# aTyr Pharma Announces Topline Results from Phase 3 EFZO-FIT™ Study of Efzofitimod in Pulmonary Sarcoidosis

September 15, 2025

*Study did not meet primary endpoint in change from baseline in mean daily oral corticosteroid (OCS) dose at week 48, although clinical benefit for efzofitimod observed across multiple study parameters.*

*52.6% of patients treated with 5.0 mg/kg efzofitimod achieved complete steroid withdrawal at week 48 vs 40.2% on placebo (p=0.0919).*

*Clinical improvement in King's Sarcoidosis Questionnaire (KSQ)-Lung score at week 48 observed in the 5.0 mg/kg efzofitimod treatment group vs placebo (p=0.0479).*

*Greater proportion of patients achieved complete steroid withdrawal at week 48 with a KSQ-Lung score improvement in the 5.0 mg/kg efzofitimod treatment group (29.5%) vs placebo (14.4%) (p=0.0199).*

*Efzofitimod was well-tolerated with a consistent safety profile.*

*Company plans to engage with the U.S. Food and Drug Administration (FDA) to determine the path forward for efzofitimod in pulmonary sarcoidosis.*

*Management to host conference call and webcast today at 8:30am ET / 5:30am PT.*

SAN DIEGO, Sept. 15, 2025 (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 topline results from the Phase 3 EFZO-FIT™ study of efzofitimod in 268 patients with pulmonary sarcoidosis, a major form of interstitial lung disease.

The study did not meet its primary endpoint of change from baseline in mean daily oral corticosteroid (OCS) dose at week 48. The change from baseline in mean daily OCS dose reduced to an average of 2.79 mg for 5.0 mg/kg efzofitimod vs 3.52 mg for placebo (p=0.3313). The study's statistical analysis plan was designed on a hierarchical assessment basis, as such since the primary endpoint was not met, all subsequent statistical testing is reported as nominal findings.

The study demonstrated a clinical improvement in the King's Sarcoidosis Questionnaire (KSQ)-Lung score at week 48 for 5.0 mg/kg efzofitimod compared to placebo (p=0.0479), with a responder analysis of patients who achieved complete steroid withdrawal at week 48 with an improved KSQ-Lung score also showing improvement in patients treated with 5.0 mg/kg efzofitimod compared to placebo (p=0.0199). Lung function as measured by forced vital capacity (FVC) at week 48 was maintained.

Based on these findings, which we believe indicate drug activity for efzofitimod as evidenced by improvements across multiple clinically relevant efficacy endpoints, the Company plans to engage with the U.S. Food and Drug Administration (FDA) to review the results and determine the path forward for efzofitimod in pulmonary sarcoidosis.

"This study demonstrates that patients with chronic, symptomatic sarcoidosis can be managed with substantially lower steroid doses than previously thought. In spite of a higher than anticipated placebo response, we found that treatment with efzofitimod was associated with a greater amount of steroid reduction and an improvement in the KSQ-Lung score," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr Pharma. "These consistent findings suggest that treatment with efzofitimod may positively impact quality of life and preserve lung function while reducing steroid burden, providing a meaningful benefit to patients. We look forward to discussing the totality of the data with the FDA in order to determine the path forward for efzofitimod in pulmonary sarcoidosis, as there remains an urgent need for a safe and effective treatment option to address the unmet needs of this underserved population."

"The results of EFZO-FIT™ demonstrate not only strong evidence of clinical efficacy for efzofitimod's effect on quality of life and its capacity to facilitate steroid withdrawal, but also increased confidence that steroids overall can be reduced to manage symptoms and lung function without the fear of worsening disease," said Daniel Culver, D.O., Chair of the Department of Pulmonary Medicine at the Cleveland Clinic and principal investigator of the trial. "This trial represents an important step forward for the field, as it is by far the largest interventional study completed in sarcoidosis to date, demonstrating the feasibility of conducting a global multi-center randomized controlled trial. The findings provide valuable insights about the treatment of pulmonary sarcoidosis, which are likely to inform treatment practices in the future."

Dr. Shukla continued, "We are encouraged by the potential of efzofitimod to improve the lives of those living with sarcoidosis, and we are incredibly grateful to the patients, investigators, patient advocacy organizations and our partner Kyorin Pharmaceutical, Ltd., who all contributed to this important study."

EFZO-FIT™ was a global Phase 3 interventional study in 268 patients with pulmonary sarcoidosis that compared the efficacy and safety of efzofitimod at 3.0 mg/kg and 5.0 mg/kg doses versus placebo after 48 weeks of treatment, which included a protocol guided steroid taper in the first 12 weeks of the study, followed by continued taper or rescue until week 48.

Presented below are the families of endpoints analyzed in the study. As the primary endpoint did not achieve statistical significance, p-values for other endpoints are reported and should be interpreted as nominal p-values.

**Study Outcome Measures at Week 48**

- **Steroid Reduction**

- Primary Endpoint: Change from baseline in mean daily OCS dose to an average of 2.79 mg for 5.0 mg/kg efzofitimod vs 3.52 mg for placebo (p=0.3313)
- Complete steroid withdrawal achieved for 52.6% of patients treated with 5.0 mg/kg efzofitimod vs 40.2% on placebo (p=0.0919)

- **KSQ-Lung Score**
  - Change from baseline in KSQ-Lung score of 10.36 for 5.0 mg/kg efzofitimod vs 6.19 for placebo (p=0.0479)
  - Proportion of patients who achieved complete steroid withdrawal with stable KSQ-Lung score was 46.9% of patients on 5.0 mg/kg of efzofitimod vs 35.7% on placebo (p=0.1241)
  - Proportion of patients who achieved complete steroid withdrawal with KSQ-Lung improvement was 29.5% of patients on 5.0 mg/kg efzofitimod vs 14.4% in placebo (p=0.0199)
- **FVC**
  - Change from baseline in absolute percent predicted FVC of -1.81 for patients in the 5.0 mg/kg efzofitimod vs -2.11 in placebo (p=0.7875)
- **Safety and Tolerability**
  - Generally well-tolerated at both the 3.0 mg/kg and 5.0 mg/kg doses, consistent with a previously observed safety profile in all trials conducted to date

The Company plans to present the EFZO-FIT™ topline results at the upcoming European Respiratory Society Congress on Tuesday, September 30, 2025, at 8:44am CEST in Amsterdam, Netherlands.

**Conference Call and Webcast**

aTyr will host a conference call and webcast to discuss the results today September 15 at 8:30am ET / 5:30am PT. Interested parties may access the call by registering here in order to obtain a dial in, personalized passcode and webcast information. Links to a live audio webcast and replay may be accessed on the aTyr website events page at: http://investors.atyrpharma.com/events-and-webcasts. An audio replay will be available for at least 90 days following the event.

**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**

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. In addition to the global Phase 3 EFZO-FIT™ study of efzofitimod in patients with pulmonary sarcoidosis, a major form of ILD, efzofitimod is also being investigated 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," "can," "could," "designed," "expects," "intends," "may," "plans," "potential," "upcoming," "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 therapeutic benefits and applications of efzofitimod; the potential for the results of EFZO-FIT™ to inform the treatment of pulmonary sarcoidosis in the future, including management of the disease with lower steroid doses; timelines and plans with respect to certain development activities (such as the timing of data from clinical trials); plans to engage the FDA to determine the path forward for efzofitimod in pulmonary sarcoidosis as well as our expectations with respect to the outcome of that meeting and next steps for the development of efzofitimod in pulmonary sarcoidosis; 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, 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.

Source: aTyr Pharma, Inc.