Historically, GISTs have been very difficult to treat due to their high resistance to traditional chemotherapy and radiation therapy. For patients with metastatic or unresectable disease, GISTs represent an incurable malignancy with a median survival of approximately 10 to 12 months.

Until now, surgery has been the only treatment option, resulting in essentially palliation of this disease. There are approximately 12,000 new cases each year of malignant GIST worldwide.

"GISTs are very difficult to treat and there are very few options beyond surgery for these patients," said Dr. David Parkinson, vice president of

clinical research for Novartis Oncology. "Gleevec is extremely active against the molecular abnormality that helps trigger GISTs, and Novartis believes it represents a significant advance in overall treatment of the disease."

Last May, the Food and Drug Administration approved Gleevec for the treatment of patients with chronic myeloid leukemia just 10 weeks after Novartis filed its application for approval — the fastest approval of any cancer drug.

The effectiveness of Gleevec in CML is based on overall hematologic and cytogenetic response rates.

There are no controlled trials demonstrating a clinical benefit such as improvement in disease-related symptoms or increased survival.

Gleevec (known as Glivec outside the U.S.) is currently approved for marketing in more than 30 countries. Gleevec received a positive recommendation by the European Union's Committee for Proprietary Medicinal Products in July, and an approval by the European Commission is anticipated shortly.

Novartis sought FDA approval to use Gleevec on GIST based on data from a Phase II, open-label, multina-

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## Approval

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tional study conducted in 147 patients with unresectable or metastatic malignant GIST. Patients were randomized to receive either 400 mg or 600 mg of Gleevec daily for up to 24 months. The overall response rate is 40 percent based on confirmed partial responses at the time of the data cut-off for the submission. An additional 32 percent of patients in this study achieved a clinically significant reduction in tumor size. Only 12 percent of patients experienced disease progressed in the study.

Gleevec has been well tolerated in patients with GIST. Although almost all patients reported an adverse reaction at least once during the trial, only a very small percentage had these recorded as either grade 3 or 4 in severity. Five patients (3.4 percent) withdrew from the study due to adverse events. In this clinical trial, the most common adverse events were nausea, diarrhea, periorbital edema, fatigue, muscle cramps, abdominal pain, dermatitis, vomiting, flatulence, lower limb edema, nasopharyngitis, insomnia, back pain and pyrexia. There is no long-term safety data on Gleevec treatment.

Gleevec is one of the first cancer drugs to be developed using rational drug design, based on an understanding of how some cancer cells work. Gleevec targets the activity of certain enzymes called tyrosine kinases that play an important role within certain cancer cells. The activity of one of these tyrosine kinases, known as c-kit, is thought to drive the growth and division of most GISTs.

Novartis, based in Basel, Switzerland, is a world leader in health care with about 70,000 employees in more than 140 countries. In 2000, Novartis had \$17.2 billion (U.S.) in sales and a net income of \$3.9 billion. Some \$2.4 billion was spent on research and development.

## Silver anniversary, going for gold



Life Rafter Cynthia and husband Jerry celebrated their 25th wedding anniversary Oct. 22 with a dinner in La-Grange, Georgia. Five grandchildren and all four of the couple's children attended along with other relatives and friends. Cynthia is a 5 1/2-year GIST survivor who underwent surgeries, chemo and RFA before learning about the Gleevec trials in July 2000. She didn't get in until Oct. 4, 2000. By that time Cynthia was in a wheelchair and wearing maternity clothes due to innumerable tumors up to 20 centimeters. The top layer of their anniversary cake "is in the freezer until we have our 50th anniversary," says Cynthia — "If I can remember where the freezer is when I'm 88 years old."

## Life Rafterers in Portland, Oregon



Gary Kirk, left, and Michael Byrne both had the one-year checkups at Oregon Health Sciences University in Portland at the end of October. They posed for this photo after dinner outside of John's Landing. Gary's tumor is a quarter the size it was before Gleevec, Michael's less than half.



# Life Raft survey draws Novartis interest

From a handful of patients battling an “orphan cancer,” the Life Raft Group has grown in numbers and influence in its first year, as evidenced by a meeting last month with Novartis officials in New Jersey.

