



2022 ANNUAL REPORT

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Dear friends,

What an exciting year this has been for our community! Recently, I was sitting in a large auditorium with hundreds of sarcoma oncologists and researchers in Vancouver, Canada as the 2022 Connective Tissue Oncology Society (CTOS) meeting hosted an entire section on desmoid tumors. Desmoid tumor/desmoid fibromatosis was named the CTOS "Sarcoma of the Year."

The energy in the room was palpable. Physicians and researchers - many of whom have been DTRF's advisors and grantees throughout the years - presented on the latest promising clinical trial results and other research.

While desmoid tumors have frequently had a presence at CTOS, this was an unprecedented level of attention and DTRF was able to shine in the spotlight. I was the opening speaker on a special October desmoid tumor CTOS webinar where I shared the issues patients deal with in navigating the desmoid tumor journey.

Also, at CTOS, we partnered with a continuing medical education (CME) company to provide an accredited session for sarcoma experts and researchers (which was later re-broadcasted virtually) on the latest in desmoid tumor treatment. The class was entitled "*On the Cusp of Change: Modernizing the Treatment of Desmoid Tumors with Novel Gamma Secretase Inhibitors.*" **We continue to educate the medical community to ensure that desmoid tumor patients get optimal care from the day they are diagnosed and throughout their journey.**

This fall, not one, but two pharmaceutical companies conducting desmoid clinical trials on gamma-secretase inhibitor (GSI) drugs announced hopeful results:

- The Phase 3 trial for nirogacestat from SpringWorks Therapeutics showed a 71% lower risk of disease progression on average versus the placebo group.
- Part A of the Phase 2/3 trial for AL102 from Ayala Pharmaceuticals showed that the drug was well tolerated across all doses and efficacy was demonstrated across all cohorts with early responses that deepened over time.

For 12 years, DTRF advocated for desmoid tumor patients as nirogacestat made its way through clinical trials. Each subsequent trial was not guaranteed. We made it a priority to help facilitate the clinical trials and stay connected with the right people to ensure that this promising treatment continued to be studied. It is so gratifying to see this promising treatment successfully complete the Phase 3 trial and to know that **a New Drug Application (NDA) has now been submitted to the FDA for approval.**

It's because of you and your support of DTRF that we are now standing on the brink of what we believe will be a groundbreaking advancement in desmoid tumor treatment options.

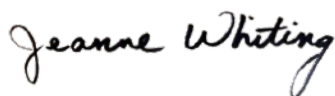
This year, we have **deepened our support for patients** as they navigate this disease in their day-to-day lives. DTRF launched "*Dealing with Desmoids: Mental Health Tools for the Desmoid Tumor Patient*", an educational webinar series facilitated by a Licensed Clinical Social Worker who is also a desmoid tumor patient. These free sessions provide resources in the context of our community's specific challenges and needs, and the recordings are available to view on our website at any time. With your support, we plan to continue this series in 2023.

In September, we also hosted our third **DTRF Together We Will Virtual Weekend** during Desmoid Tumor Awareness Month. This year's Annual Patient Meeting Webinars included highly anticipated presentations by physicians covering both the nirogacestat and AL102 clinical trial results, ensuring that our community is empowered with information on the latest treatment options and research. We celebrated our awareness month by hosting Facebook Live events featuring a patient's journey and a medical oncologist's recommendations on initial consultations and second opinions.

We've also expanded our internal team by hiring a Development Director. Growing our team will help us expand our communications and fundraising capabilities and further accelerate our mission to fund research for improved therapies, and a cure for each and every desmoid tumor patient.

We are only able to come this far and sustain and create our patient-focused initiatives because of YOUR support. Together, let's find more answers for desmoid tumor patients as we move forward!

In gratitude,



Jeanne Whiting

Executive Director and Co-founder



WHAT WE DO AND WHY IT MATTERS

OUR MISSION

Founded in 2005, the mission of The Desmoid Tumor Research Foundation (DTRF) is to aggressively fund research to accelerate the development of improved therapies, and ultimately find a cure for desmoid tumors. We collaborate with dedicated researchers and clinicians worldwide to improve the lives of patients through education, awareness and support. The founders have a deep sense of urgency in this cause motivated by a personal connection to the disease. We press forward to fund cutting-edge research that will develop new effective therapies for desmoid tumors, leading to the ultimate goal, a cure!

RESEARCH FUNDING

The DTRF is the only foundation in the country solely dedicated to funding desmoid tumor research and finding a cure for this rare disease. The funding of cutting-edge collaborative research continues to be our priority. We fund research projects at the world's top sarcoma cancer research centers in both basic science and clinical trials. DTRF-funded research seeks to determine what goes wrong in cells to generate these tumors, what systemic and local treatment options work best, and what existing drugs or potential new drugs could provide effective treatments. The DTRF facilitates collaboration among researchers from different institutions to expedite the development of a cure. DTRF-funded research has made significant advancements in our understanding of the disease. Discoveries made through desmoid tumor research can also potentially have a significant impact on many other more common cancers such as breast, colon, ovarian and other cancers.

