



Intellia Therapeutics Announces Second Quarter 2018 Financial Results

August 1, 2018

- Continued to progress lead *in vivo* program in transthyretin amyloidosis targeting submission of an Investigational New Drug application by the end of 2019
- Announced first wholly owned *ex vivo* T cell receptor program targeting a unique epitope of Wilms' Tumor 1 protein for the treatment of acute myeloid leukemia
- Strengthened foundational CRISPR/Cas9 genome editing intellectual property position through granting of new patent in the U.S.
- Reported \$306 million in cash and cash equivalents as of June 30, 2018

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

"In the first half of 2018, we focused on executing against our priorities, including initiating IND-enabling studies for our lead *in vivo* liver program, demonstrating that our lipid nanoparticle delivery platform has broad utility, and advancing our *ex vivo* R&D efforts across our key collaborations. We are excited about our progress, and we remain focused on achieving our 2018 goals," said Intellia President and Chief Executive Officer John Leonard, M.D.

Second Quarter 2018 Operational Highlights

The Company achieved several key operational milestones during the second quarter of 2018, including the following:

- Intellia continued progress in non-human primate (NHP) dose-ranging studies that support the Company's lead *in vivo* program in transthyretin amyloidosis (ATTR).
- At the 21st Annual Meeting of the American Society of Gene and Cell Therapy in Chicago, Intellia and research collaborator, Ospedale San Raffaele, announced the identification of T cell receptors (TCRs) targeting a Wilms' Tumor 1 protein (WT1) epitope, important for the treatment of acute myeloid leukemia as Intellia's first *ex vivo* program. TCRs that target WT1 may also have applicability in solid tumors and other hematological malignancies, as WT1 is over-expressed on many tumor types. The Company plans to develop these TCRs as part of its first *ex vivo* product candidate. Intellia anticipates that this program will benefit from ongoing work to develop a truly allogeneic approach for engineered cellular therapy.
- In the second quarter, Intellia progressed *ex vivo* multiplexing efforts to achieve triple knockout edits with greater than 80 percent efficiency in human cells, and observed insertion efficacy of ~50 percent with simultaneous double knockout edits. Capabilities in multiplexing will support and advance *ex vivo* efforts within the Company's expanding engineered cell therapy pipeline.
- Expanding on the *in vivo* liver editing achievements announced earlier this year, the Company progressed its primary hyperoxaluria type I (PH1) program utilizing a phenotypic mouse model of the disease. In PH1, excess oxalate produced in the liver crystallizes and accumulates in various organs eventually causing kidney failure. A knockout of the *HAO1* gene reduces levels of glyoxylate, a precursor to urinary oxalate, and thereby reduces oxalate accumulation. In a mouse model of the disease, the Company achieved 74 percent editing of *HAO1* leading to a ~90 percent protein reduction and a ~55 percent reduction in urinary oxalate after a single dose. This progress reinforces the value and speed of Intellia's modular lipid nanoparticle delivery platform, including efficient and effective delivery to hepatocytes in the liver.
- Intellia continued to expand its fully automated, next-generation sequencing and bioinformatics platform to support the identification of highly active guides with limited to no off-target cutting or unforeseen deletions. In the second quarter, Intellia increased throughput capacity to process greater than 30,000 sequencing samples per week. This capability enables measuring of on- and off-target genome editing, including indels, translocations, excisions and inversions.
- Intellia, along with other licensees, announced in June that the U.S. Patent and Trademark Office (USPTO) granted U.S. Patent No. 10,000,772 ("the '772 patent") to The Regents of the University of California, the University of Vienna and Emmanuelle Charpentier, Ph.D. (collectively, "UC"), co-owners of foundational intellectual property relating to CRISPR/Cas9 genome editing technology. The '772 patent covers the use of single- and dual-guide RNA formats having certain structural motifs in a region that interacts with the Cas9 enzyme. These guide RNA formats are widely used in the CRISPR/Cas9 field. Intellia anticipates this is the first of many patents to be granted in the U.S. to UC for the

CRISPR/Cas9 genome editing intellectual property. Outside of the U.S., UC continues to hold a strong global intellectual property position with applications from this patent estate issued in Europe, the United Kingdom, China, Japan, Australia and various other countries worldwide. The '772 patent is not involved in the appeal to the U.S. Court of Appeals for the Federal Circuit (CAFC) relating to the February 2017 interference decision from the USPTO's Patent Trial and Appeal Board. UC's appeal was heard on April 30, 2018 by the CAFC, and a decision is still pending.

