# Translate Bio Announces Second Quarter 2019 Financial Results and Provides Corporate Update

July 31, 2019

*-- Announced interim results from single-ascending dose portion of Phase 1/2 clinical trial in cystic fibrosis (CF) --*

*-- Received FDA clearance to advance 2nd program into clinical development, MRT5201 for ornithine transcarbamylase (OTC) deficiency –*

*-- Conference call to discuss interim Phase 1/2 clinical trial results for MRT5005 at 8:00 am ET today --*

LEXINGTON, Mass., July 31, 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 second quarter ended June 30, 2019 and reviewed recent corporate achievements and updates.

“We ended the second quarter with two clinical-stage programs in development for rare, genetic diseases, demonstrating the potential of our mRNA therapeutic platform and moving us closer to reaching patients with few or no treatment options today,” said Ronald Renaud, chief executive officer of Translate Bio. “We’re also excited to share our first clinical data in cystic fibrosis, and view this as a crucial step in developing a potential treatment for CF patients who do not benefit from currently available therapies. In addition, we look forward to beginning our single-ascending dose Phase 1/2 clinical trial in OTC deficiency later this year as well.”

**Second Quarter 2019 and Recent Updates**

- **Announced interim results from single-ascending dose (SAD) portion of Phase 1/2 clinical trial of MRT5005 in patients with CF:** The Company announced interim results from a first-in-human Phase 1/2 clinical trial evaluating single- and multiple-ascending doses of MRT5005 in patients with CF. MRT5005 is designed to address the underlying cause of CF regardless of genetic mutation by delivering mRNA encoding fully functional cystic fibrosis transmembrane conductance regulator (CFTR) protein to cells in the lung through nebulization. Today’s results, the first evaluation of an inhaled mRNA therapeutic, summarize the SAD portion of the clinical trial in 12 patients through one-month follow-up post treatment. The multiple-ascending dose (MAD) portion of the trial is currently ongoing with results expected in 2020.

- **Received U.S. Food and Drug Administration (FDA) clearance to proceed with a SAD Phase 1/2 clinical trial for OTC deficiency:** In June 2019, Translate Bio announced that it received clearance from the FDA to proceed with a SAD Phase 1/2 clinical trial of MRT5201 in adult patients with OTC deficiency, for which the Company plans to initiate patient screening in the second half of 2019. MRT5201 is a first-in-class treatment designed to directly address the underlying cause of OTC deficiency by providing mRNA encoding the fully functional OTC enzyme in patients with the disease. The Company is currently conducting additional preclinical studies that are required to support future clinical development of MRT5201, including a MAD clinical trial, and plans to submit data from these preclinical studies to the FDA in the fourth quarter of 2019.

- **Appointed Dr. George Demetri to the Board of Directors:** In July 2019, the Company announced the appointment of George D. Demetri, M.D., a physician-scientist at the Dana-Farber Cancer Institute and a professor of medicine at Harvard Medical School, to its Board of Directors. This appointment adds significant clinical development expertise as Translate Bio advances its clinical programs in CF and OTC deficiency.

**Anticipated Milestones**

- MRT5005 (CF): Report results from additional SAD dose group and MAD portion of Phase 1/2 clinical trial in 2020
- MRT5201 (OTC Deficiency): Initiate patient screening in SAD Phase 1/2 clinical trial in the second half of 2019; Complete additional preclinical studies and submit data from these studies to the FDA in fourth quarter of 2019
- Identify lead preclinical candidates for additional lung and liver disease targets

**Upcoming Events**

- The Company will present and host one-on-one meetings at the following conferences:
  - *SVB Leerink Spotlight Series: Rare & Genetic Diseases,* August 7-8, 2019, Boston, MA
  - *Citi’s 14 th Annual Biotech Conference*, September 4-5, 2019, Boston, MA
  - *Chardan’s 3 rd Annual Genetic Medicines Conference*, October 7-8, 2019 NY, NY
- The Company will present additional details from the Phase 1/2 clinical trial interim data set of MRT5005 at the *North American Cystic Fibrosis Conference* taking place October 31 – November 2, 2019 in Nashville, TN

**Second Quarter 2019 Financial Results and Financial Guidance**

Translate Bio ended the second quarter of 2019 with $146.9 million in cash, cash equivalents and short-term investments and 51,010,368 shares of common stock outstanding. In May 2019, the Company raised gross proceeds of $47.5 million through a private placement of 5,582,940 shares of its common stock. The Company expects that its existing cash, cash equivalents and investments will enable it to fund its operations into the second half of 2020.

Translate Bio reported a net loss of $27.8 million and $27.5 million for the three months ended June 30, 2019 and 2018, respectively.

Collaboration revenue was $1.2 million in the three months ended June 30, 2019 which was derived from the collaboration and license agreement that the Company entered into with Sanofi Pasteur in 2018. There was no collaboration revenue in the three months ended June 30, 2018.