EDUCATION AND COLLABORATION

The DTRF has established a very successful annual fall Patient Meeting which brings patients, physicians and researchers together for education, support and collaboration. In connection with the Patient Meeting, we hold the annual DTRF International Desmoid Tumor Research Workshop. This event brings together a diverse group of scientists from around the world including the brightest minds in desmoid tumor research, human genetics, drug development and related fields. The Workshop facilitates an enthusiastic and collegial atmosphere as researchers across disciplines and institutions collaborate around the shared goal of improving treatments for desmoid tumor patients, establishing research priorities and moving the field forward toward a cure. In addition, our website is a clearinghouse of information on desmoid tumors and published desmoid tumor research, reaching patients, physicians and researchers around the globe.

PATIENT SUPPORT

Patients are not fighting this disease alone. Our website provides information and helps direct patients to other sources for information, support and resources. Our annual Patient Meeting and informational webinars throughout the year provide a supportive environment for patient interaction and informative presentations by clinicians and researchers. Most of all, we seek to support patients by giving them hope through funding research for a cure. Our fundraising events around the country promote awareness and give patients and their families and friends a healing way to make a difference as proactive advocates for a cure.

**WE KNOW THAT OUR GOAL OF A CURE IS WITHIN REACH...
AND WE WILL GET THERE TOGETHER.**





Jeanne Whiting & Marlene Portnoy

2022 HIGHLIGHTS

A NEW CHAPTER FOR LEADERSHIP

In January, after 16 years of tireless work that has helped the DTRF transform the landscape for desmoid tumor patients and their families, Marlene Portnoy transitioned out of the role of Executive Director onto the DTRF Board of Directors. She began a new chapter in her life, allowing her to spend more time with her husband and their children and grandchildren, while continuing to support the Foundation on the Board of Directors.

For so many in our community, Marlene has been the first person to help guide them in the right direction after a diagnosis with a desmoid tumor. She has given reassuring support at our annual events, has been an inspiring example of perseverance and leadership, and has formed lasting bonds with patients and families. Her work with the patient and medical communities has accelerated the fight to better understand this disease.

From the bottom of our hearts, we thank Marlene for all she has given to the mission of the DTRF, and for her family's constant support. We are grateful to have her continue in her new role as we advance desmoid tumor research together.

With new opportunities on the horizon, we pressed forward with a new leader as the Executive Director role passed to Marlene's co-leader and partner in this journey, the DTRF's Co-Founder and President, Jeanne Whiting.



CLINICAL TRIALS

NIROGACESTAT

Path to approval

On May 24, 2022, SpringWorks Therapeutics announced topline results from their Phase 3, double-blind, placebo-controlled trial called the DeFi trial. This trial evaluates the gamma secretase inhibitor nirogacestat, for efficacy, safety, and tolerability in adult patients with progressing desmoid tumors. The DeFi trial's primary endpoint was met by improving progression-free survival (PFS) for usage of nirogacestat compared to placebo, with a 71% reduction in the risk of desmoid tumor progression.

"Desmoid tumors are aggressive soft-tissue tumors that can lead to severe negative outcomes for patients, including long-lasting pain, disfigurement, and amputation. In rare cases, when vital organs are impacted, desmoid tumors can also be life-threatening. Today's announcement represents a significant milestone towards our goal of bringing the first approved therapy to the desmoid tumor community. We look forward to sharing the DeFi trial data with the FDA and to presenting detailed study results at a medical meeting later this year."

-Saqib Islam, Chief Executive Officer of SpringWorks

In September, 2022, SpringWorks Therapeutics also presented the phase 3 trial data at the European Society for Medical Oncology (ESMO) medical congress. Then, in December, SpringWorks submitted a New Drug Application (NDA) to the U.S. FDA for nirogacestat.

"Our NDA submission represents an important step toward potentially delivering the first approved therapy for patients with desmoid tumors."

- Saqib Islam, CEO, SpringWorks Therapeutics

Read the [SpringWorks press release](#)

In the past, SpringWorks had applied for various regulatory designations that the FDA has in place to review drugs that treat serious conditions and fulfill unmet needs. The FDA granted nirogacestat Fast-Track and Breakthrough Therapy Designations, and "the NDA submission is now being reviewed under the FDA's Real-Time Oncology Review (RTOR) program."

All of this has been BIG NEWS and means that there is recognition that there is an important need for a new drug like nirogacestat. SpringWorks said that it expects the FDA to make a decision about approval sometime in 2023.

Pediatric clinical trial

The pediatric clinical trial of the gamma secretase inhibitor nirogacestat, Children's Oncology Group (COG) Study ARST1921, was enrolling patients aged 12 months to less than 18 years. Roughly 90 sites were recruiting, with additional locations opening at COG member institutions (>200 sites in US, Canada, and New Zealand/Australia).

(Any COG site has the opportunity to open this study while the study is activated. For a rare tumor like desmoid, it is not uncommon for a site to activate a study only when they have identified a patient.)

AL102

This is a Phase 2/3, Randomized, Multicenter Study to Evaluate AL102, a gamma secretase inhibitor, in patients with progressing desmoid tumors, sponsored by Ayala Pharmaceuticals, Inc. In March 2022, Ayala Pharmaceuticals announced the completion of enrollment in Part A of RINGSIDE, the Phase 2/3 study of the drug AL102 in desmoid tumors. Part A evaluated the safety and tolerability of AL102, tumor volume by MRI at 16 weeks, and optimal dosing. Part B of the study will begin after dose selection from Part A and will enroll up to 156 patients aged 12 and up with progressive disease. Part B will be a double-blind and placebo-controlled study. Currently known as AL102, the drug under study is a gamma secretase inhibitor (GSI) - a class of drug that has shown promise in treating desmoid tumors. The Phase 2 results in the trial were presented in September, 2022 at ESMO and at the DTRF's 2022 Virtual International Research Workshop.

