



Translate Bio Announces Second Quarter 2018 Financial Results and Highlights Recent Development and Corporate Achievements

August 9, 2018

- Initiated Phase 1/2 clinical trial of MRT5005 in patients with cystic fibrosis --
- Established strategic mRNA vaccines partnership with Sanofi Pasteur --
- Completed initial public offering raising approximately \$113.4 million in net proceeds --

LEXINGTON, Mass., Aug. 09, 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 second quarter ended June 30, 2018, and provided a summary of recent accomplishments and upcoming milestones.

"The first half of 2018 was transformational for Translate Bio as we have achieved several pivotal development and corporate milestones," said Ronald Renaud, Chief Executive Officer of Translate Bio. "We initiated a Phase 1/2 clinical trial of MRT5005, the first mRNA therapeutic to enter the clinic with targeted delivery to the lung, established a strategic partnership that validates the potential of our mRNA platform, continued to grow our team and expand our research and manufacturing capabilities at our new headquarters, and strengthened our balance sheet with a successful initial public offering. We believe that these critical achievements will enable us to continue to advance our mission of bringing novel mRNA therapeutics to patients in areas of high unmet need."

Second Quarter 2018 and Recent Highlights

Development Highlights

- **Dosed first patients in Phase 1/2 clinical trial of MRT5005:** In May 2018, Translate Bio initiated 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. 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](#).
- **Established collaboration with Sanofi Pasteur to develop mRNA vaccines for infectious diseases:** In June 2018, Translate Bio announced 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. Under the agreement, Translate Bio and Sanofi Pasteur will jointly conduct research and development activities to advance mRNA vaccines during an initial three-year research term. Translate Bio is eligible to receive up to \$805 million in payments, which includes an upfront payment of \$45 million, certain development, regulatory and sales-related milestones across several vaccine targets, and option exercise fees if Sanofi Pasteur exercises its option related to development of vaccines for additional pathogens.
- **Presented preclinical data supporting ongoing clinical trial at ECFS:** Translate Bio's Chief Medical Officer, Dr. Ann Barbier, presented preclinical *in vivo* data in June 2018 at the 41st Annual European Cystic Fibrosis Conference (ECFS), demonstrating successful widespread delivery of hCFTR mRNA in the lungs of CFTR knock-out mice. These data support the potential use of MRT5005 as a treatment for patients with CF, regardless of their mutational status.
- **Granted Orphan Drug Designation for MRT5201 for the treatment of ornithine transcarbamylase (OTC) deficiency in US and EU:** In March 2018, the U.S. Food and Drug Administration granted Orphan Drug Designation (ODD) to MRT5201, a first-in-class treatment designed to directly address the underlying cause of the disease by providing mRNA encoding the fully functional OTC enzyme in patients with OTC deficiency. In addition to U.S. ODD, the European Commission granted ODD for MRT5201 in June 2018.

Corporate Highlights

- **Completed initial public offering:** The Company closed an initial public offering in July 2018, raising approximately \$113.4 million in net proceeds. Translate Bio issued 9,714,371 shares of common stock, inclusive of the underwriters' over-allotment option, at an offering price of \$13.00 per share.
- **Relocated the corporate headquarters:** In March 2018, Translate Bio relocated to its new headquarters to accommodate a growing workforce, co-locate all employees and expand research and development footprint and in-house manufacturing capabilities. The new space expands the Company's manufacturing facilities, allowing for continued support of large-scale manufacturing process for its lipid nanoparticle drug products.
- **Strengthened leadership team:** In May 2018, Translate Bio appointed John R. Schroer, CFA, as Chief Financial Officer. With 28 years of healthcare investment experience, Mr. Schroer brings expertise in financial strategy and analysis and

strong industry relationships to Translate Bio.

