



Together, we will find a cure

DT THE DESMOID TUMOR
RF RESEARCH FOUNDATION

2021 ANNUAL REPORT



2021 Annual Report

TABLE OF CONTENTS

LETTER FROM THE CO-FOUNDERS	3
WHAT WE DO AND WHY IT MATTERS	4
2021 DTRF KEY EVENTS AND ACHIEVEMENTS	5
2021 DTRF-FUNDED GRANTS	8
FINANCIALS	9
2021 DTRF SIGNATURE EVENTS	10
OUR TEAM	15

A LETTER FROM THE CO-FOUNDERS

Dear friends,

Despite this being the second year of a world-wide pandemic, we moved forward with accelerated progress. Please see our list of events and accomplishments in 2021 below. We were thrilled to be honored with prestigious awards from two prominent rare disease organizations. In June, we received the **National Organization of Rare Disorders (NORD) Abby S. Meyers Leadership Award**. In August, we received the Global Genes RARE Champion in Advocacy award. We were honored to be in the company of our fellow nominees, for both awards, and we deeply appreciate this amazing recognition from both organizations.



These awards have led us to look back and review our history. In 2005, we co-founded DTRF with the goal to facilitate and fund desmoid tumor research and to provide patients information and support. We often say that we started as “two women working from our kitchen tables” to transform an entire rare disease community and research. We have continued as a dedicated team, along with the rest of the DTRF staff and advisors, as the Foundation has moved into its 16th year.

In a major development, Marlene announced that in early 2022 she will be transitioning from the position of Executive Director to Major Gifts Officer, giving her more time to spend with her family. The DTRF extends heartfelt gratitude to Marlene for her many years of service and the continuing work she will do as a Board Member and fundraiser. Jeanne will remain at the helm in the position of Executive Director along with Lynne Hernandez as our Director of Operations.

The two of us have pressed forward in this cause motivated by a personal connection to the disease. We first met online shortly after both Jeanne, and Marlene’s husband Steve, had been diagnosed with desmoid tumors. It was especially disconcerting when our doctors admitted, “We don’t really know these tumors.” At the time, desmoid tumors had very little research and few existing treatment options. Thousands of desmoid tumor patients and family members like us struggled for hope and support from the medical community which had little to offer in the way of a cure or even research toward a cure.

Now, researchers are making significant advancements as new clinical trials and treatments emerge. Patients know that they are not fighting this disease alone as we provide supportive programs, networking events and information. It has also been inspiring to see researchers recognize that discoveries made in desmoid tumor research can also potentially apply to many more common diseases such as colon, pancreatic, breast, lung and ovarian cancers. How exciting that our research can also impact the broader world of cancer.

The DTRF was introduced in the 2021 NORD Rare Impact Awards ceremony with the statement,

“In the community of rare diseases, DTRF has become known as a model for how patient advocates can drive research, drive collaboration, drive governmental impact in rare diseases and drive patient hope and involvement.”

We’re gratified by this recognition and appreciate this progress in our mission which is made possible by your continuing support!

We look forward to stepping up our progress and maximizing opportunities that lie ahead. Together, we will find a cure!

Sincerely and with gratitude,
DTRF Co-founders

Jeanne Whiting
President and Co-founder

Marlene Portnoy
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 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.

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 medical and surgical options work best, and what existing drugs or potential new drugs could provide effective treatments. DTRF facilitates collaboration between 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.

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.**





DTRF Board Member Kevin Reilly at Swing for a Cure - Wilmington (October 2021)

2021 DTRF ACCOMPLISHMENTS AND EVENTS

EARLY 2021

The **DTRF Patient Advocacy Committee (PAC) initiative** was implemented. Through the PAC, a committee of about thirty desmoid tumor patients and caregivers volunteer to collaborate directly with the DTRF to include the patient voice in our work. The DTRF PAC will work directly with DTRF in the following areas:

- **Providing** the patient voice for engagements with industry groups
- **Guiding** the DTRF on the needs of patients/care partners by providing insights from their perspective
- **Assisting** with fundraising
- **Shaping** and expanding patient/caregiver engagement
- **Advising** on topics of interest for patient meetings and networking events

