



SpringWorks Therapeutics Receives FDA Fast Track Designation for Nirogacestat for the Treatment of Adult Patients with Desmoid Tumors

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STAMFORD, Conn.--([BUSINESS WIRE](#))--SpringWorks Therapeutics, a clinical-stage biopharmaceutical company focused on developing life-changing medicines for patients with severe rare diseases and cancer, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for nirogacestat, an investigational, oral, small molecule, selective gamma-secretase inhibitor, for the treatment of adult patients with progressive, unresectable, recurrent or refractory desmoid tumors or deep fibromatosis.

Desmoid tumors are rare and debilitating soft-tissue tumors that can occur in both children and adults. Depending on their size and location, desmoid tumors can cause severe morbidities such as pain, internal bleeding, disfigurement, and limited range-of-motion. In some cases, desmoid tumors can be fatal if they impact vital organs.¹ It is estimated that 900 to 1,500 new cases of desmoid tumors are diagnosed each year in the United States.²

"The Fast Track designation recognizes that desmoid tumors have a substantial impact on the lives of patients, and that nirogacestat has the potential to address the significant needs faced by this community who currently do not have an FDA-approved treatment," said Saqib Islam, Chief Executive Officer of SpringWorks Therapeutics. "We look forward to continuing to work closely with the FDA throughout our development program with the hope of ultimately bringing this important medicine to patients."

The FDA's Fast Track program is designed to expedite the development and review of drugs with the potential to treat serious or life-threatening conditions, and with nonclinical or clinical data that demonstrate the potential to address unmet medical needs. Fast Track designation enables a company to have frequent communication with the FDA throughout the drug development and review process.³

In June 2018, the U.S. FDA granted Orphan Drug designation for nirogacestat for the treatment of desmoid tumors. SpringWorks expects to initiate the DeFi Study, a global Phase 3, double-blind, randomized, placebo-controlled trial in patients with desmoid tumors in the first half of 2019.

About Desmoid Tumors

Desmoid tumors (also referred to as aggressive fibromatosis or desmoid-type fibromatosis) are rare, non-metastatic tumors of connective tissue cells that can arise in any part of the body, with the most common sites being the upper and lower extremities, abdominal walls, thoracic areas, and the head and neck. The severity of desmoid tumors and associated symptoms varies based on their size and location. Desmoid tumors can cause severe morbidities such as pain, internal bleeding, disfigurement, limited range of motion, and in some cases, fatality if they impact vital organs. While they can affect children and adults, desmoid tumors are more commonly diagnosed in young adults between 20-30 years of age, with a two-to-three-fold predominance in females.^{1,4} It is estimated that desmoid tumors affect 2 to 4 per million people worldwide, and that there are 900 to 1,500 new cases diagnosed per year in the United States.² Historically, desmoid tumors were treated with surgical resection or in severe cases, amputation, but even with these interventions, high rates of tumor regrowth have been observed.⁵ There are currently no FDA-approved therapies for the treatment of desmoid tumors.

About Nirogacestat

Nirogacestat is an investigational, oral, small molecule, selective gamma-secretase inhibitor. Gamma secretase is an integral membrane protein that cleaves multiple different transmembrane protein complexes, including Notch, which is believed to play a role in activating pathways that contribute to desmoid tumor growth. Nirogacestat has been investigated in 24 patients with desmoid tumors across Phase 1 and Phase 2 clinical trials.

About SpringWorks Therapeutics

At SpringWorks Therapeutics, a clinical-stage biopharmaceutical company, we are driven to develop life-changing medicines for patients with severe rare diseases and cancer. Since our launch in 2017, we have worked to identify, re-prioritize, and advance promising science, beginning with our licensed clinical therapies from Pfizer Inc. We pioneer efficient pathways for drug development, leveraging shared-value partnerships with patient advocacy groups, innovators in industry and academia, and investors so that together, we can unlock the full potential of science for patients. Two of our therapies will be entering pivotal studies in the first half of 2019: nirogacestat, a gamma secretase inhibitor for the treatment of desmoid tumors, and PD-0325901, a MEK 1/2 inhibitor for neurofibromatosis type 1 patients with plexiform neurofibromas. PD-0325901 also holds promise as the backbone for combination therapies to treat metastatic solid tumors. Our pipeline also includes two earlier-stage assets for neurological and hematological conditions. At SpringWorks, we ignite the power of promising science to unleash new possibilities for patients. For more information, please visit www.springworkstx.com.

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References

¹ Gronder, M. M., Thomas, D. M., & Tap, W. D. (2017). Locally Aggressive Connective Tissue Tumors. *Journal of Clinical Oncology*, 36(2), 202-209. doi:10.1200/JCO.2017.75.8482.

² "Desmoid Tumor." U.S. National Library of Medicine, National Institutes of Health, ghr.nlm.nih.gov/condition/desmoid-tumor#statistics.

³ U.S. Food & Drug Administration. (2018). Fast Track. Retrieved from <https://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm>

⁴ Skubitz, K. M. (2017). Biology and Treatment of Aggressive Fibromatosis or Desmoid Tumor. *Mayo Clinic Proceedings*, 92(6), 947-964.

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⁵ Scaramussa, F.S. & Castro, U. B. (2016). Desmoid Tumor in Hand: A Case Report. *SM Journal of Orthopedics*, 2(3),1036.

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