Anticipated Milestones Over Next 12 Months

- 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

Second Quarter Financial Results and Financial Guidance

Translate Bio ended the second quarter of 2018 with \$17.5 million in cash, cash equivalents and investments. The Company expects that its existing cash, cash equivalents and investments, together with the estimated \$113.4 million of net proceeds received from the Company's IPO and the \$45.0 million upfront payment received from Sanofi Pasteur, will enable it to fund its operations into the first half of 2020.

Translate Bio reported a net loss of \$27.5 million and \$17.9 million for the three months ended June 30, 2018 and 2017, respectively.

Operating expenses for the quarter ended June 30, 2018 were \$29.1 million, compared to \$19.0 million for the same period in 2017, and were comprised of the following:

Research and development expenses were \$15.2 million during the second quarter of 2018, compared to \$13.5 million for the same period in 2017. The increase is primarily due to an increase in personnel-related costs and an increase in costs associated with the continued development of the Company's product candidates.

General and administrative expenses were \$6.0 million during the second quarter of 2018, compared to \$3.1 million for the same period in 2017. The increase is primarily due to an increase in personnel-related costs and professional fees.

In the three months ended June 30, 2018 and 2017, the Company recognized operating expenses of \$7.9 million and \$2.3 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 \$5.6 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. The Company 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. The Company'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 Translate Bio's corporate milestones in the first half of 2018 to enable it to advance its mission; potential benefits of, and plans relating to, the collaboration between Translate Bio and Sanofi Pasteur, including anticipated milestone and other payments under the collaboration; Translate Bio's estimate of the period in which it expects to have cash to fund its operations; its anticipated preclinical and clinical development milestones over the next 12 months; and statements relating to Translate Bio's research and development timing and goals, strategic plans and prospects. 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; Translate Bio's ability to maintain its collaboration with Sanofi Pasteur and risks relating to Sanofi Pasteur's ability to successfully develop vaccine-based therapeutics thereunder; the availability of significant cash required to fund operations; competitive factors; general economic and market conditions and other important risk factors set forth in Translate Bio's Form 10-Q filed with the Securities and Exchange Commission on August 9, 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 June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Operating expenses:				
Research and development	\$ 15,219	\$ 13,506	\$ 27,921	\$ 23,127

General and administrative	5,991	3,125	10,769	6,099
Change in fair value of contingent consideration	7,852	2,324	12,760	4,599
Total operating expenses	29,062	18,955	51,450	33,825
Loss from operations	(29,062) (18,955) (51,450) (33,825
Other income (expense):				
Interest income	91	79	181	158
Other income (expense), net	(32) 73	(45) 60
Total other income (expense), net	59	152	136	218
Loss before benefit from income taxes	(29,003) (18,803) (51,314) (33,607
Benefit from income taxes	1,500	870	2,602	1,721
Net loss	\$ (27,503) \$ (17,933) \$ (48,712) \$ (31,886

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

	June 30,	December 31,
	2018	2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 16,495	\$ 48,058
Short-term investments	1,000	9,997
Prepaid expenses and other current assets	4,826	3,014
Restricted cash	1,966	1,966
Total current assets	24,287	63,035
Property and equipment, net	9,868	6,778
Goodwill	21,359	21,359
In-process research and development	106,842	106,842
Deferred offering costs	3,976	511
Other assets	-	22
Total assets	\$ 166,332	\$ 198,547
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	\$ 6,219	\$ 4,594
Accrued expenses	5,758	5,888
Current portion of contingent consideration	2,387	1,296
Deferred rent	-	307
Total current liabilities	14,364	12,085
Long-term portion of contingent consideration	91,382	79,713
Deferred tax liabilities	3,437	6,039
Deferred rent, net of current portion	2,023	1,329
Total liabilities	111,206	99,166
Redeemable convertible preferred stock (Series A, B and C)	193,540	192,896
Stockholders' equity (deficit):		
Common stock	10	10
Additional paid-in capital	59,096	55,204
Accumulated deficit	(197,520) (148,808
Accumulated other comprehensive income	-	79
Total stockholders' equity (deficit)	(138,414) (93,515
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	\$ 166,332	\$ 198,547

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Source: Translate Bio, Inc.