SPRING 2021

This year we established the new position of **Research Director for The Desmoid Tumor Research Foundation**, first held by Maneesh Kumar, MD, PhD. The Research Director leads DTRF research initiatives including management of the DTRF Coordinating Center (DTRFCC). The DTRFCC was initiated in 2020 to facilitate and speed multidisciplinary desmoid tumor research through data sharing efforts and to grow the cohort of desmoid tumor researchers. Learn more; <https://dtrf.org/about-the-dtrfcc/>

MARCH 1, 2021

Jeanne Whiting, DTRF Co-founder, was selected to **present at Rare Disease Day at the National Institutes of Health (NIH), in the section entitled From Kitchen Tables to Changing Paradigms – Advocacy as a Driving Force in Advancing Progress in Rare Diseases Research**. The focus of this panel was a discussion of the increasing importance of advocacy in research and rare disease drug development. The discussion confirmed that, unlike in more widespread health challenges, advocacy has become the norm for spurring research and therapy development in rare diseases. We were honored to be chosen to present and were told that the name of the section was actually taken from our co-founders' successful experiences in the DTRF journey! Learn more; <https://patientworthy.com/2021/03/05/rare-day-nih-advocacys-advancing-rare-disease-research/>

MARCH 25, 2021

DTRF arranged a **Listening Session with the FDA on the subject of desmoid tumors**. FDA listening sessions are an opportunity to demonstrate the importance of government agencies recognizing and supporting research in rare diseases like desmoid tumors. At the meeting, desmoid tumor patients and caregivers shared their stories about living with the disease, challenges in treatments, and experiences of being a caregiver. In addition, DTRF presented information about the DTRF natural history study. Our presentation was well received by the FDA, and we felt a great sense of accomplishment in having this opportunity to meet with them and have our voices heard.

MARCH 31, 2021.

Dr. Kelly Mercier, Chair of DTRF's Coordinating Center, presented at **NORD's webinar on Driving Cures for Rare Diseases: The Role of Patient Nonprofits in Engaging Industry and Academic Researchers to Accelerate Drug Development**.

JUNE 2021.

DTRF submitted a proposal to the CDC for a new ICD-10 code specific to the diagnosis of desmoid tumor, including subcategories for tumor location. By way of background, a medical diagnosis usually has a corresponding diagnosis code known as an **ICD-10 code**. However, many diseases, like desmoid tumor, lack a specific code and are categorized under general codes that may include a variety of conditions. A diagnosis code is used by everyone from health insurance companies to medical institutions to national research databases.

Currently, desmoid tumors are categorized under a general ICD-10 code that covers a wide variety of connective and soft tissue tumors of “uncertain behavior”. From a research perspective, this has prevented understanding of the true prevalence and incidence of desmoid tumors and has made it difficult to identify desmoid tumor cases in nationwide population databases during research. This obtuse code can also sometimes impact health insurance coverage for certain treatments and does not identify the bodily location of tumors, an important factor in approaching treatment.

We believe that, if granted, DTRF’s proposal to the ICD-10 Coordination and Maintenance Committee for a new code specific to the diagnosis of desmoid tumor, including subcategories for tumor location, will improve research as well as patient care. The initial request is the beginning of what may be a long, deliberative process. We should know if the code was accepted by fall of 2022. If accepted, it would not be implemented until October of 2023.

JUNE 28, 2021.

DTRF was awarded the Abbey S. Meyers Leadership Award at the Rare Impact Awards of the National Organization of Rare Disorders. The Rare Impact Awards celebrate “the individuals, groups and companies making extraordinary contributions to the lives of rare disease patients and caregivers.” [Watch us receive the NORD’s Abby S. Meyers Leadership Rare Impact Award](#) (beginning at 55:45)

JULY 22, 2021.

DTRF and Ayala Pharmaceuticals co-sponsored **the first Global Desmoid Tumor Round Table** gathering leaders of a dozen desmoid tumor patient advocacy groups from around the world. In this half-day round table, we discussed the unmet needs of the global desmoid tumor patient community. It was a great opportunity to learn firsthand about the challenges that these groups and their patients face due to language barriers, location, lack of funding and limited access to treatments. Breakout groups brainstormed ways to provide support around the patient journey and treatment decision-making.