Novartis is the Basel, Switzerland-based pharmaceutical giant that makes Gleevec (Glivec outside the U.S.), the first molecularly targeted oral cancer drug approved for treatment of chronic myeloid leukemia and in clinical trials for GIST, gastrointestinal stromal tumor.

Life Raft Group Coordinator Norman Scherzer, his wife, Anita, and Gilles Frydman, president of the Association of Online Cancer Resources (ACOR), were invited to Novartis’ offices in East Hanover, New Jersey, on Oct. 15 to present the findings of the group’s side effects survey.

“I had been told to expect between three and five people, but that two would come a little late,” said Scherzer. “We got there just on time and walked into a conference room filled with 10 people.”

Attending was Paula Boulton, global brand director for Glivec; Sally Church, Gleevec U.S. marketing team; Dr. Deborah Dunsire, senior vice president/North American region head of Novartis Oncology; Howard Goodman, global communications manager for Glivec; Barbara Kennedy, executive director of scientific field operations; John Ketchum, head of U.S. oncology marketing; Carl Keubler, director of Gleevec marketing; Dr. Lauri Letvak, executive director/head of Glivec phase IV program; Rick Satipunwaycha, Gleevec U.S. marketing team; Dr. Sandra Silberman, PHD, international clinical leader; and Gloria Stone, director of global public relations.

For the next two hours, the Scherzers and Frydman shared information and



Photos by Gilles Frydman

Norman Scherzer, coordinator of the Life Raft Group, explains the outcome of the group’s side effects survey to Novartis officials in New Jersey on Oct. 16.



Some of those at the meeting were, from left, Barbara Kennedy, executive director, scientific field operations; Carl Keubler, director of Gleevec marketing; Life Raft member Anita Scherzer; Dr. Sandra Silberman, PHD, international clinical leader, and Gloria Stone, director of global public relations.

responded to questions from the Novartis people. “Mostly, we discussed our presentation of side effects but I also had an opportunity to review a number of concerns which this group has expressed,” said Norman. These ranged from skin problems that are really neuropathy to concern about the

cost of the drug for those not on the trial.

“The presentation was received with intense interest and there was a great deal of discussion,” said Norman. When the assembly had to vacate the conference room to make way for an-

## Novartis meet

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other scheduled group, “we continued our discussions over lunch for another hour and a half.”

The meeting was the latest in a growing list of cooperative interactions between the Life Raft Group and Novartis, said Norman. It broke ground, however, for the presentation of patient-generated data by a clinical trial group to a pharmaceutical company.

“The fact that so many senior Novartis executives spent so much time and energy to discuss data gathered by patients,” Norman said, “speaks eloquently both to the change in the role of clinical trial patients and to the far-sightedness of a large pharmaceutical company in understanding and responding to that change.”



Outside of the Novartis offices in East Hanover, New Jersey, Life Raft and ACOR representatives were shown the lovely Gleevec Garden.

# Glivec approved in European Union for CML

Glivec/GIST trial shows drug has 'potential to be a major advance'

**B**ASEL, Switzerland -- Novartis announced Nov. 8 that the European Commission has authorized Glivec in the European Union as an oral therapy for treating adult patients with Philadelphia chromosome (Bcr-Abl) positive chronic myeloid leukemia (CML) after failure of interferon-alpha therapy, or in accelerated phase or blast crisis.

The approval follows a positive recommendation by the EU's Committee for Proprietary Medicinal Products (CPMP) in July.

“We are pleased to see that medical and regulatory authorities around the world are recognizing the value of Glivec in treating CML,” said

David Epstein, president of Novartis Oncology. “With this approval, Glivec, which is already changing the paradigm of CML treatment for thousands of patients worldwide, brings the next wave of cancer treatment to the European Union.”

The authorization was based on Phase I and Phase II data from more than 1,000 patients participating in a global clinical trials program.

Similarly positive results from a Phase I study evaluating Glivec in the treatment of patients with advanced gastrointestinal stromal tumors (GIST) have been reported in the Oct. 27 issue of *The Lancet*.

