aTyr Pharma Announces Third Quarter 2024 Results and Provides Corporate Update

November 7, 2024

Enrollment completed in Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis; topline data expected in the third quarter of 2025.

Publication in European Respiratory Journal demonstrated statistically significant difference in time-to-first relapse for corticosteroid use and improvement in corticosteroid relapse rate for efzofitimod.

SAN DIEGO, Nov. 07, 2024 (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 2024 results and provided a corporate update.

"We achieved a significant milestone this quarter by completing enrollment in our global pivotal Phase 3 EFZO-FIT™ study in pulmonary sarcoidosis and topline data is expected in the third quarter of 2025," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "Additionally, our efzofitimod program was featured in this year's Best of CHEST Journals session at the CHEST 2024 annual meeting and we recently published favorable steroid relapse data for efzofitimod in the *European Respiratory Journal.* These events have generated increased interest in efzofitimod and the potential promise it holds to be a transformative therapy for patients."

Third Quarter 2024 and Subsequent Period Highlights

- **Completed enrollment 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, which exceeded the targeted enrollment. 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 (EAP).
- **Continued enrollment in the Phase 2 EFZO-CONNECT™ study to evaluate the efficacy, safety and tolerability of efzofitimod in patients with 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 with SSc-ILD and is open for enrollment at multiple centers in the United States. Patients who complete the study and wish to receive ongoing treatment with efzofitimod are eligible to participate in a 24-week open-label extension (OLE). Interim data from the study are expected in the second quarter of 2025.
- **Publication demonstrating the efficacy of efzofitimod in pulmonary sarcoidosis published in the *European Respiratory Journal*.** Findings from a post hoc analysis of the Phase 1b/2a study of efzofitimod in patients with pulmonary sarcoidosis demonstrated a statistically significant difference in time-to-first relapse for corticosteroid use and improvement in corticosteroid relapse rate in therapeutic (3.0 and 5.0 mg/kg efzofitimod) versus subtherapeutic (1.0 mg/kg efzofitimod and placebo) groups. The publication, entitled, "Therapeutic Doses of Efzofitimod Demonstrate Efficacy in Pulmonary Sarcoidosis," is available on the Journal's website and can be accessed at: https://openres.ersjournals.com/content/early/2024/07/18/23120541.00536-2024.
- **Efzofitimod for pulmonary sarcoidosis featured in the Best of CHEST Journals session at the CHEST 2024 Annual Meeting**. The session featured recent data and publications that generated significant interest and readership among the pulmonology community. Dr. Daniel A. Culver, Chair of the Department of Pulmonary Medicine at Cleveland Clinic, presented data from the Phase 1b/2a study published in *CHEST*, the post hoc analysis on corticosteroid steroid relapse published in the *European Respiratory Journal* with further elucidation on efzofitimod's mechanism of action.

Third Quarter 2024 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, cash equivalents, restricted cash and investments as of September 30, 2024, were $68.9 million. Subsequent to the end of the third quarter 2024, the Company raised approximately $19.4 million in gross proceeds from its at-the-market (ATM) offering with Jefferies LLC, before deducting commissions and offering expenses payable by the Company.
- **Financial Guidance:** The Company believes its cash runway will be sufficient to fund its operations through the filing of a Biologics License Application (BLA) for efzofitimod in pulmonary sarcoidosis.
- **R&D Expenses:** Research and development expenses were $14.8 million for the third quarter 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 $3.3 million for the third quarter 2024.

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,” “designed,” “expects,” “intends,” “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, 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 September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
	(unaudited)			
Revenues:				
License and collaboration agreement revenues	$ —	$ 353	$ 235	$ 353
Total revenues	—	353	235	353
Operating expenses:				
Research and development	14,807	10,319	42,144	29,538
General and administrative	3,336	2,649	10,185	9,775

Total operating expenses	18,143	12,968	52,329	39,313
Loss from operations	(18,143)	(12,615)	(52,094)	(38,960)
Total other income (expense), net	882	1,273	3,040	3,324
Consolidated net loss	(17,261)	(11,342)	(49,054)	(35,636)
Net loss (gain) attributable to noncontrolling interest in Pangu BioPharma Limited	2	2	(2)	7
Net loss attributable to aTyr Pharma, Inc.	$ (17,259)	$ (11,340)	$ (49,056)	$ (35,629)
Net loss per share, basic and diluted	$ (0.23)	$ (0.20)	$ (0.69)	$ (0.69)
Shares used in computing net loss per share, basic and diluted	75,801,666	57,885,393	71,419,541	51,700,864

ATYR PHARMA INC.

Condensed Consolidated Balance Sheets

(in thousands)

	September 30, 2024	December 31, 2023
	(unaudited)	
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 68,913	$ 101,650
Other receivables	1,831	2,436
Property and equipment, net	5,021	5,531
Operating lease, right-of-use assets	5,881	6,727
Financing lease, right-of-use assets	1,341	1,788
Prepaid expenses and other assets	8,629	2,521
Total assets	$ 91,616	$ 120,653
Accounts payable and accrued expenses	$ 12,907	$ 15,088
Current portion of operating lease liability	683	831
Current portion of financing lease liability	528	497
Long-term operating lease liability, net of current portion	11,331	12,339
Long-term financing lease liability, net of current portion	1,028	1,428
Total stockholders' equity	65,139	90,470
Total liabilities and stockholders' equity	$ 91,616	$ 120,653

Source: aTyr Pharma, Inc.