TEGAVIVINT

This is a Phase 1/2 Study for 38 participants of Tegavivint (NSC#826393) in Children, Adolescents, and Young Adults With Recurrent or Refractory Solid Tumors, Including Lymphomas and Desmoid Tumors sponsored by Children's Oncology Group (COG).

RECURSION TRIAL IN F.A.P.

In September 2022, Recursion initiated the TUPELO Trial, a Phase 2 trial of REC-4881, for the potential treatment of Familial Adenomatous Polyposis (FAP). The trial includes patients who have previously been diagnosed with classic Familial Adenomatous Polyposis (F.A.P.) and have undergone colectomy or subtotal colectomy. F.A.P. patients with desmoid tumors may qualify. REC-4881 was granted Fast Track and Orphan Drug designations for the potential treatment of FAP by the U.S. Food and Drug Administration as well as Orphan Drug designation by the European Commission.

WHAT AN EXCITING YEAR FOR DESMOID TUMOR PATIENTS!



CTOS Annual Meeting 2022
Session 12 - Desmoid Fibromatosis

Prof. Dr. Bernd Kasper

University of Heidelberg, Mannheim Cancer Center (MCC), Mannheim, Germany
on behalf of The Desmoid Tumor Working Group



Bringing together the world's sarcoma specialists

Network Adult Cancers (NACAC)

Sarcoma Patients EuroNet

THE DESMOID TUMOR RESEARCH FOUNDATION

Desmoid tumors were the focus of the CTOS Annual Meeting in November 2022

CONFERENCES FEATURE DESMOID TUMORS

THE CONNECTIVE TISSUE ONCOLOGY SOCIETY (CTOS) - SARCOMA OF THE YEAR!

In October, 2022, The Connective Tissue Oncology Society (CTOS) - a prestigious global organization of multidisciplinary sarcoma medical professionals - named desmoid fibromatosis the “Tumor of the Year” for their 2022 annual meeting in November.

As part of this honor, the DTRF’s Executive Director Jeanne Whiting was the opening speaker during a special October CTOS webinar dedicated to desmoid tumors. Giving a voice to our patients, Ms. Whiting presented on the topic, “Patients’ perspective: navigating the desmoid tumor journey.” The webinar also featured educational presentations on desmoid tumor pathobiology, pathology, and localized and systemic treatment options currently available. The webinar finished with a dialogue between experts on current challenges. The aim of the webinar was to bring all members to a good basic level of understanding of the “Tumor of the Year” in preparation for the CTOS Annual Meeting taking place a few weeks later in November. We appreciated this incredible opportunity to increase awareness about desmoid tumors and the patient experience with the global sarcoma community.

THE EUROPEAN SOCIETY FOR MEDICAL ONCOLOGY (ESMO)

In September 2022, the European Society for Medical Oncology (ESMO) conference was held in Paris, France. Presentations on the latest data from two promising clinical trials mentioned above - both studying new gamma-secretase inhibitor drugs in desmoid tumors - were highly anticipated by attendees.

SpringWorks Therapeutics presented the latest data from their Phase 3 DeFi clinical trial for nirogacestat (a GSI) in desmoid tumor patients during the Presidential Symposium session of ESMO. For more information, see the [SpringWorks’ press release](#), and the [ESMO article](#).

Dr. Ravin Ratan from MD Anderson Cancer Center also presented this data at DTRF’s Virtual International Research Workshop on September 23rd.

RESEARCH

DTRF INTERNATIONAL RESEARCH WORKSHOP

The 9th Annual International Desmoid Tumor Research Workshop was held virtually on Friday, September 23rd, in conjunction with our Patient Meeting Webinars the following day. The Workshop featured a forum to share research findings, develop new collaborations, bring new scientists into the research field, and discuss innovations that will lead to new treatments. Group discussions focused on gaps in desmoid tumor science and how best to address them through high impact collaborative research. The 160 registrants for the 2022 Workshop included clinicians and researchers from 28 countries and 100 institutions.

Research Workshop Speakers

Welcome: **Jeanne Whiting**, Executive Director and Co-Founder, The DTRF and **Benjamin Alman, MD**, Professor & Chair, Orthopaedic Surgery, Duke University

Kris Vleminckx, PhD, Professor of Developmental Biology, Ghent University – *“Investigating EZH2 as a druggable mediator of immune cell exclusion in desmoid tumors.”*

Paul Huang, PhD, Head, Molecular and Systems Oncology Laboratory, Division of Molecular Pathology, The Institute of Cancer Research – *“The proteomic landscape of desmoid tumours.”*

Joanna Przybyl, PhD, Assistant Professor, Department of Surgery, McGill University & Principal Investigator, Cancer Research Program, Research Institute at The McGill University Health Centre – *“Targeting hexosamine biosynthesis pathway for the treatment of desmoid tumors.”*

Jesse Boehm, PhD, Chief Science Officer, Break Through Cancer; Principal Investigator, MIT, affiliated researcher, Broad Institute – *“Progress Towards a Desmoid Tumor Dependency Map.”*

Gerlinde Wernig, MD, Assistant Professor, Department of Pathology, Stanford University School of Medicine and **Cristabelle Madona De Souza, PhD**, Postdoctoral Fellow, Department of Pathology, Stanford University – *“Studying CD47 blockade as an immunotherapy for desmoid-type fibromatosis.”*

