# aTyr Pharma Announces Phase 3 Study of Efzofitimod (ATYR1923) in Pulmonary Sarcoidosis

May 16, 2022

*Global pivotal EFZO-FIT™ study expected to begin in the third quarter of 2022*

*Primary endpoint will evaluate steroid sparing effect of efzofitimod compared to placebo*

SAN DIEGO, May 16, 2022 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE), a biotherapeutics company engaged in the discovery and development of innovative medicines from its proprietary tRNA synthetase platform, today announced a Phase 3 study evaluating the efficacy and safety of its lead therapeutic candidate, efzofitimod (ATYR1923), in patients with pulmonary sarcoidosis. The study, which will be known as EFZO-FIT ™, is expected to initiate in the third quarter of 2022.

The EFZO-FIT ™study is a global Phase 3 randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of efzofitimod in patients with pulmonary sarcoidosis. This will be a 52-week study consisting of three parallel cohorts randomized equally to either 3.0 mg/kg or 5.0 mg/kg of efzofitimod or placebo dosed intravenously once a month for a total of 12 doses. The study intends to enroll 264 subjects with pulmonary sarcoidosis at multiple centers in North America, Europe and Japan. The trial design will incorporate a forced steroid taper. The primary endpoint of the study is steroid reduction. Secondary endpoints include measures of lung function and sarcoidosis symptoms.

Efzofitimod is a first-in-class immunomodulator that downregulates innate and adaptive immune responses in uncontrolled inflammatory diseases states via selective modulation of neuropilin-2 (NRP2). Clinical proof-of-concept was recently established for efzofitimod in a Phase 1b/2a study in patients with pulmonary sarcoidosis, a major form of interstitial lung disease (ILD).

"We are very pleased with the input we received from the FDA regarding the design of this important study of efzofitimod in pulmonary sarcoidosis patients," said Sanjay S. Shukla, M.D., M.S., President and CEO of aTyr. "We aligned with the FDA on the prioritization of efficacy endpoints, with a primary focus on steroid reduction, which is clinically meaningful to patients and providers. This late-stage study is a major milestone for aTyr and the sarcoidosis community, and we look forward to the expected initiation of the study in the third quarter of this year."

"This study is a major step forward in developing a new treatment for patients with sarcoidosis," said Robert P. Baughman, M.D., Emeritus Profess of Medicine at the University of Cincinnati. "Treatment options for patients with sarcoidosis are limited. Prednisone toxicity is the most common complaint of patients on therapy. The steroid sparing primary endpoint prioritized by the FDA highlights the need for new, disease modifying treatment options that can improve clinical outcomes while reducing steroid toxicity with the goal of truly improving quality of life for patients."

**About Pulmonary Sarcoidosis**

Pulmonary sarcoidosis is an inflammatory disease characterized by the formulation of granulomas, clumps of inflammatory cells, in one or more organs of the body. Approximately 200,000 Americans live with pulmonary sarcoidosis and the prognosis ranges from benign and self-limiting to chronic, debilitating disease, permanent loss of lung function and death. Current treatment options include corticosteroids and other immunosuppressive therapies, which have limited efficacy and are associated with serious side-effects that many patients cannot tolerate long-term.

**About Efzofitimod**

aTyr is developing efzofitimod as a potential therapeutic for patients with fibrotic lung disease. Efzofitimod, a fusion protein comprised of the immuno-modulatory 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 intends to initiate EFZO-FIT ™, a Phase 3 study of efzofitimod in pulmonary sarcoidosis patients, in the third quarter of 2022.

**About aTyr**

aTyr is a biotherapeutics company engaged in the discovery and development of innovative 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 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," "expects," "intends," "may," "plans," "projects," "seeks," "should," "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 potential therapeutic benefits and applications of efzofitimod, timelines and plans with respect to certain development activities, including the timing of clinical trials; and certain development goals. 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 March 31, 2022 filed with the SEC on May 10, 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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Source: aTyr Pharma, Inc.