aTyr Pharma Announces Publication Demonstrating Efficacy of Efzofitimod in Pulmonary Sarcoidosis in the European Respiratory Journal

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Post hoc analysis of Phase 1b/2a study demonstrated statistically significant difference in time-to-first-relapse for corticosteroid use in therapeutic (3.0 and 5.0 mg/kg efzofitimod) vs subtherapeutic (1.0 mg/kg efzofitimod and placebo) groups.

54.4% of patients in the subtherapeutic group relapsed following corticosteroid taper, compared to 7.7% in the therapeutic group.

SAN DIEGO, Oct. 02, 2024 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: ATYR) ("aTyr" or the "Company"), a clinical stage biotechnology company engaged in the discovery and development of first-in-class medicines from its proprietary tRNA synthetase platform, today announced the publication of a post hoc analysis of the Phase 1b/2a clinical trial of its lead therapeutic candidate, efzofitimod, in patients with pulmonary sarcoidosis, a major form of interstitial lung disease, in the *European Respiratory Journal.* The publication, entitled, "Therapeutic Doses of Efzofitimod Demonstrate Efficacy in Pulmonary Sarcoidosis," is available on the Journal's website and at: https://doi.org/10.1183/23120541.00536-2024.

"Oral corticosteroids are considered first-line therapy in patients with sarcoidosis. However, while they may help improve symptoms, long-term use is often associated with significant toxicity and reduced quality of life," said Ogugua Ndili Obi, M.D., M.P.H., M.Sc., Associate Professor of Medicine and Clinical Director of the Sarcoidosis Program at the Brody School of Medicine at East Carolina University and lead author of the paper. "Many patients find it difficult to taper and/or maintain reduced steroid doses, as symptoms often flare or disease remains refractory, so the low relapse rate seen for the efzofitimod therapeutic group and a significant difference in time-to-first-relapse in this 24-week study is impressive. The ability of a therapy such as efzofitimod to maintain disease control while decreasing or discontinuing steroid use entirely would be clinically important and very meaningful to patients."

The publication reports findings from a post hoc analysis of a pre-specified endpoint in the Phase 1b/2a randomized, double-blind, placebo-controlled, 24-week study of efzofitimod in 37 patients with pulmonary sarcoidosis receiving oral corticosteroid treatment who underwent a forced steroid taper in the first 8 weeks of the study. In this pooled analysis, the time-to-first-relapse was significantly shorter in the subtherapeutic group (1.0 mg/kg efzofitimod and placebo) than in the therapeutic group (3.0 and 5.0 mg/kg efzofitimod). The median time-to-first-relapse in the subtherapeutic group was 126 days, whereas only one of 17 patients in the therapeutic group had relapsed by the end of the study (p=0.017). Furthermore, 54.4% of patients in the subtherapeutic group relapsed for steroid use following steroid taper, compared to 7.7% of patients in the therapeutic group.

"We continue to publish data from our Phase 1b/2a study that further demonstrate the efficacy of efzofitimod in pulmonary sarcoidosis patients and positions this first-in-class immunomodulator as a promising new treatment option that can reduce or avoid steroid-related toxicity," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "We believe we are on the cusp of a paradigm shift in the treatment for sarcoidosis, where patients may have the opportunity to receive clinically validated therapies that can treat their underlying disease without incurring added harm."

Efzofitimod is a tRNA synthetase derived therapy that selectively modulates activated myeloid cells through neuropilin-2 to resolve inflammation without immune suppression and potentially prevent the progression of fibrosis. Efzofitimod is currently being investigated in the global pivotal Phase 3 EFZO-FIT™ study in 268 pulmonary sarcoidosis patients (NCT05415137). Efzofitimod has received orphan drug designation in the U.S., E.U. and Japan for sarcoidosis and Fast Track designation in the U.S. for pulmonary sarcoidosis.

Phase 1b/2a Clinical Trial in Patients with Pulmonary Sarcoidosis

The Phase 1b/2a study was a randomized, double-blind, placebo-controlled, multiple-ascending dose clinical trial in 37 patients with pulmonary sarcoidosis. The trial consisted of three cohorts testing doses of 1.0 mg/kg, 3.0 mg/kg and 5.0 mg/kg of efzofitimod or placebo, dosed intravenously every month for six months. The primary objective of the study was to evaluate the safety, tolerability, immunogenicity and pharmacokinetic profile of multiple doses of efzofitimod compared to placebo. Secondary objectives included the potential steroid-sparing effects of efzofitimod, in addition to other exploratory assessments of efficacy, such as lung function. (NCT03824392)

About Pulmonary Sarcoidosis

Sarcoidosis is an immune-mediated disease characterized by the formulation of granulomas, clumps of inflammatory cells, in one or more organs of the body, predominantly in the lungs. Almost 200,000 Americans live with pulmonary sarcoidosis and the prognosis ranges from benign and self-limiting to chronic, debilitating disease, with 1 in 5 cases resulting in fibrosis, or scarring, of the lungs, which causes permanent loss of lung function and in many cases 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

Efzofitimod is a first-in-class biologic immunomodulator in clinical development for the treatment of interstitial lung disease (ILD), a group of immune-mediated disorders that can cause inflammation and fibrosis, or scarring, of the lungs. Efzofitimod is a tRNA synthetase derived therapy that selectively modulates activated myeloid cells through neuropilin-2 to resolve inflammation without immune suppression and potentially prevent the progression of fibrosis. aTyr is currently investigating efzofitimod in the global Phase 3 EFZO-FIT™ study in patients with pulmonary sarcoidosis, a major form of ILD, and in the Phase 2 EFZO-CONNECT™ study in patients with systemic sclerosis (SSc, or scleroderma)-related ILD. These forms of ILD have limited therapeutic options and there is a need for safer and more effective, disease-modifying treatments that improve outcomes.

About aTyr

aTyr is a clinical stage biotechnology company leveraging evolutionary intelligence to translate tRNA synthetase biology into new therapies for fibrosis and inflammation. tRNA synthetases are ancient, essential proteins that have evolved novel domains that regulate diverse pathways extracellularly in humans. aTyr's discovery platform is focused on unlocking hidden therapeutic intervention points by uncovering signaling pathways driven by its proprietary library of domains derived from all 20 tRNA synthetases. aTyr's lead therapeutic candidate is efzofitimod, a first-in-class biologic immunomodulator in clinical development for the treatment of interstitial lung disease, a group of immune-mediated disorders that can cause inflammation and progressive fibrosis, or scarring, of the lungs. 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 "anticipate," “believes,” “designed,” “can,” “expects,” “intends,” “may,” “plans,” “potential,” “will,” “would,” 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, among others, statements regarding the clinical development for efzofitimod, including the potential benefits and therapeutic application of efzofitimod as compared to the potential benefits and drawbacks of the current standard of care for sarcoidosis, timelines and plans with respect to certain development activities (such as the timing of data from clinical trials) and our publishing of additional data, the potential benefits of efzofitimod to be a new treatment option that can reduce or avoid steroid-related toxicity 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, strategies or prospects 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 geopolitical and macroeconomic events, risks associated with the discovery, development and regulation of efzofitimod, risks associated with clinical trials and their resulting data generally, the risk that we or our partners may cease or delay preclinical or clinical development activities for efzofitimod 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 most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q 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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