Intellia Therapeutics Announces Fourth Quarter and Full Year 2017 Financial Results

March 14, 2018

- *Initiated final testing of safety and efficacy in non-human primates (NHP) for our lead program intended to treat patients with transthyretin amyloidosis (ATTR)*
- *Completed successful knockout editing in mice of the SERPINA1 gene that gives rise to liver complications in certain alpha-1 antitrypsin deficiency patients*
- *Demonstrated increased complex editing capabilities by successful insertion of template DNA coding sequence in mice with partner Regeneron*
- *With partner Novartis, demonstrated that CRISPR/Cas9 editing of the erythroid specific enhancer region of the BCL11A gene in human hematopoietic stem cells led to increased production of fetal hemoglobin and the retention of multi-lineage capability in these cells when transplanted into mice*
- *$341 million in cash and cash equivalents as of December 31, 2017*

CAMBRIDGE, Mass., March 14, 2018 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR technology, announced financial results and operational progress for the fourth quarter and full year of 2017.

Using our proprietary lipid nanoparticle (LNP) delivery system, Intellia has achieved near-complete knockout editing of the transthyretin amyloidosis target gene (TTR) in rodents and most recently shared data on the progress of non-human primate (NHP) studies. In the fourth quarter, our NHP studies continued, yielding higher levels of editing as the Company improved the LNP formulation and dosing regimens, including repeated doses. Ongoing efforts in the NHP studies focus on consistently achieving a therapeutically relevant level of editing for the TTR gene as determined by transthyretin level reductions and we continue to observe and, as in prior animal studies, gather long-term durability data.

In parallel with the continuous progress on ATTR, during the fourth quarter, the Company expanded its research in the liver in two ways. First, in collaboration with partner Regeneron Pharmaceuticals, Inc. (Regeneron), we successfully inserted a functional gene into a specific site in the mouse genome, achieving therapeutically relevant levels of gene expression and demonstrating proficiency in executing complex editing procedures. These results provide a proof-of-principle for a large class of genetic defects that cannot be addressed solely by gene knockout. Second, knockout editing in livers of mice was successful for a second therapeutic target, the SERPINA1 gene that gives rise to liver complications in certain alpha-1 antitrypsin deficiency patients. The Company plans to present these data at upcoming scientific conferences and extend our evaluation in additional *in vivo* studies.

Through the ongoing collaboration with Novartis Institutes for BioMedical Research, Inc. (Novartis), the Company made further progress on complex *ex vivo* editing. Data presented at the 2017 American Society of Hematology (ASH) Annual Meeting and Exposition showed that an Intellia-qualified gRNA targeting the erythroid specific enhancer region of the BCL11A gene in human CD34+ cells could edit greater than 80 percent of these healthy bone marrow-derived cells, which led to the majority of the cells expressing fetal hemoglobin. These edited hematopoietic stem cells maintained engraftment over a 16-week period and demonstrated multi-lineage reconstitution, retaining their ability to complete normal hematopoiesis (blood cell formation) after the gene edit.

“2017 marked significant progress for Intellia with the CRISPR/Cas9 technology and our lead ATTR program. We demonstrated editing with our proprietary lipid nanoparticle delivery system across multiple animal species, including NHPs, and advanced this program towards clinical studies. After raising an additional $141 million, we are advancing the Company’s emerging pipeline of indications in the liver, including transthyretin amyloidosis and alpha-1 antitrypsin deficiency, expanding our *in vivo* editing work beyond the liver into CNS, and accelerating our *ex vivo* cellular therapy programs,” said President and Chief Executive Officer, John Leonard, M.D., Intellia Therapeutics. “As we begin 2018, our team remains focused on the patient and making genome editing-based therapies a reality. We are well-positioned to deliver against our goals and mission.”

The Company achieved several key milestones during 2017, including:

- Produced interim, top-line NHP data demonstrating, for the first time, liver genome editing using CRISPR/Cas9 delivered through a LNP system;
- Confirmed re-dosing in NHPs produced increased levels of editing, where the treatment/drug and regimen was well tolerated as assessed by clinical signs and chemistry;
- Presented 12-month data from a long-term mouse study, demonstrating robust and durable *in vivo* genome editing following a single, systemic intravenous delivery of Intellia’s proprietary, non-viral, LNP delivery system;
- Evaluated *in vivo* delivery by LNPs to a second organ, with successful genome editing by CRISPR/Cas9 in the central nervous system (cerebellum and striatum) in mice;
- In collaboration with Regeneron, produced positive insertion editing data in mice, demonstrating capability to perform complex genetic editing;
- In collaboration with Novartis, generated positive data in sickle cell anemia in transplant mouse models; and
- Continued to enhance and defend the Company’s CRISPR/Cas9 foundational and therapeutic intellectual property position, which included filing multiple patent applications covering our inventions and the issuance in Europe, the United Kingdom, Australia and China of foundational CRISPR/Cas9 patents for which we have in-licensed rights.

Upcoming Goals

The Company has set forth the following for 2018 pipeline progression:

- Mid-year: begin IND-enabling activities for lead ATTR development candidate
- Mid-year: present additional editing data following *in vivo* delivery by LNPs to organs beyond the liver
- Late 2018: advance lead development candidate for second indication
- During 2018: present additional insertion/repair editing data; and
- During 2018: present preclinical data in support of our first proprietary *ex vivo* programs on immuno-oncology and autoimmune/inflammation indications.

