

# ACOR starts lobbying for Medicare coverage

Web site offers quick, easy way to e-mail your legislators about bill

**G**illes Frydman, the founder and president of the Association for Cancer Online Resources, has launched a Web site in support of legislation that would make oral cancer medication covered by Medicare.

The Web site, [www.canceradvocate.org/hr1624/](http://www.canceradvocate.org/hr1624/), includes a brief summary of bipartisan legislation by House Resolution 1624 and Senate Bill 913, by U.S. Rep. Deborah Pryce (R-OH-15), titled the "Access to Cancer Therapies Act."

Currently, Medicare does not cover most oral anti-cancer drugs. Cancer drugs are covered by Medicare only if they can be administered through injection by health care providers.

However, biomedical research is beginning to deliver targeted therapies to treat cancer, and many of those new anticancer agents are produced only in oral form.

"Tragically," says Frydman, "Medicare beneficiaries, because of this gap in the current law, won't have access to the targeted new therapies."

Among the important drugs not currently covered by Medicare are Tamoxifen for preventing recurrence of breast cancer, hormone-blocking drugs to delay spread of metastatic prostate cancer, and the new Novartis drug Gleevec that is being touted as a miracle drug for chronic myelogenous leukemia (or CML), a deadly form of blood cancer and is having similar re-

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Battling GIST with Gleevec (STI571)



## LIFE RAFT GROUP

June, 2001 Vol. 2, No. 6 Dedicated to Amy Barney, Jim Perham

## Life Raft patients share responses, side effects

Gleevec credited with tumor shrinkage in 41 of 58 patients; 10 patients report stable disease

**By Norman J. Scherzer**  
Life Raft Group Coordinator  
with **Janet Hendrickson**  
Life Raft Group Medical Librarian

**I**n the Life Raft Group's February Newsletter, we presented the first data showing the response of 16 GIST patient members to STI571 (Gleevec). We defined response as tumor shrinkage, and reported that 87.5 percent of this group responded to Gleevec, most very quickly.

This month we report on the Gleevec response results of 58 Life Raft GIST patients on the trials for at least two months.

We reiterate our standard caveats — we are not professional researchers and any data collected by the Life Raft Group may not be representative of the clinical trials as a whole. Patient-based reporting and analysis is in its infant stages and may not always be accurate. Finally, these are relatively small numbers and small changes can have a disproportionate statistical effect.

The Life Raft Group data is based upon medical updates from 58 of the 60 evaluable member patients on the various Gleevec trials. Two member

patients did not submit medical updates in time for this report.

The table on Page 2 summarizes the Life Raft Group results. Seventy-one percent of the group demonstrated tumor shrinkage, with no differences amongst the three drug dosage groups — 400 mg/day, 600 mg/day, and 800 mg/day.

Seven percent of patients reported their cancer had progressed, and they were taken off the trial.

Seventeen percent remained stable with no change in tumor size.

Of the 41 patients reporting shrinkage, one is receiving Gleevec on a compassionate use basis (the only non-trial member included in our data).

The three patients with mixed responses are an important exception to the more common response patterns and demonstrate the effect of dose change in selected cases. Two patients initially experienced tumor growth that was subsequently reversed by higher doses (from 400 mg/day to 600 mg/day). One patient experienced the reverse — tumor growth following a dose reduction, caused by side effects, from 600 mg/day to 400 mg/day.

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## Gleevec response of the Life Raft Group, 6/12/01

Dosage	400 mg		600 mg		800 mg		Totals	
	No.	%	No.	%	No.	%	No.	%
Progression-Off Trial	1	4%	3	18%	0	0	4	7%
Stable *	5	18%	1	6%	4	31%	10	17%
Shrinkage **	20	71%	12	70%	9	69%	41	71%
Mixed Response ***	2	7%	1	6%	0	0	3	5%
Totals	28	100%	17	100%	13	100%	58	100%

\* Stable includes those with no change in tumor sizes. This is different from the definition used in the ASCO (American Society of Clinical Oncology) papers where we understand stable also included patients with tumor shrinkage under 50%.

\*\* Includes all patients with tumor shrinkage, without regard to amount.

\*\*\* Mixed Response includes the following: Patient A: Tumor growth followed by dose increase (400 to 600) followed by tumor shrinkage. Patient B: Tumor shrinkage followed by tumor growth followed by dose increase (400 to 600) followed by tumor shrinkage. Patient C: Tumor shrinkage followed by dose decrease (600 to 400) followed by tumor growth; now stable but leaving trial because cannot manage side effects.