Operating expenses for the three months ended June 30, 2019 were $29.4 million, compared to $29.1 million for the same period in 2018, and were comprised of the following:

- Research and development expenses of $16.6 million during the second quarter of 2019, compared to $15.2 million for the same period in 2018. The increase is primarily due to an increase in costs associated with the continued advancement of the Company's CF program and MRT discovery program.

- General and administrative expenses of $7.9 million during the second 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.

- In the three months ended June 30, 2019 and 2018, the Company recognized non-cash operating expenses of $4.9 million and $7.9 million, respectively, for changes in the fair value of contingent consideration liabilities, related to future potential milestone and earnout payment obligations and, prior to the Company's IPO, anti-dilution rights with respect to common stock issued to Shire. The expense recognized in the three months ended June 30, 2019 is attributed primarily to the progress of the Company's CF and OTC deficiency programs and the time value of money due to the passage of time.

**Conference Call Information**
Translate Bio will host a conference call and webcast today at 8:00 AM ET to discuss the interim results from the single-ascending dose portion of Phase 1/2 clinical trial of MRT5005 in patients with CF. The live webcast can be accessed on the investor page of Translate Bio's website at https://investors.translate.bio/investors/news-and-events. The conference call can be accessed by dialing (877) 377-8524 (toll-free domestic) or (629) 228-0742 (international) and using the conference ID 2659949. A replay of the webcast will be available on Translate Bio's website approximately two hours after the completion of the event and will be archived for up to 30 days.

**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 and central nervous system. The Company also believes its 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 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 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 2020; Translate Bio's plans to initiate patient screening for the SAD Phase 1/2 clinical trial for MRT 5201 in the second half of 2019; Translate Bio's plans to conduct additional preclinical studies for MRT5201 and its plan to submit data from these studies to the FDA in the fourth quarter of 2019; Translate Bio's plans to identify lead preclinical candidates for additional lung and liver disease targets; 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; the anticipated contributions of the new director; 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: 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 obtain additional preclinical data to support further clinical development of MRT5201; 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 Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2019 filed with the Securities and Exchange Commission on May 9, 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 June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | **2019** | **2018** | **2019** | **2018** |
| Collaboration revenue | $ 1,174 | $ — | $ 2,648 | $ — |
| Operating expenses: | | | | |
| Research and development | 16,625 | 15,219 | 34,048 | 27,921 |
| General and administrative | 7,850 | 5,991 | 14,403 | 10,769 |
| Change in fair value of contingent consideration | 4,889 | 7,852 | 16,591 | 12,760 |
| Total operating expenses | 29,364 | 29,062 | 65,042 | 51,450 |
| Loss from operations | (28,190) | (29,062) | (62,394) | (51,450) |
| Other income (expense): | | | | |
| Interest income | 358 | 91 | 878 | 181 |
| Other expense | — | (32) | — | (45) |
| Total other income (expense), net | 358 | 59 | 878 | 136 |
| Loss before benefit from income taxes | (27,832) | (29,003) | (61,516) | (51,314) |
| Benefit from income taxes | — | 1,500 | 486 | 2,602 |
| Net loss | $ (27,832) | $ (27,503) | $ (61,030) | $ (48,712) |

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

| | **June 30, 2019** | **December 31, 2018** |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 74,904 | $ 55,199 |
| Short-term investments | 71,961 | 88,904 |
| Prepaid expenses and other current assets | 6,069 | 4,474 |
| Restricted cash | 950 | 1,025 |
| Total current assets | 153,884 | 149,602 |
| Property and equipment, net | 10,903 | 10,245 |
| Right-of-use assets, net | 10,650 | — |
| Goodwill | 21,359 | 21,359 |
| Intangible assets, net | 105,630 | 106,445 |
| Deferred offering costs | 123 | — |
| Total assets | $ 302,549 | $ 287,651 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 3,347 | $ 5,168 |
| Accrued expenses | 7,246 | 6,547 |
| Current portion of deferred revenue | 9,015 | 2,572 |
| Current portion of operating lease liability | 449 | — |
| Total current liabilities | 20,057 | 14,287 |
| Long-term portion of contingent consideration | 120,233 | 103,642 |
| Deferred revenue, net of current portion | 34,192 | 41,841 |
| Deferred tax liabilities | — | 481 |
| Deferred rent | — | 2,105 |
| Operating lease liability, net of current portion | 12,370 | — |
| Total liabilities | 186,852 | 162,356 |
| Stockholders' equity: | | |
| Common stock | 51 | 45 |
| Additional paid-in capital | 422,309 | 371,257 |
| Accumulated deficit | (307,233) | (246,203) |
| Accumulated other comprehensive income | 570 | 196 |

| | | |
|---|---|---|
| Total stockholders' equity | 115,697 | 125,295 |
| Total liabilities and stockholders' equity | $ 302,549 | $ 287,651 |

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