Intellia Therapeutics Announces Fourth Quarter and Full-Year 2018 Financial Results

February 27, 2019

- *Confirmed improved non-human primate liver editing and protein reduction of greater than 95 percent in transthyretin amyloidosis program; investigational new drug application on track for 2020 submission*
- *Engineered cell therapy platform will enable wholly owned development candidate for acute myeloid leukemia by year end*
- *Demonstrated targeted insertion and genome editing in the liver of mice using proprietary DNA template designs*
- *Ended 2018 with a strong cash position of $314 million*

CAMBRIDGE, Mass., Feb. 27, 2019 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology in both *in vivo* and *ex vivo* applications, today reported operational highlights and financial results for the fourth quarter and year ended December 31, 2018. In addition, Intellia highlighted select corporate milestones for 2019 and upcoming events for the first quarter of 2019.

"We are delivering on our full-spectrum strategy, and we expect to have two candidates in development in 2019. Our *in vivo* transthyretin amyloidosis program is on track for IND filing in 2020, and by the end of this year we anticipate having our first engineered cell therapy development candidate targeting acute myeloid leukemia," said Intellia President and Chief Executive Officer John Leonard, M.D. "We have shown that a single lipid nanoparticle administration can produce very substantial transthyretin protein reduction in non-human primates, reaching levels that we believe hold great therapeutic promise for patients. We also look forward to sharing additional data on our acute myeloid leukemia program this year. We believe our TCR-based, CRISPR-engineered cell therapy will provide a much-needed option for patients."

Recent Operational Highlights

- **ATTR Program:** During the fourth quarter, **Intellia completed confirmatory non-human primate (NHP) studies**, previewed in October, using its lead candidate for the treatment of transthyretin amyloidosis (ATTR). Fifty-one days post infusion, these NHP studies verified a favorable tolerability profile across various dose levels, and a **near-complete (average of >95 percent) reduction in circulating transthyretin (TTR) protein in the liver**. The improvements in TTR protein reduction were the result of certain modifications made to the lipid nanoparticle (LNP) cargo components of the therapy, and these modifications have been incorporated into the ongoing, dose-range finding studies and scale-up activities. These modifications will also have application in subsequent programs. The ATTR program is being co-developed with Regeneron Pharmaceuticals, Inc. (Regeneron) with Intellia being the lead party. Intellia confirmed that it is on track for submitting an Investigational New Drug (IND) application in 2020 for ATTR.
- ***In Vivo* Insertion:** In October, Intellia **demonstrated *in vivo* CRISPR-mediated, targeted transgene insertion in mouse liver**. Data shared at the Annual Congress of the European Society of Gene and Cell Therapy highlighted the use of Intellia's **bi-directional DNA template**, delivered in a proprietary hybrid LNP/adeno-associated viral (AAV) template delivery system. Insertion of the human *F9* gene, the gene encoding the protein deficient in hemophilia B, yielded Factor IX protein levels in mice at or above therapeutic targets in patients. This work was done in collaboration with Regeneron. Additionally, the Company demonstrated the versatility of the hybrid LNP/AAV approach by successfully inserting a functional human *SERPINA1* gene (encoding alpha-1 antitrypsin) into the same locus. These experiments in mice yielded human protein expression levels consistent with those of normal individuals without alpha-1 antitrypsin deficiency.
- **Engineered Cell Therapies:** As part of the broad engineered cell therapy platform that the Company is developing, Intellia and its partner, Ospedale San Raffaele, isolated novel active T cell receptors (TCRs) recognizing an epitope of the Wilms' Tumor 1 (WT1) protein. This protein is overexpressed in many blood cancers, as well as in solid tumors. T cells modified with these TCRs successfully **killed acute myeloid leukemia (AML) blasts in an *in vitro* model. This work will be the foundation for the Company's first wholly owned *ex vivo* development candidate for the treatment of AML.**
- **Novartis:** In December, Intellia announced an **expansion of the existing cell therapy collaboration with Novartis Institutes for Biomedical Research, Inc. (Novartis) to include ocular stem cells.** The Company received a $10 million payment from Novartis in relation to the inclusion of this cell type. Regarding its proprietary LNP delivery system and improvements, Intellia also obtained expanded access to Novartis' LNP library, including the rights to use these lipids for *in vivo* or *ex vivo* applications in any genome editing technology.
- **Board of Directors:** In January of 2019, Intellia **appointed Fred Cohen, M.D., D.Phil, F.A.C.P., to its board of directors**, adding both biotechnology drug development experience and extensive medical expertise to the current knowledge base of the Company's board.

