4TH ANNUAL GALA AWARDS

On May 22, 2006, The Sarcoma Foundation of America (SFA) honored author and journalist Carole Radziwill, Pfizer, Dr. Laurence Baker and the Rumely Family with awards at the fourth annual SFA Gala Dinner and Auction held at the American Museum of Natural History in New York City. The event raised over $720,000 toward research for a cure for one of the rarest cancers in the world.

Pfizer received the Symbol of Caring Award for Corporate Responsibility in honor of their recently approved cancer therapy, Sutent, a new targeted anti-cancer treatment for GIST (gastrointestinal stromal tumors) resistant to imatinib, a rare form of sarcoma. The award was presented by Dr. Robert Maki of Memorial Sloan Kettering Cancer Center.

Laurence Baker, D.O., received the Nobility in Science Award acknowledging his dedication to advancing the study of sarcoma. Dr. Baker is Professor of Medicine and Pharmacology, University of Michigan Medical School. His award was presented by FDA Director Dr. Richard Pazdur.

In addition, the Rumely Family was awarded the Symbol of Caring Award in honor of their research endowment in the name of husband and father Paul Rumely, who succumbed to sarcoma. Vincent Pica presented this award.

Bestselling author Carole Radziwill was honored with the Leadership in Courage Award for penning her New York Times bestselling memoir What Remains, the moving story of Radziwill’s experience of losing her husband to sarcoma after a five-year battle the same summer that her husband’s cousin and best friend John F. Kennedy, Jr. and his wife (and Radziwill’s best friend) Carolyn Bessette Kennedy were killed in a plane crash. Her award was presented by her friend Diane Sawyer of ABC News.

“What Remains is a powerful story of love, loss and resilience that has resonated throughout the sarcoma community, and Carole’s courage and candor has touched and inspired us all,” said Mark Thornton, president of SFA. “These awardees represent our allies in the fight to cure sarcoma. In their own unique way, each has contributed to the advancement of our cause.”

2006 Dinner Chairs included JoMarie and Vincent Pica and Alison and David Rich. Major corporate sponsors for the evening include Pfizer Inc, ARIAD Pharmaceuticals Inc., Cache, Lillie Rubin, Verizon, Novartis, Savory Sojourns and HBO.

Save the Date: On May 20th-21st, 2007, SFA will host its the 5th Annual Spring Gala and Educational Conference at the New York Marriott Marquis in New York City. Look for more details on both events coming soon at www.curesarcoma.org

What Is Sarcoma?  A rare cancer of the connective tissue, sarcomas account for one percent of adult cancers and close to twenty percent of all childhood cancers. It is sometimes curable by surgery (about 20% of the time) or by surgery with chemotherapy and/or radiation (another 30-40%) but nearly half of the time sarcomas are resistant to all of these approaches—thus the extreme need for innovative new therapeutic approaches.

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President’s Letter

Greeting SFA supporters!

Much has happened over the course of 2006. This May we were very fortunate to have hosted a successful 4th Annual Gala event at the prestigious American Museum of Natural History in New York City. We had an unprecedented number of guests, a fantastic lineup of honorees, celebrity presenters and musical guests, and an amazing group of sarcoma families and supporters who joined us to raise money for sarcoma research.

For the second year and on the same day as the gala, we hosted the Sarcoma Power Lunch featuring knowledgeable sarcoma specialists who took time out of their busy schedules to educate patients and families on the latest advances in research. The lunch also updated participants on two SFA initiatives—the Sarcoma Patient Registry and the Exceptionally Rare Diseases Act. Read more about them in this newsletter.

Most recently, we welcomed new executive director Matthew Alsante. A career non-profit manager, Matthew has ten years of experience in the area of blood banking, grassroots organizing and development. He took the helm in November.

And sadly, we mourn the passing of our friend Marny Tobin, who served as SFA secretary from 2001 until 2005. In addition to being a board member, Marny led chapters, first in Pennsylvania, then in Washington. She also ran a marathon in 2002 and donated the proceeds to SFA. Herself a sarcoma survivor, Marny will be remembered for her unfailing dedication to SFA and to the sarcoma community.

Finally, we hope you will consider making a donation to support our efforts to further research and advocacy on behalf of sarcoma patients and their families. Your contributions help us to continue our work to find the cure for sarcoma in our time.

Best Regards,

Mark Thornton, MD, PhD
President

Meet Matthew Alsante, Executive Director

Matthew Alsante joined SFA as executive director in November 2006. Hailing from Syracuse, New York, Matt was most recently at the Children’s National Medical Center in Washington D.C. managing the Blood Donor Center.

Before that he was an operations manager with the American Red Cross.

When asked about his plans for SFA, Matt said, “I am working on a strategic plan focused on enhancing communications with the sarcoma community, reinvigorating our state chapters and advancing the patient registry.”

“We welcome Matt to SFA, and we are confident in his ability to move the organization forward as a major force in sarcoma patient research and advocacy” said Mark Thornton, president of SFA.

Email Matt at malsante@curesarcoma.org

DONATE TO SFA

Our mission is to act as an advocate for increased research to find new and better therapies to treat patients with sarcoma, and we welcome your support! Please consider making a donation to SFA today. Donate online at www.curesarcoma.org or make your check payable to: The Sarcoma Foundation of America, 9884 Main Street, P.O. Box 458, Damascus, MD 20872.

The Sarcoma Foundation of America is a national, not-for-profit advocacy group for the research and treatment of sarcoma (Tax ID #52-2275294).
THE EXCEPTIONALLY RARE DISEASES ACT
Advocating for Sarcoma Patients in Congress

This year, SFA brought the fight for sarcoma advocacy to Washington’s doorstep by developing and leading the charge for a new congressional bill. The Exceptionally Rare Diseases Act attempts to give those suffering from the rarest of diseases, including sarcoma patients, the hope of accessing FDA approved treatments and interventions that would otherwise remain unapproved for their conditions.

