



Translate Bio Announces Third Quarter 2019 Financial Results and Reviews Recent Highlights

November 6, 2019

-- Advanced CF program with single-ascending dose (SAD) clinical data presentation and continued enrollment and dosing in multiple-ascending dose (MAD) portion of Phase 1/2 trial --

-- Prioritized and expanded discovery efforts on additional pulmonary disease targets --

-- Strengthened financial position to support ongoing clinical development of MRT5005 for the treatment of cystic fibrosis (CF) and additional pipeline programs into the first half of 2021 --

LEXINGTON, Mass., Nov. 06, 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 third quarter ended September 30, 2019 and provided an overview of recent corporate achievements and updates.

"In 2019, we have continued to make significant progress towards our goal of developing a new class of medicines utilizing our mRNA therapeutic platform. Building on the early positive data in our ongoing Phase 1/2 clinical trial in CF, we expanded our focus in pulmonary diseases and have also continued to build out critical operations across the Company including development of our delivery platform, growth of our manufacturing capabilities and preparations for future clinical development," said Ronald Renaud, chief executive officer of Translate Bio. "In 2020, we look forward to sharing results from the multiple-ascending dose part of the Phase 1/2 CF clinical trial and continuing to advance our pipeline of mRNA therapeutics."

Third Quarter 2019 and Recent Highlights

- **Presented early positive clinical data of MRT5005 at the North American Cystic Fibrosis Conference:** Described interim safety and tolerability results from the SAD portion of an ongoing, first-in-human Phase 1/2 clinical trial of MRT5005 in patients with CF. The MAD portion of the trial is ongoing with data expected in 2020.
- **Expanded pulmonary disease focus with program pipeline update:** Announced the prioritization of the development of pulmonary disease programs including the ongoing development of MRT5005 for the treatment of CF, as well as the evaluation of targets in additional pulmonary diseases.
- **Entered into a strategic manufacturing agreement with Albany Molecular Research, Inc. (AMRI):** Announced a multi-year strategic manufacturing agreement with AMRI, a best-in-class, global clinical development manufacturing organization, in support of the planned expansion of the Company's current Good Manufacturing Practices (cGMP) clinical mRNA manufacturing capabilities. The agreement provides Translate Bio with customized and dedicated manufacturing space for cGMP mRNA production for current and future programs.
- **Completed \$90M public offering of common stock:** Completed a public offering of 9,000,000 shares of common stock at a public offering price of \$10.00 per share, for gross proceeds of \$90.0 million.

Anticipated Milestones

- MRT5005 (CF): Report results from the additional SAD dose group and MAD portion of Phase 1/2 clinical trial in 2020.
- Validate targets to support identification of development candidates for additional pulmonary diseases including primary ciliary dyskinesia, pulmonary arterial hypertension and idiopathic pulmonary fibrosis.

Upcoming Events

- The Company will present and host one-on-one meetings at the following investor conferences:
 - *Stifel 2019 Healthcare Conference*, November 19-20, 2019, New York, NY
 - *Jefferies London Healthcare Conference*, November 20-21, 2019, London, England
 - *Evercore ISI 2nd Annual HealthCONx Conference*, December 3-5, 2019, Boston, MA
- The Company will give a presentation entitled "Advancing the development of LNP-encapsulated mRNA therapeutics for pulmonary diseases" at the 7th International mRNA Health Conference taking place November 11-12, 2019 in Berlin, Germany.

Third Quarter 2019 Financial Results and Financial Guidance

Translate Bio ended the third quarter of 2019 with \$210.2 million in cash, cash equivalents and short-term investments and 60,020,725 shares of common stock outstanding, which includes gross proceeds of \$90.0 million raised through a public offering of 9,000,000 shares of its common stock in September 2019. The Company expects that its existing cash, cash equivalents and investments will enable it to fund its operations into the first half of 2021.

Translate Bio reported a net loss of \$21.2 million and \$42.6 million for the three months ended September 30, 2019 and 2018, respectively.

Collaboration revenue was \$1.3 million and \$0.2 million in the three months ended September 30, 2019 and 2018, respectively, which was derived

from the collaboration and license agreement that the Company entered into with Sanofi Pasteur in 2018.

