



aTyr Pharma

Publication in Nature Demonstrates Physiocrine Genetic Mutation Disrupts A Key Signaling Pathway in Neuronal Biology

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aTyr Sponsored Research For New Target For Rare Hereditary Peripheral Neuropathies

SAN DIEGO, Nov. 4, 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 the discovery of a new Physiocrine pathway as a potential target for the treatment of Charcot-Marie-Tooth Disease Type 2D (CMT2D). This research was co-authored by Xiang-Lei Yang, Ph.D., professor at The Scripps Research Institute and co-founder of aTyr Pharma, and was published in the October 29, 2015 issue of *Nature*. This groundbreaking research was sponsored in part by aTyr Pharma, through an agreement with The Scripps Research Institute for research into aminoacyl tRNA synthetases, the largest gene family implicated in CMT.

"Dr. Yang's research underscores the importance of Physiocrine pathways in critical areas of physiology. In this case, a mutation in a Physiocrine disrupts normal neuronal biology of peripheral nerves. This research into neurodegenerative diseases provides new insight that may benefit patients with few treatment options," said John Mendlein, Ph.D., CEO and executive chairman of aTyr Pharma. "The results of this study highlight a conceptual framework that could be used to identify other targets for defects in cell-signaling pathways, similar diseases that may be addressed by our Physiocrine-based medicines."

CMT2D is caused by a mutation in GARS, a gene that encodes a tRNA synthetase (GlyRS), which plays a canonical role in protein synthesis. This study demonstrated that the GARS mutation results in the dysregulation of a key cellular signaling pathway active in peripheral nerves: VEGF-Nrp1 that is required for cell survival. By restoring this pathway, nerve cell survival increased and disease symptoms were reduced.

Charcot-Marie-Tooth (CMT) disease is a genetic condition that affects the peripheral nervous system and is characterized by the progressive degeneration of muscle tissue and the loss of touch sensation across different parts of the body. This disease is the most common heritable neurological disorder with an incidence rate of 1 in 2,500 people. CMT2D is a subset of this disease and is characterized by muscle weakness and loss of sensation in the hands and feet. There are currently no effective treatments for this condition.

About aTyr Pharma

aTyr Pharma engages 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 (RMIC). Resolaris™ is currently in a Phase 1b/2 clinical trial in adult patients with FSHD; a Phase 1b/2 trial in adult patients with LGMD2B or FSHD; and a Phase 1b/2 trial in patients with an early onset form of FSHD. Trials are planned in additional RMIC indications as well as an initial trial in rare pulmonary diseases with an immune component (RPIC) in patients with 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 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 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.

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