IN OUR TIME
A NEWSLETTER OF THE
SARCOMA FOUNDATION OF AMERICA

Winter 2007

From the Executive Director
Matthew Alsante

Since its inception in 2000, the SFA has been working to fund early stage and translational research focused on discovering new pathways to treat all types of sarcomas. In just a few short years, we have awarded 23 research grants, two American Society of Clinical Oncologists (ASCO) Young Investigator awards, and we are in the process of supporting an ASCO Advanced Clinical Research Award (ACRA) valued at $450,000 over a three-year period.

We are especially proud of the ACRA grant, which was made possible through partnership with the SFA, ASCO and the Capon Family. We believe that by privately funding a long-term research grant of this magnitude, we can continue to attract high caliber researchers who are interested in and capable of pursuing a significant sarcoma research project. It also demonstrates to all of us who are part of the sarcoma community that the dedication of a small number of people united for a cause can truly make a difference!

CELEBRATING 5 YEARS OF PROGRESS

The 5th annual SFA Gala was held on May 21, 2007 at the New York Marriott Marquis. With over 500 people in attendance representing the sarcoma patients and their families, friends, medical professionals, corporate and philanthropic communities, this year’s gala was again a resounding success. The theme of the evening, which was chaired by Susan and Elliott Rose of Westchester, was the fifth anniversary of the event. Honorees and chairs from years past were recognized, as were founding sponsors such as Pfizer, Novartis, Savory Sojourns and Devachan. Other sponsors of the evening included ARIAD, Amgen, IDM Pharma, Genentech, ZIOPHARM, Fizzy Lizzy, WD-NY and ICC/Decision Services.

Master of Ceremonies Ed Fry introduced the event chairs, and then presented two awards: The Visionary in Medicine Award, which was given to Scott Gottlieb, M.D. a former Deputy Commissioner of the Food and Drug Administration; and the Leadership in Courage Award, which was given to sarcoma survivor and ACOR List Manager Rose Burt. A live auction raised $90,000 led by auctioneer Jeffrey Gould. Music for the evening was provided by Victor Lesser and the Manhattan City Orchestra.

The program culminated with two unforgettable moments. American Idol finalist and 2007 SFA spokesperson Anthony Fedorov performed a song he had written for his brother, Denis, whom he lost to sarcoma in 2006. And Terra Holloway, daughter of former SFA board member Marny Tobin—a longtime supporter of the SFA who had lost her battle with sarcoma earlier in the year – toasted her mother by launching a new wine created by Willow Crest Winery in her honor.

Save the Date:
The 2008 Sarcoma Foundation 6th Annual Gala Dinner will be Monday, May 5th, at 583 Park Avenue in New York City.

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**Update on the Sarcoma Patient Registry**

Due to the fractured nature of the US healthcare system, there is currently no universal database that provides access to information on sarcoma patients. This makes it challenging for public or private researchers to gather enough data to fully study genetic pathways and identify therapeutic candidates for all but the most common sarcoma sub-types. The SFA hopes to help address this issue by creating a comprehensive Sarcoma Patient Registry that will track the progress of patients with sarcomas.

Currently in a trial phase, the Registry is beginning to collect epidemiological data from existing state registries on the rarest subtypes. By combining the data from other registries around the country and gathering new data from current and future patients, the SFA’s Sarcoma Patient Registry is designed to one day be a powerful source of information for scientists on the hunt for markers, genetic pathways and effective therapies for sarcomas.

Eventually, the registry will become a repository of data for all sarcoma sub-types, capturing information on individual patient diagnosis, treatment and outcomes, while at the same time adhering to the highest principles of patient privacy rights. Its overarching goal is to be a virtual “historical control group” that would help streamline clinical trials in sarcoma sub-types.

We are in the process of registering patients with the following sub types: Dermatofibrosarcoma Protuberens, Clear cell sarcoma, Alveolar soft part sarcoma, Myxoid liposarcoma and Osteosarcoma.

