



Translate Bio Announces First Quarter 2019 Financial Results and Provides Corporate Update

May 9, 2019

-- Completed dosing in single-ascending dose portion of Phase 1/2 clinical trial in cystic fibrosis; data expected 3Q19 --

LEXINGTON, Mass., May 09, 2019 (GLOBE NEWSWIRE) -- Translate Bio (Nasdaq: TBIO), a clinical-stage messenger RNA (mRNA) therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction, today announced financial results for the first quarter ended March 31, 2019 and reviewed recent corporate achievements and updates.

"This quarter, we have continued to focus on progressing our programs for rare genetic diseases while also advancing our mRNA therapeutic (MRT) platform to fuel future pipeline programs," said Ronald Renaud, chief executive officer of Translate Bio. "We are excited to share that we have completed dosing in the single-ascending dose portion of the ongoing Phase 1/2 clinical trial for MRT5005 in patients with cystic fibrosis and anticipate reporting interim data from this trial in the third quarter of this year."

First Quarter 2019 and Recent Updates

- **Completed dosing in single-ascending dose (SAD) portion of Phase 1/2 clinical trial of MRT5005 for the treatment of cystic fibrosis(CF):** Translate Bio completed dosing of all patients in the three dose cohorts of the SAD portion of the ongoing first-in-human clinical trial of MRT5005, an mRNA therapeutic product candidate that is designed to treat all patients with CF by addressing the underlying cause of the disease, including those with limited or no cystic fibrosis transmembrane conductance regulator (CFTR) protein. The Company now expects to report interim data from this trial in the third quarter of 2019. Patient dosing continues in the ongoing multiple-ascending dose portion of this trial. Details related to this clinical trial can be found at www.clinicaltrials.gov (NCT03375047).
- **Completed \$47.5M private placement:** In May 2019, Translate Bio entered into a securities purchase agreement with several institutional accredited investors for the private placement of 5,582,940 shares of common stock at a purchase price of \$8.50 per share, yielding gross proceeds of \$47.5 million.
- **Presented preclinical data at ASGCT demonstrating successful delivery of lipid-nanoparticle encapsulated mRNA in relevant metabolic disease models:** In April 2019 at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting, Translate Bio presented preclinical results that demonstrate the potential of mRNA therapeutics as treatments for urea cycle disorders and organic acidemias using the Company's proprietary liver delivery technology. The presentation summarized findings from experiments in three mouse models of metabolic disorders including ornithine transcarbamylase (OTC) deficiency, argininosuccinate synthetase (ASS1) deficiency (citrullinemia) and methylmalonic acidemia (MMA), which are each caused by a deficiency of a single enzyme specific for each disease.
- **Strengthened leadership and board of directors with recent appointments:** In April, the Company announced the appointments of Dr. Richard Wooster as chief scientific officer and Pat Sacco as senior vice president of technical operations. The Company also announced the appointments of Dr. Robert Meyer and Dr. Robert Plenge to its board of directors. These appointments strengthen Translate Bio's leadership team and highlight the Company's commitment to innovative mRNA research and development.

Anticipated Milestones

- MRT5005 (CF): Interim data from Phase 1/2 clinical trial in the third quarter of 2019
- MRT5201 (OTC Deficiency): Complete additional preclinical studies and submit data from these studies to the FDA in fourth quarter of 2019
- Identify lead preclinical candidates for additional lung and liver disease targets

Upcoming Events

- Dr. Frank DeRosa, Translate Bio's senior vice president of Research and Development, will give a presentation entitled "Advancing the Delivery of LNP-encapsulated mRNA Therapeutics" in The State of the Art in mRNA Delivery session at the TIDES 2019 Conference on Monday, May 20, 2019 at 10:15 a.m. PT in San Diego, California.
- The Company will give a corporate presentation and host one-on-one meetings at the *Jefferies 2019 Global Healthcare Conference* taking place June 4-7 at the Grand Hyatt in New York, New York.

First Quarter 2019 Financial Results and Financial Guidance

Translate Bio ended the first quarter of 2019 with \$122.6 million in cash, cash equivalents and investments and 45,294,439 shares of common stock outstanding. In May 2019, the Company raised gross proceeds of \$47.5 million through a private placement of 5,582,940 shares of its common stock. The Company expects that its existing cash, cash equivalents and investments, together with the proceeds from the private placement, will enable it to

fund its operations into the second half of 2020.

Translate Bio reported a net loss of \$33.2 million and \$21.2 million for the three months ended March 31, 2019 and 2018, respectively.

Collaboration revenue was \$1.5 million in the three months ended March 31, 2019 which was derived from the collaboration and license agreement that the Company entered into with Sanofi Pasteur in 2018. There was no collaboration revenue in the three months ended March 31, 2018.

Operating expenses for the three months ended March 31, 2019 were \$35.7 million, compared to \$22.4 million for the same period in 2018, and were comprised of the following:

- Research and development expenses of \$17.4 million during the first quarter of 2019, compared to \$12.7 million for the same period in 2018. The increase is primarily due to an increase in costs associated with the continued advancement of the Company's CF program and MRT discovery program.
- General and administrative expenses of \$6.6 million during the first quarter of 2019, compared to \$4.8 million for the same period in 2018. The increase is primarily due to an increase in personnel-related costs.
- In the three months ended March 31, 2019 and 2018, the Company recognized non-cash operating expenses of \$11.7 million and \$4.9 million, respectively, for changes in the fair value of contingent consideration liabilities, related to future potential milestone and earnout payment obligations and, prior to the IPO, anti-dilution rights with respect to common stock issued to Shire. The increase is attributed primarily to the progress of the Company's CF and OTC deficiency programs, the time value of money due to the passage of time, as well as an adjustment in the discount rate.

