Intellia Therapeutics Announces Second Quarter 2017 Financial Results

August 1, 2017

- *In non-human primates, demonstrated robust green fluorescent protein expression throughout the liver 24 hours following a single, systemically delivered, intravenous dose, using our proprietary lipid nanoparticle delivery system*
- *Using our proprietary lipid nanoparticle delivery system, demonstrated* in vivo *durable liver genome editing in mice through nine months, post-single-dose intravenous administration*
- *$241 million cash and cash equivalents as of June 30 2017*

CAMBRIDGE, Mass., Aug. 01, 2017 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on the development of potentially curative therapeutics using CRISPR technology, announced financial and operational results for the second quarter of 2017.

The company has made significant progress using our proprietary lipid nanoparticle (LNP) delivery system throughout the quarter. We advanced into non-human primate studies, while first time data from rat studies, the second species we tested *in vivo*, confirmed the durable mouse data demonstrating high levels of sustained genome editing and serum transthyretin (TTR) reduction post-single-dose intravenous administration.

"We are very excited that our initial non-human primate data *in vivo* continue to validate our mRNA delivery technology. These preclinical data accelerate our momentum as we advance the development of potential therapies to treat patients with high unmet medical needs," said Chief Executive Officer and founder Nessan Bermingham, Ph.D., Intellia Therapeutics. "During the quarter, we have further strengthened our company by advancing partnerships, including a research agreement with Ospedale San Raffaele in Milan for our *ex vivo* technology focused on novel engineered cell therapies using CRISPR, expanded our global intellectual property portfolio, and appointed two new members to our Board of Directors."

Second Quarter 2017 Operational Highlights

The company achieved several key operational milestones during the second quarter of 2017, including:

- Commenced non-human primate studies in support of our preclinical work relating to our lead development program in TTR. We demonstrated robust green fluorescent protein expression throughout non-human primate liver 24 hours after a single systemically delivered administration dose *in vivo,* using our proprietary lipid nanoparticle delivery system.

- Extended our data set beyond the mouse and delivered CRISPR/Cas9 in the rat. Specifically, initial data from rat models post-single-dose intravenous administration *in vivo* using our proprietary lipid nanoparticle delivery system showed up to 91 percent reduction in serum TTR protein levels and up to 66 percent editing at the target DNA site, demonstrating robust and dose-responsive gene editing of TTR. We presented these results at the American Society of Gene & Cell Therapy Annual Meeting in Washington D.C. in May 2017.

- Provided an update on our ongoing durability study, demonstrating durable liver editing through nine-months post a single intravenous dose administration in mice using our proprietary lipid nanoparticle delivery system. Throughout the liver, the data continues to demonstrate durability and high editing efficiency of LNP-mediated editing of the TTR gene, showing 97 percent reduction in serum TTR protein levels and approximately 70 percent editing at the target DNA site. The intended transient nature of LNP delivery has been confirmed in rodents with undetectable Cas9 mRNA and gRNA levels in the liver by 72 hours post administration.

- Entered into a three-year research collaboration, option, and license agreement to engineer optimized T cell cancer therapies with a leading scientific research institution, Ospedale San Raffaele SRL of Milan, Italy.
    - The collaboration aims to discover innovative targets against tough-to-treat cancers, leveraging Intellia's proprietary CRISPR/Cas9 platform to generate next-generation T cell therapies to address unmet needs in hematological and solid tumors.
    - The collaboration is the first partnership of Intellia's eXtellia division, focused on advancing next generations of engineered cell therapies through unique and proprietary applications of CRISPR genome editing in areas including immuno-oncology and auto-immunity.

- Continued to defend and enhance our CRISPR/Cas9 foundational and therapeutic intellectual property position through filing and prosecution of patent applications covering our internal, collaboration and in-licensed inventions. In relation to the foundational CRISPR/Cas9 genome editing intellectual property portfolio to which we have rights for human therapeutics

and companion diagnostics, and which is co-owned by the Regents of the University of California, the University of Vienna, and Dr. Emmanuelle Charpentier (collectively “UC”), the following was achieved:

- China’s State Intellectual Property Office granted to UC a patent broadly covering CRISPR/Cas9 single-guide genome editing methods and compositions. The patent includes claims that cover methods for editing DNA in non-cellular and cellular settings, including in eukaryotic cells such as human and mammalian cells. It also includes CRISPR/Cas9 composition of matter and system claims for use in any setting, including claims covering the use of CRISPR/Cas9 in producing medicines for treating disease.
- UC submitted its opening brief July 25, 2017 to the U.S. Court of Appeals for the Federal Circuit in their appeal from a February 15, 2017 decision by the U.S. Patent and Trademark Office’s Patent Trial and Appeal Board (“PTAB”) in an interference proceeding relating to the CRISPR/Cas9 genome editing technology. In the appeal, UC requests the reversal of the PTAB’s decision to terminate the interference between certain CRISPR/Cas9 patent claims owned by UC and patents and patent applications owned by the Broad Institute, Massachusetts Institute of Technology, the President and Fellows of Harvard College and the Rockefeller University.

