European Commission Grants Orphan Drug Designation for Nirogacestat for the Treatment of Soft Tissue Sarcoma

September 24, 2019

STAMFORD, Conn., Sept. 24, 2019 (GLOBE NEWSWIRE) -- SpringWorks Therapeutics, Inc. (Nasdaq: SWTX), a clinical-stage biopharmaceutical company focused on developing life-changing medicines for patients with severe rare diseases and cancer, today announced the European Commission has granted Orphan Drug Designation for nirogacestat, an oral, selective, small molecule, gamma-secretase inhibitor, for the treatment of soft tissue sarcoma.

SpringWorks is currently enrolling patients in the Phase 3 DeFi trial of nirogacestat for the treatment of adult patients with progressing desmoid tumors, which are a type of soft-tissue tumors that are often treated by sarcoma specialists. Desmoid tumors are rare and often debilitating and disfiguring, and 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.[1] It is estimated that 1,000 to 1,500 new desmoid tumor patients are diagnosed each year in the United States.[2,3] There are currently no therapies approved by the U.S. Food and Drug Administration (FDA) for the treatment of desmoid tumors.

“This Orphan Drug Designation in the European Union is another important development for SpringWorks and follows the Orphan Drug, Fast Track and Breakthrough Therapy Designations already granted for nirogacestat in the U.S. by the FDA,” said Saqib Islam, Chief Executive Officer of SpringWorks. “We are currently enrolling adult patients in our DeFi trial and will continue to work closely with global regulators with the goal of bringing nirogacestat to patients as quickly as possible.”

The European Commission grants orphan medicinal status for products intended for the treatment, prevention or diagnosis of life-threatening or very serious conditions that affect no more than 5 in 10,000 people in the European Union, and where the product represents a significant benefit over existing treatments. Orphan designation provides companies with certain benefits and incentives in the EU, including a 10-year period of market exclusivity after product approval, reduced regulatory fees and protocol assistance.[4]

Nirogacestat previously received Orphan Drug Designation from the FDA for the treatment of desmoid tumors (June 2018), and Fast Track and Breakthrough Therapy Designations from the FDA for the treatment of adult patients with progressive, unresectable, recurrent or refractory desmoid tumors or deep fibromatosis (November 2018 and August 2019).

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 wall, 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.[1]

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.[1,5] It is estimated that there are 1,000 to 1,500 new cases diagnosed per year in the United States.[2,3]

Historically, desmoid tumors were treated with surgical resection, but this approach has become less favored due to a high recurrence rate after surgery.[6] 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

SpringWorks is a clinical-stage biopharmaceutical company applying a precision medicine approach to acquiring, developing and commercializing life-changing medicines for underserved patient populations suffering from devastating rare diseases and cancer. SpringWorks has a differentiated portfolio of small molecule targeted oncology product candidates and is advancing two potentially registrational clinical trials in rare tumor types, as well as several other programs addressing highly prevalent, genetically defined cancers. SpringWorks’ strategic approach and operational excellence in clinical development have enabled it to rapidly advance its two lead product candidates into late-stage clinical trials while simultaneously entering into multiple shared-value partnerships with industry leaders to expand its portfolio. For more information, please visit www.springworkstx.com.

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Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding SpringWorks' clinical trials and its strategy, business plans and focus. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, those related to the timing for completion of our clinical trials of our product candidates, whether and when, if at all, SpringWorks' product candidates will receive approval from the U.S. Food and Drug Administration, or FDA, or other foreign regulatory authorities, the potential benefits of receiving Orphan Drug Designation from the FDA or other foreign regulatory authorities, competition from other biopharmaceutical companies, and other risks identified in SpringWorks' SEC filings, including its final prospectus for its initial public offering and subsequent filings with the SEC. SpringWorks cautions you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. SpringWorks disclaims any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent SpringWorks' views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date.

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