Intellia Therapeutics Announces Progress with CRISPR/Cas9 at the American Society of Gene & Cell Therapy Annual Meeting

May 13, 2017

- *First-time data validates high levels of gene editing and reduction in serum transthyretin protein levels in rat models*
- *First to demonstrate single-dose, in vivo results using proprietary lipid nanoparticle delivery system in mice showing: approximately 97 percent reduction in serum transthyretin protein levels; 70 percent liver editing efficiency following a single dose; six months post-single-dose durability of stable liver editing*

WASHINGTON, May 13, 2017 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on the development of potentially curative therapeutics using CRISPR technology, presented an update on its long-term mouse genome editing and delivery studies and shared new, first-time data in rat models demonstrating consistent dose-dependent editing, at the American Society of Gene & Cell Therapy's Annual Meeting (ASGCT).

These data, featured in a platform presentation on Saturday, May 13 at ASGCT showed:

- Six-month mouse study data demonstrating both durability and high editing efficiency *in vivo,* with approximately 70 percent editing at the target DNA site with a single intravenous dose. A 97 percent reduction of serum transthyretin (TTR) protein levels also was sustained.
- In addition, robust, dose-responsive lipid nanoparticle (LNP)-mediated editing of the TTR gene in rat livers following single intravenous administration; up to 66 percent editing at the target DNA site and up to 91 percent reduction in serum TTR protein levels.
- Both studies were conducted using Intellia's proprietary LNP delivery system, providing high levels of liver delivery and rapid clearance.

"Data from the additional rat study further validates the *in vivo* CRISPR/Cas9 platform using Intellia's proprietary LNP delivery system," said David Morrissey, Ph.D., senior vice president, Platform and Delivery Technology. "In both species, we saw unprecedented *in vivo* liver editing results and consistent delivery of CRISPR/Cas9 with systemic administration using LNPs, while also showing the ability to expand our studies in larger species."

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" 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 Intellia's ability to advance and expand the CRISPR/Cas9 technology to develop into human therapeutic products; our ability to achieve stable liver editing; effective genome editing with a single treatment dose; and the potential timing and advancement of our preclinical studies and clinical trials. 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 with Novartis or Regeneron 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 filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in Intellia's 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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