aTyr Pharma Presents Efzofitimod Data Demonstrating Statistically Significant Improvements in Time to Relapse, FVC and Patient Reported Outcomes

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Post-hoc analysis from Phase 1b/2a study in pulmonary sarcoidosis presented at the European Respiratory Society (ERS) International Congress 2023.

7.7% of patients in the 3.0 and 5.0 mg/kg efzofitimod group relapsed following steroid taper, compared to 54.4% in the placebo and efzofitimod 1.0 mg/kg group (p=0.017).

Rate of change for forced vital capacity (FVC) was significantly improved for the 3.0 and 5.0 mg/kg efzofitimod group compared to placebo and the efzofitimod 1.0 mg/kg group (p=0.035).

SAN DIEGO, Sept. 11, 2023 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE) (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 results of a post-hoc analysis of data from its Phase 1b/2a study of efzofitimod in patients with pulmonary sarcoidosis. The analysis was presented in a poster at the European Respiratory Society (ERS) International Congress 2023, which is taking place September 9 – 13, 2023, in Milan, Italy. The poster is available on the Company's website.

"This new data from a post-hoc analysis, which pools efzofitimod 3.0 and 5.0 mg/kg and placebo and efzofitimod 1.0 mg/kg doses from the Phase 1b/2a study of efzofitimod in patients with pulmonary sarcoidosis, is yet another indicator of the robust efficacy demonstrated in this study," said Sanjay S. Shukla, M.D., M.S., President and CEO of aTyr. "The statistically significant difference in the relapse rate following steroid taper seen in the two highest efzofitimod dose groups, combined with significantly improved FVC and quality of life measures, suggests that efzofitimod has the potential to be the first steroid-sparing and disease-modifying treatment for sarcoidosis."

"Oral corticosteroids remain the mainstay of treatment for patients with pulmonary sarcoidosis, although long-term treatment often comes with severe side effects and toxicity. Steroid tapers in these patients are challenging, as symptoms and FVC can worsen when steroid dose is reduced," said Robert P. Baughman, M.D., Professor of Medicine at the University of Cincinnati Medical Center. "This analysis demonstrating a relapse rate limited to 7.7% for the efzofitimod therapeutic group is exciting, as we would normally expect to see a relapse rate as high as approximately 50% over the course of 6 months, which is what was observed in the subtherapeutic group, and the difference may even be more evident in a longer study. This is one of the few studies to demonstrate a steroid sparing effect of a drug associated with a significant improvement in patient outcome. A treatment such as efzofitimod that can reduce steroid burden is greatly needed."

Therapeutic Doses of Efzofitimod Significantly Improve Multiple Pulmonary Sarcoidosis Efficacy Measures

The poster presents findings from a pooled, post-hoc analysis of data from a Phase 1b/2a randomized, double-blind, placebo-controlled, multiple ascending dose (1.0, 3.0 and 5.0 mg/kg) 24-week study of efzofitimod in patients with pulmonary sarcoidosis receiving oral corticosteroid (OCS) dose ≥ 10.0 mg/day. Patients were randomized 1:2 (placebo:efzofitimod) and underwent a forced steroid taper in the first 8 weeks of the study. Dose dependent improvements in steroid burden, FVC and patient reported outcomes (PRO) were noted, though the study was not powered for efficacy.

In this pooled analysis, the 3.0 mg/kg (N=8) and 5.0 mg/kg (N=9) efzofitimod arms were considered therapeutic, and pooled. The placebo (N=12) and 1.0 mg/kg (N=8) efzofitimod arm, which was considered subtherapeutic, were pooled. Time to relapse for steroid use (defined as dose of OCS increased after OCS taper to 5.0 mg or less of prednisone or equivalent for at least five consecutive days), rate of change for FVC and proportion of patients with changes that are multiples of the minimally clinically important difference (MCID) in PRO (Kings Sarcoidosis Questionnaire-Lung, or KSQ-L) were compared. Additionally, a responder endpoint was proposed (defined as reduction in OCS from baseline without worsening in FVC or PRO) and an analysis was performed. Key findings include:

- 7.7% of patients in the therapeutic group relapsed for steroid use compared to 54.4% of patients in the placebo/subtherapeutic group (p=0.017);
- The rate of change for FVC was significantly improved for the therapeutic group compared to the placebo/subtherapeutic group (p=0.035);
- 52.9% of patients in the therapeutic group showed an increase ≥12 for KSQ-L (3 times MCID) compared with 15.0% in the placebo/subtherapeutic group (p=0.032); and
- 64.7% of patients in the therapeutic group achieved response compared to 20.0% in the placebo/subtherapeutic group (p=0.008).

aTyr is currently conducting EFZO-FIT ™, a global Phase 3 randomized, double-blind, placebo-controlled 52-week study to evaluate the efficacy and safety of 3.0 mg/kg and 5.0 mg/kg of efzofitimod in 264 patients with pulmonary sarcoidosis. The trial design incorporates a forced steroid taper. The primary endpoint of the study is steroid reduction. Secondary endpoints include measures of lung function and sarcoidosis symptoms.

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 “believes,” “can,” “expects,” “intends,” “may,” “plans,” “potential,” “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, among others, statements regarding the potential of efzofitimod to provide a differentiated approach to resolving inflammation and preventing the progression of fibrosis to be the first steroid sparing and disease-modifying treatment for sarcoidosis and to reduce steroid burden and significantly improve multiple pulmonary sarcoidosis efficacy measures, and the potential applications of efzofitimod. 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, 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 Report on Form 10-Q and in our subsequent 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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