



aTyr Pharma

aTyr Announces First FSHD Patient Study of Resolaris™

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First Physiocrine-Based Therapeutic Administered to Patients

PR Newswire

SAN DIEGO

SAN DIEGO, Jan. 28, 2015 /PRNewswire/ -- aTyr Pharma ("aTyr"), a biotherapeutics company engaged in the discovery and development of Physiocrine-based therapeutics to address rare diseases, announced today its first FSHD patient clinical trial of Resolaris™, an investigational new drug representing aTyr's first Physiocrine-based product candidate in the clinic. The study focuses on adult patients with facioscapulohumeral muscular dystrophy (FSHD), a rare and severe genetic myopathy for which there are currently no approved treatments.

The Phase 1b/2 study is a double-blind, placebo-controlled, multiple ascending dose trial in up to 44 FSHD patients at multiple sites in the European Union. The exploratory trial is designed to evaluate safety, tolerability, pharmacokinetics and the biological activity of Resolaris™ in adult patients with FSHD.

"FSHD patients suffer from a debilitating skeletal muscle disease, and we would like to thank FSHD patients, caregivers and community for their contributions to this trial. We believe therapeutic levels of Resolaris™ have the potential to promote muscle health in FSHD patients that suffer from chronic triggers of skeletal muscle damage," said John Mendlein, Ph.D., CEO and executive chairman of aTyr Pharma. "Our Resolaris™ FSHD trial represents the first patient administration of a naturally occurring protein derived from a new class of physiological modulators, Physiocrines. We believe this trial will be an important step in our plan to develop new medicines that will have a meaningful impact for patients by activating physiological pathways important to skeletal muscle health."

For additional information on this study, please visit www.clinicaltrials.gov.

About Physiocrines

Physiocrines comprise naturally occurring proteins that we believe 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™ will 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, severe 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 weakness and immune cell involvement in the muscle. 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, and in early onset FSHD, patients often die in their 20s or 30s. 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. To protect this pipeline, aTyr built an intellectual property estate comprising over 200 patents and 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. The privately held biotech was founded by Professors Paul Schimmel and Xiang-Lei Yang, two leading aminoacyl tRNA synthetase scientists at The Scripps Research Institute, and investors include Alta Partners, Cardinal Partners, Domain Associates and Polaris Partners. For more information, please visit <http://www.atyrpharma.com>.

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