



Translate Bio Announces Third Quarter 2020 Financial Results and Reviews Recent Progress

November 5, 2020

-- Advances Phase 1/2 clinical trial of MRT5005 for the treatment of cystic fibrosis (CF) with resumption of COVID-impacted enrollment and dosing --

-- Presented promising preclinical data at NACFC from next-generation CF program that leverages advances in mRNA technology --

-- Reported positive preclinical results from COVID-19 mRNA vaccine program with collaborator Sanofi Pasteur; Phase 1/2 clinical trial now anticipated to begin in first quarter 2021 --

LEXINGTON, Mass., Nov. 05, 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 for the third quarter ended September 30, 2020 and reviewed recent corporate achievements and updates.

"We continue to leverage our mRNA and lung delivery capabilities to advance our technology with a focus on addressing unmet needs in CF and other pulmonary diseases. With the resumption of enrollment and dosing in the Phase 1/2 clinical trial of MRT5005, and promising preclinical data from our next-generation CF program, we continue to demonstrate our commitment to developing a treatment for all patients with CF, particularly those non-amenable to modulator treatments," said Ronald Renaud, chief executive officer of Translate Bio.

Mr. Renaud continued, "We have also made important progress in our mRNA infectious disease vaccine programs under our collaboration with Sanofi Pasteur. We were pleased to share initial positive preclinical data from the COVID-19 vaccine program and are working to advance the lead mRNA vaccine candidate, MRT5500, into the clinic."

Third Quarter 2020 and Recent Updates

- **Expanding research and development footprint:** In November 2020, the Company entered into a lease agreement for new office and laboratory space in order to expand its research and development footprint and build upon internal capabilities with a continued focus on advancing platform innovation and driving multiple programs towards clinical development.
- **Reported positive preclinical data for MRT5500, lead COVID-19 mRNA vaccine candidate, now anticipated to begin Phase 1/2 clinical trial in first quarter 2021:** In October 2020, Translate Bio and Sanofi Pasteur announced preclinical results for MRT5500, an mRNA-based vaccine candidate against SARS-CoV-2, the virus that causes COVID-19. In preclinical studies, MRT5500 induced potent neutralizing antibodies against SARS-CoV-2 at levels significantly higher than those observed in a panel of samples from COVID-19 patients. These data supported the selection of MRT5500 for clinical development. A Phase 1/2 clinical trial was anticipated to begin in the fourth quarter of 2020, and is now expected to begin in the first quarter of 2021 due to a delay in the manufacturing of clinical trial material.
- **Presented data from mRNA clinical and preclinical product candidates for treatment of CF at NACFC:** In October 2020, Translate Bio gave oral and poster presentations at the 34th Annual North American Cystic Fibrosis Conference (NACFC) which underscored the remaining unmet need in the treatment of CF, reviewed encouraging clinical data shared to date for the Company's lead program, MRT5005, and described preclinical data for a next-generation CF program which applies advances in mRNA science, lipid nanoparticle (LNP) chemistry, and investigation of the cystic fibrosis transmembrane conductance regulator (CFTR) biology. Preclinical data presented from the next-generation CF program demonstrated that the design of novel mRNA sequences encoding wild-type and rationally engineered CFTR protein produced active ion channels and enhanced protein expression when compared to reference experiments *in vitro*. Preclinical studies are ongoing to select the optimal mRNA sequence and LNP for the Company's next-generation CFTR product candidate.
- **Resumed dosing and enrollment in Phase 1/2 clinical trial for MRT5005 for the treatment of CF:** In September 2020, Translate Bio announced that enrollment and dosing in its Phase 1/2 clinical trial for MRT5005 in CF resumed, with multiple clinical sites open for enrollment. In March 2020, the Company had announced a pause to enrollment and dosing in the clinical trial as a consequence of the COVID-19 pandemic. The Company plans to provide updated timing on the expected interim data readout of the additional single-ascending dose (SAD) group and the multiple-ascending dose (MAD) portion of the clinical trial at a later date.

Anticipated Milestones

- MRT5500 (COVID-19 vaccine): Initiate Phase 1/2 clinical trial in Q1 2021 (Sanofi Pasteur collaboration)
- Flu vaccine: Advance lead LNP/mRNA formulation to clinical proof of technology trial anticipated to begin mid-year 2021 (Sanofi Pasteur collaboration)

- MRT5005 (CF): Report results from additional SAD dose group and MAD portion of the ongoing Phase 1/2 clinical trial
- Preclinical pulmonary programs: Advance next-generation CF, primary ciliary dyskinesia, idiopathic pulmonary fibrosis and pulmonary arterial hypertension programs toward selection of development candidates
- Platform: Identify next-generation LNPs to support liver, lung and additional disease program development

Upcoming Events

- The Company will give presentations on its infectious disease vaccine and therapeutic pulmonary programs at the 8th International mRNA Health Conference taking place virtually November 9-10, 2020.
- The Company will participate in the following virtual investor conferences:
 - Jefferies Virtual London Healthcare Conference, November 17-19, 2020
 - Evercore ISI HealthCONx Conference, December 1-3, 2020

Third Quarter 2020 Financial Results and Financial Guidance

Translate Bio ended the third quarter of 2020 with \$674.1 million in cash and cash equivalents and 74,251,559 shares of common stock outstanding. The Company expects that its existing cash and cash equivalents will be sufficient to fund its operating expenses and capital expenditure requirements through 2023.

