



aTyr Pharma

aTyr Pharma Announces First Quarter 2017 Operating Results

May 11, 2017

– Announced Promising Top-Line Results from Resolaris™ Phase 1b/2 Trial in Early Onset FSHD –

– Preclinical Data from Stalaris™ Program to be Presented at American Thoracic Society International Conference in May 2017 –

– aTyr Pharma to Host Educational Webinar on Stalaris Program for the Potential Treatment of Interstitial Lung Diseases with an Immune Component –

SAN DIEGO, May 11, 2017 (GLOBE NEWSWIRE) -- 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 operating results for the first quarter ended March 31, 2017.

"We recently announced that Resolaris demonstrated promising signals of clinical activity and a favorable safety profile in the early onset FSHD patient population. These data contribute to our understanding of Resolaris and will help our team design optimal randomized, placebo-controlled trials in rare muscular dystrophies with an immune component," said John Mendlein, Ph.D., CEO of aTyr Pharma. "During 2017, we look forward to moving our second program Stalaris into the clinic, presenting preclinical findings from our Stalaris program at the American Thoracic Society (ATS) International Conference, advancing the development of pharmacodynamic (PD) assays for our programs, and initiating the next trial for Resolaris after executing a partnership related to one of our programs."

Recent Highlights

- **Promising Clinical Results for Resolaris in Early Onset FSHD** – aTyr recently announced top-line results from its Phase 1b/2 trial (003) of Resolaris in patients with early onset FSHD (diagnosis of FSHD before age 10). Overall, 63% of patients (5/8) showed an increase from baseline in their Manual Muscle Test, with a mean change from baseline of +3.8%. Moreover, 67% of patients measured (4/6) had improvement in an individualized neuromuscular quality-of-life assessment (INQoL). Resolaris was generally well-tolerated at doses up to 3.0 mg/kg once weekly in this younger patient population (patients in the trial were between the ages of 16 and 20) with no observed signs of general immunosuppression.
- **AAN Presentation for Resolaris (ATYR 1940)** – Dr. John Vissing, M.D., Ph.D., Professor of Neurology, University of Copenhagen, presented a poster titled "Results of a Phase 1b/2 Study of ATYR1940 in Adult Patients with Limb Girdle Muscular Dystrophy Type 2B (LGMD2B) and Facioscapulohumeral Muscular Dystrophy (FSHD) (ATYR-C-004)" at the American Academy of Neurology (AAN) Annual Meeting on April 25, 2017 in Boston, MA.
- **Fast Track and Orphan Drug Designations for LGMD** – During the quarter, Resolaris was granted Fast-Track designation by the FDA for the treatment of LGMD2B and was granted Orphan Drug Designation for the treatment of LGMD patients by the FDA and the European Commission.
- **Global Patent Portfolio** – During the quarter, aTyr announced the issuance of patents that cover Physiocrines derived from all 20 human tRNA synthetases. This includes patents that cover all of aTyr's three current programs in three different therapeutic areas. The current patent portfolio of issued or allowed patents that are owned or exclusively licensed totals over 190.

Upcoming Milestones & Events

- **ATS Presentations** – aTyr Pharma will present two posters on the Stalaris program, an Fc fusion protein composed of a splice-variant of the Resokine pathway, at the American Thoracic Society International Conference May 19 - 24, 2017 in Washington, D.C.:
 - "Resokine Modulates Immune Cell Infiltration into the Lung and Provides Therapeutic Activity in a Bleomycin-Induced Lung Fibrosis Model"
 - Poster Presentation at ATS on May 23rd, 2017
 - "The Resokine Pathway is Implicated in the Pathology of Interstitial Lung Disease"
 - Poster Presentation at ATS on May 24th, 2017

