aTyr Pharma Initiates Phase 1b/2 Clinical Trial in Patients with Early Onset FSHD

November 9, 2015

SAN DIEGO, Nov. 9, 2015 /PRNewswire/ -- 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 the continued expansion of its Resolaris™ clinical program in rare myopathies with an immune component ("RMICs") by initiating a Phase 1b/2 clinical trial in patients with early onset facioscapulohumeral muscular dystrophy (FSHD), a rare and severe genetic myopathy for which there are currently no approved treatments.

"The initiation of our early onset FSHD Resolaris program is an important step in addressing the needs of some of the most severely effected FSHD patients," said John Mendlein, Ph.D., CEO and executive chairman of aTyr Pharma. "With this trial, we continue to advance our new class of Physiocrine-based therapeutics to harness the body's natural immune processes in rare muscle disease."

The Phase 1b/2 clinical trial in early onset FSHD is an international multi-center, open-label, intra-patient dose escalation study designed to assess the safety, tolerability, immunogenicity, and biological activity of Resolaris™. Clinical trial sites are preparing to screen patients for inclusion in the study and are expected to enroll up to 16 early onset FSHD patients who displayed signs or symptoms of the disease prior to 10 years of age. In the first stage of the trial, up to eight patients between the ages of 16 and 25 years will be enrolled. The second stage of enrollment is expected to include up to eight patients between the ages of 12 and 15 years.

The early onset FSHD clinical research program, which will involve both young adults and children, is in addition to the Company's recently initiated Phase 1b/2 clinical trial in limb girdle muscular dystrophy (LGMD2B) and the ongoing Phase 1b/2 clinical trial evaluating Resolaris™ in adult patients with FSHD.

All three trials, adult FSHD, early onset FSHD and LGMD2B, will assess Resolaris' impact on the immune component of the disease using blood borne markers and magnetic resonance imaging (MRI) of skeletal muscle.

For additional information please visit www.clinicaltrials.gov.

About FSHD
Facioscapulohumeral muscular dystrophy (FSHD) refers to a rare genetic myopathy affecting approximately 19,000 people in the U.S. and for which there are no approved treatments. Most FSHD patients harbor molecularly definable truncations of the fourth chromosome that lead to the undesired expression of the repressed DUX4 gene in skeletal muscle. FSHD patients experience debilitating muscle weakness and immune cell involvement in the muscle. Early onset FSHD occurs in individuals who experience symptoms of progressive muscle involvement as a juvenile, some of whom suffer from a particularly severe form of the disease. Both early onset and adult FSHD patients are assessed for clinical symptoms, and skeletal muscle health may be also be assessed by MRI.

About LGMD2B
Limb girdle muscular dystrophy (LGMD) refers to a group of rare genetic myopathies, of which there are more than 20 different subtypes, none with approved therapies. LGMD affects an estimated 16,000 patients in the U.S., approximately 3,000 of whom have LGMD2B. LGMD2B is a recessive genetic disease caused by mutations in the dysferlin gene. Patients experience debilitating muscle weakness and atrophy as well as immune cell invasion in the skeletal muscle. Patients are assessed for clinical symptoms and MRI may also be used to assess skeletal muscle health.

About aTyr Pharma
aTyr Pharma engages 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 modulators. The Company's lead candidate, Resolaris™, is a first-in-class intravenous protein therapeutic for the treatment of rare myopathies with an immune component (RMIC). Resolaris™ is currently in a Phase 1b/2 clinical trial in adult patients with FSHD; a Phase 1b/2 trial in adult patients with LGMD2B or FSHD; and a Phase 1b/2 trial in patients with an early onset form of FSHD. Trials are planned in additional RMIC indications as well as an initial trial in rare pulmonary diseases with an immune component (RPIC) in patients with interstitial lung disease (ILD). To protect this pipeline, aTyr built an intellectual property estate comprising 45 issued or allowed patents and over 240 pending patent applications that are solely owned or exclusively licensed by aTyr. aTyr's key programs are currently focused on severe, rare diseases characterized by immune dysregulation for which there are currently limited or no treatment options. For more information, please visit http://www.atyrpharma.com.

Forward-Looking Statements
Statements we make in this press release may include statements which are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act, which 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 the safe harbor provisions for forward-looking statements contained in

Section 27A of the Securities Act and Section 21E of the Securities Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements regarding the potential of Resolaris, the ability of the Company to undertake certain development activities (such as clinical trial enrollment and the conduct of clinical trials) and accomplish certain development goals, and the timing of initiation of additional clinical trials 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 the prospectus for our recent offering of common stock and our most recent Quarterly Report on Form 10-Q. 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.

SOURCE aTyr Pharma, Inc.

For further information: Marcy Graham, Vice President, Investor Relations & Corporate Communications, mgraham@atyrpharma.com, 858-223-1163, or Jessi Colund, Feinstein Kean Healthcare, jessi.colund@fkhealth.com, 952-649-8600