Please contact: sfa@registypartners.com or call (336) 343-1034 or Toll Free (866) 501-6780

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**President’s Corner**

As I reflect on the year 2007, I see it as a year of both wins and losses for patients with sarcoma. Gleevec, which is currently approved to treat GIST, gained FDA approval for patients with the extremely rare dermatofibrosarcoma protuberans (DFSP). Another promising therapy, Junovan, which applied for approval to treat children suffering from osteosarcoma, was rejected by the FDA in August.

Successes and failures in gaining drug approvals for sarcoma sub-types have led us to seek more predictable pathways for drug developers to follow in their quest for new targeted therapies that effectively treat sarcoma patients. Through outreach to government leaders and others, we are creating a Sarcoma Patient Registry that will track sarcoma patient progress on various therapies. This registry will one day aid investigators by providing information on the nature of sarcoma sub-types.

This year the sarcoma community also learned that there would be significant cuts in government funding for new sarcoma research initiatives. We were disappointed to find out that the National Cancer Institute (NCI) planned to cancel its funding for all sarcoma clinical trials due to budget constraints. After instigating a well-intended but ultimately unsuccessful grassroots write-in campaign demanding the restoration of NCI funds for sarcoma clinical trials, the SFA later learned that sarcoma research projects would now be eligible to compete for the highly valued and prestigious NCI Specialized Program in Research Excellence (SPORE) grants alongside the other more “popular” cancers. So again, the message is: some progress and some setbacks.

Another element of positive news is the emergence and maturation of the Sarcoma Alliance for Research Consortium (SARC). This coalition of North American sarcoma medical centers has been very effective in attracting the interest of private pharmaceutical research investment to develop some promising therapies specifically for sarcoma patients. Farthest along is the Deforolimus (formerly known as AP23573), which has begun a Phase III clinical trial in cooperation with SARC. If Deforolimus proves successful in clinical trials and gains FDA approval, this would mean a new treatment option for sarcoma patients.

The SFA has been very fortunate to have Executive Director Matthew Alsante, the board and staff actively involved in regional fundraisers, a landmark educational conference and the production of our 5th anniversary gala dinner. We were also honored to have American Idol finalist Anthony Fedorov join with us as our celebrity spokesperson. When his brother Denis was diagnosed with Ewing’s Sarcoma in 2005, Anthony and his family were personally drawn into the world of cancer. As spokesperson, Anthony has brought a passion and energy to the cause, helping to raise awareness about sarcoma by appearing on network television news shows and performing at the gala. We cannot thank him enough for his dedication.

The upcoming year promises to have further challenges and opportunities as sarcoma fights to receive a fair share of resources and attention during times of fiscal constraint. By joining forces and working together, the sarcoma community has the power to make our voices heard. It’s up to us to roll up our sleeves and take on the challenge. With a little “do-it-ourselves” attitude, and with the help and support of donors, families and friends, we will find a cure in our time.
SFA Expands through State Chapters

In April of this year, we set out to increase the scope and reach of the SFA by further developing our reach at the state level. Just a few months later, we have increased the number of chapters from 9 to 25. State Chapters are led by dedicated Chapter Leaders, community-minded individuals who have volunteered their time to educate local communities about sarcoma, create awareness about the disease and raise funds for research.

The state-level grassroots network is an integral component to the SFA’s mission. By increasing awareness of sarcoma and the devastating effect of the disease on the children and adults diagnosed with it, we hope to create the momentum to affect public policy in a way that will result in increased funding for research into the disease.

SFA’s passionate and energetic Chapter Leaders are essential to the realization of finding a cure in our time!


Please contact us at info@curesarcoma.org

MEET THE EXPERTS EDUCATIONAL CONFERENCE

This year SFA hosted a landmark educational conference for sarcoma patients, their families, caregivers and other medical professionals. The half-day program featured a distinguished panel of experts who discussed the history and progress of treating the many forms of adult and pediatric sarcomas.

Attendees traveled from across the country to learn about the latest research, treatments, progress and myths surrounding different forms of sarcoma treatments including chemotherapy, radiation, immunotherapy and surgery.

