



aTyr Pharma Announces Fourth Quarter and Full Year 2022 Results and Provides Corporate Update

March 9, 2023

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

Phase 2 proof-of-concept study of efzofitimid in patients with SSc-ILD expected to begin in 2023.

February 2023 follow-on common stock offering of approximately \$52.0 million in gross proceeds.

Company to host conference call and webcast today, March 9th, at 5:00 p.m. EST / 2:00 p.m. PST.

SAN DIEGO, March 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 fourth quarter and full year 2022 results and provided a corporate update.

"2022 was an important year for aTyr as we advanced our lead therapeutic candidate, efzofitimid, to a global pivotal Phase 3 study in patients with pulmonary sarcoidosis, the most prevalent form of interstitial lung disease (ILD)," said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. "This study, which is known as EFZO-FIT™, is expected to be the largest interventional study in sarcoidosis to date and is currently enrolling patients at multiple centers in the U.S., Europe and Japan."

"We are also expanding the clinical development program for our novel immunomodulator efzofitimid with plans to initiate a Phase 2 proof-of-concept study in patients with systemic sclerosis (SSc, or scleroderma)-associated ILD (SSc-ILD) this year, which increases the potential market opportunity in these forms of ILD with high unmet medical need. As a result of program prioritization and proceeds from a recent public offering, we project that we will have sufficient cash to fund both of our efzofitimid clinical trials and the company's operations into 2026. We are very proud of our progress and look forward to the year ahead as we continue our pursuit to develop a new class of medicines."

Fourth Quarter 2022 and Subsequent Period Highlights

- Continued enrollment in EFZO-FIT™, a global pivotal Phase 3 randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of efzofitimid 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 efzofitimid or placebo dosed intravenously once a month for a total of 12 doses. The study intends to enroll up to 264 subjects with pulmonary sarcoidosis and is currently enrolling at multiple centers in the U.S., Europe and Japan.
- Kyorin Pharmaceutical Co., Ltd. (Kyorin), aTyr's partner for the development and commercialization of efzofitimid for ILD in Japan, dosed the first patient in Japan in EFZO-FIT™. This achievement triggered a \$10.0 million milestone payment to aTyr in the fourth quarter. Under the collaboration and license agreement with Kyorin, aTyr has received \$20.0 million in upfront and milestone payments to date and is eligible to receive up to an additional \$155.0 million in the aggregate upon the achievement of certain development, regulatory and sales milestones, as well as tiered royalties on any net sales in Japan. Kyorin has the exclusive rights to develop and commercialize efzofitimid in Japan for all forms of ILD.
- Announced U.S. Food and Drug Administration (FDA) clearance of an Investigational New Drug (IND) application for a Phase 2 randomized, double-blind, placebo-controlled, proof-of-concept study to evaluate the efficacy, safety and tolerability of efzofitimid in patients with SSc-ILD. This is expected to be a 28-week study with three parallel cohorts randomized 2:2:1 to either 270 mg or 450 mg of efzofitimid or placebo dosed intravenously monthly for a total of 6 doses. It is expected that the study will enroll 25 patients with progressive disease who are currently receiving background mycophenolate therapy at multiple centers in the U.S. The primary objective of the study will be to evaluate the efficacy of multiple doses of intravenous efzofitimid on pulmonary, cutaneous and systemic manifestations in patients with SSc-ILD. The Company plans to initiate this study in 2023.
- Received European Union (EU) orphan drug designation for efzofitimid for the treatment of sarcoidosis from the European Commission based on the opinion of the European Medicines Agency (EMA) Committee for Orphan Medicinal Products. To qualify for EMA orphan status, a product must, among other things, be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating for which either no satisfactory method of diagnosis, prevention or treatment exists, or if such 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 10 years of market exclusivity following market authorization, potential reduction in regulatory fees and a centralized EU market authorization process.
- Announced that the Company will present data on the mechanism of action and an exposure-efficacy analysis for

efzofitimod at the upcoming American Thoracic Society (ATS) 2023 International Conference, which is scheduled to take place May 19 – 24 in Washington, DC.

