aTyr Pharma Receives U.S. FDA Orphan Designation for Resolaris™

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Designation recognizes the company's first Physiocrine-based drug candidate for the treatment of facioscapulohumeral muscular dystrophy (FSHD)

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SAN DIEGO, April 27, 2015 /PRNewswire/ -- aTyr Pharma ("aTyr"), a biotherapeutics company engaged in the discovery and development of Physiocrine-based therapeutics to address rare diseases, announced today that Resolaris has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) 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.

"FSHD is a debilitating, progressive disease for which there are currently no approved treatments, and the patient community is facing significant unmet medical needs. Receiving orphan designation from the FDA will support our efforts to bring an approved therapy to patients as quickly as possible," said John Mendlein, Ph.D., CEO and executive chairman of aTyr Pharma.

Resolaris is currently being studied in a phase 1b/2, randomized, double-blind, placebo-controlled trial in adult patients with FSHD at multiple sites in the European Union. The trial is designed to evaluate the safety, tolerability, pharmacokinetics and immunogenicity of multiple intravenous doses of Resolaris in adult patients with FSHD. FSHD is a severe, rare genetic myopathy with an immune component for which there are currently no approved treatments.

The FDA's Orphan Drug Designation program is intended to advance the development of products which demonstrate promise in diagnosing or treating rare conditions that affect fewer than 200,000 people in the U.S. Sponsors developing orphan-designated products are eligible for incentives under the program, including seven years of market exclusivity following FDA approval, waiver or partial payment of application fees, and certain tax credits. The program has successfully enabled the development and marketing of more than 400 drugs and biologic products for rare diseases since 1983.

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. To protect this pipeline, aTyr built an intellectual property estate comprising 24 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 Company was founded by Professors Paul Schimmel and Xiang-Lei Yang, two leading aminoacyl tRNA synthetase scientists at The Scripps Research Institute.

For more information, please visit http://www.atyrpharma.com.

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