“There has been an evolution from neglected disease to a prime target disease,” said Dr. George Demetri of Dana Farber. “Lots of attention is now being paid to sarcoma, driving new researchers and investigators to become interested in the disease.”

Success in the field is making a difference in patient lives. The discovery that a targeted therapy called Gleevec was a powerful treatment for gastrointestinal stromal tumors or GIST, as they are now known, was eye opening to researchers who saw that they could identify pathways to stop cancers in its tracks. Sarcomas are now a model disease type for developing “mechanism-based” personalized medicines that work at the simple DNA level.

While reviewing a list of the most promising therapies for sarcoma patients-- including FDA-approved Gleevec and Sutent, and other new therapies in clinical trials that target the IGF-1, MET, MTOR and AKT receptors -- Demetri pointed out the importance of more public support for a rare disease like sarcoma. Organizations devoted to sarcoma patients and other cancers need to work together. It’s important for “…care providers and educators to break down old walls for the good of patients and for the good of the family,” said Demetri.

Moderated by SFA president Mark Thornton, M.D, Ph.D. and SFA board member Tom Tobin, M.D., the distinguished panel of presenters included the following sarcoma experts: Jack Brooks, M.D., University of Pennsylvania Hospital; David D’Adamo, M.D, Ph.D., Memorial Sloan Kettering Cancer Center; Robert Maki, M.D., Ph.D., Memorial Sloan Kettering Cancer Center; George Demetri, M.D., Dana-Farber Cancer Institute; Robert Gorlick, M.D., Montefiore Medical Center; Robert Maki, M.D., Ph.D., Memorial Sloan Kettering Cancer Center; Shreyas Kumar Patel, M.D., M.D. Anderson Cancer Center; and Samuel Singer, M.D., Memorial Sloan Kettering Cancer Center. Conference presentations can be viewed on our Web Site at www.curesarcoma.org

Save the Date: On Sunday, May 4th, 2008 the SFA will host its 2nd annual Educational Conference entitled New Frontiers in Sarcoma in New York City.
I recently participated in a focus group to evaluate the Lance Armstrong Foundation’s Survivor Handbook. Although I had received a copy of this resource book for cancer patients and their families, I had not taken the time to really delve into the book. The focus group work led me back to it, and what I found was eye opening. The LIVESTRONG Survivor Handbook is a how-to bible for cancer survivorship. Not just for the patients but for their families and caregivers as well.

It made me think back to my original experience of first being diagnosed, entering the treatment phase and moving through the cancer networks of NYC to find my place and my space in this new and foreign world—the world of cancer.

For me, it all began with a simple surgery, only it didn’t turn out to be so simple. Eight hours and 30 pints of blood later, the surgeons of Columbia Presbyterian Hospital just managed to save my life and get me stable enough for the ICU. They had removed buckets of tumors from my pelvis, all the while trying to halt the DIC (Disseminated Intravascular Coagulopathy) bleeding that occurred during the surgery. Two weeks later, my surgeon informed me that I had leiomyosarcoma. That was November 2003, and I have been undergoing some form of chemotherapy nearly continuously since that period. Yet, in spite of having to incorporate a treatment schedule that is heavy on hospital visits, scans and uncertainty, I see my life as going only up hill from the time of my diagnosis. I realized early on that I’m a survivor, and though it was not my conscious choice to get cancer, I can choose how I deal with it. The LIVESTRONG Survivor Handbook reminded me of my strength and my capacity to survive. Its first words are “Before I just lived. Now I live strong.” It’s a great motto for those of us who have faced cancer square in the face and come to the realization that we are survivors, be it for one day, three months, 10 years or more.

Along with being motivational, the handbook provides you with a wide range of resources to help you be an empowered cancer patient. It covers topics from the practical, like getting your records in order, to the physical, including a great section on the effects and after effects of various therapies, to finally the emotional—how to get help while you are dealing with cancer.

Go to www.livestrong.org to request a free copy of the The LIVESTRONG Survivor Handbook (shipping and handling charges are extra).