About Translate Bio

Translate Bio is a clinical-stage mRNA therapeutics company developing a new class of potentially transformative medicines to treat diseases caused by protein or gene dysfunction. The Company's MRT platform is designed to develop product candidates that deliver mRNA carrying instructions to produce intracellular, transmembrane and secreted proteins for therapeutic benefit. Translate Bio believes that its MRT platform is applicable to a broad range of diseases caused by insufficient protein production or where production of proteins can modify disease, including diseases that affect the lung, liver, eye, central nervous system and lymphatic system. The Company also believes its MRT platform may be applied to various classes of treatments, such as therapeutic antibodies or vaccines in areas such as infectious disease and oncology. Translate Bio's two lead programs are being developed as treatments for cystic fibrosis (CF) and ornithine transcarbamylase (OTC) deficiency. For more information about the Company, please visit www.translate.bio or on Twitter at @TranslateBio.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include, but are not limited to, those regarding: the potential for MRT5005 to address the underlying cause of CF; Translate Bio's plans to report interim data from the Phase 1/2 clinical trial of MRT5005 in the third quarter of 2019; Translate Bio's plans to conduct additional preclinical studies for MRT5201 and its plan to submit data from these studies to the FDA in the fourth quarter of 2019; Translate Bio's plans to identify lead preclinical candidates for additional disease targets; the period in which Translate Bio expects that its existing cash, cash equivalents and investments will enable it to fund its operations; Translate Bio's beliefs regarding the broad applicability of its MRT platform; the anticipated contributions of the new members of the management team; and Translate Bio's plans, strategies and prospects for its business, including its lead development programs. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from current expectations and beliefs, including but not limited to: Translate Bio's ability to advance the development of its platform and programs under the timelines it projects, demonstrate the requisite safety and efficacy of its product candidates and replicate in clinical trials any positive findings from preclinical studies; Translate Bio's ability to obtain additional preclinical data to support its IND application for MRT5201; the risk that Translate Bio may not resolve the clinical hold on MRT5201 or any other clinical hold in the near term or at all; the content and timing of decisions made by the FDA, other regulatory authorities and investigational review boards at clinical trial sites, including decisions that may arise as a result of the clinical hold on MRT5201 and as it relates to ongoing and planned clinical trials; Translate Bio's ability to obtain, maintain and enforce necessary patent and other intellectual property protection; the availability of significant cash required to fund operations; competitive factors; general economic and market conditions and other important risk factors set forth under the caption "Risk Factors" in Translate Bio's Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2019 filed with the Securities and Exchange Commission on May 9, 2019 and in any other subsequent filings made by Translate Bio. Any forward-looking statements contained in this press release speak only as of the date hereof, and Translate Bio specifically disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

TRANSLATE BIO, INC. CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS (IN THOUSANDS) (UNAUDITED)

	Three Months Ended March 31,	
	2019	2018
Collaboration revenue	\$ 1,474	\$ —
Operating expenses:		
Research and development	17,423	12,702
General and administrative	6,554	4,779

Change in fair value of contingent consideration	11,702		4,908	
Total operating expenses	35,679		22,389	
Loss from operations	(34,205)	(22,389)
Other income (expense):				
Interest income	521		89	
Other expense	—		(12)
Total other income (expense), net	521		77	
Loss before benefit from income taxes	(33,684)	(22,312)
Benefit from income taxes	486		1,103	
Net loss	\$ (33,198)	\$ (21,209)

TRANSLATE BIO, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(IN THOUSANDS)
(UNAUDITED)

	March 31, 2019		December 31, 2018	
Assets				
Current assets:				
Cash and cash equivalents	\$ 62,431		\$ 55,199	
Short-term investments	60,154		88,904	
Prepaid expenses and other current assets	5,247		4,474	
Restricted cash	1,025		1,025	
Total current assets	128,857		149,602	
Property and equipment, net	10,771		10,245	
Right-of-use assets, net	10,769		—	
Goodwill	21,359		21,359	
Intangible assets, net	105,980		106,445	
Total assets	\$ 277,736		\$ 287,651	
Liabilities and Stockholders' Equity				
Current liabilities:				
Accounts payable	\$ 4,192		\$ 5,168	
Accrued expenses	6,452		6,547	
Current portion of deferred revenue	5,708		2,572	
Current portion of operating lease liability	411		—	
Total current liabilities	16,763		14,287	
Long-term portion of contingent consideration	115,344		103,642	
Deferred revenue, net of current portion	38,017		41,841	
Deferred tax liabilities	—		481	
Deferred rent	—		2,105	
Operating lease liability, net of current portion	12,504		—	
Total liabilities	182,628		162,356	
Stockholders' equity:				
Common stock	45		45	
Additional paid-in capital	374,113		371,257	
Accumulated deficit	(279,401)	(246,203)
Accumulated other comprehensive income	351		196	
Total stockholders' equity	95,108		125,295	
Total liabilities and stockholders' equity	\$ 277,736		\$ 287,651	

Contacts for Translate Bio

Investors

Teri Dahlman
tdahlman@translate.bio
857-242-7792

Media

Maura Gavaghan
mgavaghan@translate.bio
857-242-7789