Groups represented included: Desmoid United/DFPC (UK) (Lisa Pascoe); SOS Desmoid France (Clementine Boulet); SOS Desmoid Germany (Christina Baumgarten); SPAEN (Sarcoma Patients EuroNet (Kathrin Schuster); The Italian Desmoid Tumor Association (Sabrina Mercuri); ANZSA-Australia, New Zealand (Denise Caruso); Contactgroep Desmoid-- Netherlands (Kim van der Zande); Tumor Desmoide Brasil (Carol Menezes); Sachin Sarcoma Society - India (Rashi Kapoor); SARC/Sarcoma Coalition (Steven Young).

SEPTEMBER 2021 was **an inspiring Desmoid Tumor Awareness Month**, with participation by desmoid patients around the world who shared their desmoid journeys to raise awareness. The month also included 2 special Facebook live events for patients. Christina Kosyla shared about her experience as a patient in “One Desmoid Tumor Patient’s Journey”. **Raphael Pollock, MD, PhD, Director of The Ohio State University Comprehensive Cancer Center shared on a popular topic in “What’s in a Name? ‘Cancer’ and Desmoid Tumors”.** This highly informative conversation gave context to terminology used in the field of oncology, as well as insight into why the medical community talks about desmoid tumors the way they often do and the impacts of that framing. Throughout the year, DTRF hosted many similar **informative livestream events and networking events for the patient community.**

SEPTEMBER 2021.

DTRF had multiple research posters accepted by prominent annual oncology and rare disease conferences. Two DTRF posters were accepted by the November **2021 Connective Tissue Oncology Society (CTOS) Annual Meeting: Patient reported data of desmoid tumors during and after pregnancy from an international natural history study, and Desmoid tumor and molecular testing from patient reported data in an international natural history study.** One poster was accepted by the October **2021 NORD Breakthrough Summit:** The Desmoid Tumor Research Foundation registry and **NHS: 5 year status and update.** Thanks go to Registry PI, Dr. Kelly Mercier, along with the registry team, Dr. Maneesh Kumar, Amanda Lucas and Lynne Hernandez, for their work on these submissions which helped raise awareness and advance desmoid tumor research at these prestigious events!

SEPTEMBER 24-26, 2021 was our *successful annual Together We Will weekend*. This included our 8th Annual DTRF International Desmoid Tumor Research Workshop, a virtual Community Networking Event, two 90-minute virtual Patient Meeting webinars, and our Running for Answers Virtual Challenge ceremony. These events had record attendance with our new virtual format. See more information below.

OCTOBER 2021.

This year, DTRF took the first steps to *establish a central repository for desmoid tumor data* that can be accessed and analyzed by other researchers through the DTRF Coordinating Center. We announced in October that the ***DTRF Natural History Study data is now available through the Rare Disease Cures Accelerator - Data and Analytics Platform (RDCA-DAP)***. The ***RDCA-DAP***, created by the Critical Path Institute (C-Path), is an FDA-funded initiative that provides a centralized and standardized infrastructure to support and accelerate rare disease characterization, with the goal of accelerating therapy development across rare diseases. RDCA-DAP houses integrated patient-level data from diverse sources, including clinical trials, longitudinal observational studies, patient registries and real-world data (e.g. electronic health records) across a multitude of rare diseases. C-Path has partnered with NORD to leverage the IAMRARE registry platform, which DTRF uses. Through this partnership, DTRF has made all of the de-identified data in its registry available to researchers with an approval process. Learn more here.

It should also be noted that DTRF presented data from the Natural History Study in five abstracts at three global conferences this year. These abstracts included patient-reported information on treatments, diagnosis, molecular testing, pregnancy, and more.

NOVEMBER 17, 2021.

DTRF *presented at the Wnt/Beta-Catenin Targeted Drug Discovery Summit*. The Summit brought together world leaders and thought pioneers in the Wnt pathway. Leading pharma, pioneering biotechs and reputed academics shared lessons learned to regulate this pathway and exploit untapped therapeutic potentials - bringing novel treatments for patients from oncology to autoimmune to regenerative medicine.

