



Translate Bio Announces Third Quarter 2018 Financial Results and Reviews Recent Highlights

November 8, 2018

Continued progress with ongoing Phase 1/2 clinical trial in CF and towards IND submission for OTC Deficiency

LEXINGTON, Mass., Nov. 08, 2018 (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, 2018, and provided a summary of recent updates and upcoming milestones.

"This past quarter, we made significant progress in advancing our programs, primarily focused on continuing to enroll and dose patients in our Phase 1/2 clinical trial of MRT5005 in cystic fibrosis," said Ronald Renaud, Chief Executive Officer of Translate Bio. "We also completed the IND-enabling studies for MRT5201 and are compiling the Investigational New Drug Application which will support the anticipated Phase 1/2 clinical trial initiation in patients with OTC deficiency in the first half of 2019."

Renaud continued, "Related to our discovery efforts, we are assessing data from our work in additional disease areas with the goal of advancing additional programs and leveraging the broad potential of our mRNA therapeutic platform."

Third Quarter 2018 and Recent Updates

- **Dosed multiple patients in Phase 1/2 clinical trial of MRT5005 for the treatment of CF:** Translate Bio has continued to enroll and dose patients in a first-in-human clinical trial of MRT5005, an mRNA therapeutic product candidate that is designed to treat all patients with cystic fibrosis (CF) by addressing the underlying cause of the disease, including those with limited or no cystic fibrosis transmembrane conductance regulator (CFTR) protein. The primary endpoint of the randomized, double-blind, placebo-controlled trial will be the safety and tolerability of single and multiple escalating doses of MRT5005 administered by nebulization. The trial is being conducted in collaboration with the Cystic Fibrosis Therapeutics Development Network.
- **Completed IND-enabling studies of MRT5201 for the treatment of OTC Deficiency:** With the goal of initiating a Phase 1/2 clinical trial in the first half of 2019, Translate Bio has completed its IND-enabling studies for MRT5201, a first-in-class treatment designed to directly address the underlying cause of ornithine transcarbamylase (OTC) deficiency by providing mRNA encoding the fully functional OTC enzyme in patients with the disease. MRT5201 has been granted Orphan Drug Designation for the treatment of OTC deficiency in the US and EU.
- **Presented preclinical data supporting ongoing clinical trial at NACFC:** Translate Bio's Chief Medical Officer, Dr. Ann Barbier, presented preclinical *in vivo* data in October 2018 at the 32nd Annual North American Cystic Fibrosis Conference (NACFC) demonstrating MRT5005's ability to reach the lungs in animal studies, including in a disease model with increased mucus accumulation, resulting in functional protein expression. These data support the potential use of MRT5005 as a treatment for all patients with CF, including those with limited or no CFTR protein.
- **Closed collaboration and licensing agreement with Sanofi Pasteur to develop mRNA vaccines for infectious diseases:** In July 2018, Translate Bio announced the closing of a multi-year research and development collaboration and exclusive licensing agreement with Sanofi Pasteur to develop mRNA vaccines for up to five undisclosed infectious disease pathogens. Upon closing in July, Translate Bio received an upfront payment of \$45 million.

Anticipated Milestones

- MRT5005 (CF): Initiate multiple-ascending dose portion of the Phase 1/2 clinical trial by year end 2018
- MRT5201 (OTC Deficiency): Initiate Phase 1/2 clinical trial in the first half of 2019
- Identify lead preclinical candidates for additional lung and liver disease targets

Upcoming Events

- 6th International mRNA Conference on Tuesday, November 13, 2018 at 10:45 a.m. ET, Cambridge, MA (presentation)
- Stifel 2018 Healthcare Conference on Wednesday, November 14, 2018 at 8:00 a.m. ET, New York, NY (presentation and meetings)
- Evercore ISI Healthcare Conference on Wednesday, November 28, 2018 at 12:50 p.m. ET, Boston, MA (presentation and meetings)
- Citi Global Healthcare Conference on Wednesday, December 5, 2018, New York, NY (meetings only)

Third Quarter 2018 Financial Results and Financial Guidance

Translate Bio ended the third quarter of 2018 with \$161.1 million in cash, cash equivalents and investments. The Company expects that its existing cash, cash equivalents and investments will enable it to fund its operations into the first half of 2020.

Translate Bio reported a net loss of \$42.6 million and \$18.5 million for the three months ended September 30, 2018 and 2017, respectively.

