aTyr Pharma Presents Additional Data for Resolaris™ Phase 1b/2 Trial in Adult Patients with Facioscapulohumeral Muscular Dystrophy at the 21st International Annual Congress of the World Muscle Society

October 6, 2016

SAN DIEGO, Oct. 6, 2016 /PRNewswire/ -- aTyr Pharma, Inc. (Nasdaq: LIFE), today announced that additional clinical data from aTyr's Phase 1b/2 Trial (002) in adult patients with FSHD were presented at the 21st International Congress of the World Muscle Society in Granada, Spain. The poster presentation is titled "A Randomized, Double-blinded, Placebo-controlled, Multiple Ascending Dose Study to Evaluate the Safety, Tolerability, Pharmacokinetics, Immunogenicity, and Biological Activity of ATYR1940 (Resolaris™) in Adult Patients with Facioscapulohumeral Muscular Dystrophy."

"In today's presentation we have provided additional data analyses which reaffirm encouraging trends in two meaningful clinical assessments from our adult FSHD (002) trial," commented John Mendlein, PhD, CEO of aTyr Pharma. "In December, we look forward to sharing further top-line updates from our Phase 1b/2 clinical program with Resolaris in three different rare myopathies from our early onset FSHD (003) trial, our LGMD2B/FSHD (004) trial, and our first FSHD extension (005) trial."

Today's presentation includes additional detailed data from the adult FSHD (002) trial covering safety, tolerability, PK, and clinical assessments. Encouraging activity was seen in the patient-reported outcomes, known as the Individualized Neuromuscular Quality of Life Assessment (INQoL), and the physician-reported functional assessment Manual Muscle Testing (MMT).

Patients treated with Resolaris were generally improved compared with placebo as assessed by INQoL, with patients in cohort 3 (3.0 mg/kg for 12 weeks) showing the greatest improvement compared with cohort 1 and cohort 2 (0.3 mg/kg and 1.0 mg/kg for 4 weeks respectively). Patients in cohort 3 reported a ~9.9% improvement in INQoL compared with a ~15.6% worsening in the placebo group at week 14. Five out of the six patients on Resolaris in cohort 3 showed overall improvement in their INQoL score at week 14, versus zero out of two patients on placebo.

A trend for improvement in MMT results with Resolaris treatment was also observed compared with placebo, especially in the upper limbs. In general, there was an association between changes in INQoL and MMT, whereby all patients who experienced an improvement in muscle function also showed improvement in INQoL scores. Patients in cohort 3 reported ~0.7% improvement in MMT compared with ~1.4% worsening in the placebo group at week 14. Three out of the six patients on Resolaris in cohort 3 showed overall improvement in their MMT score at week 14, versus zero out of two patients on placebo.

Conclusions of the Adult FSHD (002) Trial:

- Over the dose and duration studied, Resolaris was found to be generally safe in adult patients with FSHD, and was generally well tolerated with the exception of one patient who experienced infusion related reactions as previously reported and as discussed in more detail in our poster presentation.
- PK properties were dose-proportional and generally consistent throughout the study, with no measurable impact from anti-drug antibodies (ADAs).
- Clinical activity was supported by signals of improvement in INQoL questionnaire responses and MMT measures.
- Other exploratory measures, including lower extremity targeted MRI, did not demonstrate activity. Variability in image acquisition may have diminished the opportunity to show activity in this measurement.
- Patients in cohorts 2 and 3 were allowed to enroll in the ongoing long-term extension study (005) investigating the safety and efficacy of Resolaris, for which aTyr expects to provide an update in December 2016.

"We are excited to have observed signals of activity across two clinical assessments with both INQoL and MMT, which is a first for FSHD patients," said Sanjay Shukla, MD, MS, Chief Medical Officer of aTyr Pharma. "Directional improvement in two separate assessments that should otherwise decline over time may be evidence of how clinically meaningful Resolaris could be for FSHD patients, and we look forward to building upon the clinical activity and safety data with our additional data later this year."

For additional information please refer to aTyr Pharma's investor relations website (www.atyrpharma.investorroom.com) to access today's full poster presentation.

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. Early onset FSHD occurs in individuals who experience symptoms of progressive muscle involvement as juveniles, and some of these patients suffer from a particularly severe form of the disease. To learn more about FSHD, please visit www.fshsociety.org.

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 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. To protect this pipeline, aTyr has built an intellectual property estate 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.

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

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