Intellia Therapeutics Announces Fourth Quarter and Full-Year 2019 Financial Results

February 27, 2020

- *On track to submit an IND application for NTLA-2001 for the treatment of transthyretin amyloidosis in mid-2020 and to dose first patients in 2H 2020*
- *Plans to submit an IND application for NTLA-5001, a WT1-directed TCR-T cell therapy, for the treatment of acute myeloid leukemia in 1H 2021*
- *Expects to nominate a development candidate for the treatment of hereditary angioedema in 1H 2020*
- *Ends 2019 with a strong cash position of $284 million; cash runway through YE 2021*

CAMBRIDGE, Mass., Feb. 27, 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 fourth quarter and year ended December 31, 2019.

"In 2019, we advanced our full-spectrum strategy, guiding both our *in vivo* and *ex vivo* lead programs toward the clinic. We also continued to build on our genome editing and delivery capabilities to enable a rapid succession of candidates," said Intellia President and Chief Executive Officer, John Leonard, M.D. "We are off to a productive start in 2020. We announced the nomination of NTLA-5001, a WT1-directed TCR-T cell therapy for the treatment of AML, and plan to select our third development candidate in the first half of this year, which will be for the treatment of HAE. In addition, in the second half of the year, we expect to begin dosing ATTR patients with NTLA-2001, a potential single-course treatment for ATTR patients. This is anticipated to be the first-ever systemically delivered CRISPR/Cas9-based therapy to enter the clinic, representing an important milestone in our mission to deliver potentially curative therapies from our proprietary modular platform."

2019 and Recent Operational Highlights

- **ATTR Program:** Intellia remains on track to submit an investigational new drug (IND) application in mid-2020 for its lead *in vivo* candidate, NTLA-2001, for the treatment of transthyretin amyloidosis (ATTR). In December 2019, Intellia completed a 12-month durability study of its lead lipid nanoparticle (LNP) formulation in support of NTLA-2001, maintaining an average reduction of >95% of serum transthyretin (TTR) protein and sustained liver genome editing after a single dose in non-human primates (NHPs). NTLA-2001 is anticipated to be the first systemically delivered CRISPR/Cas9 therapy to enter the clinic, and Intellia expects to dose the first patients 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:** In January 2020, Intellia announced NTLA-5001 as its first engineered T cell therapy development candidate for the treatment of acute myeloid leukemia (AML). NTLA-5001 utilizes a T cell receptor (TCR)-directed approach to target the Wilms' Tumor 1 (WT1) intracellular antigen to develop a broadly applicable treatment for AML patients, regardless of mutational background of a patient's leukemia. At the recent Keystone Symposia's Engineering the Genome Conference, the Company presented data in support of NTLA-5001, demonstrating that the selection of a naturally-occurring, high-affinity TCR, in combination with Intellia's CRISPR-enabled engineering and targeted insertion, results in an engineered T cell capable of specific and potent killing of primary AML blasts. Importantly, data presented showed that CRISPR-enabled engineering overcomes certain key challenges of traditional TCR approaches, such as mispairing between therapeutic and endogenous TCR, therefore creating a more homogenous T cell product. The engineered T cell carrying the therapeutic TCR also exhibited no detectable off-target reactivity to bone marrow cells. The Company plans to submit an IND application for NTLA-5001 in the first half of 2021. Additional efforts are underway to evaluate the potential use of the WT1-directed TCR construct to treat other tumor types, including solid tumors.
- **HAE Program:** In January 2020, Intellia announced hereditary angioedema (HAE) as its third development program. Building on the modular LNP delivery platform developed in the ATTR program, the Company aims to knock out the *kallikrein B1 (KLKB1)* gene with a single course of treatment to reduce the spontaneous activation of biological pathways responsible for generating bradykinin and thereby ameliorate the frequency and intensity of HAE attacks. At the recent Keystone Conference, the Company reported results from an ongoing NHP study, which demonstrated a 90% reduction in kallikrein activity sustained for at least five months following a single dose. The reduction of kallikrein activity observed in this study corresponds to the reduced enzymatic levels in patients that meaningfully impact HAE attack rates (Source: Banerji et al., NEJM, 2017). Intellia expects to select its development candidate in the first half of 2020. Intellia's *KLKB1* HAE program is subject to an option by Regeneron to enter into a Co/Co agreement prior to the initiation of IND-enabling studies, with Intellia as the lead party.
- **Modular Platform:** Intellia continued to progress 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. In 2019, the Company demonstrated the first CRISPR-mediated, targeted transgene insertion in the liver of NHPs. The targeted insertion of the *Factor 9* gene generated circulating human Factor IX protein at or above normal levels. Additionally, Intellia presented the first demonstration of a consecutive *in vivo* gene knockout followed by a targeted insertion in an alpha-1 antitrypsin

deficiency (AATD) mouse model. The consecutive edits led to >98% reduction of the disease-causing protein and sustained restoration of the normal protein to therapeutically relevant circulating levels throughout the study. The Company continues to advance these platform capabilities and leverage them to develop the next wave of *in vivo* and *ex vivo* clinical candidates.
- **Board of Directors Update:** Dr. Frank Verwiel was elected Chairman of the Board of Directors in February 2020, succeeding Perry Karsen, who will remain a member of the Board.

