aTyr Pharma Announces First Quarter 2025 Results and Provides Corporate Update

May 7, 2025

Topline data from Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis on track for the third quarter of 2025.

Blinded baseline demographics and disease characteristics from Phase 3 EFZO-FIT™ study and current U.S. epidemiology and treatment practices for pulmonary sarcoidosis to be presented at ATS 2025.

SAN DIEGO, May 07, 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 first quarter 2025 results and provided a corporate update.

"Throughout the first quarter of 2025 we have maintained strong and steady progress with our clinical program for efzofitimod in interstitial lung disease (ILD)," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "The program's cornerstone is our Phase 3 EFZO-FIT™ study in pulmonary sarcoidosis, the most prevalent form of ILD for which no new treatments have been approved in more than 70 years. We look forward to reporting topline data from this study next quarter with the hopes of advancing standard of care beyond oral corticosteroids for sarcoidosis patients."

First Quarter 2025 and Subsequent Period Highlights

- **On track to announce topline data in the third quarter of 2025 from the global pivotal Phase 3 EFZO-FIT™ study to evaluate the efficacy and safety of efzofitimod in patients with pulmonary sarcoidosis.** This is a randomized, double-blind, placebo-controlled, 52-week study consisting of three parallel cohorts randomized equally to either 3.0 mg/kg or 5.0 mg/kg of efzofitimod or placebo administered intravenously monthly for a total of 12 doses. The study enrolled 268 patients with pulmonary sarcoidosis at 85 centers in nine countries. The trial design incorporates a forced steroid taper. The primary endpoint of the study is steroid reduction measured as the absolute change from baseline to week 48. Secondary endpoints include measures of sarcoidosis symptoms and lung function. Patients who complete the study and wish to receive treatment with efzofitimod outside of the clinical trial are eligible to participate in an Individual Patient Expanded Access Program.
- **Enrollment ongoing in the Phase 2 EFZO-CONNECT™ study to evaluate the efficacy, safety and tolerability of efzofitimod in patients with limited and 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. Patients who complete the study are eligible to participate in a 24-week open-label extension. Interim data from the study are expected in the second quarter of 2025 and will focus on skin assessments measured at baseline and week 12 in approximately eight patients, including patients in both the efzofitimod and placebo arms. The data will include skin histopathology, including immune biomarkers, and the modified Rodnan skin score.
- **Appointed Dalia R. Rayes as Head of Commercial, Global Efzofitimod Franchise.** Ms. Rayes has over 25 years of experience building and leading commercial organizations at biotechnology and pharmaceutical companies, including leading rare disease product launches. She will serve as a member of the Company's executive leadership team, overseeing global commercial strategy and operations for the efzofitimod program in ILD, including its lead indication in pulmonary sarcoidosis.
- **Posters for efzofitimod accepted for presentation at the upcoming American Thoracic Society (ATS) 2025 International Conference.** The conference is scheduled to take place May 16 – 21, 2025, in San Francisco, CA.
    - Poster 9320 – Real-World Treatment Patterns Among Pulmonary Sarcoidosis Patients with Parenchymal Involvement in the US on Sunday May 18, 2025, at 11:30 a.m. PDT.
    - Poster 6808 – EFZO-FIT, Largest Placebo-Controlled Trial in Pulmonary Sarcoidosis – Trial Design and Patient Characteristics on Monday, May 19, 2025, at 11:30 a.m. PDT.
    - Poster 9092 – Incidence, Prevalence, and Mortality of Pulmonary Sarcoidosis with Parenchymal Involvement in the US on Tuesday May 20, 2025, at 11:30 a.m. PDT.
- **Presented preclinical data for its neuropilin-2-targeting antibody ATYR2810 at the American Association for Cancer Research (AACR) Annual Meeting 2025.** The findings from the research, which was conducted in collaboration with Michael Lim, M.D., Chair of the Department of Neurosurgery at Stanford Medicine, demonstrate ATYR2810's ability to

enhance anti-tumor activity and increase survival in a model of glioblastoma multiforme, a primary form of brain cancer.

First Quarter 2025 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, cash equivalents, restricted cash and available-for-sale investments as of March 31, 2025, were $78.8 million. The Company believes its cash runway will be sufficient to fund its operations for a period of one year following the Phase 3 EFZO-FIT™ readout.
- **R&D Expenses:** Research and development expenses were $11.8 million for the first quarter 2025, which consisted primarily of clinical trial costs for the Phase 3 EFZO-FIT™ and Phase 2 EFZO-CONNECT™ studies, manufacturing costs for the efzofitimod program and research and development costs for the efzofitimod and discovery programs.
- **G&A Expenses:** General and administrative expenses were $4.0 million for the first quarter 2025.

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 "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 expected size of, and number of patients to be enrolled in, the EFZO-CONNECT™ study; the skin assessments and expected number of patients to be included in the interim data for the EFZO-CONNECT™ study; the potential therapeutic benefits and applications of efzofitimod; expectations regarding our financial guidance and the sufficiency of our cash runway; and timelines and plans with respect to certain development activities and development goals, including our expectation that our Phase 3 EFZO-FIT™ study of efzofitimod in patients with pulmonary sarcoidosis will report topline data in the third quarter of 2025 and expectation that our Phase 2 EFZO-CONNECT™ study will report interim data in the second quarter of 2025. 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 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 March 31,	
	2025	**2024**
	(unaudited)	
Revenues:		

License and collaboration agreement revenues	$ —	$ 235
Total revenues	—	235
Operating expenses:		
Research and development	11,814	13,364
General and administrative	3,959	3,507
Total operating expenses	15,773	16,871
Loss from operations	(15,773)	(16,636)
Total other income (expense), net	892	1,149
Consolidated net loss	(14,881)	(15,487)
Net loss (gain) attributable to noncontrolling interest in Pangu BioPharma Limited	1	(4)
Net loss attributable to aTyr Pharma, Inc.	$ (14,880)	$ (15,491)
Net loss per share, basic and diluted	$ (0.17)	$ (0.23)
Shares used in computing net loss per share, basic and diluted	86,485,126	66,080,593

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

	March 31, 2025	December 31, 2024
	(unaudited)	
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 78,781	$ 75,076
Other receivables	988	1,736
Property and equipment, net	4,683	4,850
Operating lease, right-of-use assets	5,750	5,817
Financing lease, right-of-use assets	1,043	1,192
Prepaid expenses and other assets	6,000	8,159
Total assets	$ 97,245	$ 96,830
Accounts payable and accrued expenses	$ 9,393	$ 13,715
Current portion of operating lease liability	740	711
Current portion of financing lease liability	550	541
Long-term operating lease liability, net of current portion	10,953	11,144
Long-term financing lease liability, net of current portion	747	887
Total stockholders' equity	74,862	69,832
Total liabilities and stockholders' equity	$ 97,245	$ 96,830

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Source: aTyr Pharma, Inc.