NOVEMBER 18, 2021.

DTRF was honored to be selected as one of the ***2021 RARE Champions of Hope Awardees by Global Genes***, a leading global rare disease patient advocacy organization. DTRF received the ***RARE Champion in Advocacy - Foundation award*** at the awards event which “celebrates, honors, and recognizes Champions of Hope in the rare disease community. It encourages those involved in rare diseases to stand together as a community to support those who inspire us to do more, to do better, and to help positively impact patients and their families around the globe.” [Watch part of the award ceremony](#) where we receive the Global Genes Champions of Hope “RARE Champion in Advocacy” Award (beginning at 52:42).

NOVEMBER 2021.

An important ***new Phase 2/3 clinical trial called RINGSIDE*** for a gamma-secretase inhibitor drug currently known as AL102 began recruiting patients with progressing desmoid tumors. Watch Ayala Pharmaceuticals' presentation about the trial at the 2021 DTRF Annual Patient Meeting Webinar 1 (Begins at 58:30, or click the time code in the video description).

NOVEMBER 2021 we announced that ***two, stable, long-term desmoid tumor cell lines will soon be available*** through American Type Culture Collection (ATCC). With funding from DTRF, Dr. Mushriq Al-Jazrawe, a postdoctoral fellow in Dr. Jesse Boehm's lab at The Broad Institute, has successfully created two stable cell lines that replicate the features of desmoid tumors. The cell lines can survive for long periods of time in the lab, making it easier to study desmoid tumors and perform large-scale experiments. ***One of these cell lines harbors a T41A mutation in the CTNNB1 gene, while the other has an S45F mutation***. ATCC, one of the world's largest suppliers of cell lines to labs around the world, is a private, nonprofit, global biological resource center and standards organization that provides scientists with the biomaterials and resources they need to conduct critical life science research.

DECEMBER 2021.

Shaping the Future of Radiology in Desmoid Tumors. DTRF organized a Working Group with representatives from academic institutions and pharmaceutical companies to improve how imaging is used to monitor and predict prognosis in desmoid tumors. The goal of the group is to truly shape the future of radiology in desmoid tumors by moving beyond RECIST to validate new tumor measurement endpoints that are more applicable to the unique behavior of desmoid tumors and to have them incorporated into the FDA drug approval process. The first meetings of the Working Group will be held in early 2022.



JoeStrong's Volleyball Tourney, Austin, TX (October 2021)

2021 DTRF-FUNDED GRANTS

With many labs still being impacted or delayed by the pandemic, DTRF was pleased to announce the following new or continuing projects supported by grants in 2021:

- » **Gerlinde Wernig, MD**, Stanford School of Medicine, Department of Pathology, “*Studying CD47 blockade as an immunotherapy for desmoid-type fibromatosis.*” Year 1 of 2.

SUMMARY: Immunotherapy is the process of activating a person’s own immune system to attack their cancer. It has been successful in some types of cancers, like melanoma and lung cancer, but little has been done in desmoid tumors. Dr. Gerlinde Wernig will investigate if desmoid tumors express certain proteins that help them avoid the immune system. These proteins that help tumor cells avoid the immune system are called immune checkpoints and include CD47, PDL1, and CTLA4 (among others). If Dr. Wernig’s group finds that these immune checkpoints are expressed on desmoid tumor cells, they will determine if desmoid tumors respond to certain drugs that block these proteins, called immune checkpoint inhibitors. This may lead the way for further research in immunotherapy in desmoid tumors, including possible trials with immunotherapies – several of which are already approved for other cancers.

- » **Kris Vleminckx, PhD**, Ghent University, “*Investigating EZH2 as a druggable mediator of immune cell exclusion in desmoid tumors.*” Year 1 of 2.