Fourth Quarter and Full Year 2017 Financial Results

Collaboration Revenue

Collaboration revenue was $6.7 million for the fourth quarter of 2017, compared to $5.6 million for the fourth quarter of the prior year. The increase in collaboration revenue in 2017 was primarily driven by amounts recognized under Intellia's collaboration agreement with Regeneron which was entered in April 2016.

Since inception through December 31, 2017, the Company has received $106.1 million in funding from the collaborations with Novartis and Regeneron, excluding amounts received for equity investments, and had an accounts receivable balance of $10.5 million at December 31, 2017. Excluding the $2.6 million of the upfront payment received from Novartis, which was allocated to the purchase of the Company's equity securities, Intellia has recognized $48.6 million in collaboration revenue under these agreements from inception through December 31, 2017, and had a remaining deferred revenue balance of $65.3 million at December 31, 2017.

Operating Expenses

Research and Development expenses increased by $9.8 million to $21.2 million during the fourth quarter of 2017, compared to $11.3 million during the same period of 2016. 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, and includes laboratory supplies, research materials and certain equipment. Additionally, salary and related headcount-based expenses increased, as the Company grew to 143 research and development personnel as of December 31, 2017, from 74 research and development employees as of December 31, 2016.

General and administrative expenses increased by $5.1 million to $10.2 million during the fourth quarter of this year, compared to $5.1 million in the fourth quarter of 2016. This increase was driven primarily by increased salary and related headcount-based expenses as the Company grew to 41 general and administrative employees as of December 31, 2017, from 29 general and administrative employees as of December 31, 2016, to support Intellia's larger research and development organization, public company compliance and administrative obligations. The Company also incurred increased corporate insurance, legal, and other professional expenses related to our expanding operations since becoming a public company in May 2016.

The Company's net loss was $24.0 million for the fourth quarter of 2017, compared to $10.6 million for the fourth quarter of 2016.

Balance Sheet

Cash and cash equivalents at December 31, 2017, were $340.7 million, compared to $273.1 million at December 31, 2016. The base period cash and cash equivalents were primarily attributable to $115.5 million in proceeds from the Company's initial public offering and $55.0 million in concurrent private placements in May 2016, in addition to a $75.0 million upfront payment from Regeneron in April 2016 and a follow-on public offering of $141.0 million in November 2017.

Financial Guidance

The Company's primary uses of capital will continue to be research and development programs, laboratory and related supplies, compensation costs for current and future employees, consulting, legal and other regulatory expenses, patent prosecution filing and maintenance costs for Intellia's licensed intellectual property, and general overhead costs.

As of December 31, 2017, the Company had an accumulated deficit of $121.1 million. The Company expects losses to increase as it continues to incur significant research and development expenses related to the advancement of Intellia's therapeutic programs and ongoing operations. Based on Intellia's research and development plans and expectations related to the progress with the Company's programs, the Company expects that the cash and cash equivalents as of December 31, 2017, as well as technology access and research funding from Novartis and Regeneron, will enable Intellia to fund operating expenses and capital expenditures through mid-2020, excluding 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 base case planning assumptions.

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 optimized cell therapies that can treat cancer and immunological diseases by replacing patients' diseased cells. The combination of deep scientific, technical and clinical development experience, along with our 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; Follow us on Twitter @intelliatweets.

Forward-Looking Statements

This press release contains "forward-looking statements" of Intellia 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 our ability to advance and expand the CRISPR/Cas9 technology to develop into human therapeutic products, as well as our CRISPR/Cas9 intellectual property portfolio; our ability to achieve stable or effective genome editing with a single treatment dose; the potential timing and advancement of our preclinical studies, including continuing non-human primate studies, and clinical trials; our ability to replicate results achieved in our preclinical studies in any future studies, including human clinical trials; the potential development of *ex vivo* cell therapeutics of all types using CRISPR/Cas9 technology; our ability to commence IND-enabling activities of a lead ATTR development candidate by mid-2018; our intent to present additional data for organs beyond the liver, additional insertion/repair data, and preclinical data in support of our first *ex vivo* programs on immuno-oncology and autoimmune/inflammation indications during 2018; our ability to nominate a development candidate for a second indication by late 2018; the intellectual property position and

strategy of Intellia's licensors; actions by government agencies; the impact of our collaborations on our development programs; the potential timing of regulatory filings regarding our development programs; the potential commercialization opportunities, including value and market, for product candidates; our expectations regarding our uses of capital, expenses, future accumulated deficit and other 2018 financial results; and our ability to fund operations through mid-2020. 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; 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 will be predictive of future results in connection with future studies; and the risk that Intellia's collaborations with Novartis or Regeneron 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 filed with the Securities and Exchange Commission, 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 Therapeutics undertakes no duty to update this information unless required by law.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	2016	2017	2016
Collaboration revenue	$ 6,668	$ 5,627	$ 26,117	$ 16,479
Operating expenses:				
Research and development	21,170	11,331	67,647	31,840
General and administrative	10,213	5,118	28,025	16,798
Total operating expenses	31,383	16,449	95,672	48,638
Operating loss	(24,715)	(10,822)	(69,555)	(32,159)
Interest income	752	259	2,012	525
Net loss	$ (23,963)	$ (10,563)	$ (67,543)	$ (31,634)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.61)	$ (0.31)	$ (1.88)	$ (1.42)
Weighted average shares outstanding, basic and diluted	39,155	34,507	36,006	22,222

INTELLIA THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEET DATA
(Amounts in thousands)

	December 31, 2017	December 31, 2016
Cash and cash equivalents	$ 340,678	$ 273,064
Total assets	376,235	298,969
Total liabilities	75,638	89,132
Total stockholders' equity	300,597	209,837

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