aTyr Pharma Announces Third Quarter 2025 Results and Provides Corporate Update

November 6, 2025

Results reported for Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis.

Company plans to meet with the FDA to determine path forward for efzofitimod in pulmonary sarcoidosis in the first quarter of 2026.

Company expects to complete enrollment in Phase 2 EFZO-CONNECT™ study of efzofitimod in systemic sclerosis-related interstitial lung disease (SSc-ILD) in the first half of 2026.

Ended the third quarter 2025 with $92.9 million in cash, cash equivalents, restricted cash and investments.

SAN DIEGO, Nov. 06, 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 third quarter 2025 results and provided a corporate update.

"In September, we announced results from our Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis, a major form of interstitial lung disease (ILD). While we did not meet the primary endpoint, efzofitimod is the first investigational therapy to exhibit improvements in quality of life across multiple disease-related health outcomes while also reducing steroid burden in patients with pulmonary sarcoidosis," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "Treatment options for pulmonary sarcoidosis are limited and can include a significant reliance on the use of oral corticosteroids, which often have significant side effects. There remains an urgent need for a new safe and effective treatment for patients with chronic, symptomatic disease."

"Based on the findings from EFZO-FIT™, we believe there is drug activity for efzofitimod as evidenced by improvements across multiple clinically relevant efficacy measures. Based on this, coupled with the ongoing high unmet medical need in pulmonary sarcoidosis, we plan to meet with the U.S. Food and Drug Administration (FDA) in the first quarter of 2026 to review the results of the study and determine the path forward for efzofitimod in pulmonary sarcoidosis. We plan to provide an update regarding the next steps for the program following that meeting."

Third Quarter 2025 and Subsequent Period Highlights

- **Reported results from the global Phase 3 EFZO-FIT™ study to evaluate the efficacy and safety of 3.0 mg/kg and 5.0 mg/kg of efzofitimod or placebo in 268 patients with pulmonary sarcoidosis.** The study did not meet its primary endpoint of change from baseline in mean daily oral corticosteroid dose at week 48. Clinical benefit for 5.0 mg/kg efzofitimod was observed across multiple pre-specified study efficacy parameters at week 48 compared to placebo, including the King's Sarcoidosis Questionnaire (KSQ)-Lung score, KSQ-General Health score, Fatigue Assessment Scale, and complete steroid withdrawal and improvement in KSQ-Lung score. Additionally, treatment with efzofitimod maintained lung function as a measure of forced vital capacity and was well-tolerated with a safety profile consistent with prior trials conducted to date. Based on these findings, the Company plans to meet with the FDA in the first quarter of 2026 to review the results and determine the path forward for efzofitimod in pulmonary sarcoidosis.
- **Presented the results from the Phase 3 EFZO-FIT™ study in a late-breaking oral abstract at the European Respiratory Society Congress 2025, which took place September 27 – October 1, 2025, in Amsterdam, Netherlands.** Daniel Culver, D.O., Chair of the Department of Pulmonary Medicine at the Cleveland Clinic and principal investigator of the study, delivered the presentation, titled "EFZO-FIT: The Largest Ever Interventional Trial in Pulmonary Sarcoidosis."
- **Enrollment is progressing in the Phase 2 EFZO-CONNECT™ study to evaluate the efficacy, safety and tolerability of efzofitimod in patients with limited or diffuse systemic sclerosis (SSc, or scleroderma)-related ILD (SSc-ILD).** This proof-of-concept study is a randomized, double-blind, placebo-controlled, 28-week study consisting of three parallel cohorts randomized 2:2:1 to either 270 mg or 450 mg of efzofitimod or placebo administered intravenously monthly for a total of six doses. The study intends to enroll up to 25 patients at multiple centers in the United States. Promising interim data from the study were reported in the second quarter of 2025, and the Company expects to complete enrollment in the study in the first half of 2026.
- **Presented at the Federation of European Biochemical Societies (FEBS) Special Meeting, "Expanding frontiers in aminoacyl-tRNA synthetase research," which took place September 28 – October 3, 2025, in Dubrovnik, Croatia.** Leslie Nangle, Ph.D., Vice President of Research at aTyr, delivered a presentation titled, "Advancing a therapeutic platform based on tRNA synthetases for treatment of fibrotic lung diseases."