SUMMARY: In previous research funded by DTRF, Dr. Kris Vleminckx has developed a desmoid tumor model in the frog model *Xenopus tropicalis*. Desmoid tumors can suppress the ability of the immune system to attack the tumor cells, and the current project will investigate if the drug tazemetostat can reverse this suppressive ability in their frog model. Tazemetostat is currently FDA-approved for use in epitheloid sarcoma, but the mechanism of action in desmoid tumors is unknown. Dr. Vleminckx has found that the treatment of the frog model with established desmoid tumors with tazemetostat shrinks the tumors. The team postulates that the drug affects the WNT/-catenin pathway and its mechanism of action may be related to the activation of the immune system. If successful, this research may lead to combinations of therapies or new therapies that involve activating the immune system to attack tumor cells.

- » **Manon N.G.J.A. Braat, MD**, University Medical Center Utrecht, Department of Radiology, “*Magnetic resonance image-guided high intensity focused ultrasound for patients with desmoid-type fibromatosis: the MAGNIFIED trial.*” Year 1 of 2.

SUMMARY: High-intensity focused ultrasound (HIFU) is a new way of treating desmoid tumors. It is minimally invasive and uses focused ultrasound waves to increase temperature and ablate tumors without surgery, similar to using a magnifying glass to burn a hole in a leaf. Magnetic resonance imaging (MRI) is used with HIFU to target just the tumor cells, without affecting the surrounding normal tissues. This trial is the first prospective HIFU study focused solely on desmoid tumors. The clinical trial is based in the Netherlands and aims to determine if HIFU is both safe and effective to treat desmoid tumors and leads to improved patient satisfaction. Over two years, they will assess if HIFU-treated tumors shrink in response to HIFU in addition to several patient reported outcomes. If successful, this trial may pave the way for larger clinical trials in HIFU so that HIFU becomes available for more patients who would benefit from this therapy.



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](#)
- » 2021 IRS Form 990*
- » 2021 Audited Financial Statement*

* To be added later



2021 DTRF Signature Events

TOGETHER WE WILL VIRTUAL WEEKEND, SEPT 24- 26, 2021

For over a decade, we've prioritized our annual fall weekend in Philadelphia as the time when our community comes together face-to-face. With the emergence of the COVID-19 pandemic in 2020, we pivoted to virtual Zoom meetings, seeing an opportunity to grow and reach more people than ever. Our 2021 *Together We Will* Virtual Weekend engaged a large number of medical professionals, patients and care partners, and fundraisers for a full weekend of setting research priorities, learning, networking and connecting.

- » ***Together We Will.... Advance Research***
DTRF Int'l Desmoid Tumor Research Workshop
Fri, Sept 24th
- » ***Together We Will.... Connect***
DTRF Virtual Community Networking Event
Fri, Sept 24th
- » ***Together We Will... Empower Ourselves With Information***
DTRF Patient Meeting Webinars
Sat, Sept 25th
- » ***Together We Will... Fund A Cure!***
RFA Virtual Challenge
June - Sept
- » ***RFA Virtual Challenge Ceremony***
Sept 26th



TOGETHER WE WILL... ADVANCE RESEARCH

Eighth International DTRF Desmoid Tumor Research Workshop, Sept. 24, 2021

Every year, DTRF holds an annual research workshop to facilitate international scientific education and collaboration. For the second year in a row, we were pleased to have 175 registrants from over 100 institutions in 31 different countries! Participants included those involved in desmoid tumor research, human genetics, drug development and related fields. In addition to short scientific presentations, significant time was dedicated to discussions around the important questions: What are the gaps in the science that we need to address next? How can we collaborate to move the science forward most efficiently?

Institutions and Industry represented at the Workshop included:

