



# aTyr Pharma

## aTyr Pharma Announces Second Quarter 2017 Operating Results and Provides an Update on Innovative Immunology Pipeline

August 14, 2017

- Resolaris Demonstrated Favorable Safety Profile in Rare Muscular Dystrophy Patients in Extension Studies –
- iMod.Fc Program for Interstitial Lung Disease (ILD) on Track to Commence Phase 1 Clinical Trial –
- Project ORCA: Leverages a New Immuno-Oncology Antibody Target Based on Physiocrine Biology –

SAN DIEGO, Aug. 14, 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 second quarter ended June 30, 2017.

"During the first half of 2017, our team advanced three innovative, first-in-class biologics that harness Physiocrine biology in immunology," said John Mendlein, Ph.D., CEO of aTyr Pharma. "For our Resolaris program, we report favorable safety data from two extension studies in patients with rare muscular dystrophies underscoring the potential for improved clinical outcomes over time based on stabilizing or improving muscle function. In addition, we plan to initiate our first-in-human clinical trial for our iMod.Fc program for ILD. Our third program, ORCA targets a novel, proprietary immuno-oncology pathway using antibodies to change levels of Resokine in tumor settings. We believe tumors utilize Resokine to evade immune system responses. Our Resolaris, iMod.Fc and ORCA programs demonstrate the power and leverage arising from our new insights in immunology and novel therapeutic modalities targeting homeostatic pathways."

### Resolaris Program – First Resokine Therapeutic Candidate Based on a Protein Secreted by Muscle

- **Safety Extension Study Results (005 and 006)** – aTyr recently completed two extension studies in patients with limb-girdle muscular dystrophy 2B (LGMD2B), adult facioscapulohumeral muscular dystrophy (FSHD), and early onset FSHD. Per protocol patients received 3.0 mg/kg of Resolaris weekly in these extension studies.
  - No significant adverse events or observed signs of general immunosuppression in either study.
  - Across both studies, 12 patients received at least six months of Resolaris in each study with no significant trends of worsening in either manual muscle testing (MMT) or individualized neuromuscular quality of life assessment (INQoL) scores:
    - 5 of 10 patients remained stable or improved their MMT score at 24 weeks; 3 of 6 patients remained stable or improved their MMT score at 36 weeks.
  - In the 006 trial, which commenced July 2016, 4 patients had early-onset FSHD, 2 patients had adult FSHD and 2 patients had LGMD2B.
    - All 8 patients remained Jo-1 antibody negative throughout the study; 2 patients experienced transient Jo-1 antibody levels above the protocol-defined cut off for continuation in the study.
  - In the 005 trial, which commenced August 2015, 9 patients with adult FSHD enrolled.
    - All 9 patients remained Jo-1 antibody negative throughout the study; 3 patients experienced transient Jo-1 antibody levels above the protocol-defined cut off for continuation in the study; 3 patients experienced transient, mild-to-moderate infusion related reactions and were discontinued per protocol.
  - 44 patients, across all of our trials (002, 003, 004, 005, and 006), have now received Resolaris for a total drug exposure of 204 patient months.
- **Promising Clinical Results for Resolaris in Early Onset FSHD** – During the quarter, aTyr announced top-line results from its Phase 1b/2 trial (003) of Resolaris in patients with early onset FSHD. Overall, 63% of patients (5/8) showed an increase from baseline in their MMT score, with a mean change from baseline of +3.8%. 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** – During the quarter, 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.

• **Clinical Development Plan** – Initiation of a randomized placebo-controlled trial with Resolaris is contingent upon the identification of a PD assay and execution of a partnership related to one of our pipeline programs.

### iMod.Fc Program – First Fc Fusion Based Therapeutic Candidate for Lung Diseases

• **Clinical Development** – aTyr Pharma plans to commence a Phase 1 clinical program for the iMod.Fc program later this year. This randomized, double-blind, placebo-controlled study will investigate the safety, tolerability, immunogenicity, pharmacokinetics and pharmacodynamics (PD) of intravenous iMod.Fc in healthy volunteers.