## Side effects

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### Side Effects

We had hoped to compare the side effect data presented at the recent conference of the American Society of Clinical Oncology (ASCO) with our own, but were unable to do so because of the difference in focus. The ASCO data focuses on high grade toxicity from the perspective of whether patients could stay on the trial and at what maximum dosage level. The Life Raft Group data, on the other hand, focuses upon a patient ranking of side effects by severity, using a scale of severe (or high), medium and low.

In the April newsletter we noted that 34 trial participants reported 78 occurrences of side effects, with 32 percent of the total group ranking one or more as severe. Although we found, as anticipated, that the 600 mg/day group ranked their side effects as severe more frequently than did the 400 mg/day group (54 percent versus 19 percent), we were surprised to find that gender was as significant as dosage in predicting severity of side effects, with 47 percent of all females reporting one or more severe side effects as compared to only 18 percent of males.

Various skin problems (some which we now believe to be neurological) dominated the female severe side effect list.

In a subsequent newsletter we will revisit this side effect issue and present an updated analysis. We can report now, however, based upon this earlier survey, and upon subsequent online discussions at the Life Raft Group Internet-based chat site and at small group meetings of members and loved ones, that the management of side effects is a much more serious issue from the patient's perspective than some other reports indicate.

Some of this is due to the quite natural shift in focus of GIST patients transitioning from the euphoria of surviving what had been a certain terminal disease to the very real prospect of having to take Gleevec, and cope with the management of its side effects, for the rest of their lives.

Some of this is also due to a more realistic assessment of the scope, frequency and severity of side effects when viewed solely from the quality of life concerns of the patient and caregiver than from the clinical trial con-

## Quote:

"We were surprised to learn that in the GIST/Gleevec trials, there is no system for the routine sharing of information gained about side effects between trial centers..."

cerns of the cancer researcher, whose traditional focus has been more on drug responsiveness and toxicity levels determined by the highest dosage that the patient could tolerate.

Finally, a great deal of this is the result of a clinical trial system which fails to adequately measure, evaluate and communicate side effects in a timely and consistent manner amongst clinical trial centers and to provide for adequate specialist support for their management.

For example, we were surprised to learn that in the GIST/Gleevec trials, there is no system for the routine sharing of information gained about side effects between trial centers, particularly between the Phase II and Phase III locations.

# Life Rafter's gather in the Big Apple

By Bernie Kaplan

June 3, a number of Life Raft members met for dinner in New York. Attending were Dan Cunningham and his daughter Melissa (a future doctor), Sid Locker and his wife Milly, Rita Raj and her friend Marilen Danguilan, Anita Scherzer and her husband Norman, Tanya Stutman and her husband Robert, as well as Bernie Kaplan (that's me) and my wife Bracha. Also present was Gilles Frydman, the founder and president of ACOR, the Association of Cancer Online Resources, was also present.

We started off by taking pictures of the group, followed by a toast over wine provided by the restaurant. Norman offered a meaningful toast to our

get together and our deep appreciation to the wonderful doctors and Novartis who created the breakthrough medicine. I added a few more words in praise of the caregivers present.

Over the salad, entree and dessert each of us recounted our tales of GIST and Gleevec. It was inspiring, informative and fun. We learned of each other's histories, aches and pains in the company of those who could understand us best. Interestingly, it was clear that gender is an important factor in side effects.

Norman later pointed out the need for our Life Raft group to continue to impress on Novartis and the doctors the importance of addressing the issue of side effects more seriously.

Gilles provided us with a background to the ACOR Web site which hosts our



Seated, from left, are Sid Locker, Rita Raj, Anita Scherzer and Tania Stutman. Standing, from left: Sid's wife Millie, Dan's daughter Marissa, Anita's husband Norman, Rita's friend Marilen Danguilan, Dan Cunningham, Bernie and Bracha Kaplan, Gilles Frydman, president of ACOR, and Tania's husband Robert.

## Quote:

"What began as a slight awkwardness ended up in hugs and kisses by the end of the evening as we all realized how fortunate and blessed we are."

Life Raft Internet connectivity. If I remember correctly about 75,000 people are registered with ACOR and millions of messages are shared each week. We expressed our appreciation to Gilles for his work.

