# Intellia Therapeutics Announces First Quarter 2020 Financial Results

May 7, 2020

- *On track to submit an IND or IND-equivalent for NTLA-2001 for the treatment of transthyretin amyloidosis in mid-2020; plans to dose first patient in 2H 2020*
- *On track to submit an IND or IND-equivalent for lead TCR-T cell therapy, NTLA-5001 for the treatment of acute myeloid leukemia, in 1H 2021*
- *Nominated NTLA-2002 as development candidate for the treatment of hereditary angioedema; plans to submit an IND or IND-equivalent in 2H 2021*
- *IND accepted for CRISPR/Cas9-based sickle cell disease therapeutic candidate developed under collaboration with Intellia*
- *Ended quarter with cash position of $250 million; cash runway through the end of 2021*

CAMBRIDGE, Mass., May 07, 2020 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, today reported operational highlights and financial results for the first quarter ended March 31, 2020.

"At Intellia, our mission is to develop curative, CRISPR/Cas9-based treatments for severe diseases. I am grateful to our team for remaining steadfast in our commitment to patients and putting us in the position to deliver on our goals despite the challenging circumstances presented by the global COVID-19 pandemic," said Intellia President and Chief Executive Officer John Leonard, M.D. "We look forward to advancing NTLA-2001 into clinical testing and dosing ATTR patients with the first systemically delivered CRISPR/Cas9-based therapy in the second half of 2020. Additionally, we continue to advance our full-spectrum pipeline, including IND-enabling activities for NTLA-5001, our engineered TCR-T cell therapy candidate for AML, and the nomination of NTLA-2002 as our development candidate for the treatment of HAE."

**First Quarter 2020 and Recent Operational Highlights**

- **ATTR Program:** Intellia's lead candidate, NTLA-2001, is designed to apply its *in vivo* liver knockout approach for the treatment of transthyretin amyloidosis (ATTR) in a single course of treatment. The Company has manufactured clinical-scale materials for a Phase 1 study of NTLA-2001 and remains on track to submit an Investigational New Drug application (IND) or IND-equivalent for this program in mid-2020. NTLA-2001 is anticipated to be the first systemically delivered CRISPR/Cas9 therapy to enter the clinic. Subject to the impact of COVID-19, the Company plans to dose the first patient in the second half of 2020. NTLA-2001 is part of a co-development/co-promotion (Co/Co) agreement between Intellia, which is the lead development and commercialization party, and Regeneron Pharmaceuticals, Inc. (Regeneron).
- **AML Program:** NTLA-5001 for the treatment of acute myeloid leukemia (AML) is Intellia's first T cell receptor (TCR)-T cell development candidate, which targets the Wilms' Tumor 1 (WT1) antigen. The Company's approach aims to develop a broadly applicable treatment for AML patients, regardless of mutational subtypes of a patient's leukemia. As presented at Keystone Symposia's Engineering the Genome Conference in February 2020, Intellia demonstrated that its CRISPR/Cas9-enabled approach results in an engineered T cell product capable of specific and potent killing of primary AML blasts. Intellia is continuing to advance IND-enabling activities, including process development to support clinical T cell manufacturing. The Company is on track to submit an IND or IND-equivalent for NTLA-5001 in the first half of 2021. Additional efforts are underway to evaluate the potential use of the WT1-targeted TCR construct to treat solid tumors.
- **HAE Program:** Intellia has nominated its third development candidate, NTLA-2002 for the treatment of hereditary angioedema (HAE). Building on the modular lipid nanoparticle (LNP) delivery system developed for the ATTR program, NTLA-2002 is designed to knock out the *prekallikrein B1 (KLKB1)* gene in the liver after a single course of treatment. As part of an ongoing durability study of its lead LNP formulation in support of NTLA-2002, Intellia has now demonstrated six months of sustained therapeutically relevant reduction of serum kallikrein levels and activity following a single dose in non-human primates. Intellia expects to submit an IND or IND-equivalent for NTLA-2002 in the second half of 2021. NTLA-2002 is subject to an option by Regeneron to enter into a Co/Co agreement, which must be exercised within a limited time period after development candidate selection. Intellia would be the lead party if the option is exercised.
- **Modular Platform:** Intellia continues to advance its differentiated genome editing and delivery strategies, including targeted insertion and consecutive editing, across its *in vivo* and *ex vivo* efforts. These platform capabilities enable the removal and/or restoration of a gene's function in developing treatments for life-threatening diseases. The *in vivo* research builds on data previously shared by the Company demonstrating the first CRISPR-mediated, targeted transgene insertion in the liver of non-human primates, which generated circulating human FIX protein at or above normal levels. At the upcoming American Society of Gene and Cell Therapy (ASGCT) Annual Meeting, taking place virtually from May 12 – 15,

2020, the Company will present preclinical data related to certain programs and *ex vivo* platform capabilities. This will include an oral presentation featuring Intellia's enhanced CRISPR/Cas9-based T cell engineering platform capable of highly efficient editing of multiple genes with levels of translocations indistinguishable from background levels, and favorable cell product attributes including high viability and expansion potential. This improved T cell engineering process has been incorporated into the NTLA-5001 program.

