



# aTyr Pharma

## aTyr Provides Corporate Update And Outlook For 2016

January 11, 2016

Data for Phase 1b/2 Clinical Trial of Resolaris in Adult FSHD Patients Expected at Approximately the End of First Quarter 2016

SAN DIEGO, Jan. 11, 2016 /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 provided an update on corporate developments in advance of its upcoming participation in the 34<sup>th</sup> Annual J.P. Morgan Healthcare Conference.

"We are pleased by our pipeline progress to date and look forward to further progress in 2016. We expect data from our first Resolaris™ trial in adult facioscapulohumeral muscular dystrophy (FSHD) patients at approximately the end of the first quarter that will further inform our clinical strategy moving forward for Resolaris in both muscle and lung indications," said John Mendlein, Ph.D., CEO of aTyr Pharma. "With two additional trials progressing in limb girdle muscular dystrophy (LGMD) 2B and early-onset FSHD, and our second IND candidate selected for the potential treatment of severe lung diseases, we continue to be excited by our Physiocrine-based candidates for patients with rare diseases in need of innovative therapeutic options."

### Update on Corporate Milestones

- **Completed patient dosing for Phase 1b/2 trial for Resolaris in adult FSHD patients.** Today, aTyr announced that it has completed patient dosing for its Phase 1b/2 trial for Resolaris in adult FSHD patients and is in the process of database curation and analysis. The Company expects to report data at approximately the end of the first quarter of 2016. The study is a double-blind, placebo-controlled, multiple ascending dose trial at multiple sites in the European Union and United States, and is designed to evaluate safety, tolerability, pharmacokinetics and the biological activity of Resolaris in adult patients with FSHD.
- **First patients dosed in Phase 1b/2 trial of Resolaris in patients with LGMD2B.** The first patients have been dosed in the recently initiated Phase 1b/2 trial of Resolaris in patients with LGMD2B. The international trial is an open-label, intra-patient dose escalation study designed to assess the safety, tolerability, immunogenicity and activity of Resolaris in adult patients with LGMD2B and adult patients with FSHD. The trial will further augment the Company's blinded Phase 1b/2 clinical trial of Resolaris in adult patients with FSHD and inform subsequent later-stage trial considerations.
- **Started process development for GMP manufacturing in E. coli and commenced preclinical IND-enabling studies for second IND candidate.** For aTyr's second IND candidate, iMod.Fc, the Company started process development for GMP manufacturing in E. coli, as well as preclinical IND-enabling studies. iMod.Fc represents the first, engineered Physiocrine-based product candidate. It is based on an immuno- and fibro-modulating Physiocrine domain fused to an Fc region of a human antibody. iMod.Fc has shown promising activity in a well-established preclinical rodent model of lung inflammation and pulmonary fibrosis.
- **Anticipating initiation of clinical program in rare pulmonary diseases with an immune component (RPIC).** aTyr plans to initiate a clinical program in RPIC in patients with interstitial lung disease (ILD). Later this year, the Company will make a determination as to whether to move forward with an exploratory Phase 1b/2 trial for Resolaris (by the end of 2016) and/or iMod.Fc (in 2017) in one or more ILD indications.
- **Appointed Sanuj Ravindran, M.D., as Chief Business Officer.** Dr. Ravindran was previously Senior Vice President and Global Head of Corporate Development at The Medicines Company (Nasdaq: MDCO). Prior to joining MDCO in 2012, he spent 10 years focusing on healthcare and life sciences investments with the Asian Healthcare Fund, Radius Ventures and Burrill & Company. Dr. Ravindran earned an M.B.A. from Northwestern University's Kellogg School of Management and an M.D. from Jefferson Medical College at Thomas Jefferson University.
- **Strong cash balance for YE 2015.** Today, aTyr announced an estimated cash balance of approximately \$125 million at the end of 2015.

aTyr will participate at the J.P. Morgan Healthcare Conference this week, to be held at the Westin St. Francis Hotel in San Francisco.

Dr. John Mendlein, aTyr's CEO, will provide an overview of the Company during a presentation at 10:30 a.m. PT on Wednesday, January 13, 2016.

The presentation will be webcast live through the "Investors" section of the Company website at [www.atyrpharma.com](http://www.atyrpharma.com). An audio replay will be available for 30 days following the initial presentation webcast.

### **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); a Phase 1b/2 trial in adult patients with limb girdle muscular dystrophy (LGMD) 2B or FSHD; and a Phase 1b/2 trial in patients with an early onset form of FSHD. To protect this pipeline, aTyr built an intellectual property estate comprising 70 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. The Company was founded by Professors Paul Schimmel, Ph.D., and Xiang-Lei Yang, Ph.D., two leading aminoacyl tRNA synthetase scientists at The Scripps Research Institute. 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 of 1933 and Section 21E of the Securities Exchange Act of 1934, 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 such safe harbor provisions for forward-looking statements 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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