Upcoming Milestones

The Company has set forth the following for 2019 pipeline progression:

- ATTR: Complete dose-range finding studies, initiate IND-enabling toxicology studies and commence manufacturing of lipid and CRISPR/Cas9 cargo
- Engineered Cell Therapy: Nominate first engineered cell therapy development candidate for acute myeloid leukemia by the

end of 2019
- Present additional *in vivo* NHP insertion data and ATTR formulation improvement data at upcoming scientific conferences

Upcoming Events

The Company will participate in the following investor events:

- Leerink Healthcare Conference, February 28, New York City
- Barclays Global Healthcare Conference, March 12, Miami

Fourth Quarter and Full Year 2018 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $314.1 million as of December 31, 2018, compared to $340.7 million as of December 31, 2017. The decrease was driven by cash used to fund operations of approximately $96 million, which was offset in part by $28.5 million of net equity proceeds raised from the Company's "At the Market" (ATM) agreement, $11.7 million in proceeds from employee-based stock plans, $10.4 million of ATTR cost reimbursements made by Regeneron, and $19.0 million of funding received under the Novartis collaboration. The Novartis funding received included a $10.0 million upfront payment related to the expansion of the existing collaboration in the fourth quarter of 2018.
- **Collaboration Revenue:** Collaboration revenue increased by $1.2 million to $7.9 million during the fourth quarter of 2018, compared to $6.7 million during the fourth quarter of 2017. The increase in collaboration revenue in 2018 was primarily driven by amounts recognized from the expansion of the existing collaboration with Novartis, as well as by amounts recognized under the Company's ATTR Co/Co agreement with Regeneron. As previously disclosed, Regeneron is obligated to fund approximately 50 percent of the development costs for the ATTR program.
- **R&D Expenses:** Research and development expenses decreased by $1.3 million to $19.9 million during the fourth quarter of 2018, compared to $21.2 million during the fourth quarter of 2017. This decrease was driven primarily by lower consumable costs, as well as the timing of general R&D expenses.
- **G&A Expenses:** General and administrative expenses decreased by $1.5 million to $8.7 million during the fourth quarter of 2018, compared to $10.2 million during the fourth quarter of 2017. This decrease was driven primarily by a decrease in stock-based compensation.
- **Net Loss:** The Company's net loss was $19.1 million for the fourth quarter of 2018, compared to $24.0 million during the fourth quarter of 2017.

Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of December 31, 2018, as well as technology access and funding from Novartis and Regeneron, will enable Intellia to fund its anticipated operating expenses and capital expenditure requirements into the first half of 2021. This expectation excludes 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.

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing 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, or can replace 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 Therapeutics, Inc. ("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 intellectual property portfolio; our ability to achieve stable or effective genome editing; our ability to administer our CRISPR/Cas9 product candidates; the potential timing and advancement of our preclinical studies, including continuing non-human primate studies for our ATTR program and other programs (such as alpha-1 antitrypsin deficiency (AATD) and AML), and clinical trials; the timing and potential achievement of milestones to advance our pipeline including nominating development candidates and filing INDs; our ability to replicate or apply results achieved in our preclinical studies, including those in our ATTR, AATD and AML programs, in any future studies, including human clinical trials; the potential development of our proprietary LNP/AAV hybrid delivery system to advance our complex genome editing capabilities; the potential development of other *in vivo* or *ex vivo* cell therapeutics of all types, and those targeting WT1 in AML in particular, using CRISPR/Cas9 technology; our ability to conduct successful IND-enabling studies of a lead ATTR development candidate and subsequently submitting an IND application in 2020 that will be accepted by the regulatory agencies; our intent to present additional ATTR data, additional insertion/repair data, and other preclinical data during 2019 or thereafter; our ability to advance a development candidate for a second indication by late 2019 or thereafter; our plans to commence manufacturing efforts in 2019; the intellectual property position and strategy of Intellia's licensors, or other parties from which it derives rights, as well as third-parties and competitors; actions by government agencies; our growth as a company and the anticipated contribution of the members of our board of directors and our executives to our operations and progress; 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 into the first half of 2021.

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 Intellia's relationship with third parties, including our licensors; 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 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 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,	
	2018	2017	2018	2017
Collaboration revenue	$ 7,880	$ 6,668	$ 30,434	$ 26,117
Operating expenses:				
Research and development	19,918	21,170	89,115	67,647
General and administrative	8,708	10,213	32,189	28,025
Total operating expenses	28,626	31,383	121,304	95,672
Operating loss	(20,746)	(24,715)	(90,870)	(69,555)
Interest income	1,680	752	5,527	2,012
Net loss	$ (19,066)	$ (23,963)	$ (85,343)	$ (67,543)
Net loss per share, basic and diluted	$ (0.43)	$ (0.61)	$ (1.98)	$ (1.88)
Weighted average shares outstanding, basic and diluted	44,215	39,155	43,069	36,006

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

	December 31, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 314,059	$ 340,678
Total assets	347,315	376,235
Total liabilities	69,395	75,638
Total stockholders' equity	277,920	300,597

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