Intellia Therapeutics Announces Publication in Cell Reports of Preclinical Data Demonstrating Effective CRISPR/Cas9 Genome Editing Using Lipid Nanoparticle (LNP) Delivery Technology

February 27, 2018

- *Intellia researchers achieved 97 percent reduction in serum transthyretin protein levels in mice through a single administration, with a durability of at least 12 months*
- *CRISPR/Cas9 administered via LNP was well-tolerated with no observed adverse events*
- *CRISPR/Cas9 components shown to be undetectable three days after administration*

CAMBRIDGE, Mass., Feb. 27, 2018 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company developing curative therapeutics using CRISPR/Cas9 technology, announced that *Cell Reports* will publish at noon ET today its manuscript, "A single administration of CRISPR/Cas9 lipid nanoparticles achieves robust and persistent *in vivo* genome editing."

The lipid nanoparticle (LNP) delivery of Cas9 mRNA and sgRNA resulted in 97 percent reduction in mouse transthyretin (TTR) protein levels in the liver, and the reduction was sustained for at least 12 months. The publication also documents that CRISPR/Cas9 components were undetectable in mice within three days after administration of Intellia's LNP delivery system. Researchers further demonstrated that Intellia's LNP technology is a similarly robust and effective delivery method for CRISPR/Cas9-mediated knockdown in rats, a higher rodent species.

"These data show that our proprietary lipid nanoparticle technology achieves significant and enduring editing of the *TTR* gene through a single dose," said David Morrissey, Ph.D., senior vice president, Platform and Delivery Technology, at Intellia. "Our lipid nanoparticle system is a transient expression system that enables CRISPR/Cas9 to make the intended gene edit and then clear from the cells. Minimizing the duration of CRISPR/Cas9 components in cells is desirable, as that may reduce the potential for safety issues associated with the continued presence of those components. The LNPs also allow us to re-dose, if needed, to attain the desired target effect. This paper details the most effective systemic delivery of CRISPR/Cas9 components reported to date, further supporting our IND-enabling activities this year and future potential treatments for liver-based genetic diseases."

The data included in this publication build on earlier findings initially released last year at the Le Stadium Conference on Messenger RNA Therapeutics, and later presented at the 20th Annual Meeting of the American Society of Gene and Cell Therapy and the 13th Annual Meeting of the Oligonucleotide Therapeutics Society.

About Intellia's transthyretin amyloidosis (ATTR) program

Transthyretin amyloidosis (ATTR) is a slowly progressive and debilitating disease caused by one of approximately 136 different inherited mutations in the *TTR* gene. Abnormal protein deposits caused by one of these genetic mutations may affect both the peripheral and autonomic nervous systems, resulting in a variety of symptoms that develop in people as early as age 20. (Sources: Amyloidosis Foundation and National Institutes of Health)

Intellia's sentinel *in vivo* programs focus on the use of lipid nanoparticles (LNPs) for delivery of CRISPR/Cas9 components to the liver. The company's lead *in vivo* program targets ATTR and is being co-developed with Regeneron Pharmaceuticals, Inc. Intellia aims to achieve knockout editing of specific DNA in mutated *TTR* genes within hepatocytes that cause damaging transthyretin protein deposits in heart, nerves and other tissues in the body. Non-human primate studies are ongoing and are anticipated to lead to IND-enabling activities in 2018.

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. 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. Forward-looking statement in this press release include, but are not limited to, express or implied statements regarding the successful development of CRISPR/Cas9-based therapies to treat diseases, including transthyretin amyloidosis (ATTR); the translation of the results from animal models to human therapies; and the Company's or its collaborator's ability and intention to develop, seek regulatory approval for, and commercialize therapies to treat disease using CRISPR/Cas9. Any forward-looking statements in this press release are uncertain, based on management's current expectations of future events, occurrences, actions and plans, and subject to various 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. The Company may not actually execute or obtain the results from the plans, goals, efforts or opportunities disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, goals, efforts or opportunities disclosed in these forward-looking statements as a result of various factors including: uncertainties inherent in the implementation and execution of preclinical studies and clinical trials, and preclinical and clinical development of the Company's or its collaborator's products candidates; availability and timing of results from preclinical studies and clinical trials; whether interim results from a preclinical trial will be predictive of the final results of the preclinical or clinical trials or the results of future trials; expectations and requirements for regulatory approvals to conduct trials or to market products; and availability of funding sufficient for the Company's or its collaborator's foreseeable and unforeseeable operating expenses and capital expenditure requirements. For a discussion of 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 other filings with the Securities and Exchange Commission.

All information and forward-looking statements in this press release are as of the date of the release, and Intellia Therapeutics undertakes no duty to update this information, whether because of new information, future events or otherwise, unless required by law.

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Source: Intellia Therapeutics, Inc.