- **Novartis Collaboration:** As announced in March 2020, the U.S. Food and Drug Administration (FDA) has accepted the IND application submitted by Novartis Institutes for BioMedical Research (Novartis), for a CRISPR/Cas9-based engineered cell therapy for the treatment of sickle cell disease (SCD) developed under collaboration with Intellia. The Phase 1/2 clinical trial will begin investigating OTQ923 in adult patients with severe complications of SCD. Novartis' IND application triggered a milestone payment to Intellia, and the Company is eligible to receive additional downstream success-based milestones and royalties.

**Upcoming Events**

The Company will participate in the following events during the second quarter of 2020:

- ASGCT 23rd Annual Meeting, May 12 – 15, Virtual
- Jefferies Global Healthcare Conference, June 3, Virtual

**Upcoming Milestones**

The Company has set forth the following for pipeline progression:

- ATTR:
  - Submit an IND or IND-equivalent for NTLA-2001 in mid-2020
  - Subject to the impact of COVID-19, plan to dose first patient in 2H 2020
- AML:
  - Submit an IND or IND-equivalent for NTLA-5001 in 1H 2021
- HAE:
  - Submit an IND or IND-equivalent for NTLA-2002 in 2H 2021
- Modular Platform Advancements:
  - Present preclinical data at upcoming ASGCT Annual Meeting

**First Quarter 2020 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities were $250.3 million as of March 31, 2020, compared to $284.5 million as of December 31, 2019. The decrease was driven by cash used to fund operations of approximately $40.1 million, which was offset in part by $4.5 million of net equity proceeds raised from the Company's "At the Market" (ATM) agreement, $1.0 million of funding received under the Novartis collaboration, and $0.3 million in proceeds from employee-based stock plans.
- **Collaboration Revenue:** Collaboration revenue increased by $2.5 million to $12.9 million during the first quarter of 2020, compared to $10.4 million during the first quarter of 2019. The increase in collaboration revenue in 2020 was mainly driven by the $5.0 million milestone payment earned from Novartis for the IND submission of OTQ923.
- **R&D Expenses:** Research and development expenses increased by approximately $10.9 million to $34.7 million during the first quarter of 2020, compared to $23.7 million during the first quarter of 2019. This increase was primarily driven by the advancement of our lead programs, research personnel growth to support these programs, as well as the expansion of the development organization.
- **G&A Expenses:** General and administrative expenses increased by $0.8 million to $11.3 million during the first quarter of 2020, compared to $10.5 million during the first quarter of 2019. This increase was primarily driven by an increase in intellectual property related expenses due to increased patent-related activity.
- **Net Loss:** The Company's net loss was $31.8 million for the first quarter of 2020, compared to $21.9 million during the first quarter of 2019.

**Financial Guidance**

Intellia expects that its cash, cash equivalents and marketable securities as of March 31, 2020 will enable the Company to fund its anticipated operating expenses and capital expenditure requirements at least through the end of 2021. This expectation excludes any strategic use of capital not currently in the Company's base-case planning assumptions.

**Conference Call to Discuss First Quarter 2020 Earnings**

The Company will discuss these results on a conference call today, May 7, 2020, at 8 a.m. ET.

To join the call:

- U.S. callers should dial 1-877-317-6789 and use conference ID# 10141541, approximately five minutes before the call.

- International callers should dial 1-412-317-6789 and use conference ID# 10141541, approximately five minutes before the call.

A replay of the call will be available through the Events and Presentations page of the Investors & Media section on Intellia's website at www.intelliatx.com, beginning on May 7, 2020 at 12 p.m. ET.