Upcoming Milestones

- For the remainder of 2018, Intellia's expected milestones include the following:
 - Advance a second liver knockout target in NHPs;
 - Advance candidates for a second liver indication;
 - Present additional editing data supporting more complex edits such as insertion and repair;
 - Prepare for a pre-Investigational New Drug meeting with the U.S. Food and Drug Administration for ATTR;
 - Expand preclinical data in support of Intellia's first proprietary *ex vivo* autoimmune program; and
 - Identify Intellia's first hematopoietic stem cell target from the collaboration with Novartis.

Second Quarter 2018 Financial Results

Collaboration Revenue

Collaboration revenue was \$7.7 million for the second quarter of 2018, compared to \$5.9 million during the second 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 June 30, 2018, the Company has received \$114.1 million in funding from the collaborations with Novartis and Regeneron, excluding amounts received for equity investments, and had an accounts receivable balance of \$8.6 million on June 30, 2018.

Operating Expenses

Research and development expenses increased by \$7.9 million to \$23.5 million during the second quarter of 2018, compared to \$15.6 million during the second 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 \$1.4 million to \$7.8 million during the second quarter of 2018, compared to \$6.4 million during the second 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 \$22.2 million for the second quarter of 2018, compared to \$15.6 million during the second quarter of 2017.

Balance Sheet

Cash and cash equivalents at June 30, 2018, were \$305.5 million, compared to \$241.0 million for second quarter in 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, intellectual property related costs, and general operating costs.

As of June 30, 2018, the Company had an accumulated deficit of \$159.3 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 June 30, 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 Third Quarter 2018

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

- B. Riley FBR Health Care Conference, Sept. 4, New York City
- Citi Biotech Conference, Sept. 5, Boston
- Wells Fargo Health Care Conference, Sept. 6, Boston
- Jefferies Gene Therapy Summit, Sept. 27, New York City

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing 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 improved 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; the potential timing and advancement of our preclinical studies, including non-human primate studies for our ATTR program and a second liver knockout program, 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 therapies of all types, and those targeting WT1 in particular, using CRISPR/Cas9 technology; our intent to present additional data for organs beyond the liver, *ex vivo* and *in vivo* therapeutics, additional insertion/repair data, and preclinical data in support of our first *ex vivo* programs on immuno-oncology and autoimmune/inflammation indications during 2018; the expansion of our fully automated bioinformatics platform; our ability to advance candidates for a second liver indication by late 2018; our potential ability to conduct a pre-IND meeting with the FDA for ATTR; the intellectual property position and strategy of Intellia and its 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 be predictive of future results in connection with future studies; and the risk that Intellia's 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 and quarterly reports 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,	
	2018	2017	2018	2017
Collaboration revenue	\$ 7,677	\$ 5,917	\$ 15,146	\$ 12,132
Operating expenses:				
Research and development	23,467	15,565	45,960	28,996
General and administrative	7,805	6,369	15,211	12,101
Total operating expenses	31,272	21,934	61,171	41,097
Operating loss	(23,595)	(16,017)	(46,025)	(28,965)
Interest income	1,376	424	2,450	741
Net loss	\$ (22,219)	\$ (15,593)	\$ (43,575)	\$ (28,224)
Net loss per share, basic and diluted	\$ (0.52)	\$ (0.45)	\$ (1.03)	\$ (0.81)
Weighted average shares outstanding, basic and diluted	42,836	34,916	42,441	34,820

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

	June 30, 2018	December 31, 2017
Cash and cash equivalents	\$ 305,538	\$ 340,678
Total assets	339,778	376,235
Total liabilities	60,442	75,638
Total stockholders' equity	279,336	300,597

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