Intellia Therapeutics Announces Third Quarter 2017 Financial Results

October 31, 2017

- *First company to demonstrate dose-dependent CRISPR/Cas9 liver editing in non-human primates, including editing of over 20 percent in certain animals, following systemic delivery of Intellia's proprietary, non-viral, lipid nanoparticle delivery system*
- *Well-tolerated safety profile observed to date in non-human primates after single and repeat doses, with rapid clearance of all CRISPR/Cas9 cargo and cationic lipid components, similar to Intellia's prior safety data in rodent studies*
- *Presented 52-week CRISPR/Cas9 editing durability data in mice, confirming long-term liver genome editing (approximately 70 percent) with sustained protein reduction (approximately 97 percent) after a single intravenous administration*
- *Demonstrated lipid nanoparticle-mediated delivery and editing with CRISPR/Cas9 in the central nervous system in mice*
- *$222 million in cash and cash equivalents as of September 30, 2017*

CAMBRIDGE, Mass., Oct. 31, 2017 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (Nasdaq:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR technology, announced financial and operational results for the third quarter of 2017.

The Company made considerable progress in its Transthyretin Amyloidosis (TTR) program using its proprietary lipid nanoparticle (LNP) delivery system. Based on interim, top-line data from Intellia's first exploratory non-human primate (NHP) studies, the Company has demonstrated successful editing in the livers of individual animals with NHP guide RNAs (gRNAs) as well as with exploratory human cross-reactive gRNAs. These interim results, coupled with on-going studies and further optimization efforts, are anticipated to allow for the selection of a human guide development candidate as early as the first quarter of 2018**.** In addition, Intellia completed a 12-month mouse durability study, maintaining approximately 97 percent reduction in serum TTR protein levels, accompanied by sustained editing at the target DNA site in the liver after a single intravenous administration of its CRISPR/Cas9 system.

"We accomplished some significant milestones during the third quarter, demonstrating the first CRISPR/Cas9 liver-targeted genome editing with systemic delivery of LNPs in non-human primates, the third animal species in which we have achieved editing. Our delivery system was safe and well tolerated in the studies, enabling us to redose without adverse events observed. In our 52-week mouse study, we demonstrated editing durability after a single dose of CRISPR/Cas9 using our delivery system. Moving beyond the liver, we also demonstrated the potential adaptability of our approach by showing delivery and editing in the central nervous system in mice. These data further advance Intellia's mission to develop curative genome editing therapies for current unmet medical needs," said Chief Executive Officer and Founder, Nessan Bermingham, Ph.D., Intellia Therapeutics. "Financially, the company continued to have a strong cash position at the end of the quarter."

Third Quarter 2017 Highlights

The Company achieved several key milestones during the third quarter of 2017, including:

- Produced interim, top-line NHP data demonstrating, for the first time, liver genome editing using CRISPR/Cas9 delivered through a LNP system, including observing over 20 percent editing in certain animals:
  - Observed editing ranges of 0.10 percent up to 32.0 percent after a single dose, with multiple animals showing editing greater than 20 percent after a second dose. These results were observed in experiments with various exploratory gRNA, LNP formulations and dosing regimens, similar to prior results from Intellia's initial rodent studies;
  - Confirmed re-dosing in NHPs produced increased levels of editing, with no adverse events observed;
  - Well-tolerated safety profile observed to date with both NHP-specific gRNA and exploratory human cross-reactive gRNAs. This was assessed by gross observation, as well as by clinical chemistry, hematology, and cytokine and complement levels;
  - Animals with the highest levels of editing showed a higher reduction in TTR serum protein levels; and
  - Initiated further studies to optimize editing efficiency and to explore dosing parameters, which will lead toward the selection of a development candidate.
- Presented 12-month data from the completed long-term mouse study, demonstrating robust and durable *in vivo* genome editing following a single, systemic intravenous administration:
  - Achieved and maintained approximately a 97 percent reduction in serum TTR protein levels through one year, corresponding with approximately 70 percent editing at the target DNA site in the liver;
  - Confirmed the transient nature of Intellia's LNP delivery system with 99 percent clearance of mRNA within 10 hours and clearance of sgRNA within 72 hours in the liver; and
  - The treatment was observed to be safe and well tolerated during the 52-week study.
- Made progress in evaluating *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.
- Continued to defend and enhance the Company's CRISPR/Cas9 foundational and therapeutic intellectual property position through filing and prosecution of patent applications covering its internal, collaboration and in-licensed inventions.

Third Quarter 2017 Financial Results

Collaboration Revenue

Collaboration revenue was $7.3 million for the third quarter of 2017, compared to $4.9 million for the third 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 Pharmaceuticals, Inc. (Regeneron), which was entered into in April 2016.