• **American Thoracic Society (ATS) Presentations** – During the quarter, aTyr Pharma presented two posters – on the iMod.Fc program at the ATS 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”.*
- *“The Resokine Pathway is Implicated in the Pathology of Interstitial Lung Disease”.*

In conjunction with the ATS presentations, aTyr Pharma hosted an educational webinar 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, such as idiopathic pulmonary fibrosis (IPF), sarcoidosis, and chronic hypersensitivity pneumonitis (CHP). The webinar is available on the aTyr Pharma investor website.

#### **Project ORCA – First Antibody Antagonist to a Physiocrine Immunology Pathway**

- **New Target in Immuno-Oncology** – ORCA involves a novel and proprietary target that aTyr believes is active across multiple tumor types.
- **Timeline** – aTyr plans to select an antibody as a potential IND candidate in 2017.

#### **Second Quarter 2017 Financial Results**

Research and development expenses were \$8.4 million and \$11.3 million for the quarters ended June 30, 2017 and 2016, respectively. The decrease of \$2.9 million was due primarily to a \$2.2 million decrease related to Resolaris clinical trials costs and \$0.6 million decrease related to manufacturing costs incurred in support of Resolaris.

General and administrative expenses were \$3.5 million and \$4.1 million for the quarters ended June 30, 2017 and 2016, respectively. The decrease of \$0.6 million was due primarily to a \$0.4 million reduction in professional fees.

#### **First Half 2017 Financial Results**

Research and development expenses were \$17.6 million and \$23.3 million for the six months ended June 30, 2017 and 2016, respectively. The decrease of \$5.7 million was due primarily to \$4.2 million decrease related to manufacturing costs incurred in support of Resolaris and \$2.3 million decrease related to Resolaris clinical trials costs. The decrease was partially offset by an increase of \$1.0 million related to research and non-clinical development costs incurred for iMod.Fc.

General and administrative expenses were \$7.5 million and \$8.2 million for the six months ended June 30, 2017 and 2016, respectively. The decrease of \$0.7 million was due primarily to a \$0.4 million decrease in professional fees.

#### **Financial Guidance**

As of June 30, 2017, aTyr had \$57.2 million in cash, cash equivalents and investments and 23.8 million shares of common stock outstanding.

aTyr expects 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 immunological and physiological pathways. To date, the Company has generated three innovative therapeutic programs based on its knowledge of Physiocrine biology in three different therapeutic areas and three different biologic modalities. aTyr has built an intellectual property estate, to protect its pipeline, comprising over 220 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 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™, iMod.Fc, or potential product candidates from Project ORCA, 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, and our projected cash expenditures 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**

**Six Months Ended**

	<b>June 30, 2017</b>	<b>2016</b>	<b>June 30, 2017</b>	<b>2016</b>
Operating expenses:				
Research and development	\$ 8,420	\$ 11,307	\$ 17,624	\$ 23,307
General and administrative	3,487	4,126	7,494	8,241
Total operating expenses	11,907	15,433	25,118	31,548
Loss from operations	(11,907 )	(15,433 )	(25,118 )	(31,548 )
Other income (expense), net	(231 )	50	(425 )	78
Net loss	(12,138 )	(15,383 )	(25,543 )	(31,470 )
Net loss per share, basic and diluted	\$ (0.51 )	\$ (0.65 )	\$ (1.07 )	\$ (1.33 )
Weighted average common shares outstanding, basic and diluted	23,810,112	23,672,527	23,774,736	23,655,366

#### **ATYR PHARMA INC.**

##### **Condensed Consolidated Balance Sheets**

(in thousands)

	<b>June 30, 2017 (unaudited)</b>	<b>December 31, 2016</b>
Cash, cash equivalents and available-for-sale investments	\$ 57,221	\$ 76,149
Other assets	2,248	2,954
Property and equipment, net	2,003	1,421
Total assets	\$ 61,472	\$ 80,524
Accounts payable, accrued expenses and other liabilities	\$ 6,863	\$ 8,186
Term loans, net of debt issuance costs	14,578	9,537
Stockholders' equity	40,031	62,801
Total liabilities and stockholders' equity	\$ 61,472	\$ 80,524

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