aTyr Pharma Receives FDA Fast Track Designation for Resolaris™ to Treat Facioscapulohumeral Muscular Dystrophy (FSHD)

October 24, 2016
-- First Reported Fast Track Designation for a FSHD Treatment --

SAN DIEGO, Oct. 24, 2016 /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 that its product candidate Resolaris™ was granted Fast Track designation by the US Food and Drug Administration (FDA) for the treatment of facioscapulohumeral muscular dystrophy (FSHD), making it the first known therapeutic candidate for the treatment of FSHD to receive the designation. Resolaris, a designated Orphan Drug in FSHD, is currently being studied in a Phase 1b/2 clinical program.

"This Fast Track designation, which is granted to drug candidates addressing serious conditions and that demonstrate the potential to address unmet medical needs, represents another step forward for our rare muscle disease franchise," said John Mendlein, PhD, CEO of aTyr Pharma. "This designation highlights the significant need to develop and ultimately approve meaningful new therapeutics to treat patients with rare myopathies, such as FSHD."

Fast Track is a process designed by the FDA to facilitate the development and expedite the review of new drugs, biologics or treatments. Product candidates may be eligible for priority review at the time of a Biologic License Application (BLA) filing and may also be eligible to submit completed sections of the BLA on a rolling basis. Importantly, the therapy's sponsors are eligible for more frequent written communication and meetings with the FDA, the benefit of which may be to develop a pivotal study design which more closely meets the FDA's criteria, thereby creating a more collaborative and efficient pathway to approval.

Resolaris is currently being studied in a Phase 1b/2 clinical program for three types of rare myopathies with an immune component. The Company expects to report data from the following three clinical trials in December 2016:

- Phase 1b/2 study in patients with early-onset FSHD (003 Trial)
- Phase 1b/2 study in patients with FSHD and Limb Girdle Muscular Dystrophy 2B (LGMD2B) (004 Trial)
- Phase 1b/2 Extension study in patients with FSHD (005 Trial)

The primary objectives of these trials are to establish a safety and tolerability database and to explore and establish activity signals, such as various endpoints and biomarkers, which will best inform aTyr's clinical development path forward, including endpoints for a BLA.

About Resolaris™

aTyr Pharma is developing Resolaris as a potential first-in-class intravenous protein therapeutic for the treatment of rare myopathies with an immune component. Resolaris is derived from a naturally occurring protein released *in vitro* 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 FSHD

Facioscapulohumeral muscular dystrophy (FSHD) is a rare genetic myopathy affecting an estimated 19,000 people in the United States for which there are no approved treatments. The primary clinical phenotype of FSHD is debilitating skeletal muscle deterioration and weakness. The symptoms of FSHD often appear early in the face, shoulder blades, upper arms, lower legs and trunk, and can affect certain muscles while adjacent muscles remain healthy. In addition to muscle weakness, FSHD patients often experience debilitating fatigue and chronic pain. The disease is typically diagnosed by the presence of a characteristic pattern of muscle weakness and other clinical symptoms, as well as through genetic testing. To learn more about FSHD, please visit www.fshsociety.org.

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 and extra-muscular manifestations such as 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 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 who 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 primarily assessed for clinical symptoms to assess skeletal muscle health. To learn more about LGMD2B please visit www.jain-foundation.org.

About Physiocrines

Physiocrines comprise naturally occurring proteins that aTyr believes promote homeostasis, a fundamental process of restoring stressed or diseased tissue to a healthier state. Physiocrines are extracellular signaling regions of tRNA synthetases, an ancient family of enzymes that catalyze a key step in protein synthesis. aTyr is currently focused on Physiocrines that act as endogenous modulators of the immune system. Physiocrines offer the opportunity for modulating biological pathways through newly discovered, naturally occurring mechanisms, many of which may provide advantages over other types of immune-modulatory therapeutics, including the potential for improved patient outcomes and reduced side effect profiles.

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 modulators. The Company's lead candidate, Resolaris™, is a potential first-in-class intravenous protein therapeutic for the treatment of rare myopathies with an immune component. Resolaris is currently in a Phase 1b/2 clinical trial in adult patients with facioscapulohumeral muscular dystrophy (FSHD); a Phase 1b/2 trial in adult patients with limb-girdle muscular dystrophy 2B (LGMD2B or dysferlinopathies) or FSHD; and a Phase 1b/2 trial in patients with an early onset form of FSHD. aTyr has built an intellectual property estate, to protect its pipeline, comprising over 80 issued or allowed patents and over 230 pending patent applications that are owned or exclusively licensed by aTyr, including over 300 potential Physiocrine-based protein compositions. 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

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 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 our most recent Annual Report on Form 10-K for the year ended December 31, 2015 and in our subsequent 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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