"Mauro Baschiroto" Institute for Rare Disease
A.C. Camargo Cancer Center
Ain Shams University (Egypt)
Algarve University Hospital Centre (Portugal)
All India Institute of Medical Sciences (AIIMS), New Delhi (India)
Angel H Roffo Institute of Oncology - Buenos Aires British Hospital (Argentina)
Aretaieio University Hospital, University of Athens (Greece)
Arkansas Children's Hospital
Ayala Pharmaceuticals
Bayer Pharmaceuticals
Beneficência Portuguesa de SP (Brazil)
Brigham and Women's Hospital
Broad Institute of MIT and Harvard
Cambridge University Hospital
Cancer Hospital of Pernambuco (Brazil)
Cancer Institute of Iran
Candiolo Cancer Institute, IRCCS-FPO (Italy)
Children's Hospital of Eastern Ontario (Canada)
Children's Hospital Los Angeles
Children's Hospital of Philadelphia
Children's Hospital, Westmead, Sydney (Australia)
Children's National Medical Center
Clemson University
Cleveland Clinic
Contactgroep Desmoid (Netherlands)
Daiichi - Sankyo
Department of Clinical Oncology, Copenhagen University Hospital at Herlev (Denmark)
Dmitry Rogachev National Medical Research Center of Pediatric Hematology, Oncology, Immunology (Russia)
Duke University Medical Center
Eastern Virginia Medical School
Erasmus University Medical Center (Netherlands)
European Institute of Oncology (Italy)
Fondazione IRCCS Istituto Nazionale dei Tumori (Italy)
Georgetown University
Germans Trias Hospital (Spain)
Ghent University (Belgium)
Hippokraton Hospital of Athens (Greece)
Hospital de Clinicas - Facultad de Medicina (Paraguay)
Huntsman Cancer Institute
Institut Bergonié (France)
Institute of Cancer Research (United Kingdom)
Johns Hopkins Hospital
Koch Institute for Integrated Cancer Research at MIT
Lille University and Centre Oscar Lambret (France)
Maine Medical Center
Mannheim University Medical Center (Germany)
Mayo Clinic
Medical College of Wisconsin
Memorial Sloan Kettering Cancer Center
Miami Cancer Institute
Michigan State University
Miguel Servet Hospital (Spain)
Mount Sinai Hospital/Lunenfeld-Tanenbaum Research Inst. (Canada)
N.N. Blokhin National Medical Research Center of Oncology (Russia)
N.N. Priorov Institute (Russia)
Nagoya University Hospital (Japan)
National Cancer Institute, National Institute of Health
National Organization of Rare Disorders (NORD)
Nemours Children's Hospital, Delaware
Novant Health Cancer Institute
Novant Health Orthopedics and Sports Medicine
Oncotrata (Brazil)
Oslo University Hospital (Norway)
PGH (Philippines)
Pontificia Universidad Javeriana (Columbia)
Princess Alexandra Hospital (Australia)
Rambam Medical Center (Israel)
Riley Hospital for Children
Royal Children's Hospital Melbourne (Australia)
Royal Marsden Hospital (United Kingdom)
RRD International
Rush University Medical Center
Sarcoma UK
SOS Desmoide
Sourasky Medical Center (Israel)
SpringWorks Therapeutics
St Stephens Hospital
Stanford Healthcare
Stanford University
Sylvester Comprehensive Cancer Center
The Chinese University of Hong Kong
The Institute of Cancer Research (United Kingdom)
The Ohio State University Comprehensive Cancer Center
The University of Sydney (Australia)
The University of Texas - MD Anderson Cancer Center
Thomas Jefferson university hospital
University College Hospital (United Kingdom)
University Hospital of Strasbourg (France)
University Hospitals of Leicester (United Kingdom)
University of Arizona College of Nursing
University of California at Davis
University of Colorado
University of Iowa Healthcare
University of Jos (Nigeria)
University of Miami
University of Michigan
University of Panama
University of Pretoria (South Africa)
University of Worcester
Utah Cancer Specialists
Vanderbilt University Medical Center
Washington University in Saint Louis
Westmead Hospital & The Children's Hospital at Westmead (Australia)

The following presentations were given at the 2021 Research Workshop:

