Intellia Therapeutics Announces Presentations at the 2019 Annual Congress of the European Society of Gene and Cell Therapy (ESGCT)

October 16, 2019

CAMBRIDGE, Mass., Oct. 16, 2019 (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*, announced one oral presentation and four poster presentations were accepted for the 27th Annual Congress of the European Society of Gene and Cell Therapy (ESGCT) taking place October 22-25, 2019, in Barcelona, Spain.

Intellia's data includes important updates about the company's programs and platform development activities:

Oral Presentation:

"*In Vivo* Gene Knockout Followed by Targeted Gene Insertion Results in Simultaneous Reduced Mutant Protein Levels and Durable Transgene Expression"

Intellia will present data on its alpha-1 antitrypsin deficiency (AATD) program, which uses a modular hybrid delivery system combining lipid nanoparticle (LNP) encapsulated CRISPR/Cas9 with an adeno-associated virus (AAV) donor DNA template. Intellia's gene knockout approach eliminates the production of the faulty PiZ variant of the protein, while targeted insertion of a wild-type gene copy facilitates production of a functional circulating protein. This builds on Intellia's similar approach for targeted gene insertion of *Factor 9*, which achieved increased levels of circulating human Factor IX protein through two months in non-human primates and sustained through 12 months in mice.

Presenter: Anthony Forget, Ph.D.
Abstract number: OR48
Session 5b: New delivery systems and technologies
Presentation date/time: Friday, October 25, 2019, 11:30 a.m. – 1:30 p.m. CET
Location: Room 113-115

Poster Presentations:

"In Silico, Biochemical and Cell-Based Integrative Genomics Identifies Precise CRISPR/Cas9 Targets for Human Therapeutics"

This poster presentation will highlight Intellia's approach to assess off-target activity to identify highly specific CRISPR/Cas9 guides. Researchers demonstrated that potential off-target editing profiles discovered through empirical data from biochemical approaches were the most sensitive and accurate.

Presenter: Daniel O'Connell, Ph.D.
Poster ID Number: P655
Date: Wednesday, October 23, 2019

"Generation of a Library of WT1-Specific T Cell Receptors (TCR) for TCR Gene Edited T Cell Therapy of Acute Leukemia"

This poster presentation focuses on Intellia's ongoing research collaboration with IRCCS Ospedale San Raffaele to develop CRISPR/Cas9-edited T cell therapies to address intractable cancers, such as acute myeloid leukemia (AML). Researchers have successfully established a protocol enabling consistent and efficient tumor-specific TCR isolation and characterization from healthy donors. Based on these results, Intellia has selected multiple lead TCRs, which are undergoing development candidate evaluation.

Presenter: Erica Carnevale, Ph.D., Ospedale San Raffaele
Poster ID Number: P111
Date: Wednesday, October 23, 2019

"Engineering of Highly Functional and Specific Transgenic T Cell Receptor (TCR) T Cells Using CRISPR-Mediated In-Locus Insertion Combined with Endogenous TCR Knockout"

This poster presentation focuses on the company's T cell engineering technology, which is being applied in its Wilms' Tumor 1 (WT1) lead *ex vivo* program. Intellia has identified an efficient CRISPR/Cas9-mediated process that inserts tumor-specific TCRs with high yield into the TRAC locus. Simultaneous knockout of the TRBC1 and TRBC2 loci substantially eliminates production of the endogenous T cell receptors.

Presenter: Birgit Schultes, Ph.D.
Poster ID Number: P162
Date: Thursday, October 24, 2019

"CRISPR/Cas9-Mediated Gene Knockout to Address Primary Hyperoxaluria"

This poster presentation will demonstrate the effects of independent CRISPR/Cas9-mediated knockout of each of two target genes involved in oxalate

formation, *lactate dehydrogenase A* (*LDHA)* and *hydroxyacid oxidase 1 (HAO1),* to address primary hyperoxaluria type 1 (PH1).

Presenter: Sean Burns, M.D.
Poster ID Number: P552
Date: Thursday, October 24, 2019

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing 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 IND application for NTLA-2001 in mid-2020; its plans to generate preclinical and other data necessary to nominate a first engineered cell therapy development candidate for its AML program by the end of 2019; its plans to advance and complete preclinical studies, including non-human primate studies for its ATTR program, AML program and other *in vivo* and *ex vivo* programs; develop our proprietary LNP/AAV hybrid delivery system to advance our complex genome editing capabilities, such as gene insertion; its presentation of additional data at upcoming scientific conferences regarding CRISPR-mediated, targeted transgene insertion in the liver of NHPs, using F9 as a model gene, via the Company's proprietary LNP-AAV delivery technology, and other preclinical data by the end of 2019; 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 and AML 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; the impact of its collaborations on its development programs, including but not limited to its collaboration with Regeneron Pharmaceuticals, Inc. or Ospedale San Raffaele; statements regarding the timing of regulatory filings regarding its development programs; and the ability to fund operations into the second half 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, including through our arbitration proceedings against Caribou; 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; the risk that the results of preclinical studies will not be predictive of future results in connection with future studies; and the risk that Intellia's collaborations with Novartis or Regeneron or its other *ex vivo* collaborations 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 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:

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

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

Source: Intellia Therapeutics, Inc.