aTyr Pharma Announces FDA Orphan Drug Designation for Efzofitimod (ATYR1923) for Treatment of Systemic Sclerosis

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Second U.S. orphan drug designation for efzofitimod clinical program

SAN DIEGO, April 13, 2022 (GLOBE NEWSWIRE) -- aTyr Pharma, Inc. (Nasdaq: LIFE), a clinical stage biotherapeutics company engaged in the discovery and development of innovative medicines based on novel biological pathways, today announced that the U.S. Food and Drug Administration (FDA) has granted the company orphan drug designation for its lead therapeutic candidate, efzofitimod, for the treatment of systemic sclerosis (SSc, also known as scleroderma).

Efzofitimod is a potential first-in-class immunomodulator that downregulates innate and adaptive immune responses in uncontrolled inflammatory disease states via selective modulation of neuropilin-2 (NRP2). Clinical proof-of-concept was recently established for efzofitimod in a Phase 1b/2a study in patients with pulmonary sarcoidosis, a major form of interstitial lung disease (ILD). Many patients with SSc may develop associated ILD, known as SSc-ILD. The pathology of SSc-ILD is driven by the same immune cells that are central to sarcoidosis pathology, and NRP2 is upregulated on these cells, particularly on macrophages. Furthermore, efzofitimod has been shown to reduce lung and skin fibrosis in animal models of SSc and idiopathic pulmonary fibrosis, where it matched or outperformed known anti-fibrotic agents, including nintedanib and pirfenidone.

“We are very pleased to receive orphan drug designation for efzofitimod for SSc, which marks the second such designation for our efzofitimod clinical program,” said Sanjay S. Shukla, M.D., M.S., President and CEO of aTyr. “The data we have presented in animal models of SSc along with the positive findings reported from our recent Phase 1b/2a study in pulmonary sarcoidosis patients suggest that efzofitimod has the potential to be a new treatment option that resolves inflammation and subsequent fibrosis in those living with SSc-ILD. We look forward to exploring the potential expansion of our efzofitimod clinical program into other forms of ILD with high unmet need where this novel therapeutic may be able to improve patient outcomes.”

The FDA’s Office of Orphan Drug Products grants orphan status to support the development of medicines for patients with unmet needs for rare disorders affecting fewer than 200,000 people in the United States. Orphan drug designation 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.

Systemic sclerosis is a chronic, progressive, autoimmune disease characterized by inflammation and fibrosis of connective tissues throughout the body, including the skin and other internal organs. SSc that occurs in the lungs is called SSc-ILD. It is estimated that approximately 100,000 people in the U.S. are affected by SSc and 55-65% may develop ILD. SSc-ILD causes inflammation in the lungs and, if left untreated, can result in scarring that causes permanent loss of lung function. ILD is the primary cause of death in patients with SSc. Current treatment options for SSc-ILD are limited, mainly focus on slowing disease progression and are associated with significant toxicity.

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 of efzofitimod to be a new treatment option that resolves inflammation and subsequent fibrosis in those living with SSc-ILD, the potential expansion of our efzofitimod clinical program into other forms of ILD with high unmet need, the ability of efzofitimod to improve patient outcomes, potential benefits of orphan drug designation, and the timing of our planned registrational study of efzofitimod in pulmonary sarcoidosis. 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, uncertainty regarding the COVID-19 pandemic, risks associated with the discovery, development and regulation of our product candidates, the risk that we or our partners may cease or delay preclinical or clinical development activities for any of our existing or future product candidates for a variety of reasons (including difficulties or delays in patient enrollment in planned clinical trials), the possibility that existing collaborations could be terminated early, 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 Annual Report on Form 10-K for the year ended December 31, 2021 filed with the Securities and Exchange Commission on March 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.

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