Translate Bio Announces Fourth Quarter and Full Year 2019 Financial Results and Highlights Recent Progress

March 12, 2020

LEXINGTON, Mass., March 12, 2020 (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 and business highlights for the fourth quarter and full year ended December 31, 2019.

“2019 was a year of important progress for Translate Bio as we reported positive interim data from the single-ascending dose (SAD) portion of the Phase 1/2 clinical trial of MRT5005 in patients with cystic fibrosis (CF). We look forward to sharing data from the additional SAD dose group and the multiple-ascending dose (MAD) portion of the trial in the third quarter of this year,” said Ronald Renaud, chief executive officer of Translate Bio. “As the first inhaled mRNA therapeutic designed to deliver mRNA coding for the cystic fibrosis transmembrane conductance regulator (CFTR) protein, MRT5005 has the potential to treat all patients with CF, including those considered non-amenable to currently available CFTR modulator treatments. This first-in-human clinical trial is a critical step towards our goal of addressing that unmet need.”

Renaud continued, “On the preclinical side, we have ongoing discovery efforts focused on a next-generation CF program, and beyond CF we continue our work to identify lead product candidates in additional pulmonary diseases, including primary ciliary dyskinesia, idiopathic pulmonary fibrosis and pulmonary arterial hypertension. We are excited about the advancements we’ve made in our pipeline programs and look forward to sharing more details throughout this year.”

Development Program Progress and Updates

Cystic Fibrosis

- The Company presented data at the North American Cystic Fibrosis Conference from the SAD portion of the Phase 1/2 clinical trial of MRT5005 in 12 patients with CF. MRT5005 was generally well-tolerated at low and mid-dose levels with no serious adverse events reported at any dose level. Marked increases in percent predicted forced expiratory volume in one second (ppFEV$_1$) were observed after a single dose of MRT5005, primarily at the mid-dose level.
- The Company continues to enroll and dose patients in the ongoing Phase 1/2 clinical trial of MRT5005 in patients with CF and anticipates reporting data for the additional SAD dose group and the MAD portion of the trial in the third quarter of 2020.
- The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for MRT5005 for the treatment of CF. This designation facilitates the expedited review of drugs that are intended to treat serious or life-threatening conditions and demonstrates the potential to address unmet medical needs.
- To support expansion of the Company’s pipeline opportunities in CF, discovery activities are underway to identify a next-generation CF product candidate with efforts focused on novel lipid nanoparticles (LNPs), protein engineering approaches and manufacturing process enhancements. *In vivo* studies are ongoing to evaluate preclinical safety, protein expression and duration of expression to support product candidate selection.

Additional Pulmonary Programs

Translate Bio is leveraging its lung delivery expertise towards research in additional pulmonary diseases including idiopathic pulmonary fibrosis (IPF), primary ciliary dyskinesia (PCD) and pulmonary arterial hypertension (PAH).

- IPF is a chronic lung disorder characterized by thickening, stiffening and scarring, or fibrosis, of tissue within the lungs. There are approximately 83,000 diagnosed cases of IPF in the United States. Translate Bio’s preclinical discovery efforts in IPF are primarily focused on delivering siRNA to the lung to knock down the target protein to potentially provide clinical benefit. Preclinical studies are ongoing to demonstrate proof-of-concept to support product candidate selection.

- PCD is an autosomal recessive genetic condition in which clearance of mucus from the respiratory tract is impaired due to defects in ciliary function. Cilia are tiny, hair-like structures on the cells that line the airways. Mutations in more than 30 genes are known to cause PCD and there are approximately 16,000 diagnosed cases of PCD in the United States. Translate Bio intends to use its mRNA platform to potentially restore ciliary function in the lungs of patients with PCD. The Company is conducting preclinical studies in multiple PCD genes to demonstrate proof-of-concept and support the selection of a lead PCD program.

- PAH is a rare, progressive disorder characterized by narrowing of the small arteries of the lungs resulting in increased blood pressure through the lungs, which can damage the heart. There are approximately 53,000 diagnosed cases of PAH in the United States. In developing an mRNA product candidate for PAH, Translate Bio intends to use its MRT platform to produce a number of protein targets that could potentially slow the progression of the disease. Preclinical studies are

underway to evaluate and validate target proteins for this disease.

Sanofi Pasteur Collaboration

- Translate Bio continues to work with collaborators at Sanofi Pasteur to develop mRNA vaccines in infectious diseases and has advanced its preclinical development programs including screening, optimization and production of mRNA and LNP formulations across multiple targets. Preclinical studies are being conducted with lead candidates to support an anticipated investigational new drug (IND) filing in 2021.

Lipid Nanoparticle Delivery Discovery

- A robust effort to discover proprietary next-generation delivery technologies is ongoing. Using in-house, high-throughput LNP production, as well as working with external collaborators, Translate Bio is generating novel LNPs and optimizing delivery formulations, resulting in an extensive library of LNPs. *In vivo* studies are being conducted to identify lead next-generation LNPs to support lung, liver and additional disease program development.

Fourth Quarter and Full Year 2019 Financial Results and Financial Guidance

Translate Bio ended the fourth quarter of 2019 with $188.7 million in cash, cash equivalents and short-term investments and 60,022,067 shares of common stock outstanding. The Company expects that its existing cash, cash equivalents and short-term investments will enable it to fund its operating expenses and capital expenditure requirements into the second quarter of 2021.

Translate Bio reported a net loss of $31.0 million and $6.0 million for the three months ended December 31, 2019 and 2018, respectively, and a net loss of $113.3 million and $97.4 million for the years ended December 31, 2019 and 2018, respectively.