Third Quarter 2025 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, cash equivalents, restricted cash and available-for-sale investments as of September

30, 2025, were $92.9 million.

- **R&D Expenses:** Research and development expenses were $22.1 million for the third quarter 2025, which consisted primarily of clinical trial costs for the Phase 3 EFZO-FIT™ and Phase 2 EFZO-CONNECT™ studies, manufacturing costs for a potential Biologics License Application (BLA) filing and commercial supply for the efzofitimod program, and research and development costs for the efzofitimod and discovery programs.
- **G&A Expenses:** General and administrative expenses were $4.8 million for the third quarter 2025.

About Efzofitimod

Efzofitimod is a novel 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 novel 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 “aims,” “anticipates,” “believes,” “can,” “designed,” “expects,” “hopes,” “intends,” “look toward,” “may,” “plans,” “potential,” “project,” “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 efzofitimod to improve patient quality of life across multiple disease related health outcomes in pulmonary sarcoidosis; the expected size of, and number of patients to be enrolled in the Phase 2 EFZO-CONNECT™ study; our belief that there is drug activity for efzofitimod; and timelines and plans with respect to certain development activities and development goals, including our plans to meet with the FDA in the first quarter of 2026 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 our expectation that our Phase 2 EFZO-CONNECT™ study of efzofitimod in patients with SSc-ILD will complete enrollment in the first half of 2026. 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, risks related to our reliance on third-party partners and the potential that such partners may not perform as anticipated, the fact that NRP2 and tRNA synthetase biology is not fully understood, uncertainty regarding the ultimate long-term impact of evolving macroeconomic and geopolitical conditions, the risks inherent in using the results from the EFZO-FIT™ study to pursue FDA approval for efzofitimod in pulmonary sarcoidosis, the risk of delays in our clinical trials, risks associated with the discovery, development and regulation of our product candidates, including the uncertainty of related costs and regulatory filings and the risk that results from clinical trials or other studies may not support further development, the risk that we may cease or delay preclinical or clinical development activities for any of our existing or future product candidates for a variety of reasons, the fact that our collaboration agreements are subject to early termination, 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.

ATYR PHARMA INC.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(unaudited)			
Revenues:				
License and collaboration agreement revenues	$ 190	$ —	$ 190	$ 235
Total revenues	190	—	190	235
Operating expenses:				

Research and development	22,130	14,807	49,328	42,144
General and administrative	4,805	3,336	13,693	10,185
Total operating expenses	26,935	18,143	63,021	52,329
Loss from operations	(26,745)	(18,143)	(62,831)	(52,094)
Total other income (expense), net	999	882	2,672	3,040
Consolidated net loss	(25,746)	(17,261)	(60,159)	(49,054)
Net loss (gain) attributable to noncontrolling interest in Pangu BioPharma Limited	2	2	4	(2)
Net loss attributable to aTyr Pharma, Inc.	$ (25,744)	$ (17,259)	$ (60,155)	$ (49,056)
Net loss per share, basic and diluted	$ (0.26)	$ (0.23)	$ (0.66)	$ (0.69)
Shares used in computing net loss per share, basic and diluted	97,153,541	75,801,666	91,292,046	71,419,541

ATYR PHARMA INC.
Condensed Consolidated Balance Sheets
(in thousands)

	September 30, 2025	December 31, 2024
	(unaudited)	
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 92,914	$ 75,076
Other receivables	1,042	1,736
Property and equipment, net	4,429	4,850
Operating lease, right-of-use assets	5,603	5,817
Financing lease, right-of-use assets	745	1,192
Prepaid expenses and other assets	1,919	8,159
Total assets	$ 106,652	$ 96,830
Accounts payable and accrued expenses	$ 14,249	$ 13,715
Current portion of operating lease liability	799	711
Current portion of financing lease liability	630	541
Long-term operating lease liability, net of current portion	10,532	11,144
Long-term financing lease liability, net of current portion	398	887
Total stockholders' equity	80,044	69,832
Total liabilities and stockholders' equity	$ 106,652	$ 96,830

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Source: aTyr Pharma, Inc.