

# aTyr Pharma Announces Third Quarter 2015 Operating Results

## November 10, 2015

Company Continues Expansion of Resolaris<sup>™</sup> Clinical Program and Strengthens Leadership Team with Executive Promotions and New Manufacturing Appointment

SAN DIEGO, Nov. 10, 2015 /<u>PRNewswire</u>/ -- 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 and nine months ended September 30, 2015.

## **Clinical Highlights and Upcoming Milestones:**

- The Company recently announced the continued expansion of its Resolaris clinical program in rare myopathies with an immune component (RMICs) with the initiation of a Phase 1b/2 clinical trial in patients with early onset facioscapulohumeral muscular dystrophy (FSHD), a rare and severe genetic myopathy for which there are currently no approved treatments.
- During the third quarter of 2015, the first patients were dosed in the newly initiated long-term safety extension study of Resolaris in FSHD. The open label study includes adult patients enrolled in the Company's ongoing, double-blinded, placebo-controlled, multiple ascending dose Phase 1b/2 trial of Resolaris in adult patients with FSHD. Data from the adult FSHD Phase 1b/2 study are expected in the first quarter of 2016, while the extension study is expected to continue through the third quarter of 2016.
- The Company further expanded its Resolaris clinical program in RMICs during the third quarter by initiating a Phase 1b/2 clinical trial in patients with limb girdle muscular dystrophy (LGMD) 2B and adult patients with FSHD. Both LGMD2B and FSHD are progressive, debilitating muscle diseases characterized by an immune component in the affected skeletal muscles.
- Additionally, plans to expand clinical trials of Resolaris into specific indications in interstitial lung disease, or ILD, are currently being evaluated. The Company expects to initiate a Phase 1b/2 trial in the first half of 2016 in an ILD indication.

## Additional Operational Highlights:

- In November, the Company announced the publication of research on the discovery of a new Physiocrine pathway as a potential target for the treatment of rare hereditary peripheral neuropathies. The research was co-authored by Xiang-Lei Yang, Ph.D., professor at The Scripps Research Institute and co-founder of aTyr Pharma, and was published in the October 29, 2015 issue of *Nature*.
- In October, the Company presented an overview of the therapeutic potential of Resolaris in RMICs at the International World Muscle Congress 2015 in Brighton, UK, as well as a broader overview of the therapeutic potential of Physiocrines at the 10th Annual International Symposium on Aminoacyl-tRNA Synthetases in Barcelona.
- Melissa Ashlock, M.D., has been promoted to Senior Vice President, Translational Medicine and Therapeutics, from Vice President, Translational Medicine and Therapeutics. Dr. Ashlock has been with aTyr since 2011 and previously served as the Vice President of Drug Discovery for the Cystic Fibrosis Foundation (CFF), where she was the program leader for multiple CFF-funded collaborative drug discovery programs with industry, including Kalydeco.
- Ashraf Amanullah, Ph.D., has been appointed Vice President, Manufacturing. Dr. Amanullah most recently served as Senior Director at Gilead Sciences, where he oversaw the cell line development, drug substance, analytical development, potency, stability, QC and formulation, drug product and device development functions as well as external drug substance and drug product manufacturing. Prior to joining Gilead, he was Director of Process Development at Genentech, where he headed a cross-functional department charged with process development of early stage therapeutics, late stage and post-approval processes and technology transfer, ultimately contributing to the development of Avastin and Actemra. Prior to Genentech, he held various positions at Merck & Co. Inc. over a nine-year tenure, with responsibility for process development, cGMP manufacturing, and technology transfer and commercialization of vaccines, including Gardasil and

Gardasil 9.

• John T. Blake, C.P.A., has been promoted to Vice President, Finance. Mr. Blake had served as Senior Director, Finance, and Controller of aTyr since March 2015. Prior to joining the Company, Mr. Blake served as Director, Financial Planning and Analysis, of Volcano Corporation, a global medical device company, through the acquisition by Royal Philips NV.

#### **Third Quarter Results**

Research and development expenses were \$7.7 million for the quarter ended September 30, 2015, as compared to \$4.4 million for the same period in 2014. The increase was primarily due to an additional \$2.3 million related to manufacturing costs and clinical development incurred in support of various activities for Resolaris and an \$0.8 million increase related to compensation expenses resulting from increased headcount in research and development functions, including \$0.3 million in non-cash stock-based compensation.

The Company expects its research and development expense to continue to increase with its Resolaris franchise expansion activities, including the expanding clinical development of Resolaris, the first protein therapeutic from the Resokine Pathway; advancements in the development of a second program leveraging the Resokine pathway using an iMod.Fc protein therapeutic, as well as other therapeutic modalities to harness the power of the pathway in muscle or lung disease; and continued engagement in additional research and development activities relating to the therapeutic applications of Physiocrines beyond the Resokine pathway.

