European Commission Grants Orphan Drug Designation for aTyr Pharma's Efzofitimod for Treatment of Systemic Sclerosis

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Phase 2 proof-of-concept study of efzofitimod in patients with SSc-ILD expected to begin in the third quarter of 2023

SAN DIEGO, June 22, 2023 (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 that the European Commission (EC) granted orphan drug designation for the company's lead therapeutic candidate, efzofitimod, for the treatment of systemic sclerosis (SSc, or scleroderma) based on the opinion of the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP).

Efzofitimod is a first-in-class immunomodulator that downregulates innate immune responses in uncontrolled inflammatory disease states via selective modulation of neuropilin-2. Efzofitimod is currently being investigated in a global pivotal Phase 3 clinical trial in patients with pulmonary sarcoidosis, a major form of interstitial lung disease (ILD), and a Phase 2 proof-of-concept study in patients with SSc-ILD is expected to begin in the third quarter of 2023.

"We are pleased that the EC recognizes the need for new and impactful treatments for the nearly 100,000 people living with systemic sclerosis in the European Union (EU)," said Sanjay S. Shukla, M.D., M.S., President and CEO of aTyr. "This orphan drug designation takes into account more than just the rarity of the disease. In addition to the clinical proof-of-concept data generated for efzofitimod in pulmonary sarcoidosis, the COMP considered that the non-clinical *in vivo* data for efzofitimod in systemic sclerosis, which demonstrated a reduction in the decline of lung function and skin fibrosis not achieved with an authorized medicine in the EU, constitutes a potentially clinically relevant advantage over existing therapies. We believe efzofitimod presents a much-needed opportunity to meaningfully advance treatment options for those whose lungs are affected by systemic sclerosis, and we look forward to the expected initiation of our Phase 2 clinical trial of efzofitimod in patients with SSc-ILD in the third quarter of this year."

The EMA grants orphan status to products 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. To benefit from such designation, either the prevalence of such condition must not be more than five in 10,000 people in the EU, or, if more prevalent, it must be unlikely that the marketing of the medicine would generate sufficient returns to justify the investment needed for its development. Additionally, the investigational product must be of significant benefit to those affected by the condition. EMA orphan drug designation provides certain benefits, including the potential for 10 years of marketing exclusivity following regulatory approval in the EU, reduction in regulatory fees and a centralized EU approval process. Efzofitimod received orphan drug and Fast Track designations for systemic sclerosis from the United States Food and Drug Administration (FDA) in 2022.

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 EU are affected by SSc and up to 80% 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 immuno-modulatory 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 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 "expects," "intends," "may," "plans," "forward," "potential," "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, among others, statements regarding potential therapeutic benefits and applications of efzofitimod; timelines and plans with respect to certain development activities, including the timing of our Phase 2 proof-of-concept clinical trial in patients with SSc-ILD; and the potential benefits of orphan drug designation. 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 geopolitical and macroeconomic events and any resulting delays in clinical trials, 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 most recent Annual Report on Form 10-K, Quarterly Report on Form 10-Q 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.

Contact:
Ashlee Dunston
Director, Investor Relations and Corporate Communications
adunston@atyrpharma.com

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