- Received a Notice of Allowance from the European Patent Office for a patent covering the use of efzofitimod in combination with the anti-fibrotic pirfenidone for the treatment of lung inflammation or fibrosis.
- Announced latent transforming growth factor beta binding protein 1 (LTBP1) as the target of a domain of aspartyl-tRNA synthetase (DARS). LTBP1 is a key regulator of transforming growth factor beta (TGF- β), a central player in the pathogenesis of fibrotic diseases. The Company expects to present additional findings around the interaction between LTBP1 and this domain of DARS at an upcoming scientific conference.
- Raised gross proceeds of approximately \$52.0 million through the issuance of 23,125,000 shares of common stock in February 2023 from a public offering, which included the partial exercise of the underwriters' option to purchase additional shares.

Year Ended 2022 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, restricted cash, cash equivalents and investments as of December 31, 2022, were \$69.3 million. Subsequent to the end of the year, the Company received a \$10.0 million milestone payment in connection with the Kyorin Agreement and generated gross proceeds of approximately \$52.0 million from the public offering of common stock. Based on the Company's current operational plans and existing cash, aTyr believes the Company's cash runway will extend into 2026.
- **R&D Expenses:** Research and development expenses were \$42.8 million for the year ended 2022, which consisted of clinical trial costs for the Phase 3 EFZO-FIT™ study, manufacturing costs for the efzofitimod and ATYR2810 programs, and research and development for the efzofitimod and discovery programs.
- **G&A Expenses:** General and administrative expenses were \$14.0 million for the year ended 2022.

Conference Call and Webcast Details

aTyr will host a conference call and webcast today at 5:00 p.m. EST / 2:00 p.m. PST 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 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," "project," "will," "would," 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 to fund both of our efzofitimod clinical trials and the company's operations into 2026; the expected size of, and number of patients to be enrolled in, the EFZO-FIT™ study; our expectations regarding the size of the EFOZ-FIT™ study being the largest interventional study in sarcoidosis to date; 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 begin in 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 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 on Form 10-Q for the quarter ended September 30, 2022 filed with the SEC on November 14, 2022 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 December 31,		Years Ended December 31,	
	2022	2021	2022	2021
Revenues:				
License and collaboration agreement revenues	\$ 10,386	\$ —	\$ 10,386	\$ —
Total revenues	10,386	—	10,386	—
Operating expenses:				
Research and development	\$ 14,910	\$ 5,955	\$ 42,808	\$ 23,264
General and administrative	3,426	2,685	13,982	10,751
Total operating expenses	18,336	8,640	56,790	34,015
Loss from operations	(7,950)	(8,640)	(46,404)	(34,015)
Total other income (expense), net	427	79	1,061	238
Consolidated net loss	(7,523)	(8,561)	(45,343)	(33,777)
Net loss attributable to noncontrolling interest in Pangu BioPharma Limited	2	2	5	9
Net loss attributable to aTyr Pharma, Inc.	\$ (7,521)	\$ (8,559)	\$ (45,338)	\$ (33,768)
Net loss per share, basic and diluted	\$ (0.26)	\$ (0.31)	\$ (1.60)	\$ (1.77)
Shares used in computing net loss per share, basic and diluted	29,116,524	27,791,737	28,419,569	19,080,878

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

	December 31, 2022	December 31, 2021
Cash, cash equivalents, restricted cash and available-for-sale investments	\$ 69,311	\$ 107,911
Other receivables	11,775	435
Property and equipment, net	3,059	543
Operating lease, right-of-use assets	7,250	1,267
Financing lease, right-of-use assets	1,248	—
Prepaid expenses and other assets	3,143	5,381
Total assets	\$ 95,786	\$ 115,537
Accounts payable, accrued expenses and other liabilities	\$ 12,968	\$ 5,033
Current portion of operating lease liability	630	980
Current portion of financing lease liability	264	—
Long-term operating lease liability, net of current portion	9,633	398
Long-term financing lease liability, net of current portion	1,007	—
Total stockholders' equity	71,284	109,126
Total liabilities and stockholders' equity	\$ 95,786	\$ 115,537

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Source: aTyr Pharma, Inc.