



## SpringWorks Therapeutics Receives Breakthrough Therapy Designation for Nirogacestat for the Treatment of Adult Patients with Progressive, Unresectable, Recurrent or Refractory Desmoid Tumors

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STAMFORD, Conn.--([BUSINESS WIRE](#))--SpringWorks Therapeutics, Inc. a clinical-stage biopharmaceutical company focused on developing life-changing medicines for patients with severe rare diseases and cancer, today announced the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for nirogacestat, an oral, selective, small molecule, gamma-secretase inhibitor, for the treatment of adult patients with progressive, unresectable, recurrent or refractory desmoid tumors or deep fibromatosis. The Breakthrough Therapy Designation was based on Phase 1 and Phase 2 data evaluating nirogacestat as a monotherapy in patients with desmoid tumors.

Desmoid tumors are rare and often debilitating and disfiguring soft-tissue tumors that can aggressively invade surrounding healthy tissues and cause significant morbidities, including severe pain, internal bleeding, incapacitating loss of range of motion, and, in rare cases, death.<sup>1</sup> It is estimated that 1,000 to 1,500 new desmoid tumor patients are diagnosed each year in the United States.<sup>2,3</sup> There are currently no therapies approved by the FDA for the treatment of desmoid tumors.

"We are committed to pursuing the rapid development of nirogacestat given the important need for new therapies for patients with desmoid tumors and are pleased to receive this Breakthrough Therapy Designation," said Saqib Islam, Chief Executive Officer of SpringWorks. "We are currently enrolling adult patients in our Phase 3 DeFi trial and will continue to work closely with the FDA with the goal of bringing nirogacestat to patients as quickly as possible."

The FDA's Breakthrough Therapy Designation is designed to expedite the development and regulatory review of medicines that are intended to treat a serious condition. To qualify for this designation, preliminary clinical evidence must demonstrate that the medicine may provide substantial improvement over currently available therapy on at least one clinically significant endpoint.<sup>4</sup>

Previously, the FDA had granted nirogacestat Orphan Drug Designation for the treatment of desmoid tumors (June 2018) and Fast Track Designation for the treatment of adult patients with progressive, unresectable, recurrent or refractory desmoid tumors or deep fibromatosis (November 2018).

### About Desmoid Tumors

Desmoid tumors, also referred to as aggressive fibromatosis or desmoid-type fibromatosis, are rare and often debilitating and disfiguring soft tissue tumors characterized by a growth pattern that can invade surrounding healthy tissues, including joints, muscle and viscera. While they can arise in any part of the body, the most common sites are the upper and lower extremities, abdominal walls, thoracic areas, and the head and neck. The severity of a desmoid tumor can vary based on the location of the tumor and the aggressiveness of its growth pattern. Desmoid tumors can cause significant morbidities, including severe pain, internal bleeding, incapacitating loss of range of motion, and, in rare cases, death.<sup>1</sup>

Desmoid tumors typically occur in patients between the ages of 15 to 60 years, and are more commonly diagnosed in young adults between 30-40 years of age, with a two-to-three times higher prevalence in females.<sup>1,5</sup> It is estimated that there are 1,000 to 1,500 new cases diagnosed per year in the United States.<sup>2,3</sup>

Historically, desmoid tumors were treated with surgical resection, but this approach has become less favored due to a high recurrence rate after surgery.<sup>6</sup> There are currently no FDA-approved therapies for the treatment of desmoid tumors.

### About Nirogacestat

Nirogacestat is an oral, selective, small molecule gamma-secretase inhibitor in Phase 3 clinical development for the treatment of desmoid tumors. Gamma secretase cleaves multiple 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. In these studies, treatment with nirogacestat demonstrated a 100% disease control rate as measured by RECIST criteria, and median progression free survival was not reached by the time of publication in either trial due to lack of patients progressing on therapy. Nirogacestat was generally well-tolerated in these studies, with many patients remaining on treatment for years and only one desmoid tumor patient in the combined trials discontinuing treatment due to an adverse event. The most common adverse events in the Phase 2 study were diarrhea, skin disorders and hypophosphatemia.

### About SpringWorks Therapeutics

At SpringWorks, 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 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 potential of science and bring new therapies to underserved patients.

Nirogacestat, our gamma secretase inhibitor for the treatment of desmoid tumors, is currently in a Phase 3 clinical trial (DeFi Trial), and SpringWorks expects to initiate a Phase 2b clinical trial of mirdametininib, our MEK 1/2 inhibitor for neurofibromatosis type 1 patients with plexiform neurofibromas, in the third quarter of 2019 (ReNeu Trial). Both nirogacestat and mirdametininib also hold promise as backbones for combination therapies to treat metastatic cancers. At SpringWorks, we ignite the power of promising science to unleash new possibilities for patients. For more information, please visit [www.springworkstx.com](http://www.springworkstx.com).

Follow SpringWorks Therapeutics on social media: [@SpringWorksTx](#) and [LinkedIn](#).

## References

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