Intellia Therapeutics Announces Fourth Quarter and Full-Year 2016 Financial Results

March 14, 2017

- *First to demonstrate single dose, in vivo results, showing approximately 97 percent reduction in serum transthyretin protein levels*
- *Further established a comprehensive platform, including a proprietary lipid nanoparticle delivery system, to accelerate therapeutic development*
- *Executed partnering deal with Regeneron Pharmaceuticals, and continued to advance Intellia's partnership with Novartis, to support Intellia's emerging pipeline with six distinct programs in active research and development, spanning multiple types of genome editing*
- *Continued to deploy a diversified approach to maximize CRISPR potential, including pursuing CRISPR applications in Hepatitis B Virus and hematopoietic stem cells*
- *2016 year-end cash position of $273 million*

CAMBRIDGE, Mass., March 14, 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 fourth quarter and full year 2016.

"2016 was a phenomenal year for Intellia. We signed a multi-year research and development collaboration with Regeneron Pharmaceuticals, our second partner, and became a publicly traded company with an upsized offering. We also presented the first *in vivo* CRISPR liver editing data, and significantly grew our capabilities, laboratories, and team," said Nessan Bermingham, Ph.D., chief executive officer and founder, Intellia Therapeutics. "I am extremely pleased with the progress since forming the company in May 2014. In 2017, we expect to make meaningful steps toward our goal of bringing revolutionary therapies to patients."

Business Highlights

The company achieved numerous important milestones in 2016, including:

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 August 2016, Intellia became the first company to present preclinical *in vivo* liver editing data using its novel system of delivering CRISPR/Cas9 components by lipid nanoparticle (LNP) technology. Last week, the company reported an update on these results, demonstrating increased editing efficiency *in vivo* with a significant reduction of serum transthyretin (TTR) protein levels. This effect was durable for at least four months following a single administration in mice. In summary Intellia presented:
  - 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 (versus previously reported 60 percent);
  - Decreased serum TTR protein levels of up to approximately 97 percent (versus previously reported 80 percent); and
  - Undetectable Cas9 messenger RNA (mRNA) and guide RNA (gRNA) in the liver by 72 hours, post administration.

On March 22, 2017, the company will present additional data regarding its latest *in vivo* liver editing data at Le Studium Conference on Messenger RNA Therapeutics in Orleans, France.

Completed a successful initial public offering, securing a strong cash position, fueling the company's pipeline and platform studies.

- In May 2016, the company completed its initial public offering, upsizing the offer to $115.5 million and executing a concurrent private placement of $55 million. Through December 31, 2016, Intellia raised an aggregate of approximately $352.5 million to fund its operations, of which $97.0 million was through collaboration agreements, $170.5 million was from its initial public offering and concurrent private placements, and $85.0 million was from the sale of convertible preferred stock.

Announced a multi-year, multi-target research and development collaboration with Regeneron Pharmaceuticals, representing one of the largest deals in the CRISPR/Cas9 field.

- Under the collaboration, Regeneron has the exclusive right to discover and develop CRISPR-based products against up to 10 targets, focused primarily on therapies for a broad range of diseases that may be treated by editing genes in the liver. Transthyretin amyloidosis (ATTR) is the first target to be jointly developed and potentially commercialized by the

companies.
- Intellia received access to the Regeneron Genetics Center, one of the world's most comprehensive genetics databases, and proprietary mouse models for a limited number of its liver programs.
- Intellia received a $75 million upfront payment and a $50 million equity investment and is eligible to receive significant milestone payments of up to $320 million per target, as well as high single-digit to low-teen royalties.

To advance the pipeline and explore the scope of genome edits with the CRISPR/Cas9 system, the company is working on four sentinel *in vivo* liver indications and two sentinel *ex vivo* programs employing different editing strategies.

In vivo

- Transthyretin Amyloidosis (ATTR) program, being developed with Regeneron utilizing a gene knockout strategy. Candidate guides are being progressed to non-human primate studies in 2017.
- Hepatitis B Virus (HBV) program, utilizing a knockout strategy to target covalently closed circular DNA (cccDNA). Over 400 candidate guides were characterized and evaluated, and advanced *in vitro* studies are underway using the most optimal guides identified.
- Alpha-1 Antitrypsin Deficiency (AATD) program, utilizing either a gene knockout or a gene repair strategy, therapeutic outcome dependent. The company is performing activities for guide selection activities.
- Primary Hyperoxaluria Type 1 (PH-1), our initial Inborn Errors of Metabolism (IEM) program, is in advanced guide selection, using a gene knockout strategy while we also characterize guides and edit types for several other IEMs of interest.