- » **Benjamin Alman, MD**, Professor & Chair, Orthopaedic Surgery, Duke University, and Maneesh Kumar, MD, PhD, Research Director, DTRF Coordinating Center, *Welcome/DTRF Updates.*
- » **Yoshihiro Nishida, MD**, Rehabilitation & Orthopedic Oncology, Nagoya University Graduate School of Medicine, *“Diagnostic and therapeutic strategies for extra-abdominal desmoid-type fibromatosis: a longitudinal questionnaire survey to specialized centers in Japan.”*
- » **Hyo Song Kim, MD**, Department of Internal Medicine, Yonsei University, *“Genomic and transcriptomic analysis of desmoid tumor reveals enrichment of transforming growth factor beta responsive signature.”*
- » **Afshin Gangi, MD, PhD**, Strasbourg’s University Hospitals, *“Evaluation of the Cryodestruction of Non-Abdominopelvic Desmoid Tumors in Patients Progressing despite Medical Treatment (CRYODESMO01).”*
- » **Nicholas Penel, MD, PhD**, Director of Clinical Research & Innovation Dept/ Head of General Oncology in Medical Oncology, Oscar Lambret Cancer Centre. *“Prognosis value of S45F mutation of CTNNB1 in desmoid-type fibromatosis (DF). Prospective analysis of 500 consecutive patients (pts) from ALTITUDES Trial.”*
- » **Sara Coppola, MD**, Sarcoma Department, IEO Istituto Europeo di Oncologia, *“Abdominal Desmoid Tumors: A series from a single institution.”*
- » **Ed Neilan, MD, PhD**, Chief Medical Officer, NORD, *“Data sharing and RDCA-DAP.”*
- » **KEYNOTE: Stefani Spranger, PhD**, Assistant Professor of Biology, Massachusetts Institute of Technology, *“Not all anti-tumor T cell responses are created equal.”*
- » **Kris Vlemminckx, PhD**, Professor of Developmental Biology, Ghent University, *“Investigating the mechanisms and tumor-host interactions mediating desmoid tumor regression by EZH2 inhibition in CRISPR-based Xenopus tropicalis models.”*
- » **Gerlinde Wernig, MD**, Assistant Professor, and Cristabelle De Souza, MD, Postdoctoral Fellow, Department of Pathology, Stanford University, *“Evaluating CD47 blockade as a potential immunotherapy for desmoid-type fibromatosis.”*
- » **Mushriq Al-Jazrawe, PhD**, Post-doctoral Associate, The Broad Institute of MIT and Harvard, *“Developing a physiologically relevant target validation platform for desmoid tumors.”*
- » **Daniela Segat, PhD**, Researcher, “Mauro Baschirotto” Institute for Rare Diseases – BIRD Foundation, *“CRISPR/Cas9 approach for creating a simplified cellular model to study the Desmoid cells in response to external stimuli and in cellular communication.”*
- » **Ludmilla Thome Domingos Chinen, PhD**, Senior researcher, Pesquisadora Hospital ACCamargo- Fundação Antônio Prudente, *“Circulating tumor cells in desmoid tumors.”*
- » **Sameer Rastogi, MD**, Associate Professor, Dept. of Medical Oncology, All India Institute of Medical Sciences (AIIMS), New Delhi, *“Patient-reported outcomes in patients with desmoid type fibromatosis.”*
- » **Sukanya Subramanian, MD**, Transplant Gastroenterologist, Medstar Georgetown University Hospital, *“Intestinal transplant as rescue therapy.”*
- » **Ana Oton, MD**, VP of Medical Affairs, Nirogacestat Program, SpringWorks Therapeutics, *“An Update on the Phase 3 DeFi Trial Evaluating Nirogacestat in Adult Patients with Progressing Desmoid Tumors.”*
- » **Amanda Hoffman**, Desmoid Patient Advocate
- » **Gary Gordon, MD, PhD**, Chief Medical Officer, and Jason Kaplan, MD, Medical Director, Ayala Pharmaceuticals, *“AL102, Investigational Oral Gamma-Secretase Inhibitor for the Potential Treatment of Desmoid Tumors.”*
- » **Jessica White**, PhD student, SpringWorks Therapeutics, Inc., *“The Epidemiology of Desmoid Tumors in Denmark.”*
- » **Anne-Rose Schut, MD**, PhD candidate, Surgical Oncology and Medical Oncology, Erasmus Medical Center Cancer Institute, *“The evaluation of health-related quality of life issues experienced by patients with desmoid-type fibromatosis (The QUALIFIED STUDY).”*
- » **Kelly Mercier, PhD**, Principal Investigator, DTRF Patient Registry & Chair, DTRF Coordinating Center, *“DTRF’s Natural History Study.”*
- » **Rebecca Gladdy, MD, PhD**, Associate Professor, General surgery, University of Toronto, *“Role of Multidisciplinary Care in Complex Desmoids.”*
- » **Chandrajit Raut, MD, MSc**, Professor of Surgery, Harvard Medical School Chief, Division of Surgical Oncology, Department of Surgery, Brigham and Women’s Hospital Surgery Director, Center for Sarcoma and Bone Oncology, Dana-Farber Cancer Institute, *“The International Pregnancy Study.”*

