aTyr Pharma Announces Second Quarter 2023 Results and Provides Corporate Update

August 9, 2023

Phase 3 EFZO-FIT™ study of efzofitimod in pulmonary sarcoidosis currently enrolling in the U.S., Europe and Japan.

Phase 2 EFZO-CONNECT™ study of efzofitimod in SSc-ILD expected to initiate in the third quarter of 2023.

Ended the second quarter of 2023 with $112.0 million in cash, cash equivalents and investments.

SAN DIEGO, Aug. 09, 2023 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE) ("aTyr" or the "Company"), a biotherapeutics company engaged in the discovery and development of first-in-class medicines from its proprietary tRNA synthetase platform, today announced second quarter 2023 results and provided a corporate update.

"Throughout the second quarter we have continued to progress and invest in 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. "Our global pivotal Phase 3 EFZO-FIT™ study in patients with pulmonary sarcoidosis, the most prevalent form of ILD, continues to enroll and our Phase 2 EFZO-CONNECT™ study in patients with systemic sclerosis (SSc, or scleroderma)-associated ILD (SSc-ILD), is expected to enroll the first patient in the third quarter."

Second 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.
- **Progressed plans to initiate 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 will be 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 is expected to enroll 25 patients at multiple centers in the U.S. The primary objective of the study will be to evaluate the efficacy of multiple doses of intravenous efzofitimod on pulmonary, cutaneous and systemic manifestations in patients with SSc-ILD. The study is expected to initiate in the third quarter of 2023.
- **Received European Commission orphan drug designation for efzofitimod for the treatment of SSc based on the opinion of the European Medicines Agency (EMA) Committee for Orphan Medicinal Products.** The EMA grants orphan status to products intended for the treatment, prevention or diagnosis of a disease with a prevalence no more than five in 10,000 people in the EU that is life-threatening or chronically debilitating for which either no satisfactory method of diagnosis, prevention, or treatment exists, or if such a method exists, the medicine is of significant benefit to those affected by such condition. EMA orphan drug designation provides certain benefits, including the potential for up to 10 years of marketing exclusivity following regulatory approval in the EU, reduction in regulatory fees and a centralized EU approval process.
- **Announced two posters for efzofitimod accepted for presentation at the upcoming European Respiratory Society (ERS) International Congress 2023.** The conference is scheduled to take place September 9 – 13, 2023, in Milan, Italy. The Company will present new data from a pooled, post hoc analysis from the Phase 1b/2a study of efzofitimod in patients with pulmonary sarcoidosis that further supports efficacy measures in these patients. Additionally, new mechanistic data supports the rationale for efzofitimod as a potential treatment for patients with SSc-ILD.
    - **Poster PA419 –** Efzofitimod: A Novel Therapeutic Candidate for SSc-ILD on Sunday, September 10, 2023, from 8:00 a.m. to 9:30 a.m. CEST.
    - **Poster PA1744 –** Therapeutic Doses of Efzofitimod Significantly Improve Multiple Pulmonary Sarcoidosis Efficacy Measures on Sunday, September 10, 2023, from 4:00 p.m. to 5:30 p.m. CEST.

Second Quarter 2023 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, restricted cash, cash equivalents and investments as of June 30, 2023, were $112.0 million. Based on the Company's current operational plans and existing cash, the Company maintains its prior guidance

and believes its cash runway will extend into 2026.

- **R&D Expenses:** Research and development expenses were $9.8 million for the second quarter of 2023, which consisted primarily of clinical trial costs for the Phase 3 EFZO-FIT™ study, 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 $3.7 million for the second quarter of 2023.

About Efzofitimod

aTyr is developing efzofitimod as a potential therapeutic for patients with fibrotic lung disease. Efzofitimod, a fusion protein comprised of the immunomodulatory domain of histidyl-tRNA synthetase fused to the FC region of a human antibody, is a selective modulator of neuropilin-2 that downregulates innate immune responses in inflammatory disease states. aTyr's lead indication for efzofitimod is pulmonary sarcoidosis, a major form of interstitial lung disease. Clinical proof-of-concept for efzofitimod was recently established in a Phase 1b/2a multiple-ascending dose, placebo-controlled study of efzofitimod in patients with pulmonary sarcoidosis, which demonstrated safety and a consistent dose response and trends of benefit of efzofitimod compared to placebo on key efficacy endpoints, including steroid reduction, lung function, clinical symptoms and inflammatory biomarkers. aTyr is currently conducting EFZO-FIT™, a Phase 3 study of efzofitimod in pulmonary sarcoidosis patients.

About aTyr

aTyr is a biotherapeutics company engaged in the discovery and development of first-in-class medicines from its proprietary tRNA synthetase platform. aTyr's research and development efforts are concentrated on a newly discovered area of biology, the extracellular functionality and signaling pathways of tRNA synthetases. aTyr has built a global intellectual property estate directed to a potential pipeline of protein compositions derived from 20 tRNA synthetase genes and their extracellular targets. aTyr's primary focus is efzofitimod, a clinical-stage product candidate which binds to the neuropilin-2 receptor and is designed to downregulate immune engagement in fibrotic lung disease. 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," "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 statements regarding our belief that we will have sufficient cash runway to fund both of our efzofitimod clinical trials and the Company's operations into 2026; the expected size of, and number and nationality of patients to be enrolled in, the EFZO-FIT™ and EFZO-CONNECT™ studies; certain potential benefits of EMA orphan drug designation; the potential therapeutic benefits and applications of efzofitimod and our discovery programs; and timelines and plans with respect to certain development activities and development goals, including our expectation that our Phase 2 proof-of-concept study of efzofitimod in patients with SSc-ILD will initiate in the third quarter of 2023. 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 into 2026 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 and in our subsequent 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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
	(unaudited)			
Operating expenses:				
Research and development	$ 9,840	$ 9,135	$ 19,219	$ 18,031
General and administrative	3,718	3,449	7,126	6,931
Total operating expenses	13,558	12,584	26,345	24,962
Loss from operations	(13,558)	(12,584)	(26,345)	(24,962)
Total other income (expense), net	1,216	163	2,051	387
Consolidated net loss	(12,342)	(12,421)	(24,294)	(24,575)
Net loss attributable to noncontrolling interest in Pangu BioPharma Limited	4	1	5	2
Net loss attributable to aTyr Pharma, Inc.	$ (12,338)	$ (12,420)	$ (24,289)	$ (24,573)

Net loss per share, basic and diluted	$ (0.22)	$ (0.44)	$ (0.50)	$ (0.88)
Shares used in computing net loss per share, basic and diluted	55,143,805	28,063,387	48,557,347	27,941,560

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

	June 30, 2023	December 31, 2022
	(unaudited)	
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 112,000	$ 69,311
Other receivables	1,287	11,775
Property and equipment, net	5,812	3,059
Operating lease, right-of-use assets	7,119	7,250
Financing lease, right-of-use assets	1,894	1,248
Prepaid expenses and other assets	4,635	3,143
Total assets	$ 132,747	$ 95,786
Accounts payable, accrued expenses and other liabilities	$ 11,304	$ 12,968
Current portion of operating lease liability	621	630
Current portion of financing lease liability	443	264
Long-term operating lease liability, net of current portion	12,802	9,633
Long-term financing lease liability, net of current portion	1,525	1,007
Total stockholders' equity	106,052	71,284
Total liabilities and stockholders' equity	$ 132,747	$ 95,786

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Source: aTyr Pharma, Inc.