Translate Bio reported a net income of \$17.3 million and a net loss of \$21.2 million for the three months ended September 30, 2020 and 2019, respectively.

Collaboration revenue was \$66.4 million and \$1.3 million for the three months ended September 30, 2020 and 2019, respectively, which was derived from the collaboration with Sanofi. The increase was primarily related to increased activities for the vaccine program as well as a cumulative catch-up of revenue resulting from entering into the expanded Sanofi agreement and the related increase in the transaction price.

Operating expenses for the three months ended September 30, 2020 were \$49.7 million, compared to \$22.9 million for the same period in 2019, and were comprised of the following:

- Research and development expenses of \$26.3 million during the third quarter of 2020, compared to \$17.3 million for the same period in 2019. The increase is primarily due to continued development of the Company's vaccine program associate with the Sanofi collaboration and discovery program as well as an increase in personnel-related costs, partially offset by a decrease in expenses related to the Company's MRT5005 and MRT5201 programs.
- General and administrative expenses of \$9.2 million during the third quarter of 2020, compared to \$6.9 million for the same period in 2019. The increase is primarily due to costs related to entering into the expanded Sanofi agreement.
- Operating expenses of \$14.2 million for change in the fair value of contingent consideration related to future potential milestone and earnout payment obligations. The expense recognized was primarily attributed to an increase in the fair value of the contingent consideration liability due to the time value of money due to the passage of time and a decrease 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, or to prevent infectious diseases by generating protective immunity. 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 pulmonary candidate is being evaluated as an inhaled treatment for cystic fibrosis (CF) in a Phase 1/2 clinical trial. Additional pulmonary diseases are being evaluated in discovery-stage research programs that utilize a proprietary lung delivery platform. Translate Bio believes that mRNA can be delivered to target tissues via multiple routes of administration and, consequently, its technology may apply broadly to a wide range of diseases, including diseases that affect the liver. Translate Bio is also pursuing the development of mRNA vaccines for infectious diseases under a collaboration with Sanofi Pasteur.

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 provide updated timing on the expected interim data of the Phase 1/2 clinical trial of MRT5005; Translate Bio's expectations with respect to its collaboration with Sanofi, including the anticipated initiation of a Phase 1/2 trial for MRT5500 in the first quarter of 2021 and the advancement of lead LNP/mRNA formulation for the flu vaccine to a clinical proof of technology trial to begin mid-year 2021; Translate Bio's plans to advance its pipeline of mRNA therapeutics and validate targets for additional pulmonary diseases; Translate Bio's plans to advance its additional disease program programs and platform; Translate Bio's plans to expand its research and development footprint and build upon internal capabilities; the period in which Translate Bio expects that its existing cash and cash equivalents 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," "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: the risk of adverse current and potential future impacts of the COVID-19 pandemic on the Company's business, financial condition, operations and

liquidity; the risk that Translate Bio is not able 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; whether or not Translate Bio is able to successfully advance the collaboration agreement with Sanofi; uncertainties relating to the successful discovery and development of vaccine candidates based on mRNA, and specifically as it relates to the novel coronavirus, COVID-19; 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; uncertainties related to Translate Bio's ability to obtain, maintain and enforce necessary patent and other intellectual property protection; whether or not the significant cash required to fund operations will be available; competitive factors; uncertainties and risks related to 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, 2020 filed with the Securities and Exchange Commission on November 5, 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)
(UNAUDITED)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Collaboration revenue	\$ 66,446	\$ 1,266	\$ 87,420	\$ 3,914
Operating expenses:				
Research and development	26,344	17,295	76,785	51,343
General and administrative	9,163	6,881	25,223	21,284
Change in fair value of contingent consideration	14,190	(19,834)	20,085	(3,243)
Impairment of intangible asset	—	18,559	—	18,559
Total operating expenses	49,697	22,901	122,093	87,943
Income (loss) from operations	16,749	(21,635)	(34,673)	(84,029)
Other income, net	595	408	1,447	1,286
Income (loss) before benefit from income taxes	17,344	(21,227)	(33,226)	(82,743)
Benefit from income taxes	—	—	—	486
Net income (loss)	\$ 17,344	\$ (21,227)	\$ (33,226)	\$ (82,257)

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

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	\$ 674,050	\$ 84,580
Short-term investments	—	104,098
Collaboration receivables	25,139	4,596
Prepaid expenses and other current assets	10,822	9,391
Restricted cash	950	950
Total current assets	710,961	203,615
Property and equipment, net	15,044	12,539
Right-of-use assets, net	75,650	10,400
Goodwill	21,359	21,359
Intangible assets, net	81,280	85,536
Other assets	4,334	2,752
Total assets	\$ 908,628	\$ 336,201
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 6,815	\$ 15,968
Accrued expenses	11,766	7,072
Current portion of deferred revenue	93,164	18,100
Current portion of operating lease liability	12,279	530

Total current liabilities	124,024	41,670	
Contingent consideration	123,740	103,655	
Deferred revenue, net of current portion	242,047	25,256	
Operating lease liability, net of current portion	53,151	12,084	
Total liabilities	542,962	182,665	
Stockholders' equity:			
Common stock	74	60	
Additional paid-in capital	758,314	512,231	
Accumulated deficit	(392,722)	(359,496))
Accumulated other comprehensive income	—	741)
Total stockholders' equity	365,666	153,536	
Total liabilities and stockholders' equity	\$ 908,628	\$ 336,201	

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Source: Translate Bio, Inc.