aTyr Pharma Announces Second Quarter 2016 Operating Results

August 10, 2016
- On Track to Announce Data from Three Clinical Trials for Resolaris™ in Rare Genetic Muscle Diseases in 4Q16 -
- 2nd Potential First in Class Molecule iMod.Fc, On Track to Enter Clinic in 2017 -
- Maintains Strong Cash Position: $96.6 Million at End of 2Q16 -

SAN DIEGO, Aug. 10, 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 second quarter ended June 30, 2016.

"We continue to execute on our vision to bring innovative therapeutics to patients severely afflicted by rare diseases. During this last quarter, we made considerable progress toward that goal on three fronts: first, we advanced Resolaris in three distinct opportunities to help patients with rare myopathies with an immune component (RMIC)," said John Mendlein, PhD, Chief Executive Officer of aTyr Pharma. "Second, we further elucidated the mechanism of the Resokine pathway which includes affecting T-cell activation; and finally, we have advanced our second program, iMod.Fc, to cGMP manufacturing for GLP safety studies and are on track to have that candidate enter the clinic in 2017. We also want to thank the 35 facioscapulohumeral muscular dystrophy (FSHD) and 10 limb-girdle muscular dystrophy 2B (LGMD2B) patients that have participated or are participating in our clinical trials, as well as the physicians and investigators that make these studies possible across 14 global sites."

Recent Highlights and Upcoming Milestones

Following the encouraging results from our previously announced Phase 1b/2 trial of Resolaris in patients diagnosed with adult FSHD, our 002 trial, we continued to enroll patients across three ongoing clinical studies for three different types of rare myopathies with an immune component. The primary objectives of these studies are to establish a safety and tolerability database and to explore and establish activity signals, such as various functional endpoints and biomarkers, which will best inform our clinical development path forward, including endpoints for a Biologics License Application (BLA).

Highlights from our ongoing Resolaris program include:

- **Early Onset FSHD (003) Trial –** Our Phase 1b/2 trial is enrolling patients diagnosed with early-onset FSHD, which is often the most severe form of FSHD. Patients entering the trial experienced onset of disease by the age 10, and the trial includes a Stage 1, with patients ages 16 to 25, and a Stage 2, with patients ages 12 to 15. We expect to announce data from the first four patients enrolled in Stage 1 in the fourth quarter of 2016.
- **LGMD2B/FSHD (004) Trial –** Our Phase 1b/2 trial in adult patients diagnosed with either FSHD or LGMD2B completed enrollment ahead of schedule in May with 18 total patients (8 with FSHD and 10 with LGMD2B), exceeding our stated target enrollment of 16 patients. We expect to announce results in the fourth quarter of 2016.
- **First Extension (005) Trial –** Our long-term extension study of Resolaris for our adult FSHD Phase 1b/2 trial (002) continues to dose patients that were eligible to roll over. We expect to announce an update from these patients in the fourth quarter of 2016.
- **Second Extension (006) Trial –** During the quarter, we initiated a long-term extension study of adult FSHD, early-onset FSHD, and LGMD2B patients from our ongoing 003 and 004 trials.

Our scientists, clinical operations professionals, and clinicians continued to advance our science, protein manufacturing, and programs with recent highlights, including:

- **Mechanism of Action –** The Resokine pathway includes interactions with activated T-cells. Our scientists continue to elucidate the role of the Resokine pathway in affecting the level of activation of T-cells by CD3 & CD28. Resolaris is a

56kD protein, with a naturally occurring sequence of amino acids, that harnesses the Resokine pathway and interacts with T-cells to 'place a brake' on T-cell activation.

