



aTyr Pharma

aTyr Pharma Announces Fourth Quarter and Year-End 2016 Operating Results

March 16, 2017

- Resolaris™, in multiple rare myopathy indications in four clinical trials in 2016, demonstrated signals of clinical activity and a favorable safety profile -
- Advancing second program, Stalaris™, for rare lung indications into a first in human clinical trial in 2H17 -
- Closing in on third Physiocrine-based program opportunity, code name "Project ORCA", as a 2017 IND candidate -

SAN DIEGO, March 16, 2017 /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 operating results for the fourth quarter and year ended December 31, 2016.



"During the past year, we made significant progress in our mission to develop and ultimately deliver innovative therapeutics, based on novel immunological pathways, to patients with severe and rare diseases. Last year, we announced data from four separate clinical trials for Resolaris for the treatment of patients in three rare myopathy indications where immune cells play a role," said John Mendlein, PhD, CEO of aTyr Pharma. "In addition, we advanced our Stalaris program for rare lung diseases pre-clinically and plan to initiate clinical development in the second half of 2017. Finally, we created a third program based on our proprietary immunological pathways involving Physiocrine biology in a distinctly different therapeutic area, code named 'Project ORCA', and will announce more details later this year."

Resolaris Program Highlights

Resolaris represents a potentially first-in-class intravenous protein therapeutic candidate for the treatment of rare myopathies where T-cells in the muscle contribute to disease. Resolaris is a 57kD protein secreted from human muscle cells. aTyr's initial Phase 1b/2 clinical trials focused on adult limb girdle muscular dystrophy 2B (LGMD2B), adult facioscapulohumeral muscular dystrophy (FSHD), and early onset FSHD indications with little to no treatment options available.

Resolaris Signals of Clinical Activity

- **Adult LGMD2B Patients** – Ten adult LGMD2B patients were administered Resolaris in an intra-patient, dose-escalation Phase 1b/2 clinical trial. 78% of the LGMD patients showed an improvement in their muscle function. In this open-label trial aTyr observed that LGMD2B patients showed greater signals of muscle improvement compared to those seen with FSHD patients.
- **Adult FSHD Patients** – aTyr announced data from three separate clinical trials treating adult FSHD patients with Resolaris in 2016. Approximately 50% of patients demonstrated an increase in muscle function consistent over multiple studies. In addition, a small decrease in disease burden, as measured by the Individualized Neuromuscular Quality of Life (INQoL) assessment, was observed in a majority of FSHD patients administered Resolaris consistent across multiple studies. In a long-term safety extension study, Resolaris has continued to show a favorable safety profile with patients on the drug for a longer duration and with no significant trends in worsening or improvement in muscle function.
- **Early Onset FSHD Patients** – aTyr recently announced interim data from its intra-patient, dose-escalation Phase 1b/2 clinical trial for Resolaris in patients with early onset FSHD. Three of the four (75%) patients showed an improvement in their muscle function in this open-label trial. Complete results from all eight patients enrolled in Stage 1 are expected to be announced in the first half of 2017.

Resolaris Mechanism of Action and Safety

- **Modulate Activated T-cells** – *In vitro* experiments have demonstrated that Resolaris at 100 pM significantly reduces T-cell

activation as evidenced by reduced secretion of inflammatory cytokines and reduced levels of cell-surface activation markers. Resolaris appears to work on *activated* T-cells by conferring characteristics closer to that of resting T-cells.

- **No Signals of General Immunosuppression** – Patients administered Resolaris have not demonstrated clinical symptoms of immunosuppression in the clinical trials to date. This is consistent with a homeostatic pathway at a tissue level and aTyr's hypothesis that Resolaris selectively modulates the activity of T-cells in the tissue.
- **Building a Clinical Safety Dossier** – Resolaris has been dosed in 44 patients for a total drug exposure of 185 months as of the end of 2016.

"Our clinical data and mechanism work suggest that Resolaris, as an immune modulator of activated T-cells, has been generally well-tolerated with no signs of general immunosuppression as well as promising signals of clinical activity in improved muscle function across multiple and genetically distinct T-cell mediated myopathies," commented Sanjay Shukla, MD, MS, chief medical officer of aTyr Pharma. "This supports our hypothesis that Resolaris, a potential modulator of activated T-cells in muscle, may be broadly applicable to rare myopathies with an immune component."

Regulatory Updates

- **Orphan Drug Designation** – aTyr recently announced that Resolaris was granted Orphan Drug Designation for the treatment of LGMD patients by the FDA and the European Commission. Resolaris previously received the designations from both the FDA and the European Commission for FSHD.
- **Fast Track Designations** – In January 2017 and October 2016, Resolaris was granted Fast Track designation by the FDA for the treatment of LGMD2B and FSHD, respectively, making it the first known therapeutic candidate for the treatment of LGMD2B and FSHD to receive the designation.
- **Removal of Partial Clinical Hold** – In January 2017, the FDA removed its partial clinical hold on a dosing ceiling for Resolaris.

Stalaris Program Highlights

Stalaris is an engineered Physiocrine-based protein candidate that fuses an immuno-modulatory region of Resokine to the Fc domain of a human antibody. aTyr believes Stalaris has the potential to augment aspects of the Resokine pathway to provide therapeutic benefit to patients with rare pulmonary diseases with an immune component, such as T-cells.

