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

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Global pivotal Phase 3 EFZO-FIT™ study in pulmonary sarcoidosis launched in Europe

SAN DIEGO, Jan. 18, 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 sarcoidosis 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 and adaptive immune responses in uncontrolled inflammatory disease states via selective modulation of neuropilin-2 (NRP2) and is currently being investigated in a global pivotal Phase 3 study in patients with pulmonary sarcoidosis, the main form of the disease.

"The EC's decision to grant orphan drug designation to efzofitimod acknowledges the unmet medical need in sarcoidosis for the approximately 150,000 people in the European Union (EU) living with this chronic, debilitating disease," said Sanjay S. Shukla, M.D., M.S., President and CEO of aTyr. "The criteria for orphan status in the EU go beyond the rarity of the disease. This designation takes into account the preclinical and clinical proof-of-concept data generated for efzofitimod, including data from a Phase 1b/2a study showing efzofitimod's ability to improve lung function and quality of life measures in the context of a steroid taper, and recognizes the potential benefit of this novel immunomodulator to be a transformative, disease modifying therapy with clinically meaningful outcomes for patients."

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 market 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 sarcoidosis from the United States Food and Drug Administration (FDA) in 2022.

Sarcoidosis is an immune-mediated disease characterized by the formulation of granulomas, clumps of inflammatory cells, in one or more organs of the body, predominantly in the lungs. Sarcoidosis affects over three in 10,000 people in the EU. The prognosis for sarcoidosis ranges from benign and self-limiting to chronic, debilitating disease, with one in five cases resulting in fibrosis, or scarring, of the lungs, which causes permanent loss of lung function and in many cases death. Current treatment options include corticosteroids and other immunosuppressive therapies, which have limited efficacy and are associated with serious side effects that many patients cannot tolerate long-term.

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 "potential" and variations of such word 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 potential benefits of efzofitimod to be a transformative disease modifying therapy with clinically meaningful outcomes for patients. 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 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 geopolitical and macroeconomic conditions, including 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2022 filed with the Securities and Exchange Commission (SEC) on November 10, 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.

IMMEDIATE RELEASE

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