Intellia Therapeutics Announces First Quarter 2018 Financial Results

May 1, 2018

- *New CEO puts* in vivo *and* ex vivo *genome editing on parallel tracks towards the clinic*
- *Company anticipates submitting its* in vivo *Investigational New Drug application by the end of 2019 for systemic lipid nanoparticle delivery of CRISPR/Cas9 to potentially cure transthyretin amyloidosis*
- *Consistently achieved 60 to 80 percent reduction in transthyretin protein production following a single dose of CRISPR/Cas9 delivered systemically via lipid nanoparticle to hepatocytes of non-human primates*
- *Achieved meaningful levels of genome editing using modular lipid nanoparticle platform technology against various additional liver targets in mice*
- *$328 million in cash and cash equivalents as of March 31, 2018*

CAMBRIDGE, Mass., May 01, 2018 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on the development of curative therapeutics using CRISPR/Cas9 technology, announced financial results and operational progress for the first quarter of 2018.

John Leonard, M.D., was appointed Intellia's President and Chief Executive Officer in the first quarter of 2018, and one of his first initiatives was to broaden the Company's strategy. "We are building the premier CRISPR-based genome editing company with leading *in vivo* and *ex vivo* capabilities," said Dr. Leonard. "We are very pleased with the scientific data generated from our *in vivo* non-human primate (NHP) studies, and the progress with our modular, scalable lipid nanoparticle (LNP) delivery system has allowed us to target a timeframe for our first Investigational New Drug (IND) submission. As we continue to execute on our full spectrum of *in vivo* and *ex vivo* genome editing platforms, we will share progress on our differentiated, wholly owned *ex vivo* approach, starting this month at the American Society of Gene and Cell Therapy Annual Meeting."

The Company announced today that it anticipates submitting an IND application for its lead indication, transthyretin amyloidosis (ATTR), by the end of 2019 and confirms plans to initiate IND-enabling studies in mid-2018. Over the past six months, ongoing NHP studies have demonstrated well-tolerated editing to therapeutically relevant levels of transthyretin (TTR) protein reduction (60 to 80 percent) after a single systemic administration via LNP delivery to NHP hepatocytes. Rates of editing were durable over the six-month period without re-dosing the animals. In support of the proposed IND submission, Intellia has narrowed the field of potential guides to its current development candidate for early human trials. The guide-optimization process used high-throughput screening to evaluate the entire TTR gene for those guides with high levels of activity and undetectable off-target cutting. The Company has completed studies to understand potential dosing regimens and is continuing studies on durability of the effect, both of which may expedite Phase I clinical trials. Intellia has also developed an enhanced LNP formulation through optimization campaigns that is currently being tested for multiple follow-on liver indications, and anticipates that this modular approach may minimize development timelines for each additional and subsequent liver-targeted product candidate.

Intellia has also demonstrated continued progression of its modular liver platform capability to knockout various targets of interest in the livers of mice, including SERPINA1 for alpha-1 antitrypsin deficiency (AATD) and HAO1 for primary hyperoxaluria type 1 (PH1), each of which has resulted in protein expression reductions believed to be therapeutically relevant. This initial knockout edit in AATD lays the groundwork for developing an approach that restores production of the missing protein in AATD, required for the amelioration of the disease.

The table below shows editing rates and corresponding protein reductions in the livers of mice for ATTR, AATD and PH1. ATTR and AATD both produce aberrant proteins hence treatment of these conditions requires reductions in the level of the disease-causing proteins. PH1 results from the low level activity of a particular protein for which treatment requires reducing the levels of substrate for that defective protein to metabolize, achieved by knocking out the gene that encodes HAO1. In each of these three cases, Intellia's modular LNP delivery system achieved high levels of reduction of the targeted protein. These initial editing rates and corresponding protein reductions are evidence of Intellia's ability to successfully target monogenic liver diseases by knocking out harmful genetic mutations.

Target	Indication	% editing in mouse model	% protein reduction
TTR	Transthyretin amyloidosis	70%	97%
SERPINA1	Alpha-1 antitrypsin deficiency	85%	95%
HAO1	Primary hyperoxaluria type 1	74%	90%

Beyond the liver, the Company continues to advance its application of CRISPR/Cas9 technology to the central nervous system (CNS), including through its collaboration with Beverly Davidson, Ph.D., of the Children's Hospital of Philadelphia, who will share updated LNP delivery data in a presentation at the American Society of Gene and Cell Therapy Annual Meeting later this month.

