aTyr Pharma Announces Fourth Quarter and Full Year 2021 Results and Provides Corporate Update

March 14, 2022

FDA End-of-Phase 2 meeting provides development pathway for efzofitimod (ATYR1923) in pulmonary sarcoidosis; planned registrational study to initiate in the third quarter of 2022.

Company ended 2021 with $107.9 million in cash, cash equivalents and investments.

Company to host conference call and webcast today, March 14th, at 5:00 p.m. EDT / 2:00 p.m. PDT.

SAN DIEGO, March 14, 2022 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE), a biotherapeutics company engaged in the discovery and development of innovative medicines based on novel biological pathways, today announced fourth quarter and full year 2021 results and provided a corporate update.

“2021 was a milestone year for aTyr, which culminated in clinical proof-of-concept for our lead therapeutic candidate, efzofitimod (ATYR1923), and validation for our tRNA synthetase biology platform,” said Sanjay S. Shukla, M.D., M.S., President and Chief Executive Officer of aTyr. “The positive results reported from our Phase 1b/2a study of efzofitimod in pulmonary sarcoidosis, our initial interstitial lung disease (ILD) indication, suggest that this novel immunomodulator has the potential to be a transformative, disease modifying therapy for patients with this and other fibrotic lung diseases with high unmet need.”

“We have carried this momentum into the start of 2022. The receipt of U.S. Food and Drug Administration (FDA) orphan drug designation for efzofitimod in sarcoidosis underscores the significant challenges faced by these patients. We have a path forward as a result of our positive End-of-Phase 2 meeting with the FDA and intend to initiate a planned registrational trial in pulmonary sarcoidosis in the third quarter of this year. We also remain on track with the IND-enabling work for ATYR2810, and we expect to initiate a Phase 1 study in cancer patients in the second half of this year. We ended 2021 with approximately $107.9 million in cash, and our strong balance sheet positions us well to advance our clinical programs and progress our pipeline in the year ahead.”

Fourth Quarter 2021 and Subsequent Period Highlights

- Held a Type B End-of-Phase 2 meeting with the FDA regarding the company’s lead therapeutic candidate, efzofitimod, for the treatment of pulmonary sarcoidosis. The meeting followed positive results that the company reported from a Phase 1b/2a multiple-ascending dose, placebo-controlled study of efzofitimod in 37 patients with pulmonary sarcoidosis, which demonstrated safety, tolerability and consistent dose response for efzofitimod on key efficacy endpoints and improvements compared to placebo, including measures of steroid reduction, lung function, sarcoidosis symptom measures and inflammatory biomarkers. Following the FDA’s review of the data package, including data from the nonclinical program, early clinical trials and the recently completed Phase 1b/2a study, the company will proceed with the advancement of efzofitimod. The FDA discussed endpoints detailed by the company in its proposed registrational study and prioritization of outcome measurements that would best support the evaluation of efzofitimod’s efficacy. The FDA advised the continued evaluation of multiple doses of efzofitimod in a longer duration study to establish a controlled safety database that supports the determination of the optimal dose for chronic use. The company has a path forward to initiate a planned registrational study of efzofitimod that will incorporate feedback from the FDA, and the company is proceeding with its plans to initiate this study in the third quarter of 2022.
- Received FDA orphan drug designation for efzofitimod for the treatment of sarcoidosis. Orphan drug designation is granted to support the development of medicines for patients with unmet needs for disorders affecting fewer than 200,000 people in the U.S. and provides certain benefits, including the potential for seven years of market exclusivity following regulatory approval, exemption from FDA application fees and tax credits for qualified clinical trials.
- Announced that the United States Adopted Names Council and the World Health Organization’s International Nonproprietary Name Expert Committee selected “efzofitimod” as the nonproprietary (generic) name for ATYR1923. Going forward, aTyr will use the name efzofitimod in place of ATYR1923.
- Announced an agreement with FUJIFILM Diosynth Biotechnologies, a leading contract development and manufacturing organization for biologics, viral vaccines and viral vectors, for the manufacture of efzofitimod. FUJIFILM Diosynth Biotechnologies will support process development and scale up of efzofitimod, including the manufacture of bulk drug substance for additional clinical trials in ILD.
- Had a poster accepted for presentation at the upcoming American Association for Cancer Research Annual Meeting. The poster, titled, “ATYR2810, a fully humanized monoclonal antibody targeting the VEGF-NRP2 pathway sensitizes highly aggressive and chemoresistant TNBC subtypes to chemotherapy,” will present additional preclinical data generated for ATYR2810, the company’s lead anti-Neuropilin-2 (NRP2)/VEGF antibody and IND candidate. The company expects to

initiate a phase 1 study of ATYR2810 in cancer patients in the second half of 2022.