Jason Ortwerth, Desmoid Tumor Patient Advocate

Alessandro Gronchi, MD, Chair of Sarcoma Service – Department of Surgery, Fondazione IRCCS Istituto Nazionale dei Tumori (National Cancer Institute)– *“Prospective observational study of active surveillance in primary desmoid fibromatosis.”*

Anne-Rose Schut, MD, Surgical Oncology and Medical Oncology, Erasmus Medical Center Cancer Institute – *“Unraveling Desmoid-Type Fibromatosis-specific Health-Related Quality of Life: Who Is at Risk for Poor Outcomes.”*

Ty Subhawong, MD, Associate Professor of Clinical Radiology, and Vice-Chair for Research, Department of Radiology, The University of Miami Miller School of Medicine – *“MRI T2 mapping in desmoid tumors: a quantitative imaging biomarker of tumor response with high inter-reader reliability.”*

Aimee Crago, MD, PhD, FACS, Associate Attending Surgeon, Memorial Hospital, Memorial Sloan Kettering Cancer Center/ Associate Professor, Weill Cornell Medical Center – *“Growth factor signaling and kinase inhibitors regulate oncogenesis in desmoid fibromatosis by modulating activity of the beta-catenin transcription target ABL1.”*

Philippos Costa, MD – *“Comparative Effectiveness of Systemic Treatments in Desmoid Tumors.”*

Benjamin Wilke, MD, Assistant Professor and Senior Associate Consultant in the Department of Orthopedic Surgery at Mayo Clinic – *“Pilot Study of Intralesional Injection of Triamcinolone Acetonide for Desmoid Tumors.”*

Ravin Ratan, MD, Associate Professor, Sarcoma, MD Anderson Cancer Center – *“Nirogacestat for the Treatment of Desmoid Tumors.”*

Rashmi Chugh, MD, Professor of Internal Medicine in the Division of Hematology/Oncology, Department of Internal Medicine, University of Michigan Rogel Comprehensive Cancer Center – *“Initial Results of Phase Trial of AL102 for Treatment of Desmoid Tumors (DT).”*

Diana Shuster, PhD, Senior Director of Clinical Pharmacology, Recursion Pharmaceuticals – *“TUPELO TRIAL: A Phase 2, Multicenter, Randomized, Double-Blind, Placebo- Controlled Trial to Evaluate Efficacy, Safety, and Pharmacokinetics of REC4881 in Subjects with Familial Adenomatous Polyposis (FAP) Study Design.”*

Closing: **Jeanne Whiting**, Executive Director and Co-Founder, The DTRF and **Benjamin Alman, MD**, Professor & Chair, Orthopaedic Surgery, Duke University99

List of Institution & Industry Represented at the Workshop

Addenbrooke's Hospital (Great Britain)
Advent Health
Angel H Roffo Institute of Oncology - Buenos Aires British Hospital (Argentina)
Antoni van Leeuwenhoek Hospital (Netherlands)
Aristotle University of Thessaloniki (Greece)
Ayala Pharmaceuticals
Beijing Cancer Hospital (China)
Cambridge University Hospital
Cancer Institute of Iran
Centre Oscar Lambret (France)
Children's Mercy Hospital
Children's Hospital Los Angeles
Children's Mercy
Clinical Center of Montenegro
Copenhagen University Hospital (Denmark)
Dana-Farber Cancer Institute
Duke University Medical Center
EcoR1
Edinburgh Cancer Centre (Great Britain)
Erasmus MC Cancer Institute (Netherlands)
Federal University of Health Sciences (Nigeria)
Flagler Free Clinic
Fondazione IRCCS Istituto Nazionale dei Tumori (Italy)
Georgetown University
Ghent University (Belgium)
Harvard Medical School
Hemby Children's Hospital
Hunstman Cancer Institute
Idream Consortium Ltd
Institut Bergonié Bordeaux (France)
Institut Pasteur (France)
Institute of Cancer Research (Great Britain)
Instituto Nacional de Ciencias Médicas y Nutrición Salvador Zubirán (Mexico)
IPO Coimbra (Portugal)
IRCCS ISTITUTO EUROPEO DI ONCOLOGIA (Italy)
Isssy (Mexico)
Istituto Europeo di Oncologia (Italy)
Istituto Nazionale Tumori (Italy)
Johns Hopkins
La Arrixaca University Hospital (Spain)
Lausanne University Hospital (Switzerland)
Leon BERARD Center (France)
Liverpool University Hospitals NHS Foundation Trust (United Kingdom)
Maine Medical Center
Massachusetts Institute of Technology (MIT)
Mayo Clinic
McGill University (Canada)
MD Anderson Cancer Center
MDACC
Medical College of Wisconsin
Memorial Sloan Kettering Cancer Center
Michigan State University
MKCG Hospital (India)
Moffitt Cancer Center
N.N. Blokhin NMRCO (Russia)
Nagoya University (Japan)
National Cancer Institute (NCI)
National Taiwan University Hospital
National Veterinary Services Laboratories
Netherlands Cancer Institute
Northwestern
Osteocare
Philippine Orthopedic Center
Princess Alexandra Hospital (Australia)
Princess Margaret Cancer Centre (Canada)
Queens University (United Kingdom)
Rabin Medical Center (Israel)
RadboudUMC (Netherlands)
Recursion Pharmaceuticals
Royal Children's Hospital
Ruhr University Bochum (Germany)
Ruth and Bruce Rappaport Faculty of Medicine
San Luigi University Hospital (Italy)
Sheba Medical Center (Israel)
Sidney Kimmel Medical School, Jefferson University
Smilow Cancer Center
SOS Desmoïde (France)
SPAEN
SpringWorks Therapeutics, Inc.
St Ann's
St Stephens Hospital
St. Jude Children's Research Hospital
Stanford University
Tata Memorial Centre (India)
Tel Aviv University (Israel)
The Chinese University of Hong Kong
The Koch of Integrative Cancer Research at MIT
The NemetzGroup
Trinity College
Unimed
Univ of Kansas - Wichita SOM
Università degli Studi di Torino (Italy)
Université de Montréal
University College Hospital NHS Foundation Trust
University College Hospital, London
University Health Network
University Leuven (Belgium)
University of Arkansas for Medical Sciences
University of Colorado
University of Iowa
University of Miami
University of Michigan
University of North CarolinaA106
University of Pennsylvania
University of Worcester
Yale University

