aTyr Pharma Announces Phase 2 Study of Efzofitimod in Patients with Systemic Sclerosis-Associated Interstitial Lung Disease (SSc-ILD) Following FDA Clearance of IND Application

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Proof-of-Concept study in SSc-ILD expected to begin in 2023.

Efzofitimod has been granted U.S. FDA orphan drug designation for SSc and Fast Track designation for SSc-ILD.

SAN DIEGO, March 01, 2023 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE), a clinical stage biotherapeutics company engaged in the discovery and development of first-in-class medicines from its proprietary tRNA synthetase platform, today provided additional details regarding its plans to initiate a Phase 2 study of its lead therapeutic candidate, efzofitimod, in patients with systemic sclerosis (SSc, also known as scleroderma)-associated interstitial lung disease (ILD) following the clearance of an Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA) announced earlier this year.

Efzofitimod is a potential first-in-class immunomodulator that downregulates innate and adaptive immune responses in uncontrolled inflammatory disease states via selective modulation of neuropilin-2 (NRP2). The company is currently investigating efzofitimod in patients with pulmonary sarcoidosis, a major form of ILD, in a global Phase 3 study called EFZO-FIT™.

“We are thrilled to expand our efzofitimod clinical program to include a Phase 2 study in SSc-ILD with clearance of this IND, which is supported by preclinical data showing efzofitimod reduces lung and skin fibrosis in an animal model of SSc-ILD and clinical data demonstrating proof-of-concept for efzofitimod in patients with pulmonary sarcoidosis,” said Sanjay S. Shukla, M.D., M.S., President and CEO of aTyr. “ILD is the leading cause of death in patients with scleroderma. Current treatment options may help to slow lung function decline but do not impact underlying disease or improve quality of life. There remains a medical need for more effective and safer therapies for patients with this debilitating disease.”

“Efzofitimod’s clinical proof-of-concept in sarcoidosis as well as the translational effects seen in an animal model of SSc-ILD provide strong rationale to evaluate this novel immunomodulator in patients with ILD that results from an underlying rheumatologic condition such as scleroderma,” said Kristin Highland, M.D., Director, Rheumatic Lung Disease Program at the Cleveland Clinic. “The prospect of a new therapeutic target for scleroderma-associated lung disease is welcome news for patients suffering from this disease which has limited treatment options.”

The Phase 2 study is expected to be a randomized, double-blind, placebo-controlled, proof-of-concept study to evaluate the efficacy, safety and tolerability of efzofitimod in patients with SSc-ILD. This is expected to be a 28-week study with three parallel cohorts randomized 2:2:1 to either 270 mg or 450 mg of efzofitimod or placebo dosed intravenously monthly for a total of 6 doses. The study intends to enroll 25 patients with progressive disease who are currently receiving background mycophenolate therapy (standard of care) at multiple centers in the United States. Skin biopsies are intended to be performed at baseline and week 12. The primary objective of the study will be to evaluate the efficacy of multiple doses of intravenous efzofitimod on pulmonary, cutaneous and systemic manifestations in patients with SSc-ILD. Secondary objectives include safety and tolerability.

Efzofitimod has been shown to reduce lung and skin fibrosis in animal models of SSc and idiopathic pulmonary fibrosis. The pathology of SSc-ILD is driven by the same immune cells that are central to pulmonary sarcoidosis pathology, and NRP2 is upregulated on these cells. Efzofitimod has also been shown to reduce key pro-inflammatory markers that are central to this pathology in a clinical study in patients with pulmonary sarcoidosis. Efzofitimod has been granted U.S. FDA orphan drug designation for the treatment of SSc and Fast Track designation for the treatment of SSc-ILD.

Systemic sclerosis is a chronic, progressive, autoimmune disease characterized by inflammation and fibrosis of connective tissues throughout the body, including the skin and other internal organs. SSc that occurs in the lungs is called SSc-ILD. It is estimated that approximately 100,000 people in the U.S. are affected by SSc and up to 80% may develop ILD. SSc-ILD causes inflammation in the lungs and, if left untreated, can result in scarring, or fibrosis, that causes permanent loss of lung function. ILD is the primary cause of death in patients with SSc. Current treatment options for SSc-ILD are limited, mainly focus on slowing disease progression and are associated with significant toxicity.

About Efzofitimod

aTyr is developing efzofitimod as a potential therapeutic for patients with fibrotic lung disease. Efzofitimod, a fusion protein comprised of the immunomodulatory domain of histidyl-tRNA synthetase fused to the FC region of a human antibody, is a selective modulator of neuropilin-2 that downregulates innate and adaptive immune response in inflammatory disease states. aTyr’s lead indication for efzofitimod is pulmonary sarcoidosis, a major form of interstitial lung disease. Clinical proof-of-concept for efzofitimod was recently established in a Phase 1b/2a multiple-ascending dose, placebo-controlled study of efzofitimod in patients with pulmonary sarcoidosis, which demonstrated safety and a consistent dose response and trends of benefit of efzofitimod compared to placebo on key efficacy endpoints, including steroid reduction, lung function, clinical symptoms and inflammatory biomarkers. aTyr is currently conducting EFZO-FIT™, a global pivotal Phase 3 study of efzofitimod in pulmonary sarcoidosis.

About aTyr

aTyr is a biotherapeutics company engaged in the discovery and development of first-in-class medicines from its proprietary tRNA synthetase platform. aTyr’s research and development efforts are concentrated on a newly discovered area of biology, the extracellular functionality and signaling pathways of tRNA synthetases. aTyr has built a global intellectual property estate directed to a potential pipeline of protein compositions derived from 20 tRNA synthetase genes and their extracellular targets. aTyr’s primary focus is efzofitimod, a clinical-stage product candidate which binds to the neuropilin-2 receptor and is designed to downregulate immune engagement in fibrotic lung disease. For more information, please visit http://www.atyrpharma.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are usually identified by the use of words such as “anticipates,” “believes,” “estimates,” “can,” “expects,” “intends,” “may,” “plans,” “prospect” “will,” and variations of such words or similar expressions. We intend these forward-looking statements to be covered by such safe harbor provisions for forward-looking statements and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements include statements regarding the potential of efzofitimod to be a new treatment option that resolves inflammation and subsequent fibrosis in those living with SSc-ILD as well as a first-in-class immunomodulator, the expected Phase 2 study design, including enrollment targets, and the potential effects of SSc-ILD if left untreated. These forward-looking statements also reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects, as reflected in or suggested by these forward-looking statements, are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. Furthermore, actual results may differ materially from those described in these forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, uncertainty regarding the COVID-19 pandemic, risks associated with the discovery, development and regulation of our product candidates, the risk that we or our partners may cease or delay preclinical or clinical development activities for any of our existing or future product candidates for a variety of reasons (including difficulties or delays in patient enrollment in planned clinical trials), the possibility that existing collaborations could be terminated early, and the risk that we may not be able to raise the additional funding required for our business and product development plans, as well as those risks set forth in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022 filed with the SEC on November 14, 2022 and in our other SEC filings. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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