Sid had brought a TV and VCR and

we were able to see taped news programs of Anita and Tanya and their families, all this over coffee and seven-layer cake. Dan took pictures with his digital camera (see above). Frankly, everyone looked great. Tanya handed out buttons inscribed with GIST in large letters used in her charity work.

I believe all of us attending would recommend to others to get together whenever possible. It provided an unusual opportunity to connect faces to e-mail messages and share our concerns and joys together.

What began as a slight awkwardness ended up in hugs and kisses by the end of the evening as we all realized how fortunate and blessed we are.

*(Bernie, 141 days on STI571 as of this publication..)*



## Amy Barney, 1975-2001



Amy Bowers Barney, 25, died June 10, 2001, in Salt Lake City, Utah, after a valiant three-year battle with cancer. She joined the Life Raft Group in March.

She was born Oct. 3, 1975 in Las Vegas, Nevada, the daughter of Ken and Cynthia Schoonmaker Bowers. She married Reed B. Barney in the Salt Lake Latter-Day Saints Temple on June 15, 1995.

In addition to her husband and parents, she is survived by her 4-year-old son, Joshua; sisters, Laura Crenshaw and Alyson Bowers; brothers, Michael, Paul and Kurt Bowers; and grandmother, Bernice Bowers.

This photo of Amy, Reed and Josh was taken at Disneyland in Anaheim, California just days before her death.

## Cancer advocate

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sults against GIST (gastrointestinal stromal tumor), a deadly form of connective tissue cancer.

Many more new agents, currently in clinical trials, designed to be administered with ease, will not be covered once approved, unless the Medicare program is amended.

Because cancer disproportionately affects the Medicare population., relief for this inexcusable gap in coverage must be provided expeditiously. The program must be updated immediately to allow beneficiaries with cancer to access the new oral drugs as they become available.

At Canceradvocate.org, people can simply type in their zip code and the names of their legislators automatically appear atop a form e-mail that can be customized.

The process of lobbying your lawmakers on this crucial legislation literally takes only a few moments.

## Who's new in the Life Raft Group

Welcome to all new members:  
Jim and Mae P.; she's on the trial in Wisconsin

Joe and Paula T.; he's on the trial at M.D. Anderson.

Marshall M., on the trial in Los Angeles.

Mary and Roy B.; he's on the trial at UCSF in California.

Gordon S., whose daughter Meredith is on the trial.

Melvin R., on the trial at M.D. Anderson

## Want the newsletter sent via snail mail?

Just drop a note to Linda Palmer, 76-A Akala Rd., Hilo, HI 96720. The monthly newsletter of the Life Raft Group is mailed at no charge.

The newsletter is also distributed electronically two ways: via free e-mail subscription, and on the Web site, [acor.org/lrg](http://acor.org/lrg), provided by the non-profit Association of Cancer Online Resources. It comes as a PDF (portable document format) file attachment. You'll need the free Adobe Acrobat Reader installed on your computer. It's very useful since many things on the Internet are PDF files. It lives hidden in your computer and launches only when you click a PDF file. You'll find it at [www.adobe.com/products/acrobat/readstep.html](http://www.adobe.com/products/acrobat/readstep.html)

To add your name to the e-mail mailing list, e-mail the editor at [linda@interpac.net](mailto:linda@interpac.net)

## Editorial

# GIST has become a chronic disease

**T**he dramatic response of GIST to Gleevec is changing GIST from an acute, terminal disease to a chronic, manageable one. Although no one can predict how long GIST will continue to respond to Gleevec and whether any new targeted drugs will arrive to counter any potential resistance, it seems prudent to plan for the management of GIST as a chronic disease.

### A New Paradigm

We would propose a new paradigm for both the conduct of GIST/Gleevec clinical trials and for the management of GIST treatment. We would submit that if we can fast track FDA approval of Gleevec, we can also fast track rethinking how we manage cancer clinical trials and treatment. We do so, as always, in the spirit of a constructive collaboration and dialogue between patients, caregivers and medical providers.

### Suggestions

As a discussion point with our friends and colleagues at Novartis and in the clinical research community, we would offer the following for consideration.

- Establish a minimum effective dose in lieu of the traditional goal of escalating to a maximum tolerable dose. If Gleevec is truly to be considered a targeted drug, the natural extension of that concept is to adjust the dosage to the lowest amount required to bring the disease under control and the lowest amount required to sustain a lifetime maintenance level. We now know that when Gleevec works, it seems to do so very quickly, enough time to increase dosages on a

selective case base where that seems indicated.