RESEARCH GRANTS

On the heels of another successful Desmoid Tumor Awareness Month, we were thrilled to formally announce The Desmoid Tumor Research Foundation grant awards for 2022. The funded projects offer the potential for significant advancements in desmoid tumor research and align with the research priorities set by our Scientific Advisory Board. We are grateful to our advisors who participate in the grant review process and help prioritize the projects that will move desmoid tumor science forward.

Funding for these critical projects would not be possible without the continued support of our community and donors. We would like to extend an additional special thank you to the major fundraisers and donors who have helped fund significant portions of these important projects.

Sameer Rastogi, MD, All India Institute of Medical Sciences, New Delhi, *“To see the early effect of sorafenib on cognition, cardiovascular and reproductive functions in patients with fibromatosis.”*

Cristabelle De Souza, PhD, Stanford University, *“Activation of c-JUN modulates the transcriptional activity of CD63 serving as a novel target for the treatment of Desmoid Type Fibromatosis.”* Jointly funded by DTRF and Desmoid Tumour Foundation of Canada (DFC).

Joanna Przybyl, PhD, Research Institute - McGill University Health Centre, *“Targeting hexosamine biosynthesis pathway for the treatment of desmoid tumors.”* Jointly funded by DTRF and Desmoid Tumour Foundation of Canada (DFC).

Gerlinde Wernig, MD, Stanford School of Medicine, Department of Pathology, *“Studying CD47 blockade as an immunotherapy for desmoid-type fibromatosis.”* Year 2 of 2.

Jonathan Noujaim, MD, CanSaRCC, *“Tyrosine Kinase Inhibitors in Desmoid-type Fibromatosis - A Canadian Multicenter Retrospective CanSaRCC Study.”* Jointly funded by DTRF and Desmoid Tumour Foundation of Canada (DFC).

Kris Vleminckx, PhD, Ghent University, *“Investigating EZH2 as a druggable mediator of immune cell exclusion in desmoid tumors.”* Year 2 of 2. Jointly funded by DTRF and Desmoid Tumour Foundation of Canada (DFC).

PUBLISHED RESEARCH HIGHLIGHTS

March 2022. Collaborative Paper Reveals Global Data on Sarcoma Patient Care.

A collaborative paper from the Connective Tissue Oncology Society (CTOS) and the Sarcoma Patients EuroNet (SPAEN) revealed global data on sarcoma patient care. The paper entitled “Global Patient Involvement in Sarcoma Care” points to the high percentage of misdiagnosed cases of sarcoma by physicians outside of sarcoma expert networks.

“Since the 1970s, and almost without exception, the reports of specialist pathologists suggest that approximately 30-40% of initial diagnoses made outside a sarcoma expert network are incorrect. The impact of an incorrect histological diagnosis can be substantial, and thus of great consequence for the patient.” Kasper, B. et al. Global Patient Involvement in Sarcoma Care - A Collaborative Initiative of the Connective Tissue Oncology Society (CTOS) & Sarcoma Patients EuroNet (SPAEN). *Cancers* 2022, 14, 854.

Since desmoid tumors are soft tissue tumors that are categorized within the field of sarcoma, this article emphasizes that patients diagnosed with a desmoid tumor should be evaluated by an expert in sarcoma, preferably in a multi-disciplinary setting.

June 2022. OnLive Article promoting “less surgery” for desmoid tumors.

OnLive addressed how desmoid tumor treatment has changed significantly in that clinicians have shifted from “aggressive surgical interventions” to other treatment options such as active surveillance and systemic treatments. The following physicians fielded questions about treatment challenges, emerging therapies and misdiagnosis: Alex Lazar, MD, PhD, The University of Texas MD Anderson Cancer Center; R. Lor Randall, MD, FACS, University of California Davis Comprehensive Cancer Center; Breelyn Wilky, MD, University of Colorado; Gina D’Amato, MD, University of Miami Health System; Ravin Ratan, MD, The University of Texas MD Anderson Cancer Center.

July 2022. Publication by the DTRF in Clinical Cancer Research (CCR) in partnership with Dr. Brian Van Tine of Washington University in St. Louis.