General and administrative expenses were \$3.6 million and \$1.8 million for the quarters ended September 30, 2015 and 2014, respectively. The change of \$1.8 million was due primarily to a \$0.7 million increase in personnel costs resulting from increased headcount, including \$0.3 million in non-cash stock-based compensation. Another \$0.4 million was associated with increased public company costs and intellectual property-related projects.

The Company expects general and administrative expenses to increase substantially to support the continued development of its product candidates and the costs associated with operating as a public company, which include supporting regulatory and listing requirements, insurance and investor relations. These increases will also include the cost of additional personnel and fees to outside consultants, among other expenses.

#### Year-To-Date Results

Research and development expenses for the nine months ended September 30, 2015 and 2014 were \$21.8 million and \$12.4 million, respectively. The increase of \$9.4 million was due primarily to a \$5.2 million increase related to manufacturing costs and clinical development incurred in support of various activities for Resolaris; a \$2.5 million increase related to compensation expenses, including \$1.0 million of non-cash stock-based compensation expense; and a one-time \$1.4 million non-cash expense for the assignment of certain intellectual property rights.

General and administrative expenses were \$9.3 million and \$5.1 million for the nine months ended September 30, 2015 and 2014, respectively. The change of \$4.2 million was due primarily to a \$1.8 million increase in personnel costs resulting from increased headcount, including \$0.7 million in stock-based compensation; a \$1.4 million increase in public company costs and a \$0.4 million increase in intellectual property-related projects.

Net losses for the first nine months of 2015 were \$31.5 million, as compared to \$18.3 million for the same period in 2014.

As of September 30, 2015, the number of shares outstanding was 23.6 million and the Company had cash, cash equivalents and investments totaling \$136.8 million.

#### 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 first-in-class intravenous protein therapeutic for the treatment of RMICs. Resolaris is currently in a Phase 1b/2 clinical trial in adult patients with FSHD; a Phase 1b/2 trial in adult patients with LGMD2B or FSHD; and a Phase 1b/2 trial in patients with an early onset form of FSHD. An initial trial is planned in rare pulmonary diseases with an immune component (RPIC) in patients with interstitial lung disease (ILD). To protect this pipeline, aTyr built an intellectual property estate comprising 45 issued or allowed patents and over 240 pending patent applications that are solely owned or exclusively licensed by aTyr. 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**

Statements we make in this press release may include statements which are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, which 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, 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, and the timing of initiation of additional clinical trials 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, 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 the prospectus for our recent offering of common stock and our most recent Quarterly Report on Form 10-Q. 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			Nine Months Ended					
	September 30,			September 30,					
	2015		2014		2015		2014		
Operating expenses:									
Research and development	\$	7,739	\$	4,410	\$	21,834	\$	12,436	
General and administrative	3,574		1,821		9,299		5,088		
Total operating expenses	11,31:	3	6,231		31,133		17,524		
Loss from operations	(11,313) (6,231)		1)	(31,133)		(17,524)			
Other income (expenses), net	(16)		(400)		(347)		(788)		
Net loss	(11,329)		(6,631)		(31,480)		(18,312)		
Accretion to redemption value of redeemable convertible preferred stock	-		(139)		(15)		(416)		
Net loss attributable to common stockholders	\$	(11,329)	\$	(6,770)	\$	(31,495)	\$	(18,728)	
Net loss per share attributable to common stockholders, basic and diluted	\$	(0.48)	\$	(8.02)	\$	(2.38)	\$	(22.73)	
Weighted average shares outstanding, basic and diluted	23,58	23,581,001		843,817		13,221,551		824,062	

## ATYR PHARMA INC.

## **Condensed Consolidated Balance Sheets**

(in thousands)

September 30, December 31,

## (unaudited)

Cash, cash equivalents and investments	\$	136,813	\$	15,853		
Other assets	2,715		2,866	3		
Property and equipment, net	1,841		1,925	5		
Total assets	\$	141,369	\$	20,644		
Accounts payable, accrued expenses and other liabilities	\$	6,265	\$	5,759		
Current portion of commercial bank debt	3,307		3,134	1		
Convertible promissory note	_		2,000	)		
Commercial bank debt, net of current portion 2,640				5,142		
Redeemable convertible preferred stock —			95,619			
Stockholders'equity (deficit)	129,1	57	(91,0	10)		
Total liabilities and stockholders'equity (deficit)	\$	141,369	\$	20,644		

SOURCE aTyr Pharma, Inc.

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