The European Organization for Research and Treatment of Cancer (EORTC) Phase I study was performed in 40 advanced soft tissue sarcoma patients, of whom 36 had GISTs. It was carried out by centers in Leuven, Belgium by Dr. Allan T. van Oosterom, president of the

EORTC; in London, England by Dr. Ian Judson, vice president of the EORTC Soft Tissue and Bone Sarcoma Group; and in Rotterdam, The Netherlands, by Dr. Jaap Verweij, vice president of the EORTC.

Unusual for Phase I studies, objective responses were seen in 69 percent of GIST patients. There were 27 GIST patients with tumor-related symptoms at study entry: 24 (89 percent) experienced relief of symptoms, often within one week after starting treatment.

“Our results indicate that Glivec has substantial activity against GISTs with manageable side effects,” said van Oosterom. “For patients who have historically had very few options beyond surgery, this has the potential to be a major advance.”

Glivec is currently marketed in more than 35 countries, including the U.S., Brazil, Switzerland and Australia.



# FLAMES excel in Pan-Mass Challenge

By Gary Golnik

**C**an you imagine riding 192 miles over hilly terrain during two hot and rainy August days? On a bicycle?

A team of cyclists from Dana-Farber Cancer Institute in Boston did just that to raise money for the Institute and its Jimmy Fund. The Pan-Mass Challenge starts in Sturbridge, Massachusetts and travels all the way to Provincetown at the tip of Cape Cod.

A two-day event characterized by caring and courage, this year's edition featured some 3,000 riders pedaling past their \$13 million goal and raising over \$14 million!

Jeanne Griffin, program nurse for the GIST/Gleevec clinical trial and another team member, related the story of a mother who ran out to the team bus as it was leaving Dana Farber.

The mother asked the riders to wave to her son, who was upstairs in one of the clinic windows. The 7-year-old was receiving his first chemotherapy for sarcoma. Everyone on the bus waved, and was in tears as the bus pulled out.

Joan Canniff, who many of our group know as our wonderful nurse practitioner in the Sarcoma Center at Dana Farber, set a personal goal to ride in the Challenge this year. She trained for long hours and made it through the first day, then successfully fought the battle to get back on the bike that second morning. Joan recalls what the last mile was like.

"Off in the distance, I could barely make out what I thought was



Meet the FLAMES — Fast Legs and Minds Ending Sarcoma.



Joan Canniff at the finish line of the 192-mile Pan-Mass Challenge.

the finish line. The crowds lining both sides of the street slowly began to thicken.

"The roars became louder and louder. I thought, 'Oh my God, I did it!' I have never felt more embraced."

The FLAMES raised more than \$150,000 as a team. The money will support basic sarcoma research

of the type that led to the development of Gleevec. The Pan-Mass Challenge has raised more than \$55 million since it started in 1980.

The ride is another example of the dedication and caring of the staff at Dana Farber. The Pan-Mass Challenge is one more reason why we are so blessed to have them on our side of the battle.

## Who's new in the Life Raft Group

**Suzanne and Lyn Hamm**,  
Dickinson, Texas, U.S.A.

**Ton and Ineke de Keijser**,  
The Netherlands. Ineke is on  
the trial in Rotterdam.

**Vince Luce** of Tampa, Flor-  
ida, U.S.A.

**Henrietta Olsen** of Long  
Beach, California, U.S.A., on  
the trial at University of Cali-  
fornia, Los Angeles

**Maryann Klein** of Utica,  
New York, U.S.A.

**Tuoman Hemminki** of  
Vaasa, Finland, on the trial in  
Helsinki.

**Angela R.** of Sunnyvale,  
California, U.S.A.



Life Rafter do get around: Here's new member and "future GIST survivor" Vince Luce at Abu Simbel, Egypt, in this photo taken a year ago.

## Norman's notebook

**Membership:** With the closure of the GIST clinical trials Sept. 1, the Life Raft Group expanded its membership to include GIST patients who receive Gleevec via prescription from their private physician.

**New Data Initiative:** Although most GIST patients continue to respond to Gleevec, there is a small number who do not. The Life Raft Group has now begun a new database to track these patients and focus on alternative treatments — usually Gleevec in combination with another drug.

**Medicare Initiative:** The Sept. 11 attacks have sidetracked a number of important legislative initiatives, one of which is a bill to amend Medicare to cover oral cancer drugs like Gleevec. The Life Raft Group is about to launch its own advocacy initiative to support this legislation, joining a growing list of professional and consumer organizations in this regard.