Through experience we have learned that with many rare cancers there are simply not enough patient volunteers available to conduct the clinical trials in the manner presently mandated by the FDA.

In view of this dilemma, the FDA sought guidance on what could be done to resolve it. In November 2005, the FDA Oncology Drugs Advisory Committee (ODAC) met, heard the issues involved and recommended that reforms be made. ODAC urged the adoption of the European model here in the U.S. However to date, new regulations or guidelines have not been implemented to deal with this problem.

To encourage progress in this area and advocate for action, SFA and its legal counsel, Van Ness Feldman and Arnold & Porter, carefully studied Federal Advisory Board recommendations and worked to create legitimate legislation that captures both the spirit and specific remedies of the ODAC.

The resulting Exceptionally Rare Diseases Act is based upon the European model, already in place there, which allows the number of participants required in clinical studies to be reduced when approval is sought for the introduction of a drug aimed at an exceptionally rare disease, such as sarcoma. The legislation is also aimed at establishing a new mechanism to verify the clinical benefits of drugs for the treatment of rare cancers and other extraordinarily rare diseases which have been approved under accelerated approval procedures.

SFA was also instrumental in forming a separate lobbying group, The Alliance Against Exceptionally Rare Diseases (www.taaerd.org) which is attempting to unite all ultra-rare disease groups to this common cause.

In practical terms, passing this legislation means that patients who are suffering from ultra-rare diseases will have access to many more drug therapies than currently exist. It is our hope that those who were once doomed to die because they were unable to obtain necessary treatment might instead be saved.

Driving to have this legislation enacted, SFA representatives met with U.S. Senator Mark Pryor, himself a sarcoma survivor, and congress members Steve Israel and Nydia Valezquez of New York; and Deborah Pryce of Ohio, among others, to advocate on behalf of the Exceptionally Rare Diseases Act.

For a copy of the Exceptionally Rare Diseases Act, go to www.curesarcoma.org

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2006 SFA Research Awards

Shelby L. Richter Memorial Research Award
Tsz-Kwong Man, Ph.D., Assistant Professor of Pediatrics, Baylor College of Medicine Texas Children’s Cancer Center for “Early identification of chemoresistant patients for improved risk-based therapy in pediatric osteosarcoma”

Bradley J. Breidinger Memorial Research Award
Brent R. Stockwell, Ph.D., Assistant Professor, Departments of Biological Sciences and Chemistry, Columbia University for “Discovery of small molecule inhibitors of the MDM2-ubcH5 interaction”

Close Family Research Award
Rula Geha, M.D., Department of Surgery, Memorial Sloan-Kettering Cancer Center for “Ribonucleotide reductase as a novel target in liposarcoma”

Brian J. Monaghan Memorial Research Award
Stacie Goldberg, M.D., Department of Pediatrics, Memorial Sloan-Kettering Cancer Center for “Identifying native and heteroclitic epitopes for T-cell immunity to PAX3-FKHR alveolar rhabdomyosarcoma”

Paul M. Rumely Memorial Research Award
Yuechueng Liu, Ph.D., Associate Professor, Department of Pathology, University of Oklahoma Health Sciences Center for “Blocking SYT-SSX activities using an inhibitory peptide”

Seth A. Bailey Memorial Research Award
Karl G. Sylvester, M.D., Assistant Professor, Department of Surgery, Stanford University for “Cancer stem cells and sarcoma”

ASCO Young Investigator Award 2006
Raymond Meng, MD, PhD, Memorial Sloan-Kettering Cancer Center for “Targeting the Notch Signaling Pathway: A Novel Method to Inhibit Sarcomas with Constitutive Ras Activation”
Project 53 – Path to a Patient Registry

Early in 2006, the FDA announced a sweeping program entitled the “Critical Path Initiative” which is designed to speed new drug developments for patients suffering from rare diseases. Of the 76 topics on the “Critical Path List”, Item #53 was specifically relevant to the sarcoma community.

Item 53 addresses a major problem in drug development for ultra-rare diseases – not enough patients to properly perform the clinical trials necessary for FDA approval. It also states that if a rare disease group can properly (i.e., to FDA’s high standards) profile the natural history of the disease, this natural history database can be used as a “virtual historical control group” in a clinical trial of a drug to be tested to treat the disease.

Establishing a virtual historical control group for sarcoma patients would mean the ability to greatly streamline clinical trials for some of the rarer sarcoma subtypes, and the mere existence of a streamlined pathway might entice drug developers to aim their investment in cancer drug development towards the sarcoma community.

So what does all of this have to do with establishing a Sarcoma Patient Registry? Simply put, the first step on the path of developing the natural history database is to acquire as much medical information as possible on as many sarcoma patients as possible.

To do this, the SFA has begun the process of data acquisition via establishment of a patient registry. We are starting small and will learn as we grow. Our first step was to ask our Medical Advisory Board (MAB) if any of the 100+ sarcoma subtypes had a more desperate diagnosis than others. In other words, the patients with sarcoma subtypes who are most likely to be told that nothing we have works at all against your type of sarcoma. The MAB gave us a list of 5 to 7 very rare subtypes that meet these criteria and these groups (totaling less than 1,000 patients) will be the first types to be gathered in the registry. All information gathered will carefully follow all patient privacy rights as directed by federal law.

As time goes on and funding for this project grows, we will add more and more sarcoma subtypes. Our goal is to have all 50,000+ sarcoma patients in what we are calling “Project 53.”

Learn more about “Project 53” by visiting www.curesarcoma.org