- Named Moncef Slaoui, Ph.D. and Frank Verwiel, M.D. to its Board of Directors. In addition the Board established a Science & Technology Committee:
  - Moncef Slaoui, Ph.D. and Frank Verwiel, M.D. joined the Board of Directors. Drs. Slaoui and Verwiel began Board responsibilities on July 25, 2017. Intellia’s Board of Directors also established a Science and Technology Committee, which will be chaired by Dr. Slaoui. Additionally, Carl L. Gordon, Ph.D., CFA, General Partner, OrbiMed Advisors, LLC, informed the company of his resignation from the Intellia Board of Directors effective at the close of business on July 25, 2017.

Second Quarter 2017 Financial Results

Collaboration Revenue

Collaboration revenue was $5.9 million for the second quarter of 2017, compared to $4.2 million for the second quarter of the prior year. The increase in collaboration revenue in 2017 was primarily driven by amounts recognized under our collaboration agreement with Regeneron Pharmaceuticals, Inc. (Regeneron), which was entered into in April 2016.

Through June 30, 2017, the company received $104.1 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 $2.8 million. Excluding the $2.6 million of the upfront payment received from Novartis, which was allocated to the purchase of equity securities, we recognized $34.7 million in collaboration revenue under these agreements through June 30, 2017, and had remaining deferred revenue of $69.6 million as of June 30, 2017.

Operating Expenses

Research and development expenses increased $8.2 million to $15.6 million during the second quarter 2017, compared to $7.4 million during the same period of 2016. This increase was driven primarily by greater support for the advancement of our early-stage research programs and includes laboratory supplies and research materials. Additionally, salary and related headcount-based expenses increased as the company grew to 110 research and development employees as of June 30, 2017, from 56 research and development employees as of June 30, 2016.

General and administrative expenses increased $2.7 million to $6.4 million during the second quarter of this year, compared to $3.7 million in the second quarter of 2016. This increase was driven primarily by increased salary and related headcount-based expenses as the company grew to 33 general and administrative employees as of June 30, 2017, from 19 general and administrative employees as of June 30, 2016, to support our 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.

Our net loss was $15.6 million for the second quarter 2017, compared to $6.9 million for the second quarter of 2016.

Balance Sheet

Cash and cash equivalents at June 30, 2017, were $241 million, compared to $301 million for the same quarter in 2016. The base period cash and cash equivalents were primarily attributable to $115.5 million in proceeds from our initial public offering, $55 million in concurrent private placements, and a $75 million upfront payment from Regeneron in April 2016. The year-over-year change is attributed to cash used in ongoing operations.

Financial Guidance

Our 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 our licensed intellectual property, and general overhead costs.

During 2017, the company expects expenses to continue to increase compared to prior periods relating to our ongoing activities, particularly as research and development and preclinical activities gather further momentum toward human clinical trials, and we spend a full year occupying our new office and laboratory facility, which we began to occupy in the fourth quarter of 2016.

As of June 30, 2017, the company had an accumulated deficit of $81.8 million. We expect our losses to increase as we continue to incur significant research and development and other expenses related to the advancement of our therapeutic programs and our ongoing operations. Based on our research and development plans and expectations related to the progress of the company’s programs, we expect that the cash and cash equivalents as of June 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 our collaboration agreements with Novartis and Regeneron.

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on the development of 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. Our combination of deep scientific, technical and clinical development experience, along with our leading intellectual property portfolio, puts us 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 liver 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; 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 June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Collaboration revenue	$ 5,917	$ 4,206	$ 12,132	$ 5,983
Operating expenses:				
Research and development	15,565	7,423	28,996	12,648
General and administrative	6,369	3,729	12,101	6,975
Total operating expenses	21,934	11,152	41,097	19,623
Operating loss	(16,017)	(6,946)	(28,965)	(13,640)
Interest income	424	46	741	51
Net loss	$ (15,593)	$ (6,900)	$ (28,224)	$ (13,589)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.45)	$ (0.36)	$ (0.81)	$ (1.37)
Weighted average shares outstanding, basic and diluted	34,916	19,121	34,820	9,899

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

	June 30, 2017	December 31, 2016
Cash and cash equivalents	$ 241,047	$ 273,064
Total assets	264,975	298,969
Total liabilities	77,390	89,132
Total stockholders' equity	187,585	209,837

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