

# resTORbio Reports Second Quarter 2018 Financial Results

August 9, 2018

Phase 2b results identify dose and patient population for pivotal program with RTB101

#### Initiation of pivotal program with RTB101 expected in the first half of 2019

BOSTON, Aug. 09, 2018 (GLOBE NEWSWIRE) -- restORbio, Inc. (NASDAQ:TORC), a clinical-stage biopharmaceutical company focused on helping people live healthier longer through the development and commercialization of novel therapeutics for the treatment of aging-related diseases, today provided a corporate update and reported financial results for the second quarter ended June 30, 2018.

"We have made great strides in advancing RTB101, our TORC1 inhibitor, for enhancing immune function and reducing the incidence of respiratory tract infections (RTIs)," said Chen Schor, President and CEO of resTORbio. "In addition to publishing the results from our Phase 2a trial in <u>Science</u> <u>Translational Medicine</u>, we announced positive topline results from our Phase 2b trial. We successfully identified the dose and patient populations for our planned pivotal program, and we expect to report additional data from this trial in the second half of this year. We look forward to working closely with the U.S. Food and Drug Administration (FDA) on our pivotal program for RTB101 in reducing the incidence of RTIs."

# **Recent Highlights and Outlook**

**Positive Topline Results from Phase 2b Trial**: In July 2018, resTORbio reported results from its exploratory dose-finding Phase 2b clinical trial examining the safety and efficacy of RTB101 in reducing the incidence of RTIs. The two-part, randomized, double-blinded, placebo-controlled trial enrolled a diverse population of 652 elderly patients at increased risk of morbidity and mortality associated with RTIs. RTB101 10 mg once daily demonstrated a statistically significant and clinically meaningful reduction in the percentage of patients with one or more laboratory-confirmed RTIs during the 16-week treatment period compared to placebo (p=0.026). Prespecified analyses of patient subgroups enrolled in the study showed that RTB101 10 mg once daily also had a profound reduction of more than 65% in the incidence of laboratory-confirmed RTIs in patients with asthma and patients 85 years and older, compared to placebo (p=0.0002 and p=0.007 respectively). All doses of RTB101 were observed to be well-tolerated. The study successfully identified the dose and patient populations for pivotal trials, and the Company plans to hold an end-of-Phase 2 meeting with the FDA in the fourth quarter of 2018.

**Analyses of Phase 2b Study to Inform Trials in Additional Aging-Related Diseases**: The Company intends to leverage learnings from its Phase 2b clinical trial, together with preclinical data, to further develop RTB101 for the treatment of additional aging-related indications where TORC1 inhibition may have therapeutic benefit. Additional data from the Phase 2b clinical trial on the incidence of urinary tract infections and a potential efficacy signal for heart failure based on echocardiograms are expected in the second half of 2018.

Initiation of Phase 2 Trial in Parkinson's Disease Expected by the First Quarter 2019 : Selective and broad inhibition of TORC1 has been shown to extend lifespan and ameliorate several aging-related diseases in preclinical studies, including neurodegenerative diseases. Neurodegenerative diseases, including Parkinson's disease, are associated with accumulation of aggregated proteins that may contribute to neuronal death. Inhibition of TORC1 with RTB101 in combination with rapalogs, such as everolimus or sirolimus, induces autophagy, the process in which a cell breaks down and recycles damaged aggregated proteins and cellular components. Therefore, induction of autophagy with RTB101 in combination with rapalogs may have potential therapeutic benefit for patients with Parkinson's disease. The Company plans to initiate a proof-of-concept trial in patients with Parkinson's disease by the first quarter 2019.

**Positive Phase 2a Results Published in Science Translational Medicine:** Data from resTORbio's Phase 2a clinical trial of RTB101 alone and in combination with everolimus were published in the journal Science Translational Medicine in July 2018 demonstrating that TORC1 inhibition improved immune function and decreased incidence of all infections, including RTIs, in people aged 65 years and older. The study was a randomized, double-blinded, placebo-controlled Phase 2a study of 264 elderly volunteers at least 65 years of age.

## **Corporate Updates**

- In June 2018, resTORbio announced the appointment of Kerry Russell, M.D., Ph.D., as Vice President of Clinical Development.
- In May 2018, resTORbio hosted a key opinion leader symposium on the potential of TORC1 inhibition to treat multiple aging-related diseases, as well as the unmet medical need for reducing the incidence of respiratory tract infections.