**About Intellia Therapeutics**
Intellia Therapeutics is a leading genome editing company focused on developing proprietary, curative therapeutics using the CRISPR/Cas9 system. Intellia believes the CRISPR/Cas9 technology has the potential to transform medicine by permanently editing disease-associated genes in the human body with a single treatment course, and through improved cell therapies that can treat cancer and immunological diseases, or can replace patients' diseased cells. The combination of deep scientific, technical and clinical development experience, along with its leading intellectual property portfolio, puts Intellia in a unique position to unlock broad therapeutic applications of the CRISPR/Cas9 technology and create a new class of therapeutic products. Learn more about Intellia Therapeutics and CRISPR/Cas9 at intelliatx.com and follow us on Twitter @intelliatweets.

**Forward-Looking Statements**
This press release contains "forward-looking statements" of Intellia Therapeutics, Inc. ("Intellia" or the "Company") within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements include, but are not limited to, express or implied statements regarding Intellia's beliefs and expectations regarding its: planned submission of an investigational new drug ("IND") or equivalent regulatory filing for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR") in mid-2020 and its planned dosing of first patients in the second half of 2020; plans to complete manufacturing activities and submit an IND application or equivalent regulatory filing for NTLA-5001, its first T cell receptor ("TCR")-directed engineered cell therapy development candidate for its acute myeloid leukemia ("AML") program in the first half of 2021; plans to submit an IND application or equivalent regulatory filing for NTLA-2002, a development candidate for its hereditary angioedema ("HAE") program in the second half of 2021; plans to advance and complete preclinical studies, including non-human primate studies for its ATTR program, AML program, HAE program and other in vivo and ex vivo programs; development of a proprietary LNP/AAV hybrid delivery system, as well as its modular platform to advance its complex genome editing capabilities, such as gene insertion; presentation of additional data at upcoming scientific conferences, and other preclinical data in 2020; advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, as well as its ability to maintain and expand its related intellectual property portfolio; ability to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies, including those in its ATTR, AML and HAE programs, in any future studies, including human clinical trials; ability to develop other in vivo or ex vivo cell therapeutics of all types, and those targeting WT1 in AML in particular, using CRISPR/Cas9 technology; expectations of the potential impact of the coronavirus disease 2019 pandemic on strategy, future operations and timing of its clinical trials or IND submissions; ability to continue its growth and realize the anticipated contribution of the members of its board of directors and executives to its operations and progress; ability to optimize the impact of collaborations on its development programs, including but not limited to its collaborations with Novartis or Regeneron, and a decision by Regeneron on whether to enter into a co-development and co-promotion agreement for the HAE program; statements regarding the timing of regulatory filings, actions and decisions regarding its development programs; use of capital, expenses, future accumulated deficit and other 2020 financial results or in the future; and ability to fund operations through the end of 2021.

Any forward-looking statements in this press release are based on management's current expectations and beliefs of future events, and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: risks related to Intellia's ability to protect and maintain its intellectual property position; risks related to Intellia's relationship with third parties, including its licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; uncertainties related to the initiation and conduct of studies and other development requirements for its product candidates; the risk that any one or more of Intellia's product candidates will not be successfully developed and commercialized; the risk that the results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies; and the risk that Intellia's collaborations with Novartis or Regeneron or its other ex vivo collaborations will not continue or will not be successful. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause Intellia's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in Intellia's most recent annual report on Form 10-K, as well as discussions of potential risks, uncertainties, and other important factors in Intellia's other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Intellia undertakes no duty to update this information unless required by law.

**INTELLIA THERAPEUTICS, INC.**
**CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)**
**(Amounts in thousands, except per share data)**

| | Three Months Ended Mar 31, | |
|---|---|---|
| | **2020** | **2019** |
| Collaboration revenue | $ 12,916 | $ 10,433 |
| Operating expenses: | | |
| Research and development | 34,650 | 23,709 |
| General and administrative | 11,314 | 10,533 |
| Total operating expenses | 45,964 | 34,242 |
| Operating loss | (33,048) | (23,809) |
| Interest income | 1,242 | 1,869 |
| Net loss | $ (31,806) | $ (21,940) |
| Net loss per share, basic and diluted | $ (0.63) | $ (0.49) |

| | | |
|---|---|---|
| Weighted average shares outstanding, basic and diluted | 50,491 | 45,234 |

**INTELLIA THERAPEUTICS, INC.**
**CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)**
**(Amounts in thousands)**

| | Mar 31, 2020 | December 31, 2019 |
|---|---|---|
| Cash, cash equivalents and marketable securities | $ 250,259 | $284,472 |
| Total assets | 314,752 | 334,280 |
| Total liabilities | 66,993 | 64,399 |
| Total stockholders' equity | 247,759 | 269,881 |

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