



aTyr Pharma Announces Positive Data from Phase 1b/2a Clinical Trial Demonstrating Consistent Dose Response for ATYR1923 in Pulmonary Sarcoidosis

September 13, 2021

Trial met primary endpoint, ATYR1923 was safe and well-tolerated.

Efficacy observed in key endpoints including steroid reduction of 58% in the 5.0 mg/kg treatment group with 33% of patients in the group able to taper completely off of steroids.

Clinically meaningful improvements in forced vital capacity (FVC) of 3.3% and all sarcoidosis symptom measures, including shortness of breath, cough, and fatigue, observed in the 5.0 mg/kg treatment group.

Management to host conference call and webcast today, September 13th at 8:30am ET/5:30am PT

SAN DIEGO, Sept. 13, 2021 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE), a clinical stage biotherapeutics company engaged in the discovery and development of innovative medicines based on novel biological pathways, today announced positive results from its Phase 1b/2a double-blind, placebo-controlled clinical trial of its lead therapeutic candidate, ATYR1923, in 37 patients with pulmonary sarcoidosis, a major form of interstitial lung disease (ILD). ATYR1923 was safe and well-tolerated at all doses with no drug-related serious adverse events or signal of immunogenicity. Additionally, the study demonstrated consistent dose response for ATYR1923 on key efficacy endpoints and improvements compared to placebo, including measures of steroid reduction, lung function, sarcoidosis symptom measures and inflammatory biomarkers.

"We are delighted by the results of this study, which provide the first clinical proof-of-concept for ATYR1923, as well as validation for our tRNA synthetase biology platform and Neuropilin-2 as a target. The consistency in dose response and clinically meaningful benefit observed, along with ATYR1923's favorable safety and tolerability profile, give us great confidence that ATYR1923 could be a transformative, disease modifying therapy for pulmonary sarcoidosis patients," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "Based on the results of this study, we plan to meet with the U.S. Food and Drug Administration to present these data and our plans for subsequent clinical development and path to registration for ATYR1923 for pulmonary sarcoidosis, and we expect to initiate a registrational trial next year."

"I am very impressed by this study, which is one of the best that I have seen conducted in sarcoidosis, a patient population that is highly underserved by current treatment options," said Robert Baughman, M.D., Professor of Medicine and Pulmonologist at the University of Cincinnati Medical Center. "The dose response and consistent response seen across multiple efficacy measures, without added toxicity, in this patient population with advanced disease is notable. Importantly, ATYR1923 demonstrated an improvement in several indicators of quality of life, a high priority for patients, by a much larger margin than I would expect in a trial of this size and duration."

"The dose response and consistent results across almost every endpoint are remarkable findings, and as good as could be expected in this small study. The ability to taper patients off steroids while controlling disease symptoms in the ATYR1923 treatment groups is particularly compelling and supports advancement of ATYR1923 into the next phase of development," said Daniel Culver, D.O., Chair of the Department of Pulmonary Medicine and Director of Diffuse Parenchymal Lung Disease at The Cleveland Clinic.

Key Safety and Clinical Efficacy Findings for ATYR1923

- Safe and well-tolerated at all doses
 - No dose-relationship with most common adverse events associated with underlying disease
 - No drug-related serious adverse events
 - No signal of immunogenicity
- Dose response and consistent positive findings across key efficacy endpoints
 - Steroid reduction of 58% overall from baseline and 22% relative reduction compared to placebo in steroid usage post taper in the 5.0 mg/kg treatment group
 - Complete steroid taper to 0 mg achieved and maintained for 33% of patients in the 5.0 mg/kg treatment group compared to no patients in any other group
 - Absolute improvement in forced vital capacity (FVC) as a measure of lung function at week 24 of 3.3% in the 5.0 mg/kg treatment group compared to placebo, with an improvement in FVC of > 2.5% considered clinically meaningful
 - Clinically meaningful improvement over placebo observed for dyspnea (shortness of breath), cough, fatigue and the King's Sarcoidosis Scores for Lung and General Health in 5.0 mg/kg treatment group
 - Dose dependent trends of improvement in key inflammatory biomarkers compared to placebo including IL-6, MCP-1, IFN- γ , IP-10 and TNF α as well as key sarcoidosis markers including ACE, IL-2Ra and SAA with tightest control in the 5.0 mg/kg treatment group
 - FDG-PET-CT was not evaluable due to incomplete data primarily caused by operational issues related to the COVID-19 pandemic

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 ATYR1923 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 ATYR1923 compared to placebo. Secondary objectives included the potential steroid-sparing effects of ATYR1923, in addition to other exploratory assessments of efficacy, such as lung function.

Conference Call and Webcast

aTyr Pharma will host a conference call and webcast to discuss the results today, September 13th at 8:30am ET/5:30am PT. Interested parties may access the call by dialing toll-free 844-358-9116 from the US, or 209-905-5951 internationally and using conference ID 1957829. Links to a live webcast and replay may be accessed on the aTyr website events page at: <http://investors.atyrpharma.com/events-and-webcasts>. A replay will be available for at least 90 days following the event.

About Pulmonary Sarcoidosis and Other ILD

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.

Pulmonary sarcoidosis is a major form of ILD, which is an umbrella term used for a large group of diseases that cause scarring (fibrosis) of the lung. The scarring causes stiffness in the lungs which makes it difficult to breathe and get oxygen to the bloodstream. Lung damage from ILD is often irreversible and gets worse over time. Other major forms of ILD include connective-tissue disease related ILD (e.g., scleroderma-related ILD), chronic hypersensitivity pneumonitis and idiopathic pulmonary fibrosis (IPF).

About ATYR1923

aTyr is developing ATYR1923 as a potential therapeutic for patients with severe inflammatory lung diseases. ATYR1923, 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 ATYR1923 is pulmonary sarcoidosis, a major form of interstitial lung disease. Clinical proof-of-concept for ATYR1923 was recently established in a Phase 1b/2a multiple-ascending dose, placebo-controlled study of ATYR1923 in patients with pulmonary sarcoidosis, which demonstrated safety and a consistent dose response and trends of benefit of ATYR1923 compared to placebo on key efficacy endpoints, including steroid reduction, lung function, clinical symptoms and inflammatory biomarkers.

About aTyr

aTyr is a biotherapeutics company engaged in the discovery and development of innovative medicines based on novel biological pathways. 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 ATYR1923, a clinical-stage product candidate which binds to the neuropilin-2 receptor and is designed to down-regulate immune engagement in inflammatory lung diseases. 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," "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 ATYR1923; timelines and plans with respect to certain development activities (such as the timing of additional clinical trials and planned interactions with regulatory authorities); 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 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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