



INSIDER

THE DESMOID TUMOR RESEARCH FOUNDATION — FUNDING RESEARCH ~ PROVIDING SUPPORT

The DTRF Supports Research to Find a Cure

NEWLY-FUNDED PROJECTS

Benjamin Alman, MD

The Hospital for Sick Children, Toronto

"Identifying novel drug therapies for desmoid tumors"

Dina Lev, MD – MD Anderson Cancer Center

"A rational search for novel anti-desmoid therapies"

ONGOING RESEARCH PROJECTS

Stephen X. Skapek, MD – The University of Chicago

"Deregulated mTOR in desmoid-type fibromatosis: identification and validation of a new therapeutic agent"

Matthew van de Rijn, MD, PhD – Stanford University

"ROR2, a potential novel therapeutic target in desmoids"

Richard Lackman, MD – University of Pennsylvania

"The role of hydroxyurea as first line treatment for primary and recurrent/refractory desmoid tumors"

RECENTLY-COMPLETED PROJECTS

Andrew Beck, MD – Stanford University, gift in support of Dr. Andrew Beck's research evaluating large intergenic non-coding RNA's in desmoid-type fibromatosis.

Benjamin Alman, MD

The Hospital for Sick Children, Toronto

"Identifying the desmoid tumor initiating cell"

Dina Lev, MD – MD Anderson

"Molecular determinants of desmoid tumor development and progression"

DTRF-Funded Research Seeks Identification and Validation of a New Therapeutic target.

Two major clinical problems are unresolved for patients with desmoid-type fibromatosis. First, what can be done to prevent disease recurrence in those patients with resectable disease? And second, how can unresectable disease be controlled? Stephen X. Skapek, MD, an Associate Professor in the Department of Pediatrics, Hematology/Oncology, at the University of Chicago is seeking answers to these problems.



Dr. Skapek has led two multi-institutional clinical trials in children with desmoid tumors that were either recurrent or not easily amenable to surgical resection or radiation. His current project, funded by DTRF, is a pilot study examining the role of Sirolimus (rapamycin) in the treatment of children and young adults with desmoid tumors that are deemed likely to recur following resection.

Sirolimus (rapamycin), is a drug that is currently being evaluated as an anti-cancer agent in a variety of tumor types. Sirolimus and several related drugs have been used in children and adults to suppress the immune system following transplant and, more recently, as an anti-neoplastic agent in a variety of cancer types. It is attractive as a potential drug for desmoid tumors because it is well-tolerated in children and young adults and it can be given orally.

Sirolimus directly targets and inhibits a pathway that may be critical for the development and growth of desmoid tumors called the mTOR pathway. Dr. Skapek's project is testing the hypotheses that mTOR is active in desmoid tumors in children and young adults. The study will determine whether sirolimus can decrease tumor size, decrease tumor-associated pain, and block the mTOR pathway.

Activity is being assessed by clinical and histological studies following a course of pre-operative chemotherapy using sirolimus. The data obtained on this pilot study will be used to leverage additional resources to support a national collaborative study, such as one run through the Children's Oncology Group (COG), in children and young adults.

DR. RAPHAEL POLLOCK

Raphael E. Pollock, M.D., Ph.D., F.A.C.S.

*Professor of Molecular and Cellular Oncology,
The University of Texas Health Sciences Center Houston, Houston, TX*

*Professor of Surgery, Department of Surgery, The University of Texas
MD Anderson Cancer Center, Houston, TX*



How did you become interested in desmoid tumors?

My interest began during my fellowship training from 1982-1984 at the University of Texas MD Anderson Cancer Center. The Chairman of the Department of Surgery, Dr. Richard G. Martin, was an outstanding surgical oncologist who had a strong professional interest in desmoids and other types of connective tissue tumors. Dr. Martin kindled my personal interest in these diseases because we both recognized that this was an area of focus that clearly lay beyond the typical general surgical practice, and therefore was a disease cluster about which little was (and still is!) known. So for me personally I was at the right place at the right time and with the right mentoring to embark on a career under Dr. Martin's initial guidance – a professional trajectory that has sustained me ever since.

What research interests have you pursued?

I share a deep personal and professional interest in connective tissue oncology with my spouse Dina Lev, MD. Our joint focus is manifested in the research environment that we have been able to create in our work together at the University of Texas MD Anderson Cancer Center: the Sarcoma Research Center. This investigative facility now includes faculty from six different departments at MD Anderson who work collaboratively with the additional support of five dedicated research staff personnel.

The Sarcoma Research Center also includes pre- and post-doctoral MD, PhD, and MD-PhD trainees from the United States and abroad. Although desmoid tumors are not the exclusive focus of research in this Center, it is a major area of our research. Many of the scientific issues relevant to the study of other connective tissue tumors also apply directly to desmoid questions, so there are economies of research scale as well as potent intellectual cross-pollination that routinely occurs within this specialized research environment.

Can you tell us about the impact of DTRF support at MD Anderson?

The support of the DTRF has been critical to our work. Certainly the financial wherewithal generously provided by the DTRF has made our desmoid research efforts possible by helping address salary components, supply costs, desmoid tissue retrieval expenses and other associated financial realities. Equally important, The DTRF has been a portal of collegial contact with other desmoid clinicians and investigators.