#### Second Quarter 2018 Financial Results

- **R&D Expenses**: R&D expenses were \$11.8 million for the three months ended June 30, 2018 compared to \$3.4 million for the three months ended June 30, 2017. The increase was primarily attributable to the Company's Phase 2b study.
- **G&A Expenses**: General and administrative expenses were \$2.3 million for the three months ended June 30, 2018 compared to \$0.6 million for the three months ended June 30, 2017. The increase was primarily attributable to an increase in headcount as well as increased operating costs as a result of the Company's transition from a private company to a public company, including legal, accounting, insurance and investor relations expenses.
- Net Loss: Net loss was \$13.6 million, or \$0.48 per share, for the three months ended June 30, 2018 compared to a net loss of \$4.1 million, or \$0.94 per share, for the three months ended June 30, 2017.

• Cash Position: Cash, cash equivalents and marketable securities were \$125.9 million as of June 30, 2018. The Company expects that its cash, cash equivalents and marketable securities as of June 30, 2018 will be sufficient to fund its operating expenses through 2020.

#### About resTORbio

resTORbio, Inc. is a clinical stage biopharmaceutical company targeting TORC1 and other biological pathways that regulate aging to develop innovative medicines with the potential to extend healthy lifespan. resTORbio's lead program is selectively targeting TORC1, an evolutionarily conserved pathway that contributes to the decline in function of multiple organ systems, including the immune, cardiovascular and central nervous systems.

#### Forward Looking Statements:

This press release contains forward-looking statements that are made pursuant to the safe harbor provisions of the federal securities laws. Investors are cautioned that statements in this press release which are not strictly historical statements, including, without limitation, express or implied statements or guidance regarding our plans to develop and commercialize RTB101 alone or in combination with everolimus, including the therapeutic potential and clinical benefits thereof, and the potential patient populations that may be addressed by our product candidates, our ongoing and future clinical trials for RTB101 alone or in combination with everolimus, including the timing of the initiation and anticipated results of these trials, the intended regulatory path for our product candidates and interactions with regulatory authorities, and our cash position and expected runway, constitute forward-looking statements identified by words like "believe," "expect," "may," "will," "should," "seek," "anticipate," or "could" and similar expressions. Such forward-looking statements are subject to a number of risks and uncertainties that could cause actual results to differ materially from those anticipated, including, without limitation, risks associated with: the delay of any planned clinical trials and/or development of RTB101, either alone or in combination with everolimus; our ability to successfully demonstrate the efficacy and safety of our lead product candidate; the clinical results for our lead product candidate which may not support further development of additional indications; and obtaining, maintaining and protecting our intellectual property; as well as those risks more fully discussed in the section entitled "Risk Factors" in the Annual Report on Form 10-K filed by resTORbio, Inc. with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent our views only as of today and should not be relied upon as representing its views as of any subsequent date. resTORbio explicitly disclaims any obligation to update any forward-looking statements.

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# RESTORBIO, INC. CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (unaudited) (in thousands, except per share data)

	Three Months	Ended June 30,	Six Months Ended June 30,	
	2018	2017	2018	2017
Operating expenses:				
Research and development	\$ 11,845	\$ 3,420	\$ 19,951	\$ 6,714
General and administrative	2,268	637	4,362	700
Total operating expenses	14,113	4,057	24,313	7,414
Loss from operations	(14,113	) (4,057	) (24,313	) (7,414 )
Other income, net	522	—	863	_
Net loss	\$ (13,591	) \$ (4,057	) \$ (23,450	) \$ (7,414 )
Net loss per share —basic and diluted	\$ (0.48	) \$ (0.94	) \$ (0.95	) \$ (2.09 )
Weighted-average number of common shares used in net loss per share —basic and diluted	28,046	4,302	24,803	3,550

#### **RESTORBIO, INC.**

## CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited)

(in thousands)

	June 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	\$ 125,923	\$ 53,349
Prepaid expenses and other current assets	2,449	876
Deferred offering costs	_	929

Total current assets Restricted cash	128,372 84		55,154 —	
Property and equipment, net	324		39	
Total assets	\$ 128,78	) :	\$ 55,193	
Liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)				
Current liabilities:				
Accounts payable	\$ 4,523	:	\$ 1,515	
Accrued liabilities	6,568		3,987	
Funding advance	500			
Total current liabilities	11,591		5,502	
Other liabilities	25			
Total liabilities	11,616		5,502	
Redeemable convertible preferred stock	_		81,620	
Stockholders' equity (deficit):				
Common stock	3		1	
Additional paid-in capital	174,420		1,849	
Accumulated deficit	(57,229	)	(33,779	)
Accumulated other comprehensive loss	(30	)	_	
Total stockholders' equity (deficit)	117,164		(31,929	)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	\$ 128,78	) :	\$ 55,193	

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