- **cGMP Manufacturing** – aTyr is on track to support a clinical trial of Stalaris in the second half of 2017.
- **Clinical Development on Track** – aTyr currently anticipates initiating a clinical development program for its second Physiocrine-based therapeutic candidate in the second half of 2017.

Project ORCA Program Highlights

- **Third Biologics Program** – aTyr's third biologics program based on aTyr's knowledge of immunological pathways involving Physiocrine biology, which is currently in preclinical development. The company looks forward to providing additional details on the Project ORCA program later this year.

Intellectual Property Update

- **Global Patent Portfolio** – aTyr recently announced the issuance of US Patent Number 9,428,743, which represents the successful completion of an important company milestone – the issuance of patents that cover Physiocrines derived from all 20 human tRNA synthetases. In addition, the patent portfolio covers all of aTyr's three current programs in three different therapeutic areas.

Fourth Quarter 2016 Financial Results

Research and development expenses were \$9.1 million and \$12.7 million for the quarters ended December 31, 2016 and 2015, respectively. The decrease of \$3.6 million was due primarily to a \$4.5 million decrease related to cGMP manufacturing of Resolaris, a \$0.8 million decrease in non-cash stock-based compensation. These decreases were partially offset by a \$1.7 million increase in cGMP manufacturing development costs for Stalaris. aTyr has substantially completed its cGMP manufacturing runs for Resolaris at commercial scale to support future clinical trials and does not anticipate additional commercial scale runs in 2017.

General and administrative expenses were \$3.4 compared to \$3.8 million for the quarters ended December 31, 2016 and 2015, respectively. The decrease was primarily related to non-cash stock-based compensation.

Full Year 2016 Financial Results

Research and development expenses were \$42.8 million and \$34.5 million for the years ended December 31, 2016 and 2015, respectively. The increase of \$8.3 million was due primarily to a \$3.2 million increase in development costs for Resolaris, a \$2.4 million increase in manufacturing development costs for Stalaris, a \$2.0 million increase in other pre-clinical development costs, a \$1.2 million increase related cGMP manufacturing of Resolaris and a \$0.9 million increase related to compensation expenses resulting from increased headcount in research and development functions, net of a \$0.6 million reduction of non-cash stock-based compensation. These increases were offset by a decrease related to a one-time \$1.4 million non-cash expense for the assignment of certain intellectual property rights in the prior year period.

General and administrative expenses were \$15.1 million and \$13.1 million for the years ended December 31, 2016 and 2015, respectively. The increase of \$2.0 million was due primarily to a \$1.8 million increase in personnel costs resulting from increased headcount, inclusive of \$0.8 million of

non-cash stock-based compensation.

Financial Guidance

As of December 31, 2016, aTyr had \$76.1 million in cash, cash equivalents and investments and 23.7 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 175 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 of Resolaris™ or Stalaris™, 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, the timing of initiation of additional clinical trials and of reporting results from our clinical trials and 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.

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ATYR PHARMA, INC.

Condensed Consolidated Statements of Operations

(in thousands, except share and per share data)

	Three Months Ended		Years Ended	
	December 31,		December 31,	
	2016	2015	2016	2015
	(unaudited)			
Operating expenses:				
Research and development	\$9,144	\$12,670	\$42,846	\$34,504

General and administrative	3,383	3,812	15,094	13,112
Total operating expenses	12,527	16,482	57,940	47,616
Loss from operations	(12,527)	(16,482)	(57,940)	(47,616)
Interest income (expense), net	(59)	(11)	65	(386)
Loss on extinguishment of debt	(29)	—	(29)	—
Change in fair value of warrant liabilities	—	—	—	29
Total other income (expense)	(88)	(11)	36	(357)
Loss before income taxes	(12,615)	(16,493)	(57,904)	(47,973)
Income tax benefit	49	—	49	—
Net loss	(12,566)	(16,493)	(57,855)	(47,973)
Accretion to redemption value of redeemable convertible preferred stock	—	—	—	(15)
Net loss attributable to common stockholders	\$(12,566)	\$(16,493)	\$(57,855)	\$(47,988)
Net loss per share attributable to common stockholders, basic and diluted	\$(0.53)	\$(0.70)	\$(2.44)	\$(3.03)
Weighted average common stock shares outstanding, basic and diluted	23,716,904	23,603,661	23,681,019	15,838,353

ATYR PHARMA, INC.

Condensed Consolidated Balance Sheets

(in thousands)

	December 31, December 31,	
	2016	2015
Cash, cash equivalents and available-for-sale investments	\$ 76,149	\$ 125,349
Other assets	2,954	1,793
Property and equipment, net	1,421	2,533
Total assets	\$ 80,524	\$ 129,675
Accounts payable, accrued expenses and other liabilities	\$ 8,186	\$ 9,483
Current portion of long-term debt	339	3,366
Long-term debt, net of current portion and issuance costs	9,198	1,776
Stockholders' equity	62,801	115,050

Total liabilities and stockholders' equity	\$ 80,524	\$ 129,675
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SOURCE aTyr Pharma, Inc.