TOGETHER WE WILL... CONNECT

DTRF Virtual Community Networking Event, Sept. 24, 2021

In the spirit of our previous “Speed Desmoidian-ing” in-person event, and our virtual “Timely Tumor Talks” event in the spring, this September virtual networking event provided an opportunity for patients and care partners to be paired off at random for one-on-one, 5-minute conversations. To ensure a more personal setting in our virtual Zoom environment, there were multiple breakout groups and interactive engagement tools.

We send a special thank you to Patient Advocate and FAP survivor Dakota Fisher-Vance who has led these networking events on our behalf for a number of years.

TOGETHER WE WILL... EMPOWER OURSELVES WITH INFORMATION

DTRF Virtual Patient Meeting Webinars, Sept. 25, 2021

Our virtual Patient Meeting consisted of two separate 90-minute webinars, focusing on topics important to the patient community. Roughly 200 community members joined us internationally. Patients submitted questions via the written Q&A as well as live after each panelist presentation.

Our patient meeting webinars included the following presentations:

- » ***An Overview of Medical Desmoid Tumor Treatments*** with Dr. Bernd Kasper (Mannheim University Medical Center, Germany)
- » ***Cryoablation*** with Dr. Afshin Gangi (University Hospital Strasbourg, France)
- » ***Ayala Pharmaceuticals’ Phase 2/3 Clinical Trial for Desmoid Tumors using AL102 (a gamma-secretase inhibitor)*** with Dr. Gary Gordon and Dr. Jason Kaplan (Ayala Pharmaceuticals)
- » ***The International Pregnancy Study*** with Marianna Coppola (Desmoid Foundation of Italy) & Dr. Chandrajit Raut (Dana Farber Cancer Institute)
- » ***Learning about the Clinical Trial Experience*** with Dr. Rashmi Chugh (University of Michigan) & Jon Fields, a Clinical Trial Participant
- » ***Learning about Compassionate Use*** with Dr. Ravin Ratan (MD Anderson Cancer Center) & Amanda Hoffman, a Beneficiary of Expanded Access (aka: Compassionate Use)
- » ***Learning about Prescription Assistance from Pharmaceutical Companies*** with Dr. Joe Germino (Bayer Corporation)

Special thanks goes to SpringWorks Therapeutics for its generous sponsorship of the Patient Meeting.

TOGETHER WE WILL... FUND A CURE!

RFA Virtual Challenge, June - Sept., 2021

The DTRF is principally funded by grassroots donations raised through signature DTRF events and patient-organized fundraising events. Volunteers, including patients, care partners and supporters, are encouraged to plan and host fundraisers around the country. We cannot accomplish our mission without this support and are always here to help!

With the pandemic in year 2, most in-person fundraisers were canceled again. The patient-care partner community rallied around our RFA Virtual Challenge. Patients participated from all over the world, feeling connected to each other while helping us achieve our collective fundraising goal. “Challengers” were invited to create their own personal challenges, match them with a fundraising goal, document their successes and share them with the community. We were so inspired by the ideas, support and drive to achieve these goals. There were opportunities for virtual check-ins and virtual celebrations daily around personal achievements and fundraising milestones.



Swing For A Cure - St. Louis (August 2021)

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THE DESMOID TUMOR
RESEARCH FOUNDATION

The Desmoid Tumor Research Foundation, Inc.
P.O. Box 273
Suffern, New York 10901

FOR INFORMATION PLEASE CONTACT:

Jeanne Whiting
Executive Director and Co-founder
jeanne@dtrf.org

dtrf.org