- **iMod.Fc Program –** iMod.Fc, our second product development candidate, comprises an Fc region of an antibody genetically engineered to a protein, derived from the Resokine pathway. Fc fusion proteins, such as iMod.Fc, include an additional antibody domain to enhance pharmacokinetic, or PK, and distribution characteristics. We plan to evaluate its therapeutic potential in patients with rare pulmonary diseases with an immune and/or fibrotic component. We continue to prepare for clinical trial initiation in 2017 by initiating large scale drug substance cGMP manufacturing.
- **Natural History Study Collaboration –** We recently entered into a collaboration with The Cooperative International Neuromuscular Research Group on a longitudinal natural history study, comprised of 53 patients, to advance knowledge of early onset FSHD. We provide financial support to this study, which was initially implemented by the FSH Society, the FSHD Global Research Foundation and Muscular Dystrophy Canada.
- **Expanding Leadership –** In May, we appointed Grove Matsuoka as Senior Vice President, Product Programs and Planning. Mr. Matsuoka's most recent role was Senior VP, Commercialization at CoDa Therapeutics, Inc. Prior to that, he worked in various positions at Amgen, Inc., most recently as Director, Medical Affairs, Strategic Planning and Operations, where he established and managed the strategic and operational function for the newly formed Medical Affairs organization. He was also Clinical Research Project Team Leader for an Fc Fusion Program while at Amgen.
- **Robust Patent Estate –** We have recently been issued or allowed an additional 17 patents that are now part of an intellectual property estate comprising over 80 issued or allowed patents and over 230 pending patent applications that we own or exclusively license, including over 300 potential Physiocrine-based protein compositions.

Second Quarter 2016 Financial Results

Research and development expenses were $11.3 million and $7.5 million for the quarters ended June 30, 2016 and 2015, respectively. The increase of $3.8 million was due primarily to a $1.9 million increase in clinical and non-clinical development costs for Resolaris, a $0.8 million increase related to compensation expenses resulting from increased headcount in research and development functions, including $0.1 million in non-cash stock-based compensation, a $0.7 million increase related to cGMP manufacturing of Resolaris to support future clinical trials, and a $0.4 million increase in other pre-clinical development costs.

Sequentially, there was a $2.4 million decrease related to the cGMP manufacturing of Resolaris in the second quarter of 2016 versus the first quarter of 2016, as much of the investment required to supply future clinical trials was spent in the first quarter of 2016, partially offset by a $1.2 million increase in clinical and non-clinical development costs for Resolaris.

General and administrative expenses were $4.1 million and $3.4 million for the quarters ended June 30, 2016 and 2015, respectively. The increase of $0.7 million was due primarily to a $0.5 million increase in non-cash stock-based compensation and a $0.2 million increase in costs associated with being a public company.

First Half 2016 Financial Results

Research and development expenses were $23.3 million and $14.1 million for the six months ended June 30, 2016 and 2015, respectively. The increase of $9.2 million was due primarily to a $4.7 million increase related to cGMP manufacturing of Resolaris to support future clinical trials, a $3.5 million increase in clinical and non-clinical development costs for Resolaris, a $1.6 million increase related to compensation expenses resulting from increased headcount in research and development functions, including $0.3 million of non-cash stock-based compensation, and $0.7 million in other pre-clinical development costs. 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 $8.2 million and $5.7 million for the six months ended June 30, 2016 and 2015, respectively. The increase of $2.5 million was due primarily to a $1.8 million increase in personnel costs resulting from increased headcount inclusive of $1.0 million of non-cash stock-based compensation and a $0.6 million increase in costs associated with being a public company.

Financial Guidance

As of June 30, 2016, we had $96.6 million in cash, cash equivalents and investments and 23.7 million shares of common stock outstanding.

We currently expect that our cash, cash equivalents and investments will be sufficient to fund our anticipated operations into 2018.

About Physiocrines

Physiocrines comprise naturally occurring proteins that aTyr believes promote homeostasis, a fundamental process of restoring stressed or diseased tissue to a healthier state. Physiocrines are extracellular signaling regions of tRNA synthetases, an ancient family of enzymes that catalyze a key step in protein synthesis. aTyr is currently focused on Physiocrines that act as endogenous modulators of the immune system. Physiocrines offer the opportunity for modulating biological pathways through newly discovered, naturally occurring mechanisms, many of which provide advantages over engineered immune-modulatory therapeutics, including the potential for improved patient outcomes and reduced side effect profiles.