- Make the patient's assessment of type and severity of side effects as important as that of the clinician's assessment of high grade drug toxicity.

- Strengthen the clinical trial and patient treatment infrastructure to improve the gathering and dissemination of information between clinical trial physicians and private practice physicians, and provide a core specialist team to evaluate and support the management of side effects. In the GIST/Gleevec trials, a small team consisting of a dermatologist, a neurologist, and a gastroenterologist, coordinated perhaps by an internist and an oncologist, could make a major

difference to the lives of both patients and physicians.

- As quickly as technology and resources allow, make genetic testing a routine part of patient treatment strategy, including finishing the job of teasing out the subtleties of genetic mutations and treatment strategy.

As an aside, we would note how the management of GIST has changed. Only a very short time ago, we were not even distinguishing GIST from broader category diagnoses like leiomyosarcoma and here we are today looking at whether a GIST patient tested for a c-kit oncogene mutation, in what exon that occurred and in what part of the exon. Incredible.



*By Norman Scherzer,  
Life Raft Group coordinator*

# Life Rafter Jim Perham: Never forgotten

A daughter's tribute,  
a Father's Day essay

By **Kathy Perham-Hester**

**M**y sons, age 7 and 9, were silent. We had sat them down to tell them the news that Grandpa Jim, my dad, had died.

Corey, my eldest, spoke first. His head was downcast; his eyes were cherry red but he didn't allow himself to cry.

"Will we get a new Grandpa?"

Corey's first words struck me as peculiar but innocent. It wasn't until a few hours later that I had a better understanding of where he was going with his query.

"Mommy," he said matter-of-factly, "I don't want another Grandpa."

As I write this it has only been three weeks since I lost my dad, Jim Perham, to heart disease. What a shock of a lifetime. It still doesn't seem real. He was only two weeks shy of his 64th birthday and oh what a celebration we were going to have.

You see, he had been given a second chance at life. Two years ago he had had surgery to remove a very rare malignant tumor. Last Thanksgiving the cancer returned, leaving hopelessness in its wake because there was no cure.

Then miraculously, a lifeline was thrown out — an experimental drug showed promising results of combating his type of cancer, gastrointestinal stromal tumor, or GIST. He got into a clinical trial in Oregon. He had just had a checkup and by all accounts, this drug (now known as Gleevec) was taking his cancer to the cleaners after only three months.

Unfortunately, everyone was so focused on the cancer that the buildup in one artery of his heart was somehow missed.

Dad came to Alaska the year of statehood, 1959. For 35 of the 42 years he lived here, he worked for the Federal Aviation Administration (FAA). Whether at work or not, you could count on Dad to have an opinion and to share it.

An FAA co-worker shared with me that years ago Dad put a block of wood inside a used box of soap and would actually "get on his soap box" to share his view. He wasn't content with banal statements either. A simple "How're you doin'?" would elicit "Oh, I'm shuckin' and jivin' down the bike path of life pursuing my chosen avocation with vim and vigor." He had a quit wit and a laughter that embraced you.

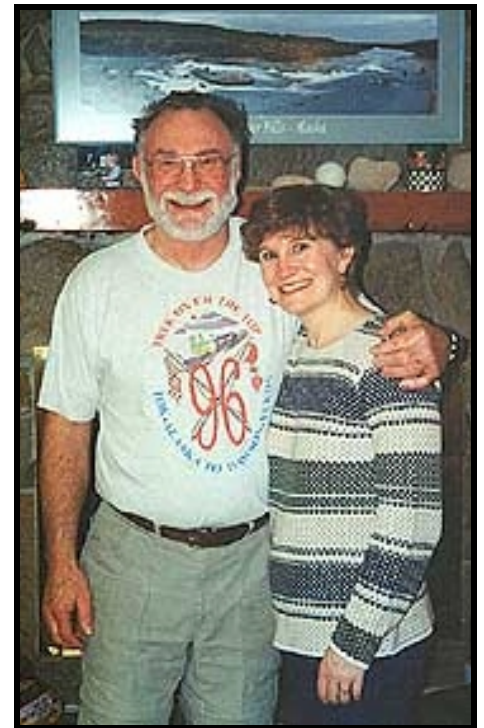
As far as fathers go, he was always there for me, as a child and as an adult. As a teen-ager, I remember him teaching me self-defense moves after I was attacked while running. Dad actually held a black belt in karate and, at that time, was a karate instructor for the Anchorage Police Department. I thought it was so cool to say that my dad trained the SWAT team in karate moves.