Candace Sue has been an SFA board member since 2005.
Recently, SFA co-founder Patricia Thornton sat down with Dr. Malay Haldar to learn about his scientific research using mouse models to unlock new treatment pathways for synovial cell sarcomas.

**Patricia Thornton:** How closely do the mouse tumors resemble their human counterparts?

**Malady Haldar:** The tumors in our mouse model significantly resemble their human counterparts clinically and pathologically. However, the tumors in our model were generated by expressing the SYT-SSX2 fusion protein in only one population of cells -- myoblasts of skeletal muscle lineage. The situation might be somewhat different in humans where more than one type of cells could potentially be a source of this disease. We are currently investigating whether expressing SYT-SSX2 in other cell types could also generate synovial sarcoma in mice. The clinical significance of this issue is that response to therapy or tumor prognosis could be dependent upon its cell of origin.

**Patricia:** How will your mouse model lead to new treatments for synovial cell sarcoma and other sarcoma sub-types?

**Malady:** The process of identifying a candidate drug to its first experimental use in humans in a clinical trial is a long process involving significant investment of time and resources. Very few potential drugs ever make it to the bed side. Most candidate drugs that seem to perform well in vitro in a Petri dish fail to perform in vivo in a complex living being.

The availability of a mouse model facilitates this process greatly. Mice are quite similar to humans genetically and physiologically and therefore a candidate drug having promising performance in a mouse model has good prospects of performing well in humans. Moreover, large scale, well designed trials can be performed easily in mice rendering the trial more powerful while such large scale studies are often fraught with problems in human subjects for ethical, technical and medical reasons.

The mouse model of synovial sarcoma that we have recently generated can be used to evaluate any new candidate drug or treatment. Such drugs or treatment could be either synovial sarcoma specific or for sarcomas in general. The tumor-inducing event is unique in synovial sarcoma (expression of SYT-SSX oncprotein) but it most likely shares a lot of downstream pathways with other similar sarcomas. A new drug, similar to the majority of existing chemotherapeutic agents, could be effective for several different types of sarcomas and hence our mouse model can be used flexibly in pre-clinical trials.

Apart from using the mouse model as a preclinical platform for evaluating new drugs, this model can be used very effectively to study the genetic and molecular anomalies that underlie this disease and that knowledge can be used to design more effective therapies.

**Patricia:** If you had funding, what would be your next step to develop a novel sarcoma therapy?

**Malay:** In my opinion we still need more biological insight to design a targeted novel therapy. Therefore, I would continue to study the pathogenesis of this disease especially looking into mechanisms that could be vulnerable to therapeutic interventions. One intriguing possibility that is specific to synovial sarcoma is to target the SYT-SSX protein itself. We know that SYT-SSX protein induces synovial sarcoma. However, we don’t know whether its continuous presence is required for the survival and proliferation of tumor cells. If this is the case, then SYT-SSX offers a unique therapeutic target since it is only expressed in tumor cells and not in the normal tissue. We are currently investigating whether taking away the SYT-SSX protein after the tumor has progressed makes the tumor regress or at least stop growing.

The results of these studies have important implications in determining whether SYT-SSX offers a real target for drug development in synovial sarcoma.

**Malay Haldar, M.B.B.S., Ph.D.,** is a research associate at the Howard Hughes Medical Institute at the University of Utah. A 2005 and 2007 SFA grantee, Dr. Haldar published an article based on his work called “Cancer Research: Progress Against Sarcoma” in the April issue of Science Daily.

Patricia Thornton is a co-founder of SFA. At the age of three, her son faced a rare type of sarcoma, called Alveolar Soft Part Sarcoma.

Spotlight On: Cure Magazine’s fall issue provides a compelling perspective on sarcoma called The New Sarcoma Story: Strategies for Diagnosing and Treating Sarcomas Shift to a More Patient-Specific Approach. View the article at www.curetoday.com
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The mission of the Sarcoma Foundation of America is to act as an advocate for increased research to find new and better therapies with which to treat patients with sarcoma.

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