aTyr Pharma Announces Promising Top-Line Results from Resolaris™ Phase 1b/2 Clinical Trial in Patients with Early Onset Facioscapulohumeral Muscular Dystrophy

April 24, 2017

- 63% of Patients Observed Increased Muscle Strength -

- Resolaris Demonstrated a Generally Well-Tolerated Safety Profile in Younger Patient Population -

SAN DIEGO – April 24, 2017 – aTyr Pharma, Inc. (Nasdaq: LIFE), a biotherapeutics company engaged in the discovery and development of Physiocrine-based therapeutics to address severe, rare diseases, today announced promising clinical results from its Phase 1b/2 003 trial assessing the safety and potential activity of Resolaris™ in patients with early onset facioscapulohumeral muscular dystrophy (FSHD).

aTyr's exploratory trials in rare muscular dystrophies were designed to assess:

- Potential signals of clinical activity informative for developing clinical endpoints to be assessed in later-stage, placebo-controlled efficacy trials;
- Safety and tolerability of a new biologic protein, Resolaris, in patients;
- Biomarkers in these patients; and
- Data that would support further evaluation of Resolaris in rare muscular dystrophies with an immune component.

The 003 trial was an international, multi-center, open-label, intra-patient, placebo run-in, dose escalation Phase 1b/2 study designed to evaluate the safety, tolerability, immunogenicity and exploratory clinical assessments of Resolaris at weekly doses of 0.3, 1.0 and 3.0 mg/kg in patients with early onset FSHD for a total of 12 weeks. Eight patients, ages 16 to 20, participated in the study.

Potential Signals of Clinical Activity

- 63% of patients (5 of 8) had increases from baseline in their Manual Muscle Test (MMT), a validated assessment tool that measures muscle strength, with a mean change from baseline of +3.8%.
- 67% of patients measured (4 of 6) had improvement in their Individualized Neuromuscular Quality of Life (INQoL) score, a validated patient reported outcome measuring a patient's level of disease burden. On average, patients did not have a worsening of their disease burden as measured by INQoL.

Safety and Tolerability

Resolaris was generally well-tolerated at doses up to 3.0 mg/kg once weekly in early onset FSHD. There have been no observed signs of general immunosuppression and low-level anti-drug antibody signals did not result in clinical symptoms. Adverse events were mild or moderate in intensity. There were no clinically significant changes in other safety assessments. aTyr believes the observed safety results of Resolaris to date are supportive of further advancement of Resolaris.

"We would like to congratulate our patients, collaborators and team who helped us accomplish the fundamental objectives for this clinical trial," said John Mendlein, PhD, CEO of aTyr Pharma.

"We are developing Resolaris, derived from a naturally occurring protein that we believe acts on a newly discovered immunological pathway to potentially treat patients with rare muscular dystrophies characterized by immune cell imbalance," said Sanjay Shukla, MD, MS, Chief Medical Officer of aTyr Pharma. "These results are important as they reinforce previous clinical results (in adult FSHD and adult LGMD2B) with Resolaris in a younger patient population, with a potentially more aggressive progression of disease. We look forward to the advancement of Resolaris in the clinic in rare muscular dystrophies upon the identification of a pharmacodynamic assay and the successful execution of our pipeline partnering efforts."

About Resolaris™

aTyr Pharma is developing Resolaris as a potential first-in-class intravenous protein therapeutic candidate for the treatment of rare myopathies with an immune component. Resolaris is derived from a naturally occurring protein released by human skeletal muscle cells. aTyr believes Resolaris has the potential to provide therapeutic benefit to patients with rare myopathies with an immune component characterized by excessive immune cell involvement.

About Early Onset FSHD

While FSHD can manifest at any age, the onset of symptoms in many patients occurs before the age of 18. We refer to this patient population as early onset FSHD. aTyr has selected those patients with onset of symptoms before the age of ten for its current clinical trial. Within the early onset population are individuals with symptom onset at less than five years of age, with progression in disease prior to age ten. These individuals have generally the most severe muscle symptoms as well as extra-muscular manifestations including auditory deficits and retinal complications that may result in vision loss. This sub-group of early onset patients are often referred to as having "infantile onset" FSHD. Estimates of prevalence vary; however, aTyr believes the "infantile onset" FSHD population is approximately 1,000 in the U.S.

About aTyr Pharma

aTyr Pharma is engaged in the discovery and clinical development of innovative medicines for patients suffering from severe, rare diseases using its knowledge of Physiocrine biology, a newly discovered set of physiological pathways. To date, the company has generated three innovative therapeutic candidate programs based on its knowledge of Physiocrine biology in three different therapeutic areas. aTyr has built an intellectual property estate, to protect its pipeline, comprising over 175 issued patents or allowed patent applications that are owned or exclusively licensed, including over 300 potential Physiocrine-based protein compositions. aTyr's key programs are currently focused on severe, rare diseases characterized by immune imbalance for which there are currently limited or no treatment options. 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 Litigation Reform Act. 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, including statements regarding the potential and potential and potential therapeutic benefits of Resolaris™, the ability of the Company to successfully advance its pipeline or product candidates, undertake certain development activities (such as clinical trial enrollment and the conduct of clinical trials) and accomplish certain development goals and the timing of such activities and development goals, the timing of initiation of additional clinical trials, the scope and strength of our intellectual property portfolio, our ability to receive regulatory approvals for, and commercialize, our product candidates and of reporting results from our clinical trials 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 those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, risks associated with the discovery, development and regulation of our Physiocrine-based product candidates, as well as those set forth in our most recent Annual Report on Form 10-K for the year ended December 31, 2016 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.

Contact:
Mark Johnson
Sr. Director, Investor Relations
mjohnson@atyrpharma.com
858-223-1163