Upcoming Milestones

The Company has set forth the following for pipeline progression:

- ATTR:
  - Submit IND application for NTLA-2001 in mid-2020
  - Dose first patients in 2H 2020
- AML:
  - Submit IND application for NTLA-5001 in 1H 2021
- HAE:
  - Nominate a development candidate in 1H 2020
- R&D Advancements:
  - Present preclinical data at upcoming scientific conferences in 2020

Upcoming Event

The Company will participate in the following investor event during the first quarter of 2020:

- Barclays Capital Global Healthcare Conference, March 10, Miami

Fourth Quarter and Full Year 2019 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $284.5 million as of December 31, 2019, compared to $314.1 million as of December 31, 2018. The decrease was driven by cash used to fund operations of approximately $124.9 million, which was offset in part by $72.3 million of net equity proceeds raised from the Company's "At the Market" (ATM) offerings, $9.0 million of funding received under the Novartis collaboration, $9.9 million of ATTR cost reimbursements made by Regeneron, and $4.2 million in proceeds from employee-based stock plans.
- **Collaboration Revenue:** Collaboration revenue increased by approximately $3.1 million to $10.9 million during the fourth quarter of 2019, compared to $7.9 million during the fourth quarter of 2018. The increase in collaboration revenue in 2019 was primarily driven by amounts recognized under the Company's ATTR Co/Co agreement with Regeneron. As previously disclosed, Regeneron funded approximately 50% of the development costs for the ATTR program throughout 2019. Starting in June 2020, Regeneron will share approximately 25% of worldwide development costs and commercial profits for the ATTR program.
- **R&D Expenses:** Research and development expenses increased by $11.8 million to $31.7 million during the fourth quarter of 2019, compared to $19.9 million during the fourth quarter of 2018. This increase was mainly due to IND-enabling activities for NTLA-2001, research efforts supporting the selection of NTLA-5001 and the expansion of the Company's research and development team.
- **G&A Expenses:** General and administrative expenses increased by $0.3 million to $9.0 million during the fourth quarter of 2019, compared to $8.7 million during the fourth quarter of 2018. This increase was driven primarily by employee-related expenses.
- **Net Loss:** The Company's net loss was $28.3 million for the fourth quarter of 2019, compared to $19.1 million during the fourth quarter of 2018.

Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of December 31, 2019 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 Fourth Quarter and Full Year 2019 Earnings

The Company will discuss these results on a conference call today, February 27, 2020, at 8 a.m. ET.

To join the call:

- U.S. callers should dial 1-877-317-6789 and use conference ID# 10138773, approximately five minutes before the call.
- International callers should dial 1-412-317-6789 and use conference ID# 10138773, approximately five minutes before the call.

A replay of the call will be available through the Events and Presentations page of the Investor Relations section on Intellia's website, beginning on February 27, 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") application or similar clinical trial application 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 submit an IND application 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 nominate a development candidate for its hereditary angioedema ("HAE") program in the first half of 2020; 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; 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 optmize the impact of its collaborations on its development programs, including but not limited to its collaborations with Novartis or Regeneron Pharmaceuticals, Inc., and Regeneron's ability to enter into a co-development and co-promotion agreement for the HAE program; statements regarding the timing of regulatory filings regarding its development programs; use of capital, expenses, future accumulated deficit and other 2019 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 December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Collaboration revenue	$ 10,936	$ 7,880	$ 43,103	$ 30,434
Operating expenses:				
Research and development	31,731	19,918	108,413	89,115
General and administrative	8,976	8,708	41,058	32,189
Total operating expenses	40,707	28,626	149,471	121,304
Operating loss	(29,771)	(20,746)	(106,368)	(90,870)
Interest income	1,495	1,680	6,835	5,527
Net loss	$ (28,276)	$ (19,066)	$ (99,533)	$ (85,343)
Net loss per share, basic and diluted	$ (0.57)	$ (0.43)	$ (2.11)	$ (1.98)
Weighted average shares outstanding, basic and diluted	49,350	44,215	47,247	43,069

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

	December 31, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 284,472	$ 314,059
Total assets	334,280	347,315

Total liabilities	64,399	69,395
Total stockholders' equity	269,881	277,920

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