About Resolaris™

aTyr Pharma is developing Resolaris as a potential first-in-class intravenous protein therapeutic for the treatment of rare myopathies with an immune component. Resolaris is derived from a naturally occurring protein released in vitro by human skeletal muscle cells. aTyr believes Resolaris has the potential to provide therapeutic benefit to patients with rare myopathies with an immune component characterized by excessive immune cell involvement.

About FSHD

Facioscapulohumeral muscular dystrophy (FSHD) is a rare genetic myopathy affecting an estimated 19,000 people in the United States for which there are no approved treatments. The primary clinical phenotype of FSHD is debilitating skeletal muscle deterioration and weakness. The symptoms of FSHD often appear early in the face, shoulder blades, upper arms, lower legs and trunk, and can affect certain muscles while adjacent muscles remain healthy. In addition to muscle weakness, FSHD patients often experience debilitating fatigue and chronic pain. The disease is typically diagnosed by the presence of a characteristic pattern of muscle weakness and other clinical symptoms, as well as through genetic testing. Early onset FSHD occurs in individuals who experience symptoms of progressive muscle involvement as juveniles, and some of these patients suffer from a particularly severe form of the disease. To learn more about FSHD please visit www.fshsociety.org.

About LGMD2B

Limb girdle muscular dystrophy (LGMD) refers to a group of rare genetic myopathies, of which there are more than 20 different subtypes, none with approved therapies. LGMD affects an estimated 16,000 patients in the U.S., approximately 3,000 of whom have LGMD2B. LGMD2B is a recessive genetic disease caused by mutations in the dysferlin gene. Patients experience debilitating muscle weakness and atrophy as well as immune cell invasion in the skeletal muscle. Patients are primarily assessed for clinical symptoms to assess skeletal muscle health. To learn more about LGMD2 please visit www.jain-foundation.org.

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 clinical trial in adult patients with facioscapulohumeral muscular dystrophy (FSHD); 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. To protect this pipeline, aTyr has built an intellectual property estate 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.

ATYR PHARMA INC.

Condensed Consolidated Statements of Operations

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

	Three Months Ended June 30,		**Six Months Ended June 30,**	
	2016	**2015**	**2016**	**2015**
Operating expenses:				
Research and development	$11,307	$7,502	$23,307	$14,095
General and administrative	4,126	3,396	8,241	5,725
Total operating expenses	15,433	10,898	31,548	19,820

Loss from operations	(15,433)	(10,898)	(31,548)	(19,820)
Other income (expenses), net	50	(182)	78	(331)
Net loss	(15,383)	(11,080)	(31,470)	(20,151)
Accretion to redemption value of redeemable convertible preferred stock	—	(15)	—	(15)
Net loss attributable to common stockholders	(15,383)	(11,095)	(31,470)	(20,166)
Net loss per share attributable to common stockholders, basic and diluted	$(0.65)	$(0.74)	$(1.33)	$(2.53)
Weighted average shares outstanding, basic and diluted	23,672,527	14,901,473	23,655,366	7,955,973

ATYR PHARMA INC.

Condensed Consolidated Balance Sheets

(in thousands)

	June 30, 2016	**December 31, 2015**
	(unaudited)	
Cash, cash equivalents and available-for-sale investments	$ 96,581	$ 125,349
Other assets	2,139	2,533
Property and equipment, net	1,766	1,793
Total assets	$ 100,486	$ 129,675
Accounts payable, accrued expenses and other liabilities	$ 10,428	$ 9,483
Total commercial bank debt	3,489	5,142
Stockholders' equity	86,569	115,050
Total liabilities and stockholders' equity	$ 100,486	$ 129,675

Contact:
Mark Johnson
Sr. Director, Investor Relations
mjohnson@atyrpharma.com
858-223-1163

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

SOURCE aTyr Pharma, Inc.