— From Coordinator Norman Scherzer

## Call this Life Rafter 'Dad'



Photo by Mia B.

Life Rafter member Michael B. and his son, 6-year-old Matthew, pose for a picture on Litchfield Beach, South Carolina. Mom Mia captured this image of father and son in early August during a weeklong vacation. Michael has been on Gleevec for more than a year.



# New drug offers hope

By Lorraine Thompson  
The Olympian

**M**arcel Szyszkowski waited through three years of medical tests, surgeries and experimental procedures, but found it excruciating in early June to wait just a few more days.

After all, the results he waited for could mean his life or death. They could also be his last hope.

Szyszkowski, an environmental engineer with the Department of Ecology, is those taking an experimental drug, Gleevec, being tested for its ability to halt or shrink a rare and deadly form of cancer.

Szyszkowski was diagnosed in 1998 with gastrointestinal stromal tumor, or GIST — and was told in January he would likely die within a year.

Surgeries and an experimental photodynamic therapy had not halted the fast-growing tumor. At one point, the tumor grew 50 percent in a month.

When Szyszkowski heard about the Gleevec trial, he eagerly signed on.

He had to be persistent — it appeared at first that he did not have the enzyme needed for Gleevec to work. He pleaded with doctors to test him again for the enzyme, and the tests came back positive.

By early June, the engineer had been taking the drug for about eight weeks, and the time had come for a CT scan to measure his tumor.

Szyszkowski knew the technician was not supposed to say anything about what she saw, that he was supposed to wait a few days and talk to his Olympia oncologist.

He begged for just a little indication that the Gleevec was working.

A slight nod of the head and a slight smile filled him with relief, telling him

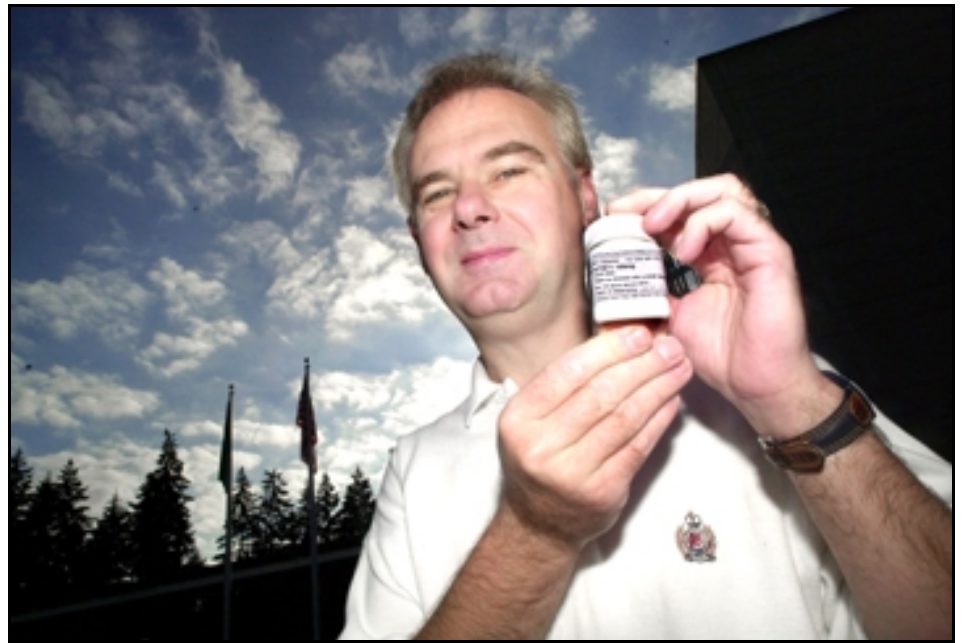


Photo by Mike Salsbury/The Olympian

Marcel Szyszkowski said his GIST tumor has shrunk 35 percent since beginning treatment using an experimental cancer drug called Gleevec.

the news would be good.

His tumor had shrunk 35 percent.

"Which is excellent news. I was hoping it would at least stop the disease and it did better than that," said Szyszkowski, 46, who was born and raised in Poland, and has been in Thurston County since 1989.