But even more fundamental to us, the DTRF has provided the connectivity between our therapy and research team and desmoid patients and their families. In this way we have been better enabled to serve a much broader community of affected people, most of whom are located outside of Houston, Texas. The constant contact with desmoid patients and their family members helps to almost daily reinforce our commitment to helping solve the riddle of this disease while enabling us to partake of that most important privilege: the possibility of being able to use whatever knowledge and expertise we have on behalf of someone else—nothing is more deeply gratifying than this opportunity, for which we will always be indebted to the DTRF!



Quest Diagnostics Steps Up in the Fight Against Desmoid Tumors

Quest Diagnostics has a long history of giving back to the local communities in which its employees live and work. In the summer of 2010, an opportunity presented itself to again make a difference.

DTRF was in high gear planning its November patient symposium and the Running for Answers Run in Philadelphia. At the same time, Robin McLaughlin, whose husband John works for Quest Diagnostics in Collegeville PA, learned that the desmoid tumor she had had removed from her chest wall via radical surgery one year before had recurred and had already grown to almost its original size.

Overcome with fear at the idea of Robin facing another radical surgery, John reached out to DTRF for information on treatment options. Grateful to DTRF for its overwhelming support, John and Robin signed up for the DTRF Patient Meeting and Running for Answers.

Struck by the compatibility of the missions of DTRF and Quest Diagnostics, John approached his company about spreading the word and sponsoring the DTRF event. Quest responded immediately by providing sponsorship, promotional items and runners!

Meanwhile, Robin's story became known around the company. Three Philadelphia Quest facilities held bake sales to benefit DTRF, with over 50 employees taking time to bake goods for the sales. By the time Running for Answers rolled around, 30 Quest employees and family members had signed-up, and 71 employees had made personal contributions. The overall contribution from Quest Diagnostics and its employees topped \$15,000. What an amazing success story of genuine, caring people!



“ ... the most heart-warming (and heart-breaking) moments were meeting our fellow desmoid patients and hearing their stories in person. I was struck once again by everyone’s strength, courage and shared concern...”

**SAVE THE DATE:
October 22-23, 2011
Patient Symposium and
Running for Answers**

2010 PATIENT SYMPOSIUM

The 2010 Patient Symposium was held at the Loews Philadelphia Hotel on November 13th. Over 75 patient and family members attended the meeting and received valuable information from our distinguished panel of experts. Benjamin Alman, MD, spoke about his ongoing research on desmoid tumor initiating cells; Andrea Marrari, MD, discussed the role of medical therapy in managing desmoid tumors; Marcovalerio Melis, MD, F.A.C.S, discussed surgical treatments and trends in desmoid tumors; and, Dr. Stephen Skapek spoke about the unique dilemma of treating desmoid tumors in the pediatric population. One patient commented, “I just wanted to tell you how helpful and moving the DTRF patient symposium and ‘Running for Answers’ fundraiser turned out to be this past weekend. The four doctors that presented did a wonderful job in giving an overview of the disease and current treatment options... The most important thing they imparted was HOPE.”

Following the talks, we held the patient roundtable, where we had the opportunity to share our own stories, learn from each other and ask more questions of the experts.

This session is always a highlight of the event. One of our presenters commented, “It was particularly nice to hear, first-hand, how your foundation has been able to touch so many individual’s lives. It’s an inspiring story.” A patient said, “... the most heart-warming (and heart-breaking) moments were meeting our fellow desmoid patients and hearing their stories in person. I was struck once again by everyone’s strength, courage and shared concern. I’m so glad that I was able to give real hugs and not just virtual ones. There was a tremendous showing of support.”



RUNNING FOR ANSWERS WALK/RUN

Our Running for Answers Walk/Run was held the next day at Philadelphia’s Fairmont Park on a crisp, sunny fall day along the beautiful Schuylkill River. Over 260 people participated in the Five Mile Race, One Mile Walk or Kids Fun Run. Pat Ciarrocchi from CBS 3 kicked off the event as our Master of Ceremonies.

Personal fundraising by participants was a key to our success! A fun example was from a desmoid patient, Lynn Parks, who said when she added up all of her sponsorships, they totaled exactly \$1,776 – a meaningful number for an event in Philadelphia!

DTRF extends thanks to all of the individuals who raised funds and supported this event.

In addition, we are grateful to our corporate sponsors which included Quest Diagnostics, City Sports, Whole Foods, Starbucks and Vita Coco.



An Evening of Wine Tasting, Food and Music

DTRF held a special fundraising evening May 25th in Piermont, NY. Approximately 120 people tasted 90+ point wines from around the world! This wonderful event was coordinated through the efforts of K&D Wines and Spirits of Manhattan and featured wines from select vineyards, including Antinori, Chateau St. Michele and Nicolas Feuillatte Champagne.



SAVE THE DATE

October 22-23, 2011

Patient Symposium and Running for Answers

The 2011 Patient Meeting will be held at The Philadelphia Downtown Courtyard Marriott from 2-6pm, followed by patient roundtables. The Downtown Courtyard is located at 21 North Juniper Street, Philadelphia, PA 19107, (215) 496- 3200, and we have reserved special rates for participants. Our Walk/Run will be held the next morning followed by a brunch back at the Marriott from 10:30 - 2:00.

For more information visit runningforanswers.com. If you have any questions please contact dtrf.marlene@yahoo.com.



For up-to-date information visit our site, www.dtrf.org



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