



THE DESMOID TUMOR  
RESEARCH FOUNDATION

**THE DESMOID TUMOR RESEARCH FOUNDATION ANNOUNCES THAT GROUNDBREAKING RESULTS FROM THE PHASE 3 SPRINGWORKS DEFI TRIAL WERE PUBLISHED TODAY IN THE NEW ENGLAND JOURNAL OF MEDICINE IN THE ARTICLE, "NIROGACESTAT, A  $\gamma$ -SECRETASE INHIBITOR FOR DESMOID TUMORS." THE FDA RECENTLY ACCEPTED THE NEW DRUG APPLICATION (NDA) FOR NIROGACESTAT AND IT IS NOW UNDER PRIORITY REVIEW.**

(Pittsburgh, PA) – Thursday, March 9th, 2023 – Years of advocacy by The Desmond Tumor Research Foundation (DTRF) facilitated clinical trials in nirogacestat in desmoid tumors. On February 27, 2023, SpringWorks Therapeutics announced FDA acceptance and Priority Review of their New Drug Application (NDA) for nirogacestat, for the treatment of adults with desmoid tumors. The NDA is based on the previously announced positive results from the DeFi (NCT03785964) phase 3 trial, a global, randomized, double-blind, placebo-controlled trial evaluating nirogacestat in adult patients with desmoid tumors. Results from the DeFi trial were published today in *The New England Journal of Medicine* in the article, "Nirogacesat, a  $\gamma$ -Secretase Inhibitor for Desmoid Tumors," by Drs. Gounder, Ratan, Kummar, Kasper et al.

This exciting news follows a 14 year, arduous path. Nirogacestat, a  $\gamma$ -secretase inhibitor, was first studied in patients with a range of solid tumors including seven desmoid tumor patients. A poster presentation of the promising results of this work in desmoid tumors caught the eye of a member of the National Cancer Institute's (NCI) Rare Tumors Initiative. The NCI team, Shivaani Kummar, MD and Abby Sandler, PhD, wanted to run a phase 2 trial, but due to the rare nature of the disease, concluded that they couldn't be successful without the support of a patient advocacy organization. After they found the DTRF, NCI and the DTRF enthusiastically collaborated to bring the phase 2 trial forward. While it had been anticipated

that it might take up to two years to fill this trial, with DTRF's outreach to patients, the trial was filled in less than 5 months.

Despite promising results that were coming out of the phase 2 study, a business decision resulted in halting the development of this molecule. Once again, the DTRF galvanized its resources and continued discussions with Pfizer staff, determined not to let this potential treatment stall because it showed such promise for desmoid tumor patients who have limited treatment options and no approved therapies. Supportive clinicians, as well as encouraging staff at Pfizer, supported the effort to move the drug forward. Eventually SpringWorks Therapeutics was created to acquire nirogacestat and other assets that showed potential in underserved patient populations.

Under newly-formed SpringWorks, the phase 3 DeFi trial for nirogacestat eventually opened. Over the following 15 months, with the help of the DTRF's outreach, the trial was fully enrolled. In September 2022, final trial data were presented in a Presidential Symposium during the ESMO Congress and at the DTRF's annual International Desmoid Tumor Research Workshop. In December 2022, SpringWorks filed a New Drug Application for nirogacestat in desmoid tumors which was accepted for Priority Review on February 27, 2023. If approved, nirogacestat could be available to patients as the first FDA-approved therapy in desmoid tumors.

With the support of the DTRF, three additional clinical trials on desmoid tumors are currently underway. For a rare disease with an incidence rate of 5-6 per million, these are remarkable accomplishments that demonstrate the power of collaboration between patient groups, researchers and industry.

#### About The Desmoid Tumor Research Foundation

The Desmoid Tumor Research Foundation (DTRF) was co-founded in 2005 by Jeanne Whiting and Marlene Portnoy, with the mission of facilitating and funding desmoid tumor research and supporting patients with information. The DTRF's mission is to aggressively fund research to accelerate the development of improved therapies, and ultimately find a cure for desmoid tumors. The DTRF collaborates with dedicated researchers and clinicians worldwide to facilitate research and improve the lives of patients through education, awareness and support. Every year the foundation's progress has gained momentum with new clinical trials, emerging therapies and important

new discoveries about the disease coming to fruition. The DTRF is the recipient of the National Organization of Rare Disorders *Abby S. Meyers Leadership Rare Impact Award*, and the Global Genes' Champions of Hope *Rare Champion in Advocacy Award*. For more information, please visit [www.dtrf.org](http://www.dtrf.org).

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