Collaboration revenue was $3.9 million and $1.2 million for the three months ended December 31, 2019 and 2018, respectively, and $7.8 million and $1.4 million for the years ended December 31, 2019 and 2018, respectively. The collaboration revenue was derived from the collaboration and license agreement that the Company entered into with Sanofi Pasteur in 2018. The increase was related to the advancement of the vaccine program during the year ended December 31, 2019 compared to the same period in 2018.

Operating expenses for the three months ended December 31, 2019 were $35.6 million, compared to $8.5 million for the same period in 2018, and were comprised of the following:

- Research and development expenses of $25.0 million during the fourth quarter of 2019, compared to $17.2 million for the same period in 2018. The increase is primarily due to an increase in costs associated with the continued advancement of the Phase 1/2 trial of MRT5005 for the treatment of patients with CF and continued development of the vaccine programs as well as an increase in personnel-related costs.

- General and administrative expenses of $7.3 million during the fourth quarter of 2019, compared to $5.9 million for the same period in 2018. The increase is primarily due to an increase in personnel-related costs and professional fees.

- Operating expense of $3.3 million for changes in the fair value of contingent consideration related to future potential milestone and earnout payment obligations. The operating expense was attributed to 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.

Operating expenses for the year ended December 31, 2019 were $123.6 million, compared to $105.7 million for the same period in 2018, and were comprised of the following:

- Research and development expenses of $76.4 million during the year ended December 31, 2019, compared to $58.0 million for the same period in 2018. The increase is primarily due to an increase in costs associated with the continued advancement of the Phase 1/2 trial of MRT5005 for the treatment of patients with CF and continued development of MRT discovery and vaccine programs as well as an increase in personnel-related costs.

- General and administrative expenses of $28.6 million during the year ended December 31, 2019, compared to $22.6 million for the same period in 2018. The increase is primarily due to an increase in personnel-related costs.

- Operating expense of less than $0.1 million for changes in the fair value of contingent consideration related to future potential milestone and earnout payment obligations, compared to $25.0 million for the same period in 2018. The operating expense in 2019 was attributed primarily to 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 offset by the decision to discontinue the ornithine transcarbamylase (OTC) deficiency program, which the Company discontinued in the third quarter of 2019 and which resulted in the removal of $23.2 million in contingent consideration liability related to the OTC deficiency program.

- An impairment charge of $18.6 million representing the value of the indefinite-lived in-process research and development

intangible asset related to the discontinuation of the OTC deficiency program.

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. Translate Bio is primarily focused on applying its technology to treat pulmonary diseases caused by insufficient protein production or where the reduction of proteins can modify disease. 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. The Company also believes its technology is applicable to a broad range of diseases, including diseases that affect the liver. Additionally, the platform may be applied to various classes of treatments, such as therapeutic antibodies or vaccines in areas such as infectious disease and oncology. 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 portion of the Phase 1/2 clinical trial of MRT5005 in the third quarter of 2020; Translate Bio's plans to advance its pipeline of mRNA therapeutics and validate targets for additional pulmonary diseases; Translate Bio's expectations with respect to its collaboration with Sanofi and the anticipated IND filing in 2021; the period in which Translate Bio expects that its existing cash, cash equivalents and short-term investments will enable it to fund its operating expenses and capital expenditure requirements; 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 FDA, 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2019 filed with the Securities and Exchange Commission on March 12, 2020 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.
CONSOLIDATED STATEMENTS OF OPERATIONS
(IN THOUSANDS)

	Three Months Ended December 31,		Years Ended December 31,	
	2019	**2018**	**2019**	**2018**
Collaboration revenue	$ 3,890	$ 1,182	$ 7,804	$ 1,420
Operating expenses:				
Research and development	25,025	17,170	76,369	58,024
General and administrative	7,348	5,879	28,632	22,606
Change in fair value of contingent consideration	3,256	(14,569)	13	25,020
Impairment of intangible asset	—	—	18,559	—
Total operating expenses	35,629	8,480	123,573	105,650
Loss from operations	(31,739)	(7,298)	(115,769)	(104,230)
Other income (expense):				
Interest income	703	825	2,010	1,323
Other expense	—	(2)	(20)	(53)
Total other income (expense), net	703	823	1,990	1,270
Loss before benefit from income taxes	(31,036)	(6,475)	(113,779)	(102,960)
Benefit from income taxes	—	438	486	5,565
Net loss	$ (31,036)	$ (6,037)	$ (113,293)	$ (97,395)

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

	December 31, 2019	December 31, 2018

Assets		
Current assets:		
Cash and cash equivalents	$ 84,580	$ 55,199
Short-term investments	104,098	88,904
Short-term collaboration receivables	4,596	833
Prepaid expenses and other current assets	9,391	3,641
Restricted cash	950	1,025
Total current assets	203,615	149,602
Property and equipment, net	12,539	10,245
Right-of-use assets, net	10,400	—
Goodwill	21,359	21,359
Intangible assets, net	85,536	106,445
Other assets	2,752	—
Total assets	$ 336,201	$ 287,651
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 15,968	$ 5,168
Accrued expenses	7,072	6,547
Current portion of deferred revenue	18,100	2,572
Current portion of operating lease liability	530	—
Total current liabilities	41,670	14,287
Contingent consideration	103,655	103,642
Deferred revenue, net of current portion	25,256	41,841
Deferred tax liabilities	—	481
Deferred rent	—	2,105
Operating lease liability, net of current portion	12,084	—
Total liabilities	182,665	162,356
Stockholders' equity:		
Common stock	60	45
Additional paid-in capital	512,231	371,257
Accumulated deficit	(359,496)	(246,203)
Accumulated other comprehensive income	741	196
Total stockholders' equity	153,536	125,295
Total liabilities and stockholders' equity	$ 336,201	$ 287,651

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