Intellia Therapeutics Demonstrates Pioneering CRISPR/Cas9 Genome Editing Efficiency Data Using Lipid Nanoparticle Delivery Technology

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- *First to demonstrate single dose, in vivo results, showing approximately a 97 percent reduction in serum transthyretin protein levels*
- *Durability data show stable liver editing for at least four months*
- *Increased liver editing efficiency reported to date at 70 percent, following a single dose*

CAMBRIDGE, Mass., March 08, 2017 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on the development of potentially curative therapies, today reported updated data showing increased levels of genome editing efficiency *in vivo* and durability results with its CRISPR/Cas9 technology, following a single administration. Using its lipid nanoparticle (LNP) technology, Intellia achieved approximately a 97 percent reduction in serum transthyretin (TTR) protein driven by 70 percent gene editing efficiency in the mouse liver.

"These results are extremely promising, as they demonstrate compelling activity with lipid nanoparticles observed in the liver following a single dose," said Executive Vice President, R&D John Leonard, M.D. "We are excited by the extent of the effect, which confirms the power of CRISPR/Cas9 for potential therapeutic uses. These high levels of gene editing are the result of Intellia's effort to identify highly efficient delivery of CRISPR/Cas9 using lipid nanoparticles. The data advance our efforts as we look to transform the way we treat disease."

The data are being presented for the first time at the Cowen and Company 37th Annual Health Care Conference in Boston on Wednesday, March 8, 2017 at 8:40 am ET. Complete data are being presented on March 22, 2017 at the Le Stadium Conference on Messenger RNA Therapeutics in Orleans, France. Data showed robust editing and sustained results including:

- Progress in achieving *in vivo* gene editing in the TTR locus, with an efficiency of approximately 70 percent in the total mouse liver at the target DNA site, after a single intravenous administration (versus previously reported 60 percent);
- An associated decrease in serum TTR protein levels of up to approximately 97 percent (versus previously reported 80 percent);
- Undetectable Cas9 mRNA and guide RNA (gRNA) in the liver for 72 hours post administration; and
- Durable and stable liver editing for at least four months following a single administration.

Study Background

The ongoing, preclinical editing studies were designed to explore the use of lipid nanoparticles for delivery of CRISPR/Cas9 components to the liver in mice and to mediate editing of target DNA within hepatocytes. For the LNPs in the studies, Cas9 mRNA was co-formulated with chemically synthesized gRNAs targeting the mouse TTR gene, and administered via one intravenous tail vein injection. Additional studies were performed to evaluate the impact of editing of variables in guide format, degree of guide chemical modification, and dose response on editing efficiency. The durability of the liver editing was evaluated through a four-month time period, and pharmacokinetic (PK) parameters for Cas9 mRNA and sgRNA were measured.

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company, focused on the development of proprietary, potentially 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. These forward looking statements include, but are not limited to, express or implied statements regarding our ability to advance CRISPR/Cas9 into therapeutic products for severe and life-threatening diseases; the potential timing and advancement of our clinical trials; the potential targets or indications we may pursue; and potential commercialization opportunities for product candidates. Any forward-looking statements in this press release are based on management's current expectations 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, the risk that any one or more of our product candidates will not be successfully developed and commercialized, the risk that positive results from a preclinical or clinical study may not necessarily be predictive of the results of future preclinical or clinical studies, risks related to our ability to protect and maintain our intellectual property position, the risk of cessation or delay of any of the ongoing or planned clinical trials and/or our development of our product candidates, the risk that the results of previously conducted studies involving similar product candidates will not be repeated or observed in ongoing or future studies involving current product candidates, and the risk that our collaboration with Novartis or Regeneron will not continue or will not be successful. For a discussion of other 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 subsequent 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.

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