aTyr Pharma Announces FDA Orphan Drug Designation for ATYR1923 for Treatment of Sarcoidosis

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Company expects to initiate registrational trial of ATYR1923 in pulmonary sarcoidosis in 2022

SAN DIEGO, Jan. 06, 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, ATYR1923, for the treatment of sarcoidosis. ATYR1923 is a potential first-in-class immunomodulator that downregulates innate and adaptive immune responses in uncontrolled inflammatory disease states. Clinical proof-of-concept was recently established for ATYR1923 in a Phase 1b/2a study in patients with pulmonary sarcoidosis, the main form of the disease, and the company expects to initiate a registrational trial in this indication this year.

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.

"The receipt of orphan drug designation for ATYR1923 for sarcoidosis is an important milestone and recognizes the unmet need for the close to 200,000 patients living with this chronic, debilitating disease," said Sanjay S. Shukla, M.D., M.S., President and CEO of aTyr. "Current treatment options for patients with pulmonary sarcoidosis are limited. By reducing steroid burden while improving lung function and measures of sarcoidosis symptoms, ATYR1923 has the potential to be a transformative, disease modifying therapy with clinically meaningful outcomes for patients. We look forward to initiating a registrational trial in pulmonary sarcoidosis this year."

Sarcoidosis is an inflammatory disease characterized by the formulation of granulomas, clumps of inflammatory cells, in one or more organs of the body. The lungs are affected in >90% of cases. Almost 200,000 Americans live with sarcoidosis and the prognosis ranges from benign and self-limiting to chronic, debilitating disease, in some cases with scarring of the lung, or fibrosis, resulting in permanent loss of lung function and 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 ATYR1923

aTyr is developing ATYR1923 as a potential therapeutic for patients with severe inflammatory lung diseases. ATYR1923, 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 ATYR1923 is pulmonary sarcoidosis, a major form of interstitial lung disease. Clinical proof-of-concept for ATYR1923 was recently established in a Phase 1b/2a multiple-ascending dose, placebo-controlled study of ATYR1923 in patients with pulmonary sarcoidosis, which demonstrated safety and a consistent dose response and trends of benefit of ATYR1923 compared to placebo on key efficacy endpoints, including steroid reduction, lung function, clinical symptoms and inflammatory biomarkers.

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 ATYR1923, a clinical-stage product candidate which binds to the neuropilin-2 receptor and is designed to down-regulate immune engagement in inflammatory lung diseases. 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 potential therapeutic benefits and applications of ATYR1923; timelines and plans with respect to certain development activities (such as the timing of additional clinical trials and planned interactions with regulatory authorities); and certain development goals. 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 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.

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