Ex vivo

- Chimeric antigen receptor T Cell (CAR-T) and hematopoietic stem cell (HSC) applications in partnership with Novartis, where Intellia retains the right to develop and commercialize rights to certain HSC programs, advanced to late preclinical development stage.

Beyond providing the company multiple potential product opportunities, each of these programs potentially enable CRISPR/Cas9 translation into a broader set of disease indications requiring the same types of DNA edits.

Gained exclusive access to two leading genetic database centers that will bring critical disease insights to Intellia's preclinical and clinical process.

- Through its agreement with Regeneron, the company has access to the Regeneron Genetics Center, one of the world's most comprehensive genetic databases, which pairs sequenced exomes with anonymized electronic health records.
- Intellia joined Genomics England's GENE Consortium as the first dedicated genome editing company to participate in the 100,000 Genome Project. Participation in the GENE Consortium, alongside leading global biotech and pharmaceutical companies, gives Intellia access to important data to better understand the basis of rare genetic diseases, which could lead to identifying novel targets and possible treatment approaches.

Enhanced our CRISPR/Cas9 foundational and delivery intellectual property position.

- Executed an invention management agreement (IMA) with the owners (University of California, University of Vienna, and Emmanuelle Charpentier) and other key licensees of the CRISPR/Cas9 foundational intellectual property, thus ensuring alignment in the prosecution, defense and enforcement of, as well as global freedom to operate regarding this intellectual property.
- Secured issuance in the United Kingdom of two critical CRISPR/Cas9 patents, which are the first two issued patents from the UCal/Vienna/Charpentier CRISPR/Cas9 foundational patent application family.
- Filed and prosecuted patent applications covering the company's intellectual property, including the company's LNP delivery system, treatment and analytical methods, and chemical modifications to guide RNA.
- Worked with collaborators to protect jointly developed and licensed intellectual property including RNA guide improvements and disease specific applications.

Expanded our highly qualified expert leadership team by augmenting the Board of Directors and company management, while significantly growing R&D operations and laboratories, in preparation for clinical studies.

- Perry Karsen, formerly CEO of Celgene's Cellular Therapeutics Division, was appointed Chairman of the Board, bringing decades of biopharmaceutical C-suite management and board experience.
- Graeme Bell, formerly CFO of Anacor Pharmaceuticals (acquired by Pfizer in 2016), was named Executive Vice President and Chief Financial Officer, bringing extensive financial and operational experience and strong relationships with the investment community.
- Andrew Schiermeier, formerly Senior Vice President and Global Head of Merck KGaA's Oncology, was named Senior Vice President of eXtellia, responsible for the strategic direction and oversight of all operations within the division. eXtellia is the company's division focused on delivering *ex vivo* therapies in the areas of immuno-oncology and autoimmune/inflammatory

diseases.
- In December 2016, Intellia opened a new state-of-the-art headquarters facility in Cambridge, Massachusetts, increasing the company's total laboratory and office space to more than 80,000 sq. ft.

Full-Year 2016 Financial Results

Cash and cash equivalents at December 31, 2016, were $273.1 million, compared to $75.8 million at December 31, 2015. The increase in cash and cash equivalents was primarily attributable to proceeds from our initial public offering and concurrent private placements in May 2016, as well as a $75.0 million upfront payment from Regeneron in April 2016, partially offset by cash used in operations.

The company is not profitable and has incurred losses in each period since our inception. Our net loss was $31.6 million for the year ended December 31, 2016, and $10.6 million for the fourth quarter of 2016, compared to $12.4 million for the year ended December 31, 2015, and $5.2 million for the fourth quarter of 2015.

Collaboration Revenue

Collaboration revenue was $16.5 million for the year ended December 31, 2016, and $5.6 million for the fourth quarter of 2016, as compared to $6.0 million for the year ended December 31, 2015, and $1.7 million for the fourth quarter of 2015. The increase in collaboration revenue in 2016 was primarily driven by amounts recognized under our collaboration agreement with Regeneron, which was entered into in April 2016.

Through December 31, 2016, the company received $97.0 million in funding under our collaborations with Novartis and Regeneron, excluding amounts received for equity investments, and recorded accounts receivable of $6.5 million as of December 31, 2016. Excluding the $2.6 million of upfront payment received from Novartis that was allocated to the purchase of equity securities, we recognized $22.5 million in collaboration revenue under these agreements through December 31, 2016, and had remaining deferred revenue of $78.3 million as of December 31, 2016.

