aTyr Pharma Announces Second Quarter 2025 Results and Provides Corporate Update

August 7, 2025

Last patient visit completed in Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis; topline results expected in mid-September 2025.

SAN DIEGO, Aug. 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 second quarter 2025 results and provided a corporate update.

"With the recent completion of the last patient visit in our Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis, a major form of interstitial lung disease (ILD), we are on track to report topline data in mid-September," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "This upcoming readout represents a major inflection point for aTyr, our clinical program for efzofitimod in ILD, and the broader sarcoidosis community, and we look forward to sharing the results."

Second Quarter 2025 and Subsequent Period Highlights

- **Completed the last patient visit in the global pivotal Phase 3 EFZO-FIT™ study to evaluate the efficacy and safety of efzofitimod in patients with pulmonary sarcoidosis. Topline data from the study are expected in mid-September 2025.** 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 across 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.
- **Announced interim data from the ongoing 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. Enrollment in the study is ongoing, and the study intends to enroll up to 25 patients at multiple centers in the United States. The interim analysis evaluated skin assessments and serum biomarkers at baseline and week 12 for efzofitimod and placebo patients. Eight patients were evaluated, including five with diffuse and three with limited SSc-ILD. Key findings for efzofitimod-treated patients to date included:
    - Stable or improved modified Rodnan Skin Score *(*mRSS), a key measure of skin fibrosis, for all patients and an improvement of 4 points or greater for three out of four efzofitimod-treated patients with diffuse SSc-ILD, where the minimal clinically important difference (MCID) is a 4 to 6 point improvement at 12 months
    - Preliminary signals of improvement for inflammatory biomarkers including interferon gamma (IFN-γ) and monocyte chemoattractant protein-1 (MCP-1) and disease biomarkers Krebs von den Lungen-6 (KL-6) and surfactant protein-D (SP-D)
    - Efzofitimod was generally safe and well tolerated at all doses, with no treatment related serious adverse events
- **Advanced ATYR0101 to investigational new drug (IND) candidate stage for pulmonary fibrosis.** ATYR0101 is a fusion protein derived from a proprietary extracellular domain of aspartyl-tRNA synthetase (DARS) that binds to latent transforming growth factor beta binding protein 1 (LTBP-1) to induce cell death of myofibroblasts, which are key cells responsible for driving the progression of fibrosis. The Company anticipates filing an IND application in the second half of 2026.
    - Preclinical data generated to date demonstrating ATYR0101's unique anti-fibrotic mechanism through LTBP-1 were presented in an oral presentation at the American Thoracic Society 2025 Respiratory Innovation Summit
- **Announced that the Company was added to the Russell 2000® Index and broad market Russell 3000® Index**. These additions were a part of the 2025 Russell U.S. Indexes annual reconstitution.

Second Quarter 2025 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, cash equivalents, restricted cash and available-for-sale investments as of June 30, 2025, were $83.2 million. Subsequent to the end of the second quarter 2025, the Company raised approximately $30.7 million in gross proceeds from its at-the-market (ATM) offering with Jefferies LLC. 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 $15.4 million for the second 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 $4.9 million for the second 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 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 mid-September 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 June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
	(unaudited)			
Revenues:				
License and collaboration agreement revenues	$ —	$ —	$ —	$ 235
Total revenues	—	—	—	235
Operating expenses:				
Research and development	15,384	13,973	27,198	27,337
General and administrative	4,929	3,342	8,888	6,849
Total operating expenses	20,313	17,315	36,086	34,186
Loss from operations	(20,313)	(17,315)	(36,086)	(33,951)
Total other income (expense), net	781	1,009	1,673	2,158
Consolidated net loss	(19,532)	(16,306)	(34,413)	(31,793)
Net loss (gain) attributable to noncontrolling interest in Pangu BioPharma Limited	1	—	2	(4)
Net loss attributable to aTyr Pharma, Inc.	$ (19,531)	$ (16,306)	$ (34,411)	$ (31,797)

Net loss per share, basic and diluted	$ (0.22)	$ (0.23)	$ (0.39)	$ (0.46)
Shares used in computing net loss per share, basic and diluted	90,120,235	72,284,351	88,312,722	69,204,401

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

	June 30, 2025 (unaudited)	December 31, 2024
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 83,224	$ 75,076
Other receivables	498	1,736
Property and equipment, net	4,526	4,850
Operating lease, right-of-use assets	5,678	5,817
Financing lease, right-of-use assets	894	1,192
Prepaid expenses and other assets	6,714	8,159
Total assets	$ 101,534	$ 96,830
Accounts payable and accrued expenses	$ 14,200	$ 13,715
Current portion of operating lease liability	769	711
Current portion of financing lease liability	562	541
Long-term operating lease liability, net of current portion	10,745	11,144
Long-term financing lease liability, net of current portion	602	887
Total stockholders' equity	74,656	69,832
Total liabilities and stockholders' equity	$ 101,534	$ 96,830

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Source: aTyr Pharma, Inc.