The commentary is entitled, “Are the pieces starting to come together for management of desmoid tumors?” The paper’s summary states that desmoid tumor research is changing how desmoid tumors are managed with the prospective documentation that growing desmoid tumors spontaneously regress one-third of the time. Patient partnership through The Desmoid Research Tumor Foundation and academia is leading to rapid advancement in desmoid tumor biology understanding and treatment. Studies continue to support the growing consensus to use active surveillance (also known as “watch and wait”) as first-line treatment in primary desmoid tumors. Surgery is not recommended as an initial treatment except for progressive abdominal wall tumors; medical therapies are recommended for other tumor locations after progression. There are always exceptions, and decisions regarding treatment should be discussed with a patient’s medical team.

This commentary by Brian Van Tine, MD, PhD of Washington University in St. Louis and DTRF’s Research Director Anna Greene, PhD, provides an overview of new research supporting these treatment guidelines, referencing newly published desmoid tumor studies including:

Featured study #1

A study by Dr. Chiara Colombo and colleagues found that about half of 114 sporadic desmoid tumor patients under active surveillance as first-line treatment experienced spontaneous tumor regression, including those who experienced prior progression by RECIST standards. Just one-third of patients in the study eventually required treatment. (See Colombo et al.: <https://bit.ly/activesurveillance>)

Featured study #2

A DTRF-funded study by Dr. Breelyn Wilky and team revealed that, at this point in time, a tumor’s mutational status cannot be used as a prediction for response to therapy and cannot help determine treatment protocols. They found that certain treatments such as doxorubicin/dacarbazine, single-agent doxorubicin, sorafenib, and methotrexate/vinca alkaloids had better outcomes when compared to surgery and treatments such as tamoxifen/sulindac. (See Nathenson et al.: <https://bit.ly/mutationalstatus>). NOTE: It is still recommended that patients have tumor mutational testing to 1.) confirm the diagnosis and 2.) prompt germline testing for FAP.

These findings further support global consensus guidelines (<https://dtrf.org/consensus-paper/>) on active surveillance. The Global Consensus Paper includes a treatment algorithm that proposes how best to treat desmoid tumors based on their location. All desmoid tumor patients should make it a priority to be seen by a sarcoma expert, preferably with experience in desmoid tumors, at a sarcoma center to receive proper care.



PATIENT POWERED RESEARCH

TISSUE DONATION

We continued working with Pattern.org to provide the means for patients to help support research through donating excess tumor tissue from surgery. Since surgeries are becoming less frequent, there is a continual need to increase the availability of fresh tissue for research. All a patient needs to do is fill in an online consent form prior to surgery at Pattern.org and Pattern handles all the arrangements, working with the patient's surgical institution, to facilitate the transfer of tissue to researchers. There is no cost or any additional effort on the patient's part to participate.

PATIENT REGISTRY & NATURAL HISTORY STUDY

Thank you to Kelly Mercier, PhD, Amanda Lucas, and Lynne Hernandez for their management of this important DTRF initiative which collects de-identified survey data inputted by patients about their desmoid tumor patient experience. The data is aggregated and analyzed to create a natural history study of the disease, accessible to researchers. In 2022, members of the DTRF team attended the National Organization for Rare Disorders, Inc. (NORD) IAMRARE Registry Community Leaders Meeting, part of NORD's Breakthrough Summit in Washington, D.C. This meeting provided a chance to meet and confer with other organizations that are part of the FDA and NORD cooperative IAMRARE Patient Registry project. Learn more about the DTRF Patient Registry and Natural History Study at <https://dtrf.org/patient-registry/>.

DESMOID TUMOR PREGNANCY STUDY

This year, we worked with international healthcare and patient advocacy organizations to learn more about the decisions women make regarding pregnancy after a desmoid tumor diagnosis, as well as to understand how pregnancy may influence the behavior of a desmoid tumor. This may one day lead to improved tools to support women who are making decisions regarding pregnancy. *The study is no longer recruiting participants and will be publishing results in 2023-2024.*



Researchers and medical professionals joined us for the DTRF International Research Workshop in September 2022

PROFESSIONAL INITIATIVES

THE DTRF-SPONSORED VIRTUAL TUMOR BOARD

The DTRF continues to host our quarterly Virtual Tumor Board with 3-4 cases presented during each board. Desmoid tumor experts in all related fields are invited to join. We rely on their expertise to make this important project succeed for the benefit of patients as they assist other physicians in dealing with difficult desmoid tumor cases. Other medical professionals are also welcome to observe.

RADIOLOGY WORKING GROUP

In February, 2022, DTRF hosted a Working Group to Shape the Future of Radiology in Desmoid Tumors. As many patients know first-hand, “Responsive Evaluation Criteria in Solid Tumors” (known as RECIST) - a standard methodology for measuring a patient’s tumor response to treatment - is not always the best method to measure treatment outcomes for desmoid tumor patients. RECIST criteria are used in clinical trials to assess if a tumor is progressing, stable, or responding to treatment. RECIST is strictly measurement-based and does not factor in other positive indicators that do not involve changes in tumor size. Additionally, RECIST relies on certain measurements to monitor response - such as “longest dimension” - which may not accurately reflect the typical response of desmoid tumors to treatments. To address this issue, The DTRF and members of its Advisory Boards have formed a working group with top radiologists, as well as pharmaceutical medical professionals who design clinical trials for desmoid patients. This working group will help define other imaging features beyond RECIST that can be used to better evaluate patient outcomes. The first working group meeting, held in February, was a resounding success. We would like to thank our working group chairs, Drs. Mrinal Gounder and Larry Schwartz, for hosting this meeting as an ongoing project which promises to positively impact the future of radiology in desmoid tumors.



