



FDA APPROVES OGSIVEO™ (NIROGACESTAT), AS THE FIRST MEDICAL THERAPY FOR THE TREATMENT OF ADULTS WITH PROGRESSING DESMOID TUMORS.

(Pittsburgh, PA) – November 27, 2023 – The U.S. Food and Drug Administration (FDA) announced approval today of OGSIVEO (nirogacestat) for the treatment of adults with progressing desmoid tumors. This is the first FDA-approved medical therapy in this rare disease. FDA approval follows positive results from the SpringWorks Therapeutics, Inc. (Nasdaq: SWTX) DeFi (NCT03785964) phase 3 clinical trial, a global, randomized, double-blind, placebo-controlled trial evaluating nirogacestat in adult patients with desmoid tumors. Results from the DeFi trial were published on March 9, 2023, in *The New England Journal of Medicine* in the article, “Nirogacesat, a γ -Secretase Inhibitor for Desmoid Tumors,” by Drs. Gounder, Ratan, Kummar, Kasper et al.

OGSIVEO (nirogacestat) is an oral gamma secretase inhibitor. For more information, visit www.ogsiveo.com.

“The significance of FDA approval of OGSIVEO (nirogacestat) to desmoid tumor patients cannot be overstated. This is an extraordinary victory in the area of rare diseases, and demonstrates the power of collaboration among patients, industry and researchers.”

- Jeanne Whiting, Executive Director Emeritus, Desmoid Tumor Research Foundation

“SpringWorks is grateful to the Desmoid Tumor Research Foundation, as well as the patients and investigators who participated in our trials, whose enduring commitment and enthusiasm helped us get to this point. We are honored to provide the first FDA-approved therapy and committed to offering ongoing support to the desmoid tumor community throughout their treatment journey on OGSIVEO.”

- *Saqib Islam, Chief Executive Officer, SpringWorks Therapeutics*

The collaborative path to approval

The Desmoid Tumor Research Foundation (DTRF) has been instrumental in the nearly 15-year path to approval of OGSIVEO (nirogacestat). The drug 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 early work in desmoid tumors caught the eye of members of the National Cancer Institute's (NCI) Rare Tumors Initiative, Shivaani Kummar, MD and Abby Sandler, PhD. The pair were interested in a phase 2 trial, but due to the rare nature of the disease, concluded that in order to be successful they needed the support of a patient advocacy organization. Synergistic collaboration between the DTRF and NCI brought the phase 2 trial forward, and with the DTRF's outreach efforts, filled it in less than five months.

Despite promising results that were coming out of the phase 2 study, a business decision resulted in halting the development of the asset. Determined not to let this potential treatment stall, the DTRF once again galvanized its resources and continued discussions with Pfizer staff. Supportive clinicians, as well as encouraging staff at Pfizer, supported the effort to move the drug forward. Eventually SpringWorks Therapeutics was created to license nirogacestat and other assets that showed potential in underserved patient populations.

Under newly-formed SpringWorks, the phase 3 DeFi trial for nirogacestat opened. During the conduct of the DeFi trial, and with the help of DTRF's patient outreach, the trial was fully enrolled in July 2020. In September 2022, trial data were presented in a Presidential Symposium during the ESMO Congress and at the annual DTRF International Desmoid Tumor Research Workshop. In December 2022, SpringWorks filed a New Drug Application for nirogacestat which was accepted for Priority Review on February 27, 2023. Today, FDA approval of OGSIVEO (nirogacestat), makes it the first FDA-approved drug for the treatment of adults with desmoid tumors.

"This breakthrough represents the power of patient advocacy. The Desmoid Tumor Research Foundation's role in OGSIVEO's path to approval represents collaboration at its finest. Were it not for their advocacy work to shepherd the drug through the trial phases, facilitate communication among stakeholders, and use their outreach to help fill the trial, we may not be here today. I'm honored to be part of the process that brings the first-ever FDA-approved medical therapy to desmoid tumor patients."

- Mrinal Gounder, MD, Memorial Sloan Kettering Cancer Center

About desmoid tumors

Desmoid tumors (also known as aggressive fibromatosis) are rare, invasive, soft tissue tumors that form in the connective tissues of the body. They affect 5-6 per 1 million people per year. Although they do not metastasize, desmoid tumors can be locally aggressive and severely damage surrounding tissues and vital structures as they grow. They are highly recurrent and can be multifocal. These tumors may occur anywhere in the body (abdominal, intraabdominal, and extra-abdominal locations) and approximately 5-10% arise in the context of a rare genetic condition known as Familial Adenomatous Polyposis (F.A.P.). Previously, there have been no approved therapies for desmoid tumors.

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 a 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.

For more information about this important milestone for desmoid tumor patients, read the FDA's and SpringWorks' press releases linked below.

[SpringWorks Therapeutics Press Release](#)

[US FDA Press Release](#)

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