In *ex vivo* applications, Intellia seeks to develop allogeneic cellular therapies, which are cells derived from unmatched tissue donors, which are modified outside of the human body to allow them to be administered to an unrelated patient. This endeavor is supported through multiple efforts, including recently acquired access to intellectual property from researchers at the Karolinska Institutet and Intellia's collaboration with Ospedale San Raffaele, announced in June of 2017.

In February of 2018, *Cell Reports* published Intellia's first peer-reviewed paper entitled "A single administration of CRISPR/Cas9 lipid nanoparticles achieves robust and persistent *in vivo* genome editing." This landmark paper documented Intellia's delivery of Cas9 mRNA and single guide RNA using its proprietary LNPs to achieve a 97 percent reduction in mouse TTR protein levels in the liver, which was sustained for at least 12 months.

During the course of 2018, Intellia plans to share additional preclinical data on its TTR genome editing program, including the achievement of a near ten-fold reduction in the required dose, derived via improvements in potency, as well as other knockout targets and data on delivery via LNPs to the CNS of NHPs. Additionally, Intellia plans to share preclinical data on both immuno-oncology and autoimmune disease targets in 2018.

First Quarter 2018 Financial Results

Collaboration Revenue

Collaboration revenue was $7.5 million for the first quarter of 2018, compared to $6.2 million during the first quarter of 2017. The increase in collaboration revenue in 2018 was primarily driven by amounts recognized under Intellia's collaboration agreement with Regeneron.

Since inception through March 31, 2018, the Company has received $112.1 million in funding from the collaborations with Novartis and Regeneron, excluding amounts received for equity investments, and had an accounts receivable balance of $7.5 million at March 31, 2018.

Operating Expenses

Research and development expenses increased by $9.1 million to $22.5 million during the first quarter of 2018, compared to $13.4 million during the first quarter of 2017. 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 and research materials such as reagents.

General and administrative expenses increased by $1.7 million to $7.4 million during the first quarter of 2018, compared to $5.7 million during the first quarter of 2017. This increase was driven primarily by increased salary and related headcount-based expenses to support Intellia's larger research and development organization, public company compliance, and administrative obligations.

The Company's net loss was $21.4 million for the first quarter of 2018, compared to $12.6 million during the first quarter of 2017.

Balance Sheet

Cash and cash equivalents at March 31, 2018, were $327.8 million, compared to $340.7 million at December 31, 2017.

Financial Guidance

The Company's primary uses of capital will continue to be for 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 March 31, 2018, the Company had an accumulated deficit of $137.0 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 of the Company's programs, the Company expects that the cash and cash equivalents as of March 31, 2018, 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.

Upcoming Events During the Second Quarter 2018

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

- The American Society of Gene and Cell Therapy Annual Meeting, May 16, Chicago
- Jefferies Global Health Care Conference, June 5, New York City
- JMP Securities Life Sciences Conference, June 20, New York City

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on the development of 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 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 and 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 conduct successful IND-enabling studies of a lead ATTR development candidate and subsequently submitting an IND application by the end of 2019 that will be accepted by the regulatory agencies; 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 not be predictive of future results in connection with future studies; and the risk that Intellia's collaborations with Novartis or Regeneron or its other *ex vivo* collaborations 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 (UNAUDITED)
(Amounts in thousands, except per share data)

	Three Months Ended March 31,	
	2018	**2017**
Collaboration revenue	$ 7,469	$ 6,215
Operating expenses:		
Research and development	22,493	13,431
General and administrative	7,406	5,732
Total operating expenses	29,899	19,163
Operating loss	(22,430)	(12,948)
Interest income	1,074	317
Net loss	$ (21,356)	$ (12,631)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.51)	$ (0.36)
Weighted average shares outstanding, basic and diluted	42,043	34,723

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

	March 31, 2018	**December 31, 2017**
Cash and cash equivalents	$ 327,778	$ 340,678
Total assets	359,545	376,235
Total liabilities	64,046	75,638
Total stockholders' equity	295,499	300,597

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