aTyr Pharma Receives EMA Orphan Drug Designation for Resolaris™ in FSHD

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SAN DIEGO, Feb. 18, 2015 /PRNewswire/ -- aTyr Pharma, Inc. ("aTyr"), a biotherapeutics company engaged in the discovery and development of Physiocrine-based therapeutics to address rare diseases, announced today the European Commission (EC) has granted orphan drug designation to Resolaris™ for the treatment of facioscapulohumeral muscular dystrophy (FSHD). Resolaris™, an investigational new drug representing aTyr's first Physiocrine-based product candidate in the clinic, is being developed as a first-in-class intravenous protein therapeutic for the treatment of rare myopathies with an immune component.

"Our mission is to develop medicines that will make a meaningful difference to patients impacted by debilitating rare diseases. We are very pleased that the EMA has recognized the potential of Resolaris™ for patients suffering from FSHD," said John Mendlein, Ph.D., CEO and executive chairman of aTyr Pharma. "The Agency's decision is an important advancement for our promising Physiocrine-based medicines, and marks a key milestone in our strategy to deliver Resolaris™ to address the unmet needs of patients with this rare disease."

aTyr previously announced the first patient study of Resolaris™ in adults with 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 currently enrolling at multiple sites in the European Union (EU).

EMA orphan designation is intended to encourage development of medicines for the diagnosis, treatment, or prevention of life-threatening or chronically debilitating conditions that affect no more than 5 in 10,000 people in the EU. Medicines that receive orphan designation are eligible for a number of incentives, including assistance with development of the medicine; reduced fees for marketing-authorization applications; and extended market exclusivity once the medicine is authorized. Orphan designation is conferred following a positive opinion by the EMA's Committee for Orphan Medicinal Products (COMP).

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, and received orphan product designation in the EU for the treatment of FSHD. aTyr believes Resolaris™ will provide therapeutic benefit to patients with rare myopathies with an immune component characterized by excessive immune cell involvement.

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, which may provide advantages over engineered immuno-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. To protect this pipeline, aTyr built an intellectual property estate comprising 13 patents and over 200 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. 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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