



aTyr Pharma

aTyr Pharma Announces Third Quarter 2016 Operating Results

November 14, 2016

- Data Readouts from Three Clinical Trials of Resolaris™ in Rare Myopathies On Track for December 2016 -



SAN DIEGO, Nov. 14, 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 announced operating results for the third quarter ended September 30, 2016.

"During the third quarter, we continued to make significant progress in our clinical development of Resolaris™ for the treatment of patients with facioscapulohumeral muscular dystrophy (FSHD), early onset FSHD, and limb-girdle muscular dystrophy 2B (LGMD2B). We look forward to announcing data from our three ongoing clinical trials in these indications next month," said John Mendlein, PhD, CEO of aTyr Pharma. "We anticipate initiating clinical development for our iMod.Fc program for rare pulmonopathies next year. Finally, we continue to advance our understanding of the Resokine pathway to aid in therapeutically intervening in homeostatic pathways in a fiscally prudent manner."

Recent Highlights

- **Fast Track Designation** – In October, Resolaris was granted Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of FSHD, making it the first known therapeutic candidate for the treatment of FSHD to receive the designation.
- **World Muscle Society Presentation on Resolaris for FSHD** – In October, aTyr presented clinical data from its adult FSHD (002) trial at the 21st International Annual Congress of the World Muscle Society in Granada, Spain. The presentation included detailed data covering the safety, tolerability and pharmacokinetics of Resolaris, and further detailed activity seen in the patient-reported outcomes, known as the Individualized Neuromuscular Quality of Life Assessment (INQoL), and the physician-reported functional assessment of Manual Muscle Testing (MMT).
- **Early Onset FSHD Enrollment Completed** – During the third quarter, aTyr completed enrollment in Stage 1 of its early onset FSHD (003) trial testing Resolaris in that indication.
- **Research Expertise** – In September, David J. King, Ph.D., joined aTyr Pharma as Senior Vice President, Research. Dr. King's industry experience is highlighted by his tenures at Medarex, Inc. (acquired by Bristol-Myers Squibb in 2009) and Celltech Therapeutics Ltd. (acquired by UCB in 2004). At Medarex, he led programs to identify therapeutic antibodies and played a key role in programs targeting novel biologics (including PD-1), and at Celltech, he directed the protein biochemistry and antibody engineering activities that led to the discovery and development of Cimzia®. Dr. King also served as Chief Scientific Officer at AnaptysBio, Inc. where he led research that developed a novel technology for generating antibody therapeutics.

Upcoming Milestones

- **Early Onset FSHD (003) Trial:**
 - Data from first four (of eight) patients enrolled in Stage 1 expected to be announced in December 2016.
 - Results from all eight patients enrolled in Stage 1 expected to be announced in first half of 2017.
- **LGMD2B/FSHD (004) Trial:** Top-line results expected to be announced in December 2016.
- **First Extension (005) Trial:** Update from the rollover patients expected to be announced in December 2016.
- **Second Extension (006) Trial:** Update from the rollover patients expected to be announced in first half of 2017.

Third Quarter 2016 Financial Results

Research and development expenses were \$10.4 million and \$7.7 million for the quarters ended September 30, 2016 and 2015, respectively. The increase of \$2.7 million was due primarily to a \$0.9 million increase related to cGMP manufacturing of Resolaris to support future clinical trials, a \$0.8 million increase in clinical and non-clinical development costs for Resolaris and a \$0.6 million increase in GMP manufacturing development costs for iMod.Fc. For Resolaris, we have substantially completed our GMP manufacturing runs at commercial scale and do not anticipate additional commercial scale runs in 2017.

General and administrative expenses remained relatively flat at \$3.5 million compared to \$3.6 million for the quarters ended September 30, 2016 and 2015, respectively.

Year-to-Date 2016 Financial Results

Research and development expenses were \$33.7 million and \$21.8 million for the nine months ended September 30, 2016 and 2015, respectively. The increase of \$11.9 million was due primarily to a \$5.7 million increase related to cGMP manufacturing of Resolaris to support future clinical trials, a \$3.9 million increase in clinical and non-clinical development costs for Resolaris, a \$1.7 million increase related to compensation expenses resulting from increased headcount in research and development functions, including \$0.2 million of non-cash stock-based compensation, a \$1.3 million increase in other pre-clinical development costs and a \$0.8 million increase in GMP manufacturing development costs for iMod.Fc. The increase was 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 \$11.7 million and \$9.3 million for the nine months ended September 30, 2016 and 2015, respectively. The increase of \$2.4 million was due primarily to a \$2.1 million increase in personnel costs resulting from increased headcount, inclusive of \$1.2 million of non-cash stock-based compensation.

Financial Guidance

As of September 30, 2016, we had \$80.9 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 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 modulators. The Company's lead candidate, Resolaris™, is a potential first-in-class intravenous protein therapeutic for the treatment of rare myopathies with an immune component. Resolaris is currently in a Phase 1b/2 trial in adult patients with limb-girdle muscular dystrophy 2B (LGMD2B or dysferlinopathies) or FSHD; and a Phase 1b/2 trial in patients with an early onset form of FSHD. In addition, Resolaris is currently being evaluated in two Phase 1b/2 long-term safety extension studies, one in patients with adult FSHD and the other in patients with adult FSHD, early onset FSHD, and LGMD2B. aTyr has built an intellectual property estate, to protect its pipeline, comprising over 80 issued or allowed patents and over 230 pending patent applications that are owned or exclusively licensed by aTyr, including over 300 potential Physiocrine-based protein compositions. 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

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 iMod.Fc, 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 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, 2015 and in our subsequent 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.

Contact:

Mark Johnson

Sr. Director, Investor Relations

mjohnson@atyrpharma.com

858-223-1163

ATYR PHARMA INC.

Condensed Consolidated Statements of Operations

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Operating expenses:				
Research and development	\$10,395	\$7,739	\$33,702	\$21,834
General and administrative	3,470	3,574	11,711	9,299
Total operating expenses	13,865	11,313	45,413	31,133
Loss from operations	(13,865)	(11,313)	(45,413)	(31,133)
Other income (expenses), net	46	(16)	124	(347)
Net loss	(13,819)	(11,329)	(45,289)	(31,480)
Accretion to redemption value of redeemable convertible preferred stock	—	(15)	—	(15)
Net loss attributable to common stockholders	(13,819)	(11,344)	(45,289)	(31,495)
Net loss per share attributable to common stockholders, basic and diluted	\$(0.58)	\$(0.48)	\$(1.91)	\$(2.38)
Weighted average shares outstanding, basic and diluted	23,696,511	23,581,001	23,669,154	13,221,551

ATYR PHARMA INC.
Condensed Consolidated Balance Sheets
(in thousands)

	September 30, December 31,	
	2016	2015
	(unaudited)	
Cash, cash equivalents and available-for-sale investments	\$ 80,859	\$ 125,349
Other assets	3,470	2,533
Property and equipment, net	1,596	1,793
Total assets	\$ 85,925	\$ 129,675
Accounts payable, accrued expenses and other liabilities	\$ 9,296	\$ 9,483
Total commercial bank debt	2,640	5,142
Stockholders' equity	73,989	115,050
Total liabilities and stockholders' equity	\$ 85,925	\$ 129,675

Logo - <http://photos.prnewswire.com/prnh/20120809/MM55538LOGO>

SOURCE aTyr Pharma, Inc.