aTyr Pharma Announces Second Quarter 2022 Results and Provides Corporate Update

August 15, 2022

First sites initiated in Phase 3 EFZO-FIT™ study in patients with pulmonary sarcoidosis.

FDA granted Fast Track designation for efzofitimod for the treatment of pulmonary sarcoidosis.

Ended the second quarter 2022 with $89.3 million in cash, restricted cash, cash equivalents and investments.

Company to host conference call and webcast today, May 15th, at 5:00 p.m. EDT / 2:00 p.m. PDT.

SAN DIEGO, Aug. 15, 2022 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE), a biotherapeutics company engaged in the discovery and development of first-in-class medicines from its proprietary tRNA synthetase platform, today announced second quarter 2022 results and provided a corporate update.

"We are pleased with our second quarter progress as we announced our plans to initiate EFZO-FIT™, a Phase 3 study of efzofitimod in patients with pulmonary sarcoidosis," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "This global pivotal study is a major milestone for the sarcoidosis community and is projected to be the largest interventional study for patients with sarcoidosis to date. We are on track to enroll the first patient in this study this quarter."

Second Quarter 2022 and Subsequent Period Highlights

- In May 2022, announced plans to initiate the global pivotal EFZO-FIT ™ study, a global pivotal Phase 3 randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of efzofitimod in patients with pulmonary sarcoidosis. This is a 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 once a month for a total of 12 doses. The study intends to enroll 264 subjects with pulmonary sarcoidosis at multiple centers in North America, Europe and Japan. The trial design will incorporate a forced steroid taper. The primary endpoint of the study is steroid reduction. Secondary endpoints include measures of lung function and sarcoidosis symptoms.
- Accomplished several operational milestones for the EFZO-FIT™ study since its announcement in May 2022, including multiple interactions with regulatory authorities in the United States, European Union and Japan along with the submission of study protocol and clinical trial applications to regulatory authorities, ethics committees and institutional review boards. Site selection, qualification and initiations for several sites have occurred, as well as an investigator meeting for U.S. sites. The company is on track to enroll the first patient in the study in the third quarter of 2022.
- Received U.S. Food and Drug Administration (FDA) Fast Track designation for efzofitimod for the treatment of pulmonary sarcoidosis. Fast Track designation helps facilitate development and expedite the review of drugs to treat serious or life-threatening diseases with unmet medical need. Fast Track designation provides certain benefits, including more frequent interactions with the FDA throughout the development program, as well as eligibility for accelerated approval, priority review and rolling review.
- Presented clinical data from the recently completed Phase 1b/2a study of efzofitimod in patients with pulmonary sarcoidosis at the American Thoracic Society (ATS) 2022 International Conference in San Francisco, California.
- Announced fibroblast growth receptor 4 (FGFR4) as the target receptor for a fragment of the Alanyl-tRNA Synthetase (AARS) in a poster presented at the Keystone Symposia on Tissue Fibrosis and Repair: Mechanisms, Human Disease and Therapeutics. FGFR4 is known to play a role in diseases related to inflammation and fibrosis, including conditions where unchecked fibrosis can precede the development of certain cancers. The company intends to interrogate the interaction between this fragment of AARS and FGFR4 and the implications for disease in order to explore this synthetase fragment as a potential pipeline candidate.

Second Quarter 2022 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, restricted cash, cash equivalents and investments as of June 30, 2022, were $89.3 million.
- **R&D Expenses:** Research and development expenses were $9.1 million for the second quarter of 2022, which consisted of product development and manufacturing costs for the efzofitimod and ATYR2810 programs, as well as startup costs for the Phase 3 EFZO-FIT™ study.
- **G&A Expenses:** General and administrative expenses were $3.4 million for the second quarter of 2022.

- **Shares Outstanding:** Common shares outstanding were 28,127,458 as of June 30, 2022.

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: http://investors.atyrpharma.com/events-and-webcasts. An audio replay will be available for at least 90 days following the event.

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 and adaptive immune response 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 http://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," "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 statements regarding the trial design of EFZO-FIT™ and the expected number of patients to be enrolled in the study; our plans to enroll the first patient in the EFZO-FIT™ study in the third quarter of 2022; our plan to interrogate the interaction between AARS-1 and FGFR4 and explore such synthetase fragment as a potential pipeline candidate; 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. 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, the fact that NRP2 and tRNA synthetase biology is not fully understood, uncertainty regarding the COVID-19 pandemic, and geopolitical conflicts, including 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2022 filed with the SEC on August 15, 2022 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,	
	2022	2021	2022	2021
	(unaudited)			
Operating expenses:				
Research and development	$ 9,135	$ 7,655	$ 18,031	$ 12,171
General and administrative	3,449	2,790	6,931	5,476
Total operating expenses	12,584	10,445	24,962	17,647
Loss from operations	(12,584)	(10,445)	(24,962)	(17,647)
Total other income (expense), net	163	53	387	100
Consolidated net loss	(12,421)	(10,392)	(24,575)	(17,547)
Net loss attributable to noncontrolling interest in Pangu BioPharma Limited	1	1	2	5
Net loss attributable to aTyr Pharma, Inc.	$ (12,420)	$ (10,391)	$ (24,573)	$ (17,542)

Net loss per share, basic and diluted	$ (0.44)	$ (0.64)	$ (0.88)	$ (1.16)
Shares used in computing net loss per share, basic and diluted	28,063,387	16,128,473	27,941,560	15,121,721

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

	June 30, 2022	December 31, 2021
	(unaudited)	
Cash, cash equivalents, restricted cash and available-for-sale investments	$ 89,287	$ 107,911
Other receivables	426	435
Property and equipment, net	558	543
Operating lease, right-of-use assets	827	1,267
Financing lease, right-of-use assets	368	—
Prepaid expenses and other assets	4,550	5,381
Total assets	$ 96,016	$ 115,537
Accounts payable, accrued expenses and other liabilities	$ 8,284	$ 5,033
Current portion of operating lease liability	906	980
Current portion of financing lease liability	70	—
Long-term operating lease liability, net of current portion	—	398
Long-term financing lease liability, net of current portion	298	—
Total stockholders' equity	86,458	109,126
Total liabilities and stockholders' equity	$ 96,016	$ 115,537

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Source: aTyr Pharma, Inc.