As an adult, I continued to be on the receiving end of his generosity and good graces. They extended to my husband and children, just as they did to anyone he came in contact with.

At his memorial service, Pastor Oldfield's sermon title was "I Want to be Like Jim Perham!" Just reading the title for the first time made me weep for in those seven words was a deep, personal, undeniable respect for a man who loved life but who was no longer around to share life with. And the fact of the matter is that that opinion was not just something one man said about another in isolation. Every person in church that evening paid their respects to a man who was a role model for all - who loved life and who loved his Lord and wasn't afraid to share that

fact. Jim Perham made an impact on many lives; he will not be easily forgotten.

This Father's Day will be very bitter-sweet. It will be the first time without



Jim Perham, with daughter Kathy Perham-Hester of Anchorage, Alaska.

someone there to say "Happy Father's Day, Dad." I guess this will have to be the first of many. It's already been six years since we lost Mom. It's not a good feeling to know you're an orphan of sorts.

There is one feeling, though, that I can share with conviction — I am very proud to be recognized as Jim and Sandy Perham's daughter. I am very proud of my Perham family name and there is nothing on God's green Earth that can diminish that feeling in my heart and soul.

That's my tribute to Dad this Father's Day and every one hereafter. Love you, Dad.

*Kathy Perham-Hester lives in Anchorage.*



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**Who are we and what do we do?** We are a group of GIST patients and caregivers (spouses and others) in the STI571 (Gleevec) clinical trials who have come together to share our experiences and support each other. Persons not in the trial are encouraged to seek support from the broader leiomyosarcoma (LMS) community. We focus on side effects, symptoms and other drug-related issues. Members correspond privately to each other and to the wider 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 listserv does not include professional members of the various study sites. However, this newsletter does serve as an outreach 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.

## Dr. Alex Matter wins Harvard's Alpert award

EAST HANOVER, N.J.  
(PRNewswire)

**D**r. Alex Matter, therapeutic area head of Oncology Research at Novartis Oncology, is one of several recipients of the 13th annual Warren Alpert Foundation Scientific Prize for his work in the discovery and preclinical research on Gleevec (imatinib mesylate), a unique oral medication that has demonstrated efficacy against chronic myeloid leukemia (CML).

Administered by Harvard University, the prize is awarded to scientists who have harnessed their basic-science discoveries into practical applications that have a dramatic impact on patients.

Matter will share the \$150,000 prize with his co-awardees — David Baltimore, Ph.D., president and professor of biology at California Institute of Technology; Dr. Brian J. Druker, professor of medicine, Oregon Health Sciences University; Nicholas B. Lydon, Ph.D., vice president for small mole-

cule drug discovery, Amgen, Inc.; Dr. Owen N. Witte, Howard Hughes Medical Institute investigator, and David Saxon, presidential professor in developmental immunology, UCLA.

The prize was presented at a May 1 ceremony in Boston.

"I am honored to be recognized, along with my co-awardees, by Harvard Medical School and the Alpert Foundation," said Matter. "I am also proud to represent the scientists at Novartis that worked so diligently on this drug, which is helping thousands of people each year. Working on the discovery and development of Gleevec has been a once-in-a-lifetime opportunity for everyone involved."

### **Matter and the discovery of Bcr-Abl**

Matter began building a cancer research unit focused primarily on inhibition of kinases (Bcr-Abl is one) in 1983, when he headed the research team at the company then known as Ciba-Geigy (now Novartis) in Basel,

Switzerland. By early 1990, Matter and his team, including Lydon, discovered Bcr-Abl inhibitors and focused their efforts (in close collaboration with Druker) on one particularly promising compound, which eventually became Gleevec.

Gleevec is an oral signal transduction inhibitor. The first Phase I study on CML patients began in June 1998, led by Druker.

The initial results were extremely promising and the company devoted significant resources to accelerate the compound's development. On Feb. 27, 2001 — 32 months after the first clinical trial was initiated — Novartis submitted new-drug applications to marketing authorities globally. In the United States, the application was granted priority review by the Food and Drug Administration (FDA) on March 7, and was approved for use in treating CML on May 10 — one of the fastest approvals ever for a cancer therapy.