aTyr Pharma Initiates Phase 1b/2 Clinical Trial for Patients with Limb Girdle Muscular Dystrophy 2B

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SAN DIEGO, Oct. 13, 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 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 limb girdle muscular dystrophy (LGMD) 2B, a severe genetic muscle disease with immune cell invasion in afflicted skeletal muscle for which there are currently no approved treatments.

This international Phase 1b/2 clinical trial is an open-label, intra-patient dose escalation study designed to assess the safety, tolerability, immunogenicity and activity of Resolaris™ in adult patients with LGMD2B. This new trial includes adult patients with facioscapulohumeral muscular dystrophy (FSHD) to further augment the Company's ongoing, blinded Phase 1b/2 clinical trial of Resolaris™ in adult patients with FSHD and to inform subsequent later-stage trial considerations. Both LGMD2B and FSHD are progressive, debilitating muscle diseases characterized by an immune component in the effected skeletal muscles.

"Our initiation of a LGMD2B patient trial represents our first indication expansion in RMICs with Resolaris," said John Mendlein, Ph.D., CEO and executive chairman of aTyr Pharma. "Although the genetic basis for LGMD2B is substantially different from FSHD, both patient groups develop an immune component as a part of the pathophysiology of the disease. The current standard of care potentially includes surgical interventions or use of devices, but no approved therapies. We believe Resolaris, derived from a protein released by human skeletal muscle cells, will promote the immune system's natural homeostatic processes and will result in improved outcomes for those suffering from these rare diseases."

Clinical trial sites for this study will screen patients for potential enrollment in the United States and Europe with plans to enroll an estimated 16 patients in total, including up to eight patients with LGMD2B and up to eight adult patients with FSHD. The Company expects to initiate a third Phase 1b/2 clinical trial this month to specifically study patients with early onset FSHD. All three trials, adult patients with FSHD, early onset FSHD and LGMD2B, will assess Resolaris' impact on the immune component using blood borne markers and magnetic resonance imaging (MRI).

For additional information please visit www.clinicaltrials.gov.

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 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. 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 who 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 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, as well a Phase 1b/2 trial in adult patients with LGMD2B or FSHD. Trials are planned in additional RMIC indications, including a study of patients with an early onset form of FSHD 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 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.

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