Year Ended 2021 Financial Highlights and Cash Position

- **Cash & Investment Position:** Cash, cash equivalents and investments as of December 31, 2021, were $107.9 million.
- **R&D Expenses:** Research and development expenses were $23.3 million for the year ended 2021, which consisted primarily of product development costs for the efzofitimod and ATYR2810 programs. Program costs for efzofitimod included preparation for the upcoming planned registrational trial in pulmonary sarcoidosis, which included the manufacture of clinical trial material and initiation of technology transfer activities with FUJIFILM Diosynth Biotechnologies. Program costs for ATYR2810 included costs related to IND-enabling activities and the initiation of manufacturing activities with Lonza.
- **G&A Expenses:** General and administrative expenses were $10.8 million for the year ended 2021. This included an increase in the number of employees as the company prepares for the efzofitimod planned registrational trial in pulmonary sarcoidosis and a phase 1 clinical trial of ATYR2810 in cancer.
- **Shares Outstanding:** Common shares outstanding were 27,793,035 as of December 31, 2021.

Financial Guidance

- The company expects its research and development expenses to increase in 2022 as it continues to develop efzofitimod and ATYR2810 as well as its discovery programs.

Conference Call and Webcast Details

aTyr will host a conference call and webcast today at 5:00 p.m. Eastern Time / 2:00 p.m. Pacific Time to discuss its financial results and provide a corporate update. Interested parties may access the call by dialing toll-free 844-358-9116 from the US, or 209-905-5951 internationally and using conference ID 3686825. 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 intends to initiate a planned registrational study of efzofitimod in pulmonary sarcoidosis in the third quarter of 2022.

About aTyr

aTyr is a biotherapeutics company engaged in the discovery and development of innovative medicines based on novel biological pathways. 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 "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "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 potential therapeutic benefits and applications of efzofitimod, ATYR2810 and our discovery programs; timelines and plans with respect to certain development activities (including the further development of efzofitimod and ATYR2810 and the timing and design of future clinical trials) and value to be derived therefrom; certain development goals; and expected trends in future expenses. 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, 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 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 December 31,		Twelve Months Ended December 31,	
	2021	**2020**	**2021**	**2020**
Revenues:				
License and collaboration agreement revenues	$ —	$ 2,053	$ —	$ 10,455
Total revenues	—	2,053	—	10,455
Operating expenses:				
Research and development	5,955	4,698	23,264	17,291
General and administrative	2,685	2,295	10,751	9,075
Total operating expenses	8,640	6,993	34,015	26,366
Loss from operations	(8,640)	(4,940)	(34,015)	(15,911)
Total other income (expense), net	79	5	238	(319)
Consolidated net loss	(8,561)	(4,935)	(33,777)	(16,230)
Net loss attributable to noncontrolling interest in Pangu BioPharma Limited	2	3	9	6
Net loss attributable to aTyr Pharma, Inc.	$ (8,559)	$ (4,932)	$ (33,768)	$ (16,224)
Net loss per share, basic and diluted	$ (0.31)	$ (0.47)	$ (1.77)	$ (1.77)
Shares used in computing net loss per share, basic and diluted	27,791,737	10,573,584	19,080,878	9,160,269

ATYR PHARMA INC.
Consolidated Balance Sheets
(in thousands)

	December 31, 2021	December 31, 2020
Cash, cash equivalents and available-for-sale investments, short-term	$ 107,911	$ 31,689
Other receivables	435	2,039
Property and equipment, net	543	899
Right-of-use assets	1,267	2,083
Prepaid expenses and other assets	5,381	2,016
Total assets	$ 115,537	$ 38,726
Accounts payable, accrued expenses and other liabilities	$ 5,033	$ 5,003
Current portion of operating lease liability	980	861
Long-term operating lease liability, net of current portion	398	1,378
Total stockholders' equity	109,126	31,484
Total liabilities and stockholders' equity	$ 115,537	$ 38,726

Source: aTyr Pharma, Inc.