Collaboration revenue was \$0.2 million in the three months ended September 30, 2018, which was derived from the collaboration and license agreement that the Company entered into with Sanofi Pasteur in June 2018, which became effective in July 2018. There was no collaboration revenue in the three months ended September 30, 2017.

Operating expenses for the quarter ended September 30, 2018 were \$45.7 million, compared to \$20.8 million for the same period in 2017, and were comprised of the following:

- Research and development expenses of \$12.9 million during the third quarter of 2018, compared to \$11.1 million for the same period in 2017. The increase is primarily due to increases in personnel-related costs and an increase in costs associated with the continued development of the Company's product candidates.
- General and administrative expenses of \$6.0 million during the third quarter of 2018, compared to \$3.6 million for the same period in 2017. The increase is primarily due to an increase in personnel-related costs, professional fees and insurance costs.
- In the three months ended September 30, 2018 and 2017, the Company recognized operating expenses of \$26.8 million and \$6.1 million, respectively, for changes in the fair value of the contingent consideration liabilities related to future potential milestone and earnout payment obligations and anti-dilution rights with respect to common stock issued to Shire that the Company recorded in connection with its acquisition of the MRT Program in December 2016. The \$20.7 million increase in expense was attributed primarily to an increase in the fair value of the contingent consideration liability for future earnout payments that could be due.

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, lymphatic system and circulatory system. The Company also believes its platform may be applied to produce therapeutic antibodies and 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 treat patients with CF; Translate Bio's plans to continue to enroll and dose patients in its Phase 1/2 clinical trial of MRT5005 in CF and to initiate the multiple-ascending dose part of the study by the end of 2018; Translate Bio's plans to submit an IND and initiate a Phase 1/2 clinical trial of MRT5201 in the first half of 2019; its plans to advance additional programs and leverage its mRNA therapeutic platform, including its plans to identify lead preclinical candidates for additional lung and liver disease targets; and the period in which Translate Bio expects that its existing cash, cash equivalents and investments will enable it to fund its operations. 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 enroll patients in its ongoing clinical trial; 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; 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, 2018 filed with the Securities and Exchange Commission on November 8, 2018 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,	
	2018	2017	2018	2017
Collaboration revenue	\$ 238	\$ —	\$ 238	\$ —
Operating expenses:				
Research and development	12,933	11,097	40,854	34,224
General and administrative	5,957	3,607	16,726	9,706

Change in fair value of contingent consideration	26,829	6,132	39,589	10,731
Total operating expenses	45,719	20,836	97,169	54,661
Loss from operations	(45,481)	(20,836)	(96,931)	(54,661)
Other income (expense):				
Interest income	318	72	499	230
Other income (expense), net	(7)	—	(52)	59
Total other income (expense), net	311	72	447	289
Loss before benefit from income taxes	(45,170)	(20,764)	(96,484)	(54,372)
Benefit from income taxes	2,524	2,299	5,126	4,020
Net loss	\$ (42,646)	\$ (18,465)	\$ (91,358)	\$ (50,352)

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

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 38,864	\$ 48,058
Short-term investments	122,236	9,997
Prepaid expenses and other current assets	4,194	3,014
Restricted cash	1,025	1,966
Total current assets	166,319	63,035
Property and equipment, net	10,068	6,778
Goodwill	21,359	21,359
Intangible assets, net	106,842	106,842
Deferred offering costs	—	511
Other assets	—	22
Total assets	\$ 304,588	\$ 198,547
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	\$ 3,111	\$ 4,594
Accrued expenses	5,806	5,888
Current portion of contingent consideration	—	1,296
Current portion of deferred revenue	1,042	—
Deferred rent	—	307
Total current liabilities	9,959	12,085
Long-term portion of contingent consideration	118,211	79,713
Deferred revenue, net of current portion	44,024	—
Deferred tax liabilities	913	6,039
Deferred rent, net of current portion	2,064	1,329
Total liabilities	175,171	99,166
Redeemable convertible preferred stock (Series A, B and C)	—	192,896
Stockholders' equity (deficit):		
Common stock	45	10
Additional paid-in capital	369,614	55,204
Accumulated deficit	(240,166)	(148,808)
Accumulated other comprehensive income (loss)	(76)	79
Total stockholders' equity (deficit)	129,417	(93,515)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	\$ 304,588	\$ 198,547

Contacts for Translate Bio

Investors

Teri Dahlman

tdahlman@translate.bio

857-242-7792



Source: Translate Bio, Inc.

Media

Maura Gavaghan

mgavaghan@translate.bio

857-242-7789