Operating expenses for the three months ended September 30, 2019 were \$22.9 million, compared to \$45.7 million for the same period in 2018, and were comprised of the following:

- Research and development expenses of \$17.3 million during the third quarter of 2019, compared to \$12.9 million for the same period in 2018. The increase is primarily due to an increase in costs associated with the continued advancement of the CF program and continued development of MRT and vaccine discovery programs.
- General and administrative expenses of \$6.9 million during the third quarter of 2019, compared to \$6.0 million for the same period in 2018. The increase is primarily due to an increase in personnel-related costs.
- An impairment charge of \$18.6 million representing the value of the indefinite-lived in-process research and development related to the ornithine transcarbamylase (OTC) deficiency program, the development of which the Company discontinued in the third quarter of 2019.
- Operating income of \$19.8 million for changes in the fair value of contingent consideration related to future potential milestone and earnout payment obligations. The operating income was attributed primarily to the decision to discontinue the OTC deficiency program, which resulted in the removal of \$23.2 million in contingent consideration liability related to the OTC deficiency program, largely offset by an increase in the fair value of the contingent consideration liability related to the CF program due to its continued progress and the time value of money due to passage of time.

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 is primarily focused on applying its MRT platform to treat pulmonary diseases caused by insufficient protein production or where production of proteins can modify disease. The Company also believes its technology is applicable to a broad range of diseases, including diseases that affect the liver, eye and central nervous system. Additionally, the 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 lead program is being developed as a treatment for cystic fibrosis (CF) and is in an ongoing Phase 1/2 clinical trial. 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 and benefit patients; Translate Bio's plans to report data from the additional SAD dose group and MAD part of the Phase 1/2 clinical trial of MRT5005 in 2020; Translate Bio's plans to advance its pipeline of mRNA therapeutics and validate targets for additional pulmonary diseases; 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; and Translate Bio's plans, strategies and prospects for its business, including its lead development programs. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "forward," "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; the content and timing of decisions made by the U.S. Food and Drug Administration, other regulatory authorities and investigational review boards at clinical trial sites, including decisions 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 September 30, 2019 filed with the Securities and Exchange Commission on November 6, 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 September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Collaboration revenue	\$ 1,266	\$ 238	\$ 3,914	\$ 238
Operating expenses:				
Research and development	17,295	12,933	51,343	40,854
General and administrative	6,881	5,957	21,284	16,726
Change in fair value of contingent consideration	(19,834)) 26,829	(3,243)) 39,589
Impairment of intangible asset	18,559	—	18,559	—
Total operating expenses	22,901	45,719	87,943	97,169

Loss from operations	(21,635)	(45,481)	(84,029)	(96,931)
Other income (expense):								
Interest income	428		318		1,306		499	
Other expense	(20)	(7)	(20)	(52)
Total other income (expense), net	408		311		1,286		447	
Loss before benefit from income taxes	(21,227)	(45,170)	(82,743)	(96,484)
Benefit from income taxes	—		2,524		486		5,126	
Net loss	\$ (21,227)	\$ (42,646)	\$ (82,257)	\$ (91,358)

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

	September 30, 2019	December 31, 2018		
Assets				
Current assets:				
Cash and cash equivalents	\$ 98,930	\$ 55,199		
Short-term investments	111,233	88,904		
Prepaid expenses and other current assets	6,648	4,474		
Restricted cash	950	1,025		
Total current assets	217,761	149,602		
Property and equipment, net	10,680	10,245		
Right-of-use assets, net	10,527	—		
Goodwill	21,359	21,359		
Intangible assets, net	86,695	106,445		
Other long-term assets	2,492	—		
Total assets	\$ 349,514	\$ 287,651		
Liabilities and Stockholders' Equity				
Current liabilities:				
Accounts payable	\$ 3,407	\$ 5,168		
Accrued expenses	8,767	6,547		
Current portion of deferred revenue	12,603	2,572		
Current portion of operating lease liability	489	—		
Total current liabilities	25,266	14,287		
Long-term portion of contingent consideration	100,399	103,642		
Deferred revenue, net of current portion	30,046	41,841		
Deferred tax liabilities	—	481		
Deferred rent	—	2,105		
Operating lease liability, net of current portion	12,230	—		
Total liabilities	167,941	162,356		
Stockholders' equity:				
Common stock	60	45		
Additional paid-in capital	509,294	371,257		
Accumulated deficit	(328,460)	(246,203)
Accumulated other comprehensive income	679	196		
Total stockholders' equity	181,573	125,295		
Total liabilities and stockholders' equity	\$ 349,514	\$ 287,651		

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Source: Translate Bio, Inc.