aTyr Pharma Announces Fourth Quarter and Full Year 2024 Results and Provides Corporate Update

March 13, 2025

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

Fourth positive DSMB review for efzofitimod in Phase 3 EFZO-FIT™ study reinforces its favorable safety profile.

Publication in Science Translational Medicine validates efzofitimod's mechanism of action.

Company to host conference call and webcast today, March 13th at 5:00 p.m. EDT / 2:00 p.m. PDT.

SAN DIEGO, March 13, 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 fourth quarter and full year 2024 results and provided a corporate update.

"2024 was an important year for aTyr, as we completed enrollment in our global pivotal Phase 3 EFZO-FIT™ study, which is the largest interventional study ever to be conducted in pulmonary sarcoidosis, a disease where incidence and prevalence are rising yet there are still limited treatment options," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "The stage is set for 2025 to be a potential milestone year as we look toward reporting topline data for EFZO-FIT™ in the third quarter. Leading into this pivotal readout, we are very pleased with the execution of the study to date and the continued favorable safety profile demonstrated for efzofitimod, including our most recent positive DSMB review, which recommended the study continue as designed. Efzofitimod is poised to potentially become a safe and effective alternative to the current standard of care and improve patients' lives."

Fourth Quarter 2024 and Subsequent Period Highlights

- **Enrollment complete in 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 dosed 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. Secondary endpoints include measures of sarcoidosis symptoms and lung function. Topline data from the study are expected in the third quarter of 2025. 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.
- **Held a Type C meeting with the U.S. Food and Drug Administration (FDA) to discuss the statistical analysis plan (SAP) for the Phase 3 EFZO-FIT™ study.**The main objective of the meeting was to finalize the way in which the study endpoints are assessed from a statistical standpoint. Based on FDA feedback, steroid reduction will be measured as the absolute change from baseline to week 48.
- **Announced the fourth positive data and safety monitoring board (DSMB) review for the Phase 3 EFZO-FIT™ study.** The independent DSMB recommended that the study continue without modifications based on the pre-planned analysis.
- **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 interstitial lung disease** (**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 dosed 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 8 patients, including patients on drug and placebo. The data will include skin histopathology, including immune biomarkers, and the modified Rodnan skin score.
- **Manuscript demonstrating the immunomodulatory activity for efzofitimod in lung inflammation and fibrosis published in the peer-reviewed journal *Science Translational Medicine*.** The publication describes efzofitimod's unique anti-inflammatory effect on macrophages through neuropiln-2 (NRP2) and details the preclinical data supporting its development. The publication, which is entitled, "A human histidyl-tRNA synthetase splice variant therapeutic targets NRP2 to resolve lung inflammation and fibrosis," is available on the journal's website and at: https://www.science.org/doi/10.1126/scitranslmed.adp4754.
- **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 research demonstrating anti-fibrotic effects of ATYR0101 in lung and kidney fibrosis in two posters at the Keystone Symposia on Fibrosis: Inflammation, Drivers, and Therapeutic Resolution.** The findings demonstrated that ATYR0101 interacts with LTBP-1 to induce myofibroblast apoptosis through a novel anti-fibrotic mechanism and suggest that ATYR0101 has the potential to be a next generation anti-fibrotic drug for lung and kidney fibrosis.
- **Appointed Eric Benevich to the Company's Board of Directors.** Mr. Benevich currently serves as Chief Commercial Officer at Neurocrine Biosciences, Inc., where he is responsible for all aspects of commercial development, marketing, and sales of the Neurocrine product portfolio.

Year Ended 2024 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, cash equivalents, restricted cash and available-for-sale investments as of December 31, 2024, were $75.1 million. Subsequent to the end of the fourth quarter 2024, the Company raised approximately $18.8 million in gross proceeds from its at-the-market (ATM) offering with Jefferies LLC.
- **Financial Guidance:** The Company updated its prior guidance and believes its cash runway will be sufficient to fund its operations for a period of one year following the Phase 3 EFZO-FIT™ readout, which includes the potential filing of a Biologics License Application (BLA) for efzofitimod in pulmonary sarcoidosis.
- **R&D Expenses:** Research and development expenses were $54.4 million for the year ended 2024, 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 $13.8 million for the year ended 2024.
- **Collaboration and License Revenue:** Collaboration and license revenue related to the Kyorin Agreement was $0.2 million for the year ended 2024, which consisted of drug product material sold to Kyorin for the Japan portion of the EFZO-FIT™ study.

Conference Call and Webcast Details

aTyr will host a conference call and webcast today at 5:00 p.m. EDT / 2:00 p.m. PDT to discuss its financial results and provide a corporate update. 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: https://investors.atyrpharma.com/events-and-webcasts. An audio replay will be available for at least 90 days following the event.

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," "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 interactions with the FDA relating to the SAP for the Phase 3 EFZO-FIT™ study, including the measurement of steroid reduction; 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 the potential filing of a BLA for efzofitimod in pulmonary sarcoidosis and 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.

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

	Three Months Ended December 31,		Years Ended December 31,	
	2024	**2023**	**2024**	**2023**
Revenues:				
License and collaboration agreement revenues	$ —	$ —	$ 235	$ 353
Total revenues	—	—	235	353
Operating expenses:				
Research and development	12,228	12,755	54,372	42,293
General and administrative	3,592	3,204	13,777	12,979
Total operating expenses	15,820	15,959	68,149	55,272
Loss from operations	(15,820)	(15,959)	(67,914)	(54,919)
Total other income (expense), net	852	1,198	3,892	4,522
Consolidated net loss	(14,968)	(14,761)	(64,022)	(50,397)
Net (gain) loss attributable to noncontrolling interest in Pangu BioPharma Limited	1	1	(1)	8
Net loss attributable to aTyr Pharma, Inc.	$ (14,967)	$ (14,760)	$ (64,023)	$ (50,389)
Net loss per share, basic and diluted	$ (0.18)	$ (0.25)	$ (0.86)	$ (0.94)
Shares used in computing net loss per share, basic and diluted	82,724,659	59,261,219	74,261,265	53,606,488

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

	December 31, 2024	December 31, 2023
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 75,076	$ 101,650

Other receivables	1,736	2,436
Property and equipment, net	4,850	5,531
Operating lease, right-of-use assets	5,817	6,727
Financing lease, right-of-use assets	1,192	1,788
Prepaid expenses and other assets	8,159	2,521
Total assets	$ 96,830	$ 120,653
Accounts payable and accrued expenses	$ 13,715	$ 15,088
Current portion of operating lease liability	711	831
Current portion of financing lease liability	541	497
Long-term operating lease liability, net of current portion	11,144	12,339
Long-term financing lease liability, net of current portion	887	1,428
Total stockholders' equity	69,832	90,470
Total liabilities and stockholders' equity	$ 96,830	$ 120,653

Source: aTyr Pharma, Inc.