Intellia Therapeutics Announces First Quarter 2017 Financial Results

May 2, 2017

- *First to demonstrate single dose,* in vivo *results using a proprietary lipid nanoparticle delivery system showing: approximately 97 percent reduction in serum transthyretin protein levels; 70 percent liver editing efficiency following a single dose; six months post-single-dose durability of stable liver editing*
- *Appeal of U.S. Patent Trial and Appeal Board decision on CRISPR/Cas9 interference involving Intellia-licensed patent family filed by the University of California and other co-owners*
- *European Patent Office announced decision to grant CRISPR/Cas9 genome editing technology patent and the United Kingdom Intellectual Property Office granted national chimeric CRISPR/Cas9 patent*
- *First quarter 2017 cash position of $258 million*

CAMBRIDGE, Mass., May 02, 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 results for the first quarter 2017.

"The first quarter of 2017 has been highly productive for Intellia as we continue to advance our preclinical programs toward developing life-transforming therapies. Building on our earlier results showing 97 percent *in vivo* reduction of serum transthyretin protein levels, our durability data also continue to demonstrate stable liver editing and support our vision that genome editing could be effective with just a single dose," said Nessan Bermingham, Ph.D., chief executive officer, and founder, Intellia Therapeutics. "We expect to advance preclinical studies significantly this year by progressing our technology in non-human primate studies and look toward its potential broad clinical applications to help treat patients with unmet medical needs."

Business Highlights

During the first quarter of 2017, the company achieved numerous important milestones:

Significantly advanced the genome editing field with unprecedented liver editing and lipid nanoparticle delivery data using CRISPR/Cas9 as a potential one-time treatment.

- In March, the company reported editing efficiency *in vivo* with a significant reduction of serum transthyretin (TTR) protein levels. More recent durability study data show stable liver editing in mice for at least six months following a single administration.
    - Enhanced *in vivo* gene editing in the TTR locus, with an efficiency of approximately 70 percent in the total mouse liver at the target DNA site, after a single intravenous administration;
    - Decreased serum TTR protein levels of up to approximately 97 percent; and
    - Achieved undetectable Cas9 messenger RNA (mRNA) and guide RNA (gRNA) in the liver by 72 hours, post administration.

Defended and enhanced our CRISPR/Cas9 foundational and therapeutic intellectual property position.

- The European Patent Office (EPO) decided to grant to co-owners the Regents of the University of California, University of Vienna, and Dr. Emmanuelle Charpentier (collectively, UC) a patent broadly covering the CRISPR/Cas9 genome editing technology. The patent includes claims covering compositions of the widely adopted CRISPR single guide RNA technology for use in any non-cellular or cellular setting, including eukaryotic cells such as human or mammalian cells, as well as for use in human therapeutics. This European patent, which is expected to grant on May 10, 2017, will be nationalized in, and cover, approximately forty European countries that are member states of the European Patent Organisation.
- In addition to the EPO decision, earlier this year, the United Kingdom's (U.K.) Intellectual Property Office granted to UC a second national patent on the CRISPR/Cas9 genome editing system. This February 7, 2017 U.K. patent covers chimeric CRISPR/Cas9 systems in which the Cas9 protein is modified to provide alternative DNA-modulating activities while the first U.K. patent obtained by UC covers the single guide RNA for uses in both non-cellular and cellular settings.
- In April 2017, UC appealed to the U.S. Court of Appeals for the Federal Circuit the decision by the Patent Trial and Appeal Board (PTAB) to terminate the interference between certain CRISPR/Cas9 patent claims owned by UC and patents and patent applications owned by the Broad Institute, Harvard University and the Massachusetts Institute of Technology. UC is seeking review and reversal of the PTAB's February 15, 2017 decision, which concluded that, although the claims overlap, the respective scope of UC and Broad's claim sets do not define the same patentable invention and, accordingly, terminated the interference without deciding which party first invented the use of the CRISPR/Cas9 technology in

eukaryotic cells.

First Quarter 2017 Financial Results

Collaboration Revenue

Collaboration revenue was $6.2 million for the first quarter of 2017, compared to $1.8 million for the first 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 March 31, 2017, the company received $103.1 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 $2.4 million. Excluding the $2.6 million of the upfront payment received from Novartis which was allocated to the purchase of equity securities, we recognized $28.8 million in collaboration revenue under these agreements through March 31, 2017 and had remaining deferred revenue of $74 million as of March 31, 2017.

Operating Expenses

Research and development expenses increased $8.2 million to $13.4 million during the first quarter 2017, compared to $5.2 million during the same period of 2016. This increase was driven primarily by increased salary and related headcount-based expenses, including equity-based compensation expenses, as the company grew to 88 research and development employees as of March 31, 2017, from 44 research and development employees as of March 31, 2016. This increase supported the advancement of our early-stage research programs including, consumption of laboratory supplies and research materials.

General and administrative expenses increased $2.5 million to $5.7 million during the first quarter of this year, compared to $3.2 million in the first quarter of 2016. This increase was driven primarily by increased salary and related headcount-based expenses, including equity-based compensation expenses, as the company grew to 31 general and administrative employees as of March 31, 2017, from 17 general and administrative employees as of March 31, 2016. 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 $12.6 million for the first quarter 2017, compared to $6.7 million for the first quarter of 2016.

Balance Sheet

Cash and cash equivalents at March 31, 2017, were $258 million, compared to $64 million for the same quarter in 2016. The increase in cash and cash equivalents was 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, partially offset by cash used in operations.

Financial Guidance

Our primary uses of capital will continue to be research and development services, compensation and related expenses, laboratory and related supplies, 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 in connection with our ongoing activities, particularly as research and development and preclinical activities gather momentum, 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 March 31, 2017, the company had an accumulated deficit of $66.2 million. We expect our losses to increase as we continue to incur significant research and development and other expenses related to our ongoing operations. Based on our research and development plans and expectations related to the progress of the company's programs, we expect that cash and cash equivalents as of March 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-2019, without giving effect to any potential milestone payments or extension fees received under our collaboration agreements with Novartis and Regeneron.

Upcoming Events During the Second Quarter 2017

The company expects to make presentations at the following upcoming scientific and investor conferences:

- The American Society of Gene & Cell Therapy, May 13, Washington
- The TechConnect World Innovation Conference, May 16, Washington
- Jefferies Healthcare Conference, June 6, New York City
- JMP Securities Life Sciences Conference, June 20, New York City
- Goldman Sachs Third Annual Innovation Symposium, June 27, New York City

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 non-human primate studies, and clinical trials; the intellectual property position and strategy of Intellia's licensors; actions by government agencies; the impact of our collaborations with 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 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 subsequent 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 March 31,	
	2017	**2016**
Collaboration revenue	$ 6,215	$ 1,777
Operating expenses:		
Research and development	13,431	5,225
General and administrative	5,732	3,246
Total operating expenses	19,163	8,471
Operating loss	(12,948)	(6,694)
Interest income	317	5
Net loss	$ (12,631)	$ (6,689)
Net loss per share, basic and diluted	$ (0.36)	$ (9.89)
Weighted average shares outstanding, basic and diluted	34,723	676

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

	March 31, 2017	**December 31, 2016**
Cash and cash equivalents	$ 257,551	$ 273,064
Total assets	279,263	298,969
Total liabilities	79,380	89,132
Total stockholders' equity	199,883	209,837

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