



# aTyr Pharma

## **aTyr Pharma Doses First Patient In Long-Term Safety Extension Study Of Resolaris™ for Treatment of Adult Patients with FSHD**

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Physiocrine-Based Therapy Harnesses Natural Immunomodulation Pathway to Address Rare Genetic Muscular Dystrophy

SAN DIEGO, Sept. 10, 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 that the first patient has been dosed in a newly initiated long term-safety extension study of Resolaris™ for adult patients with facioscapulohumeral muscular dystrophy (FSHD), a rare and severe genetic myopathy for which there are currently no approved treatments.

The open label study is designed to assess the long-term safety, tolerability and biological activity of Resolaris™ and will include adult patients who are currently enrolled in an ongoing, double-blind, placebo-controlled, multiple ascending dose Phase 1b/2 trial of Resolaris™ in adult patients with FSHD. Data from the Phase 1b/2 study are expected in the fourth quarter of 2015 or first quarter of 2016. The extension study is expected to continue through mid-2016.

"Our Resolaris extension study takes the next step in developing a new class of Physiocrine-based medicines for patients who currently have few options. The trial will also expand our growing safety and tolerability database over a longer treatment period," said John Mendlein, Ph.D., CEO and executive chairman of aTyr Pharma. "When given in therapeutic doses, we believe Physiocrine-based therapies may productively modulate immune processes by rebalancing the immune systems in chronic rare muscle diseases to provide new options for these severely affected patients."

Resolaris™ is the first Physiocrine-based therapeutic under investigation for any disease and is based on a naturally occurring protein identified by aTyr Pharma. The Company is also planning trials in additional indications, including early onset FSHD and limb-girdle muscular dystrophy (LGMD) 2B, and both are expected to initiate later this year.

For additional information on this study, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### **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 engineered immuno-modulatory therapeutics, including the potential for improved patient outcomes and reduced side effect profiles.

### **About Resolaris™**

aTyr Pharma is developing Resolaris™ as a 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 approximately 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 develop in a descending pattern, starting with the face and upper body to the lower body and progressing in a "muscle by muscle" fashion. 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.

### **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 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). Trials are planned in additional indications, including early onset FSHD and limb-girdle muscular dystrophy (LGMD) 2B. Trials are also planned for indications in 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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