- **ATS Educational Webinar** – In conjunction with the ATS presentations, aTyr Pharma will host an educational webinar on Tuesday, May 23rd, 2017 at 8:30 am ET featuring Dr. Steven D. Nathan, M.D., FCCP, Director of the Advanced Lung Disease Program and Lung Transplant Program at Inova Fairfax Hospital, to provide disease education on interstitial lung diseases that are characterized by an immune component. The company will also provide an overview of the Stalaris program, in development for the potential treatment of patients with these severe rare diseases for which there are limited treatment options.
- **Long-Term Safety Extension Trials for Resolaris** – An update from rollover patients of the previously announced Resolaris Phase 1b/2 studies is expected mid-year. The focus of the trials is the long-term safety of Resolaris in patients with adult LGMD2B, adult FSHD, and early onset FSHD.
- **PD Assays** – aTyr Pharma's research team is working on the identification of pharmacodynamic assays for the Resokine pathway that may be used in future clinical trials for both the Resolaris and Stalaris programs.
- **Initiate Next Resolaris Clinical Trial** – Following the successful completion of a robust Phase 1b/2 program for Resolaris in multiple rare muscular dystrophies with an immune component, aTyr plans to initiate a randomized placebo-controlled trial with Resolaris following the identification of a PD assay and execution of a partnership related to one of aTyr's pipeline programs.
- **Initiate Stalaris Clinical Development** – aTyr plans to initiate its clinical development program for Stalaris, its second Physiocrine-based therapeutic candidate in the second half of 2017.
- **Project ORCA** – aTyr's third biologics program based on aTyr's knowledge of immunological pathways involving Physiocrine biology is currently in preclinical development. This represents a third therapeutic modality for which the company plans to provide additional details on later this year.

First Quarter 2017 Financial Results

Research and development expenses were \$9.2 million and \$12.0 million for the quarters ended March 31, 2017 and 2016, respectively. The decrease of \$2.8 million was due primarily to a \$4.1 million decrease related to manufacturing costs incurred in support of various activities for Resolaris. The decrease was partially offset by an increase of \$1.5 million related to manufacturing and non-clinical development costs incurred for the Stalaris program.

General and administrative expenses were relatively flat at \$4.0 million and \$4.1 million for the quarters ended March 31, 2017 and 2016, respectively.

Financial Guidance

As of March 31, 2017, aTyr had \$61.9 million in cash, cash equivalents and investments and 23.8 million shares of common stock outstanding.

aTyr continues to expect that its cash, cash equivalents and investments will be sufficient to fund its anticipated operations into the third quarter of 2018.

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 pathways. To date, the company has generated three innovative therapeutic candidate programs based on its knowledge of Physiocrine biology in three different therapeutic areas. aTyr has built an intellectual property estate, to protect its pipeline, comprising over 190 issued patents or allowed patent applications that are owned or exclusively licensed, including over 300 potential Physiocrine-based protein compositions. aTyr's key programs are currently focused on severe, rare diseases characterized by immune imbalance for which there are currently limited or no treatment options. For more information, please visit <http://www.atyrpharma.com>.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Litigation Reform Act. Forward-looking statements 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 and potential therapeutic benefits of Resolaris™ or Stalaris™, the ability of the Company to successfully advance its pipeline or product candidates, undertake certain development activities (such as clinical trial enrollment and the conduct of clinical trials) and accomplish certain development goals and the timing of such activities and development goals, the timing of initiation of additional clinical trials, the scope and strength of our intellectual property portfolio, our ability to receive regulatory approvals for, and commercialize, our product candidates 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 our most recent Annual Report on Form 10-K for the year ended December 31, 2016 and in our other SEC filings. 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.

ATYR PHARMA INC.

Condensed Consolidated Statements of Operations

(unaudited, in thousands, except share and per share data)

	Three Months Ended	
	March 31,	
	2017	2016
Operating expenses:		
Research and development	\$ 9,204	\$ 12,000
General and administrative	4,007	4,115
Total operating expenses	13,211	16,115
Loss from operations	(13,211)	(16,115)
Other income (expense), net	(194)	28
Net loss	(13,405)	(16,087)
Net loss per share, basic and diluted	\$ (0.56)	\$ (0.68)
Weighted average common stock shares outstanding, basic and diluted	23,739,057	23,631,133

ATYR PHARMA INC.

Condensed Consolidated Balance Sheets

(in thousands)

	March 31,	December 31,
	2017	2016
	(unaudited)	
Cash, cash equivalents and available-for-sale investments	\$ 61,934	\$ 76,149
Other assets	2,178	2,954
Property and equipment, net	1,623	1,421
Total assets	\$ 65,735	\$ 80,524
Accounts payable, accrued expenses and other liabilities	\$ 5,455	\$ 8,186
Current portion of long-term debt	1,094	339
Long-term debt, net of current portion and issuance costs	8,480	9,198
Stockholders' equity	50,706	62,801
Total liabilities and stockholders' equity	\$ 65,735	\$ 80,524

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aTyr Pharma Inc.