EDUCATION, ADVOCACY AND AWARENESS

VIRTUAL PATIENT MEETING WEBINARS

The DTRF Annual Patient Meeting - our signature event of the 2022 DTRF Together We Will Virtual Weekend - was held virtually in the form of three, 90-minute webinars, each focusing on valuable topics of interest to educate and empower patients and caregivers. The webinars were recorded (including Q&As) and transcribed to make this a dynamic resource on our website.

Webinar #1 focused on the basics of desmoid tumors, including treatment options, as well as ways patients and caregivers can advocate for themselves and take part in essential projects that will help us advance desmoid tumor research.

Desmoid Tumors 101 presented by Seth Pollack, MD, Director of the Sarcoma Program, Lurie Cancer Center, Northwestern University

Suggested Questions For Your Doctors: A Patient's View presented by Bill Lundgren, Desmoid tumor caregiver

Tissue Donation Through Pattern.org presented by Barbara Van Hare, Director of Partnerships, The Rare Cancer Research Foundation

Sharing Your Desmoid Journey presented by Kelly Mercier, PhD, Chair, Coordinating Center, The Desmoid Tumor Research Foundation

Webinar #2 featured a mock Tumor Board of five multidisciplinary experts from UPMC Hillman Cancer Center. This sample discussion, which evaluated de-identified desmoid tumor cases, gave the community a chance to experience a Tumor Board first-hand and to learn how a multidisciplinary approach to treatment can improve patient outcomes. This webinar also included presentations on recent results from promising clinical trials from SpringWorks Therapeutics (nirogacestat) and Ayala Pharmaceuticals (AL-102).

The Tumor Board: A Multidisciplinary Treatment Approach presented by University of Pittsburgh Hillman Cancer Center

Nirogacestat for the Treatment of Desmoid Tumors presented by Ravin Ratan, MD, Associate Professor, Sarcoma, MD Anderson Cancer Center

Initial Results of RINGSIDE, a Phase 2/3 Trial of AL102 for Treatment of Desmoid Tumors presented by Mark Agulnik, MD, Professor and Section Chief of the Sarcoma Program, Department of Medical Oncology and Therapeutics Research, City of Hope

Cryoablation as a Treatment for Desmoid Tumors presented by Joseph Erinjeri, MD, Interventional Radiologist & Physician Scientist, Memorial Sloan Kettering Cancer Center

Webinar #3: This webinar session was dedicated to the topic of desmoids in the context of the genetic syndrome familial adenomatous polyposis (F.A.P.). Three physicians from the Cleveland Clinic presented in their areas of expertise on this topic.

Building Resilience: Psychosocial Issues in Desmoid Disease presented by Karen Hurley, PhD, Clinical Psychologist, Center for Adult Behavioral Health, Neurological Institute, Cleveland Clinic

Medical decision making for systemic treatment of FAP-associated desmoid tumors presented by Dale R. Shepard, MD, PhD, FACP, Director, Phase I and Sarcoma Programs, Cleveland Clinic Taussig Cancer Institute

Surgical considerations for desmoid disease in familial adenomatous polyposis presented by Joshua Sommovilla, MD, Associate Staff & Colorectal Surgeon, Cleveland Clinic

THE DTRF INTERNATIONAL VIRTUAL COMMUNITY NETWORKING EVENT

On September 24, 2022, members from the desmoid patient/caregiver community around the world joined our virtual networking event. This event was produced in partnership with the following desmoid tumor patient advocacy organizations: Sachin Sarcoma Society (India), Desmoide Brasil (Brazil), and Contactgroep Desmoid (Netherlands). Facilitators - including American desmoid survivor, F.A.P. survivor, and patient advocate, Dakota Fisher-Vance - guided the discussion and provided breakout groups based on language and location. Community connections can be transformative and are essential to the patient and caregiver journey. This is a wonderful opportunity to have one-on-one, real-time conversations with fellow patients and care partners. The event is structured to provide a natural way to get to know attendees and share experiences in a safe and private setting.

“PICNIC IN THE PARK” IN PHILADELPHIA

On Sunday morning, September 25, we hosted a “Picnic in the Park” event at Fairmount Park across from the Please Touch Museum in Philadelphia, Pennsylvania. It was a relaxing, outdoor event giving attendees the chance to mingle with other members of the community face-to-face. Event sponsor Connor Catering provided some light snacks for attendees, and we heard from some desmoid survivors, including the inspiring former NFL-player and desmoid patient Kevin Reilly!

2022 DESMOID TUMOR AWARENESS MONTH

Patients submitted their desmoid tumor stories to help us get ready to celebrate Desmoid Tumor Awareness Month, DTRF’s worldwide annual campaign to create and increase awareness for desmoid tumors and amplify the stories, struggles, and triumphs of desmoid tumor patients around the globe. Patients were also encouraged to start Facebook fundraisers. During the month, we also held three Facebook LIVE events on topics of interest to patients such as: “One Desmoid Tumor Patient’s Journey” with Jackie Kucskar; “Getting the Most Out of Your Consultation and Treatment Plan” with Dr. Candace Haddox from Dana-Farber Cancer Institute; and, “Five Mental Health Tools for Desmoid Patients” with Ashley Williams, Licensed Clinical Social Worker (LCSW) and desmoid tumor patient.