Before he learned of the study, "I was very depressed. I was thinking of

undertakers and all of that," he said. Now, "it looks like the diagnosis of my death within a year is put on the shelf."

Szyszkowski is hoping he can continue taking the drug as long as it keeps his tumor at bay, keeps his terminal diagnosis on hold.

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## In Memoriam

There have been eight deaths of Life Raft Members 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, 16, and Scott, 9
- **Ron Martinez**, 60, July 2, 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.

## THE LIFE RAFT GROUP

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**Who are we and what do we do?** We started as GIST patients and caregivers (spouses and others) in the Gleevec (STI571) clinical trials, and have since extended membership to all GIST patients on Gleevec. We come together to share our experiences and support each other. We focus on symptoms, side effects and other drug-related issues. Members correspond privately to each other and to the group as appropriate.

**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. To assist in that goal, the secure e-mail listserve does not include professional members of the various study sites. However, this newsletter does serve as an out-

reach and is widely distributed. Hence, all items in the newsletter are edited to maintain the anonymity of members, unless members have granted publication of more detailed information.

**Method:** Our primary means of communication is through a confidential, secure listserv operated by the Association of Cancer Online Resources, ACOR ([www.acor.org](http://www.acor.org)).

**Disclaimer:** We are patients and caregivers, not doctors. Any information shared among the group should be used with caution, and is not a substitute for careful discussion with your doctor.

**Newsletter note:** Read at your own risk! Every effort to achieve accuracy is made, but we are human and errors occur. Please advise the newsletter editor of any errors you may find.

## Editor's letter

From the front page

ing me a three-day weekend. He's been good about that, has been since I was diagnosed in June 2000. He covered for me when I went to Houston for surgery and continues to do so when I fly to Oregon for regular checkups.

A few days later, Linda and I were on Saddle Road heading to Kona, driving between the Big Island's two 13,700-foot peaks, Mauna Loa and Mauna Kea. The barely-two-lane road rises up out of jungle and forest before crossing the vast lava plain and dropping to the Kona coast.

"Nervous?" Linda asked as we neared our destination.

"There isn't much to be nervous about. More like excited," I replied.

Meeting people from the list is a lot like Christmas eve. You know something good is coming, even if you don't know what. You imagine and guess, but you're still surprised.

We'd left Linda's cell number at the resort where the Gibsons were staying. When Roberta called, I had Linda's cell phone clipped to my belt. Since I

never use the cell phone, I couldn't figure out why my waistline was ringing. (Fat alarm? Backup alarm for my butt?)

An hour or so later, Linda and I were knocking on the front door. Still excited. And by this time, a bit nervous.

I still haven't figured out which blonde actress Roberta looks like, but she in no way looks like anyone suffering from a life-threatening disease. More like a model for Nordic Track or a fitness center.

And she may be one of the toughest ladies I've met. Standing about 5-foot-7, and tipping the scales at 112, Roberta has been on 800 mg for nearly five months now. She's successfully controlled both edema and severe rash.

Doug is equally impressive. Tall, bearded, friendly yet focused, with an authoritative but quiet voice.

We were supposed to go out to eat, but found it hard to stop talking. That's one of the amazing things about this rare cancer, and the Life Raft Group. It takes strangers and makes them friends. People who live thousands of miles apart become close. Barriers dissolve and intimate revelations become easy.

Roberta and Doug were traveling with Doug's brother and sister-in-law, who eventually got too hungry to wait and too tired to go out, and ordered pizza in.

We did make it to a restaurant that night, and the following night. Two days later, Roberta and Doug drove over to our side of the island and we got to show them around Hilo and Hawaii Volcanoes National Park.

Later, Linda and I both agreed it'd be great to see them again. Which is exactly the message Roberta left on our answering machine the day they left.

If the Life Raft Group is the silver lining of the cancer cloud, meeting other Life Rafterers is the rainbow.

Whenever I see an e-mail from Roberta, I'll see her face and hear her voice, and smile.

Linda and I are ready for a gathering of Life Rafterers. So are the Gibsons. All we need to do is pick the time (next spring?) and place (Las Vegas?). Now is the time to start making plans; we'll chat online.

Roberta and Doug are great people. Maybe *that's* the common denominator with this cancer: it only strikes great people.