Intellia Therapeutics Announces Third Quarter 2019 Financial Results

October 31, 2019

- *On track to submit in mid-2020 an investigational new drug application for NTLA-2001 for the treatment of transthyretin amyloidosis*
- *Expects to nominate its first T cell receptor-directed engineered cell therapy development candidate for the treatment of acute myeloid leukemia by the end of 2019*
- *Presented preclinical data including first demonstration of consecutive* in vivo *gene knockout and insertion for the treatment of alpha-1 antitrypsin at the 2019 European Society of Gene and Cell Therapy Annual Meeting*
- *Ends quarter with strong cash position of $296 million*

CAMBRIDGE, Mass., Oct. 31, 2019 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), reported operational highlights and financial results for the third quarter ended September 30, 2019.

"In 2019, we continued to leverage the breadth of our genome editing platform to advance our *in vivo* and engineered cell therapy programs. We have demonstrated that we can knock out a disease-causing gene as well as introduce a functional gene to restore normal protein production. Now, we have achieved consecutive editing *in vivo* by combining both these edit types, further highlighting the versatility of our modular platform," said Intellia President and Chief Executive Officer, John Leonard, M.D. "Our full-spectrum strategy and platform capabilities are enabling Intellia's development of a robust pipeline to address a range of severe diseases. We look forward to the planned nomination of our first engineered cell therapy development candidate for acute myeloid leukemia by year-end and the submission of our first IND application for NTLA-2001 for the treatment of transthyretin amyloidosis in mid-2020."

Third Quarter 2019 and More Recent Operational Highlights

- **ATTR Program:** Intellia remains on track to submit in mid-2020 an investigational new drug (IND) application for NTLA-2001 for the treatment of transthyretin amyloidosis (ATTR). NTLA-2001 is anticipated to be the first systemically delivered CRISPR/Cas9 therapy to enter the clinic. As part of an ongoing durability study of its lead lipid nanoparticle (LNP) formulation in support of NTLA-2001, Intellia has demonstrated 10 months of durable liver editing with sustained reduction of circulating transthyretin (TTR) protein (average reduction >95%) following a single dose in non-human primates. In preparation for the submission of the IND application, the Company announced today it has commenced clinical-scale manufacturing for Phase 1 materials. NTLA-2001 is part of a co-development/co-promotion (Co/Co) agreement with Regeneron Pharmaceuticals, Inc., with Intellia as the lead development and commercialization party.
- **WT1-TCR for AML Program:** Intellia is on track to nominate its first T cell receptor (TCR)-directed engineered cell therapy development candidate by the end of 2019. The initial application of the in-locus TCR-based approach consists of autologous T cells directed towards the intracellular antigen Wilms' Tumor 1 (WT1) for the treatment of acute myeloid leukemia (AML). During the third quarter and more recently, Intellia continued to generate data from ongoing studies of multiple lead TCRs in patient-derived xenograft models to support the nomination of a development candidate for AML. Concurrently, the Company advanced GMP manufacturing-related development activities in support of a Phase 1 clinical trial.
- ***In Vivo* Platform:** At the recent 2019 European Society of Gene and Cell Therapy (ESGCT) Annual Meeting, Intellia presented the first demonstration of consecutive *in vivo* gene knockout and 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 novel genome editing strategy is enabled by the ability to sequentially dose with LNPs, one of the key advantages to Intellia's non-viral delivery system.

Upcoming Milestones

The Company has set forth the following for pipeline progression:

- ATTR:
  - Submit IND application for NTLA-2001 in mid-2020
- AML:
  - Nominate first engineered cell therapy development candidate by the end of 2019

Upcoming Events

The Company will participate in the following investor events:

- Credit Suisse Healthcare Conference, November 12, Scottsdale, Arizona
- Barclays Gene Editing and Gene Therapy Summit, November 13, New York City

