Intellia Therapeutics Demonstrates Sustained and Durable Genome Editing with CRISPR/Cas9 in One-Year Animal Study

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- *First-in-class, one-year data demonstrate sustained TTR protein reduction of approximately 97 percent, corresponding to 70 percent liver editing, following a single* in vivo *systemic dose in mice*
- *Non-human primate studies ongoing and leading to development candidate designation in first half 2018*
- *Investigational new drug-enabling activities on track for initiation in first half 2018*

CAMBRIDGE, Mass., Sept. 26, 2017 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on the development of potentially curative therapeutics using CRISPR/Cas9 technology, today presented the full 12-month data of its completed long-term mouse study, demonstrating robust and durable *in vivo* genome editing post single-dose, intravenous administration using its proprietary lipid nanoparticle (LNP) delivery system.

The data presented at the 13th Annual Meeting of the Oligonucleotide Therapeutics Society in Bordeaux, France, demonstrated robust editing and durability:

- Achieved and maintained approximately 97 percent reduction in serum TTR protein levels through one-year, following a single dose
- Attained approximately seventy percent editing at the target DNA site in the liver through one year
- The transient nature of LNP delivery was confirmed with 99 percent clearance of mRNA within 10 hours and of sgRNA within 72 hours in the liver
- The genome editing treatment was well tolerated with no adverse events during the course of the 12-month study

“These results show the long-term persistence of edited TTR genes in the mouse liver, indicating the potential for sustained efficacy and tolerability of our CRISPR/Cas9 platform as we move to higher species,” said David Morrissey, Ph.D., senior vice president, Platform and Delivery Technology. “These data validate Intellia’s approach as we remain on track with our non-human primate studies, which will permit us to designate our development candidate and perform IND-enabling activities in 2018 for the development of potential therapies in humans.”

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.

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