aTyr Pharma Announces Third Quarter 2023 Results and Provides Corporate Update

November 9, 2023

Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis expected to complete enrollment early in the second quarter of 2024.

Phase 2 EFZO-CONNECT™ study of efzofitimod in SSc-ILD initiated patient dosing.

Ended the third quarter of 2023 with $105.6 million in cash, cash equivalents and investments.

SAN DIEGO, Nov. 09, 2023 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE) ("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 2023 results and provided a corporate update.

"During the third quarter we made meaningful progress with our clinical development program for our lead therapeutic candidate, efzofitimod, in interstitial lung disease (ILD)," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "We had a positive data and safety monitoring board (DSMB) review for our global pivotal Phase 3 EFZO-FIT™ study in patients with pulmonary sarcoidosis, a major form of ILD with high unmet medical need. This study continues to enroll in the U.S., Europe and Japan and based on current projections, we expect to complete enrollment in this study early in the second quarter of 2024. Additionally, we dosed the first patient in our Phase 2 EFZO-CONNECT™ study in patients with systemic sclerosis (SSc, or scleroderma)-related ILD (SSc-ILD), which is currently enrolling in the U.S."

Third Quarter 2023 and Subsequent Period Highlights

- **Continued 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 intends to enroll up to 264 subjects with pulmonary sarcoidosis. The study is open for enrollment at nearly all of the centers intended in the U.S., Europe and Japan and is expected to expand to include centers in Brazil. Based on current enrollment projections, the Company expects to complete enrollment in the study early in the second quarter of 2024.
- **Completed a positive DSMB review for the Phase 3 EFZO-FIT™ study.**Data from the pre-planned, interim analysis of safety and tolerability of efzofitimod in patients with pulmonary sarcoidosis, which included the evaluation of patients who completed treatment, was evaluated. There were no drug-related serious adverse events, consistent with prior studies. The DSMB assessed that the study could continue unmodified and that the drug does not pose any undue risk to the patient that warrants additional safety measures.
- **Dosed the first patient in the Phase 2 EFZO-CONNECT™ study to evaluate the efficacy, safety and tolerability of efzofitimod in patients with 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 6 doses. The study intends to enroll 25 patients at multiple centers in the U.S. The primary objective of the study is to evaluate the efficacy of multiple doses of intravenous efzofitimod on pulmonary, cutaneous and systemic manifestations in patients with SSc-ILD.
- **Peer-reviewed article for efzofitimod published in the journal *Frontiers in Pharmacology*.** The publication, titled, "Exposure-response analyses of efzofitimod in patients with pulmonary sarcoidosis," highlights a positive exposure response demonstrated by efzofitimod across multiple clinically relevant endpoints in the Phase 1b/2a study in patients with pulmonary sarcoidosis.
- **Presented two posters for efzofitimod at the European Respiratory Society (ERS) International Congress 2023.** The posters presented new data from a pooled, post hoc analysis from the Phase 1b/2a study of efzofitimod in patients with pulmonary sarcoidosis that further demonstrates efficacy and findings that identify the expression of neuropilin-2 (NRP2), efzofitimod's binding partner, in the skin of patients with SSc-ILD.
- **Poster for efzofitimod accepted for presentation at the upcoming American College of Rheumatology (ACR) Convergence 2023.** The conference is scheduled to take place November 10 – 15, 2023, in San Diego, CA. The poster presents new data demonstrating the effects of efzofitimod in preclinical models of rheumatoid arthritis (RA) and RA-associated lung fibrosis.
    - Poster 1322 – Efzofitimod, a First-in-Class NRP2-targeting Immunomodulator, Ameliorates Rheumatoid Arthritis and Associated Lung Fibrosis in Preclinical Models on Monday, November 13, 2023, from 9:00 a.m. to 11:00 a.m. PST.

Third Quarter 2023 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, restricted cash, cash equivalents and investments as of September 30, 2023, were $105.6 million. Based on the Company's current operational plans and existing cash, the Company updates its prior guidance and believes its cash runway will be sufficient to fund the Company's operations through the filing of a Biologics License Application (BLA) for efzofitimod in pulmonary sarcoidosis.
- **R&D Expenses:** Research and development expenses were $10.3 million for the third quarter of 2023, 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 $2.6 million for the third quarter of 2023.
- **Collaboration and License Revenue:** Collaboration and license revenue related to the Kyorin Agreement was $0.4 million for the third quarter of 2023, which consisted of drug product material sold to Kyorin for the Japan portion of the EFZO-FIT™ study.

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 "believes," "expects," "intends," "may," "plans," "potential," "will," "project," 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 statements regarding our belief that we will have sufficient cash runway to fund the Company's operations through the filing of a BLA for efzofitimod for pulmonary sarcoidosis; the expected size of, and number and nationality of patients to be enrolled in, the EFZO-FIT™ and EFZO-CONNECT™ studies; the potential therapeutic benefits and applications of efzofitimod; 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 complete enrollment early in the second quarter of 2024. 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 or strategies 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, our assumptions and expectations underlying our belief that we will have sufficient cash runway to fund the Company's operations through the filing of a BLA for efzofitimod for pulmonary sarcoidosis may not be accurate, 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 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.
Consolidated Statements of Operations
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
	(unaudited)			
Revenues:				
License and collaboration agreement revenues	$ 353	$ —	$ 353	$ —
Total revenues	353	—	353	—

Operating expenses:				
Research and development	$ 10,319	$ 9,867	$ 29,538	$ 27,898
General and administrative	2,649	3,625	9,775	10,556
Total operating expenses	12,968	13,492	39,313	38,454
Loss from operations	(12,615)	(13,492)	(38,960)	(38,454)
Total other income (expense), net	1,273	247	3,324	634
Consolidated net loss	(11,342)	(13,245)	(35,636)	(37,820)
Net loss attributable to noncontrolling interest in Pangu BioPharma Limited	2	1	7	3
Net loss attributable to aTyr Pharma, Inc.	$ (11,340)	$ (13,244)	$ (35,629)	$ (37,817)
Net loss per share, basic and diluted	$ (0.20)	$ (0.46)	$ (0.69)	$ (1.34)
Shares used in computing net loss per share, basic and diluted	57,885,393	28,663,047	51,700,864	28,184,698

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

	September 30, 2023	December 31, 2022
	(unaudited)	
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 105,582	$ 69,311
Other receivables	2,087	11,775
Property and equipment, net	5,644	3,059
Operating lease, right-of-use assets	6,812	7,250
Financing lease, right-of-use assets	1,791	1,248
Prepaid expenses and other assets	3,153	3,143
Total assets	$ 125,069	$ 95,786
Accounts payable, accrued expenses and other liabilities	$ 11,648	$ 12,968
Current portion of operating lease liability	774	630
Current portion of financing lease liability	459	264
Long-term operating lease liability, net of current portion	12,548	9,633
Long-term financing lease liability, net of current portion	1,437	1,007
Total stockholders' equity	98,203	71,284
Total liabilities and stockholders' equity	$ 125,069	$ 95,786

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Source: aTyr Pharma, Inc.