Third Quarter 2019 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $295.8 million as of September 30, 2019, compared to $314.1 million as of December 31, 2018. The decrease was driven by cash used to fund operations of approximately $90.5 million, which was offset in part by $54.1 million of net equity proceeds raised from the Company's "At the Market" (ATM) agreement, $8.0 million of funding received under the Novartis collaboration, $7.3 million of ATTR cost reimbursements made by Regeneron, and $2.8 million in proceeds from employee-based stock plans.
- **Collaboration Revenue:** Collaboration revenue increased by approximately $3.2 million to $10.6 million during the third quarter of 2019, compared to $7.4 million during the third quarter of 2018. The increase in collaboration revenue in 2019 was primarily driven by amounts recognized from the expansion of the existing collaboration with Novartis, as well as by amounts recognized under the Company's ATTR Co/Co agreement with Regeneron. As previously disclosed, Regeneron funds approximately 50% of the development costs for the ATTR program.
- **R&D Expenses:** Research and development expenses increased by approximately $4.3 million to $27.5 million during the third quarter of 2019, compared to $23.2 million during the third quarter of 2018. This increase was driven primarily by the advancement of Intellia's research 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 approximately $0.2 million to $8.4 million during the third quarter of 2019, compared to $8.3 million during the third quarter of 2018. This increase was driven primarily by employee-related expenses to support Intellia's growing research and development efforts.
- **Net Loss:** The Company's net loss was $23.6 million for the third quarter of 2019, compared to $22.7 million during the third quarter of 2018.

Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of September 30, 2019, as well as technology access and funding from Novartis and Regeneron, will enable Intellia to fund its anticipated operating expenses and capital expenditure requirements into the second half of 2021. This expectation excludes any potential milestone payments or extension fees that could be earned and distributed under the collaboration agreements with Novartis and Regeneron or any strategic use of capital not currently in the Company's base-case planning assumptions.

Conference Call to Discuss Third Quarter 2019 Earnings

The Company will discuss these results on a conference call today, October 31, 2019, at 8 a.m. ET.

To join the call:

- U.S. callers should dial 888-208-1711 and use conference ID# 7693636, approximately five minutes before the call.
- International callers should dial + 1 856-344-9299 and use conference ID# 7693636, 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 October 31, 2019 at 12 p.m. ET.

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing 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 for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR") in mid-2020; its plans to nominate a first T cell receptor ("TCR")-directed engineered cell therapy development candidate for its acute myeloid leukemia ("AML") program by the end of 2019; its plans to advance and complete preclinical studies, including non-human primate studies for its ATTR program, AML program and other *in vivo* and *ex vivo* programs; develop our proprietary LNP-AAV hybrid delivery system to advance our complex genome editing capabilities, such as gene insertion; its presentation of additional data at upcoming scientific conferences, and other preclinical data by the end of 2019; the advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, as well as maintain and expand its related intellectual property portfolio; the ability to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies, including those in its ATTR and AML programs, in any future studies, including human clinical

trials; its 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; the 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; the impact of its collaborations on its development programs, including but not limited to its collaborations with Regeneron Pharmaceuticals, Inc. and Novartis Institutes for BioMedical Research; statements regarding the timing of regulatory filings regarding its development programs; its use of capital, including ATM receivables, expenses, future accumulated deficit and other financial results during the third quarter of 2019; and the ability to fund operations into the second half 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 our intellectual property position, including through our arbitration proceedings against Caribou; risks related to Intellia's relationship with third parties, including our licensors; risks related to the ability of our licensors to protect and maintain their intellectual property position; uncertainties related to the initiation and conduct of studies and other development requirements for our 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; the risk that Novartis will not continue to pursue programs it has selected through its collaboration with Intellia; 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 September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Collaboration revenue	$ 10,616	$ 7,408	$ 32,167	$ 22,554
Operating expenses:				
Research and development	27,513	23,237	76,682	69,197
General and administrative	8,431	8,270	32,082	23,481
Total operating expenses	35,944	31,507	108,764	92,678
Operating loss	(25,328)	(24,099)	(76,597)	(70,124)
Interest income	1,694	1,397	5,340	3,847
Net loss	$ (23,634)	$ (22,702)	$ (71,257)	$ (66,277)
Net loss per share, basic and diluted	$ (0.49)	$ (0.53)	$ (1.53)	$ (1.55)
Weighted average shares outstanding, basic and diluted	48,554	43,161	46,547	42,684

INTELLIA THERAPEUTICS, INC.
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(Amounts in thousands)

	September 30, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 295,790	$ 314,059
Total assets	346,635	347,315
Total liabilities	70,983	69,395
Total stockholders' equity	275,652	277,920

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