RARE DISEASE DAY

DTRF helped celebrate Rare Disease Day on February 28th. Patients submitted their stories which help explain the impact of this rare disease, highlight the need for research, and raise #DesmoidAwareness.

ANNUAL INTERNATIONAL DTRF GLOBAL DESMOID TUMOR ROUNDTABLE

In October, we held the second annual global roundtable— a meeting hosted by DTRF for leaders of international advocacy groups that support desmoid tumor patients in their own language and country. This meeting is growing in scope and attendance and is an important opportunity for all of us to connect and support each other.

DTRF PARTNERS WITH PEERVIEW AT CTOS

In November, during the Connective Tissue Oncology Society (CTOS) Annual Meeting, the symposium entitled “On the Cusp of Change: Modernizing the Treatment of Desmoid Tumors with Novel Gamma Secretase Inhibitors” was presented by Bernd Kasper, MD, PhD (University of Heidelberg), Mrinal Gounder, MD (Memorial Sloan Kettering Cancer Center), and Alessandro Gronchi, MD (Fondazione IRCCS Istituto Nazionale dei Tumori). The faculty provided expert insights and practical guidance for navigating the latest treatment advances, including gamma-secretase inhibitors (GSIs), for desmoid tumors. Illustrative cases and clinical scenarios were discussed to demonstrate the emerging role and integration of novel GSIs in real-world patient care settings.



Jeanne Whiting, DTRF’s Executive Director, at hybrid CME-accredited program for desmoid tumors



Team Rachel gathered in September for their 5K fundraiser to support DTRF, and their loved one, Rachel Sitta

FUNDRAISING

RFA VIRTUAL CHALLENGE, SPRING-FALL, 2022

In June, the 2022 RFA Virtual Challenge officially began. Patients took the DTRF's summer-long, virtual community fundraiser by going the #Distance4Desmoids. RFA Challengers were encouraged to set an achievable step or mileage goal to reach by September 25th. Whether walking 10,000 steps or cycling 100 miles, or creating a team of people who could donate steps/miles to the goal, patients matched their physical challenge with a fundraising goal. Some patients created their own unique creative challenge. The RFA Virtual Challenge culminated with a LIVE Virtual ceremony to close the DTRF Together We Will Virtual Weekend, complete with awards, team and patient spotlights, and more! We're grateful to all those who participated and raised funds this year to help us advance desmoid tumor research and programs supporting patients!

PATIENT-LED EVENTS

The DTRF relies on the patient and caregiver community for grassroots local fundraising support. We're so grateful to the patients who led fundraisers in 2022 as the world began to emerge from the Covid 19 pandemic. If you are interested in running a fundraising event, please contact development@dtrf.org.

SWING FOR A CURE- ST. LOUIS, SEPTEMBER 2022

Fore! 'Swing For A Cure - St. Louis' Hits Another Hole in One for Desmoid Research. Congratulations and THANK YOU to desmoid survivor Jason Ortwerth and his wife Katie on a successful 4th annual "Swing For A Cure - St. Louis" golf tournament on Sept. 26th, which raised more than \$50,000 for desmoid tumor research! Jason was diagnosed with a desmoid tumor in 2017. Just two years later, he and his wife organized the first Swing For A Cure event.

"Following my diagnosis, I was scared and confused. It was determined that the immediate course of action was surgery followed by medical therapy. After my recovery, my wife and I attended our first DTRF Annual Patient Meeting in Philadelphia in 2018. We were eager to get involved and make a difference on any scale. We later asked ourselves 'what do we know,' and 'what does our network enjoy?' For me, golf was an obvious answer - and that was when we started Swing For A Cure - St. Louis. We are so gratified to see frequent DTRF updates about research and development. We want to do whatever we can to make sure a cure is in sight."

Thank you, Jason and Katie, for your continued commitment to raising funds for research!



JoeStrong Volleyball Tourney in Austin during October 2022

JoeStrong Volleyball Tournament, October 2022

Congratulations and THANK YOU to desmoid survivor Joe Erickson on his 6th JoeStrong Volleyball Tournament which raised ~\$5,000 for the DTRF this Halloween! Attendees once again sported impressive and creative costumes and played beach volleyball outside for the chance to win prizes from local sponsors. Over the years, Joe’s tournament has raised a grand total of over \$32,000 for desmoid tumor research!

ATHINA’S RIDE for the DESMOID TUMOUR FOUNDATION OF CANADA, Summer 2022

Congratulations and THANK YOU to Athina’s Ride which benefits the Desmoid Tumour Foundation of Canada (DFC). The DFC is the only registered charity in Canada working to fund desmoid tumor research. Funds raised go toward co-funding some of the DTRF’s grant awards.

ABOUT ATHINA: Athina Merckx was diagnosed with a desmoid tumor in her knee at 13-years-old. Turning pain into action. The Merckx family launched Athina’s Ride to help fund hope and a cure for desmoid tumors.



What a day! Jason Ortwerth joins the warmup for Swing for a Cure - St. Louis in September 2022

FINANCIALS

The Desmoid Tumor Research Foundation depends on the generosity of donors to carry out its mission and we highly value every dollar received. DTRF is a 501(c)(3) tax-exempt organization and donations are tax-deductible to the extent allowed by law.

VIEW OUR:

- [IRS 501\(c\)\(3\) determination letter](#)
- [2022 IRS Form 990](#)
- [2022 Audited Financial Statement](#)



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