Since its inception through September 30, 2017, the Company has received $105.1 million in funding under its collaborations with Novartis Institutes for BioMedical Research, Inc. (Novartis) and Regeneron, excluding amounts received for equity investments and recorded accounts receivable of $3.5 million. Excluding the $2.6

million of the upfront payment received from Novartis, which was allocated to its purchase of the Company's equity securities, Intellia recognized $42.0 million in collaboration revenue under these agreements through September 30, 2017, and had remaining deferred revenue of $65.0 million as of September 30, 2017.

Operating Expenses

Research and development expenses increased by $9.6 million to $17.5 million during the third quarter 2017, compared to $7.9 million during the same period of 2016. This increase was driven primarily by personnel growth and the advancement of Intellia's research programs, as well as the initial build-out of the development organization, and includes laboratory supplies, research materials and bench-top equipment. Additionally, salary and related headcount-based expenses increased, as the Company grew to 135 research and development personnel as of September 30, 2017, from 66 research and development employees as of September 30, 2016.

General and administrative expenses increased by $1.0 million to $5.7 million during the third quarter of this year, compared to $4.7 million in the third quarter of 2016. This increase was driven primarily by increased salary and related headcount-based expenses as the Company grew to 34 general and administrative employees as of September 30, 2017, from 23 general and administrative employees as of September 30, 2016, to support Intellia's public company compliance and administration obligations. The Company also incurred increased corporate insurance, legal, and other professional expenses related to its expanding operations since becoming a public company in May 2016.

The Company's net loss was $15.4 million for the third quarter 2017, compared to $7.5 million for the third quarter 2016.

Balance Sheet

Cash and cash equivalents at September 30, 2017, were $222 million, compared to $291 million for the same quarter in 2016. The base period cash and cash equivalents were primarily attributable to $115.5 million in proceeds from the Company's initial public offering, $55 million in concurrent private placements, and a $75 million upfront payment from Regeneron in April 2016.

Financial Guidance

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

During the remainder of 2017, the Company expects expenses to continue to increase compared to prior periods relating to its ongoing activities, particularly as certain research and development activities progress toward human clinical trials, and as the Company spends a full year in its new office and laboratory facility, which it has occupied since the fourth quarter of 2016.

As of September 30, 2017, the Company had an accumulated deficit of $97.2 million. The Company expects its losses to increase as it continues to incur significant research and development and other 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 September 30, 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-2019, excluding any potential milestone payments or extension fees received under its collaboration agreements with Novartis and Regeneron.

Conference Call to Discuss Third Quarter 2017 Earnings

The Company will present third quarter 2017 results to investors and analysts in a conference call on October 31, 2017 at 8:30am EDT, hosted by Nessan Bermingham, Ph.D., Chief Executive Officer and Founder, John Leonard, M.D.,Executive Vice President, R&D, and Graeme Bell, Chief Financial Officer. The analyst and investor presentation can be downloaded starting at 8:00am EDT from the Investor Relations section of the company's website at www.intelliatx.com.

To participate on the day, U.S. callers should dial 888-752-0423 and use Conference ID# 9089048, approximately five minutes before the call. International callers should dial +1 918-398-4936 and use Conference ID# 9089048, approximately five minutes before the call.

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing proprietary, potentially 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. 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; 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 Intellia's 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 editing; 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 the *ex vivo* cell therapeutics through our eXtellia division, including the development of next-generation T cell therapies that address unmet needs in hematological and solid tumors, immuno-oncology and auto-immunity; the intellectual property position and strategy of Intellia's licensors; actions by government agencies; the impact of our collaborations with Ospedale San Raffaele, Novartis and Regeneron 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 2017 financial results; and our ability to fund operations through mid-2019. 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 quarterly report on Form 10-Q 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 (UNAUDITED)
(Amounts in thousands except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Collaboration revenue	$ 7,317	$ 4,869	$ 19,449	$ 10,852
Operating expenses:				
Research and development	17,481	7,861	46,477	20,509
General and administrative	5,711	4,705	17,812	11,680
Total operating expenses	23,192	12,566	64,289	32,189
Operating loss	(15,875)	(7,697)	(44,840)	(21,337)
Interest income	519	215	1,260	266
Net loss	$ (15,356)	$ (7,482)	$ (43,580)	$ (21,071)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.44)	$ (0.22)	$ (1.25)	$ (1.16)
Weighted average shares outstanding, basic and diluted	35,189	34,316	34,945	18,098

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

	September 30, 2017	December 31, 2016
Cash and cash equivalents	$ 222,264	$ 273,064
Total assets	249,170	298,969
Total liabilities	73,323	89,132
Total stockholders' equity (deficit)	175,847	209,837

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