



THE DESMOID TUMOR  
RESEARCH FOUNDATION

**DTRF'S EFFORTS ENABLE LARGEST CLINICAL STUDY ON DESMOID TUMORS, AND  
NEW ENGLAND JOURNAL OF MEDICINE PUBLISHES BREAKTHROUGH STUDY RESULTS**

**PLUS, DTRF PATIENT ADVOCACY WORK HELPS ENABLE FDA FAST TRACK  
DESIGNATION FOR NIROGACESTAT FOR SPRINGWORKS THERAPEUTICS**

(New York, NY) – Thursday, December 20<sup>th</sup>, 2018 – The efforts of The Desmoid Tumor Research Foundation (DTRF) enabled the largest clinical study ever in desmoid tumors. The New England Journal of Medicine just released the breakthrough study results in their December 20, 2018 article, “Sorafenib for Advanced and Refractory Desmoid Tumors.”

To date, there are no approved drugs for desmoid tumors. This international study, a first of its kind, showed that patients treated with sorafenib reduced the risk of tumor growth or death by 7-fold, which are groundbreaking results.

In 2010, when DTRF heard news of promising results from a small pilot study conducted by Dr. Mrinal Gounder at Memorial Sloan Kettering Cancer Center with 26 desmoid tumor patients responding to sorafenib, they wasted no time in encouraging him to set a Phase III clinical trial in motion. Dr. Gounder then began a series of conversations with the FDA and the National Cancer Institute to start an international trial in this rare cancer with the purpose of getting sorafenib approved for patients with desmoid tumors.

It took over three years of efforts to finally open this study as there were countless barriers to conducting a study in a rare cancer. The Phase III study, which opened in 2014 across the United States and Canada, accrued 87 patients, a remarkable number for an ultra-rare cancer. Due to the tireless efforts of DTRF founders Jeanne Whiting and Marlene Portnoy, DTRF played a significant role in the recruitment for the trial, filling it in half the time expected-- a record time of just under 17 months.

This enabled the first clinical trial of its kind on desmoid tumors, and one of the largest Phase III clinical trials on a rare disease, to take place. Prior to this study, many cancer experts had declared that conducting a trial like this would be impossible or futile in desmoid tumors.

The implications of this study are immense as researchers now try to identify how research in desmoid tumors can be applicable to fighting other types of cancers, particularly colon cancer which is a leading cause of death worldwide.

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DTRF's efforts also played a role in moving forward another investigational treatment that was "stuck" on the shelves of a large pharmaceutical company after being de-prioritized for business reasons. Nirogacestat, a gamma secretase inhibitor, showed encouraging results in a Phase II study sponsored by the NCI. However, the drug's clinical development program was halted by the pharmaceutical company. DTRF advocated for nirogacestat because it showed such promise and patients with desmoid tumors had limited treatment options. SpringWorks Therapeutics was then conceived by Pfizer as an innovative way to advance investigational therapies that may hold significant promise for underserved patients.

In just a year since the company's formation, nirogacestat received fast-track designation from the FDA and SpringWorks is preparing to start a Phase III study in adult patients with desmoid tumors in first quarter 2019. Once again, when nirogacestat showed promising results in an early study, the two fearless women who co-founded DTRF advocated to move the drug forward. They galvanized the patient community they had built and filled up the Phase II trial in record-time, enabling SpringWorks to be able to spring into action with a Phase III trial to deliver treatments to patients who are in great need and without alternatives.

With the support of DTRF's efforts, there also will be two additional clinical trials on desmoid tumors in 2019. For a rare disease with an incidence rate of 5-6 per million, these are remarkable accomplishments.

For more information on The Desmoid Tumor Research Foundation please visit [www.dtrf.org](http://www.dtrf.org).

### **About The Desmoid Tumor Research Foundation**

**DTRF was founded in 2005 with the mission of facilitating and funding desmoid tumor research and supporting patients with information. 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. DTRF collaborates with dedicated researchers and clinicians worldwide to improve the lives of patients through education, awareness and support. Every year the foundation has gained momentum in its progress and has seen new clinical trials, emerging therapies and important new discoveries about the disease come to fruition.**

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