Intellia Therapeutics Highlights Recent Progress and Anticipated 2020 Milestones

January 9, 2020

- *On track to submit IND application for lead candidate, NTLA-2001 for transthyretin amyloidosis, in mid-2020 and dose first patients in 2H 2020*
- *Nominated NTLA-5001 for the treatment of acute myeloid leukemia as first WT1-TCR-directed engineered cell therapy development candidate; plan to submit IND in 1H 2021*
- *Announced third development program, which will be for treatment of hereditary angioedema; expects to nominate a development candidate in 1H 2020*
- *Ended 2019 with strong cash position of $284 million; cash runway extended through YE 2021*

CAMBRIDGE, Mass., Jan. 09, 2020 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ: NTLA), a leading genome editing company focused on the development of curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, today provided an update on recent progress and the Company's 2020 priorities and expected milestones.

"2020 will be a significant year for Intellia, as we execute on our full-spectrum strategy. With milestones anticipated across our pipeline, we are making important progress towards the development of curative treatments for severe diseases. In particular, we expect to dose ATTR patients with the first-ever systemically delivered CRISPR/Cas9-based therapy this year, and we are beginning IND-enabling activities for our newly announced development candidate, NTLA-5001, a WT1-TCR-directed engineered cell therapy, for treatment of AML," said Intellia President and Chief Executive Officer, John Leonard, M.D. "We are focused on developing a robust platform with modular genome editing capabilities that enable a fast and reproducible path to development. Today's update reflects this strategy, and it also features the announcement of our third development program, an *in vivo* knockout approach for HAE. Importantly, this program leverages the infrastructure and insights from NTLA-2001 and underscores our ability to produce a rapid succession of new clinical candidates. We are excited by the strong momentum across our diverse pipeline and look forward to providing updates on our development programs in the upcoming year."

Program Updates and Anticipated 2020 Milestones:

- **ATTR Program**: Intellia remains on track to submit an investigational new drug (IND) application in mid-2020 for its lead *in vivo* candidate, NTLA-2001, for treatment of transthyretin amyloidosis (ATTR). NTLA-2001 is anticipated to be the first systemically delivered CRISPR/Cas9 therapy to enter the clinic, and Intellia anticipates dosing the first patients in the second half of 2020. In addition, Intellia completed a 12-month durability study of its lead lipid nanoparticle (LNP) formulation in support of NTLA-2001, maintaining an average reduction of >95% of serum transthyretin (TTR) protein and sustained liver genome editing after a single dose in non-human primates (NHPs). NTLA-2001 is part of a co-development/co-promotion (Co/Co) agreement between Intellia, which is the lead development and commercialization party, and Regeneron Pharmaceuticals, Inc. (Regeneron). Intellia and Regeneron have a 75% and 25% share of worldwide development costs and profits, respectively.
- **AML Program**: Intellia today announced NTLA-5001 as its first engineered T cell therapy development candidate, utilizing its T cell receptor (TCR)-directed approach to target the Wilms' Tumor 1 (WT1) intracellular antigen for the treatment of acute myeloid leukemia (AML). Intellia's WT1-TCR-directed approach aims to develop a broadly applicable treatment for AML patients, regardless of mutational background of a patient's leukemia. This approach employs CRISPR/Cas9 to efficiently knock out and replace the endogenous TCR with a natural, high affinity therapeutic TCR. The resulting cells are capable of specific and potent killing of AML blasts, and have no detectable bone marrow cell toxicity. The Company expects to present preclinical data in support of NTLA-5001 at an upcoming scientific meeting in the first quarter of 2020 and plans to submit an IND application in the first half of 2021. Additional efforts are underway to evaluate the potential use of the WT1-TCR construct to treat other tumor types, including solid tumors.
- **HAE Program**: Today, Intellia announced that the Company is committed to developing a CRISPR/Cas9-based therapy for hereditary angioedema (HAE) as its third development program. HAE is a rare genetic disorder characterized by recurring and unpredictable severe swelling attacks in various parts of the body, and is significantly debilitating or even fatal in certain cases. The disease is caused by increased levels of bradykinin, a protein which leads to swelling. Most patients with HAE have a C1 esterase inhibitor (C1-INH) protein deficiency, which normally prevents the unregulated release and

buildup of bradykinin. Using its modular LNP-based CRISPR/Cas9 delivery system, Intellia aims to knock out the *kallikrein B1 (KLKB1)* gene, which is part of a biological pathway that results in release of bradykinin. Knocking out this gene should reduce the undesired bradykinin activity in HAE patients. The Company plans to present preclinical data at an upcoming scientific meeting in the first quarter of 2020. In addition, Intellia is evaluating several potential guide RNAs and expects to nominate a development candidate in the first half of 2020. Intellia's *KLKB1* HAE program is subject to an option by Regeneron to enter into a Co/Co agreement, in which Intellia would remain the lead party.

Cash Position and Financial Guidance:

- Intellia ended the fourth quarter of 2019 with approximately $284.5 million in cash, cash equivalents and marketable securities. Intellia expects that its cash, cash equivalents and marketable securities as of December 31, 2019 will enable the Company to fund its anticipated operating expenses and capital expenditure requirements at least through the end of 2021. This expectation excludes any strategic use of capital not currently in the Company's base-case planning assumptions.

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, 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" or the "Company") 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 beliefs and expectations regarding its planned submission of an investigational new drug ("IND") application for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR") in mid-2020; its plans to submit an IND application for NTLA-5001, its first T cell receptor ("TCR")-directed engineered cell therapy development candidate for its acute myeloid leukemia ("AML") program in the first half of 2021; its plans to nominate a development candidate for its hereditary angioedema ("HAE") program in the first half of 2020; its plans to advance and complete preclinical studies, including non-human primate studies for its ATTR program, AML program, HAE program and other in vivo and ex vivo programs; its presentation of additional data at upcoming scientific conferences, and other preclinical data in 2020; the advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, as well as maintain and expand its related intellectual property portfolio; the ability to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies, including those in its ATTR, AML and HAE programs, in any future studies, including human clinical trials; its ability to develop other in vivo or ex vivo cell therapeutics of all types, and those targeting WT1 in AML in particular, using CRISPR/Cas9 technology; its business plans and objectives for its preclinical studies and clinical trials, including the therapeutic potential and clinical benefits thereof, as well as the potential patient populations that may be addressed by its ATTR program, AML program, HAE program and other in vivo and ex vivo programs; the impact of its collaborations on its development programs, including but not limited to its collaboration with Regeneron Pharmaceuticals, Inc. ("Regeneron") and Regeneron's ability to enter into a Co/Co agreement for the HAE program; statements regarding the timing of regulatory filings for its development programs; its use of capital, including expenses, future accumulated deficit and other financial results during 2019 or in the future; and the ability to fund operations through the end 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; and the risk that the results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies. 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 Contacts:

Investors:
Lina Li
Associate Director
Investor Relations
+1 857-706-1612
lina.li@intelliatx.com

Media:
Jennifer Mound Smoter
Senior Vice President
External Affairs & Communications
+1 857-706-1071
jenn.smoter@intelliatx.com

Source: Intellia Therapeutics, Inc.