Operating Expenses

Research and development expenses increased $20.6 million to $31.8 million during the year ended December 31, 2016, as compared to $11.2 million during the year ended December 31, 2015, and increased by $6.9 million to $11.3 million during the fourth quarter of 2016, as compared to $4.4 million during the fourth quarter of 2015. This increase was primarily driven by our growth to 77 research and development employees as of December 31, 2016, from 38 research and development employees as of December 31, 2015, and the advancement of our early-stage research programs collectively resulting in increases in salaries and related compensation expenses, as well as laboratory supplies and research materials.

General and administrative expenses increased by $8.5 million to $16.8 million during the year ended December 31, 2016, as compared to $8.3 million during the year ended December 31, 2015, and increased by $2.3 million to $5.1 million during the fourth quarter of 2016, as compared to $2.8 million during the fourth quarter of 2015.

This increase was primarily related to increased salary and related headcount-based expenses, including equity-based compensation expenses, as the company grew to 27 general and administrative employees as of December 31, 2016, from 14 general and administrative employees as of December 31, 2015, as well as increased corporate insurance, legal, and other professional expenses related to our operations as a public company beginning in May 2016.

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 increase compared to prior periods in connection with our ongoing activities, particularly as research and development and preclinical activities continue, 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 December 31, 2016, the company had an accumulated deficit of $53.6 million. We expect our losses to increase as we continue to incur significant research and development and other expenses related to our ongoing operations, seek regulatory approvals for our future product candidates, scale-up manufacturing capabilities, maintain, expand and protect our intellectual property portfolio, and hire additional personnel to support the development of our product candidates and to enhance our operational, financial and information management systems. 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 December 31, 2016, 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

The company will participate in the following upcoming investor conferences:

- Oppenheimer Healthcare Conference, March 22, 2017, New York City; and
- Needham Healthcare Conference, April 5, 2017, 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” 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 CRISPR/Cas9 into therapeutic products for severe and life-threatening diseases; the potential timing and advancement of our clinical trials; the impact of our collaborations with Novartis and Regeneron on our development programs; the impact of our research relationships with Regeneron and the GENE Consortium on our ability to potentially identify disease targets and possible treatments; the future effects of our invention management agreement on our intellectual property strategy and position; the potential indications we may pursue, including our sentinel indications; the potential timing of regulatory filings regarding our development programs; potential commercialization opportunities for product candidates; our expectations regarding our uses of capital, expense, future accumulated deficient and other 2017 financial results; and our expectations regarding our ability to fund operations through mid-2019. Any forward-looking statements in this press release are based on management's current expectations 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, the risk that any one or more of our product candidates will not be successfully developed and commercialized, the risk that positive results from a preclinical or clinical study may not necessarily be predictive of the results of future preclinical or clinical studies, the risk of cessation or delay of any of the ongoing or planned clinical trials and/or our development of our product candidates, the risk that the results of previously conducted studies involving similar product candidates will not be repeated or observed in ongoing or future studies involving current product candidates, the risk that our collaboration with Novartis or Regeneron will not continue or will not be successful, and risks related to our ability to protect and maintain our intellectual property position.

For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in our 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 our subsequent filings with the Securities and Exchange Commission, including our upcoming Annual Report on Form 10-K for the year ended December 31, 2016. 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 December 31,		Twelve Months Ended December 31,	
	2016	2015	2016	2015
Collaboration revenue	$ 5,627	$ 1,693	$ 16,479	$ 6,044
Operating expenses:				
Research and development	11,331	4,375	31,840	11,170
General and administrative	5,118	2,809	16,798	8,283
Total operating expenses	16,449	7,184	48,638	19,453
Operating loss	(10,822)	(5,491)	(32,159)	(13,409)
Interest income	259	-	525	-
Loss before income taxes	(10,563)	(5,491)	(31,634)	(13,409)
Income tax benefit	-	246	-	1,012
Net loss	$ (10,563)	$ (5,245)	$ (31,634)	$ (12,397)
Net loss per share, basic and diluted	$ (0.31)	$ (9.57)	$ (1.42)	$ (51.02)
Weighted average shares outstanding, basic and diluted	34,507	548	22,222	243

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

	December 31, 2016	December 31, 2015
Cash and cash equivalents	$ 273,064	$ 75,816
Total assets	298,969	82,139
Total liabilities	89,132	14,783
Convertible preferred stock	-	88,557
Total stockholders' equity (deficit)	209,837	(21,201)

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