Intellia Therapeutics Announces Three Oral Presentations on In Vivo and Engineered Cell Therapy Data at the 22nd Annual Meeting of the American Society of Gene and Cell Therapy

April 15, 2019

CAMBRIDGE, Mass., April 15, 2019 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), announced three oral presentations at the 22nd Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), taking place April 29-May 2, 2019, in Washington, D.C.

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

"CRISPR/Cas9-Mediated Targeted Insertion of Human F9 Achieves Therapeutic Circulating Protein Levels in Mice and Non-Human Primates"

Intellia will present data showing that its targeted gene insertion platform achieved therapeutic levels of Factor IX protein in non-human primates (NHP). The company employs a proprietary hybrid delivery system, comprised of both lipid nanoparticles (LNPs) and adeno-associated virus (AAV), to insert the desired gene sequence. *Factor 9* (*F9*) is a gene that encodes Factor IX (FIX), a blood-clotting protein that is missing or defective in hemophilia B patients.

The data showing therapeutic levels of FIX achieved in NHPs is from an ongoing research collaboration between Intellia and Regeneron Pharmaceuticals, Inc.

Presenter: Hon-Ren Huang, Ph.D., associate director, Vector Biology, Intellia
Abstract number: 11
Session: Advances in Genome Editing and Hemophilia Gene Therapies
Presentation date/time: Mon., April 29, 2019, 9-9:15 a.m. ET
Location: Heights Courtyard 2

"Exploiting Clonal Tracking of WT1-Specific T Cells to Generate a Library of Tumor-Specific T Cell Receptors (TCR) for TCR Gene Editing of Acute Leukemia"

This presentation will focus on Intellia's ongoing research collaboration with IRCCS Ospedale San Raffaele in Italy to develop CRISPR-edited T cell therapies to address intractable cancers, such as acute myeloid leukemia (AML). Researchers generated and tested a library of TCRs with different epitope specificities and human leukocyte antigen (HLA) restrictions.

Presenter: Eliana Ruggiero, Ph.D., Experimental Hematology Unit, Division of Immunology, Transplantation and Infectious Diseases, IRCCS Ospedale San Raffaele, Italy
Abstract number: 123
Session: Cancer Adoptive Immunotherapy
Presentation date/time: Mon., April 29, 2019, 5-5:15 p.m. ET
Location: Georgetown

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

Intellia will provide information demonstrating successful knockout of two targets of interest, *lactate dehydrogenase A* (*LDHA*) and *hydroxyacid oxidase 1* (*HAO1*), to address primary hyperoxaluria type 1 (PH1) in a PH1 mouse model. The data shows the continued progression of the company's modular platform capability using CRISPR to knock out liver gene targets. The data being presented includes results from an ongoing collaboration with researchers at the University of Alabama at Birmingham.

Presenter: Anette Hübner, Ph.D., associate director, Liver Biology, Intellia
Abstract number: 1000
Session: Use of New Technologies for Hepatic Therapy
Presentation date/time: Thur., May 2, 2019, 12-12:15 p.m. ET
Location: Heights Courtyard 3

"Delivering on the Therapeutic Promise of CRISPR/Cas9"

Intellia also will participate in the ASGCT Gene Editing Workshop, which will provide an overview of current gene editing technologies and approaches, as well as emerging uses and applications.

Presenter: Sean Burns, M.D., senior director, Hematology and New Therapeutic Areas
Session: Corporate Review II

Session date/time: Sun., April 28, 2019, 5-6 p.m. ET
Location: Lincoln

Abstracts are available on the ASGCT website.

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 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 and expand the CRISPR/Cas9 technology to develop into human therapeutic products, as well as our CRISPR/Cas9 intellectual property portfolio; our ability to achieve stable or effective genome editing with a single treatment dose; our ability to perform genomic editing, such as knock-out and insertion, to treat disease by modulating, replacing or correcting genetic function; the potential timing and advancement of our preclinical studies, including continuing non-human primate studies, and clinical trials; our ability to replicate results achieved in our preclinical studies in any future studies, including human clinical trials; the potential development of *ex vivo* cell therapeutics of all types using CRISPR/Cas9 technology; our ability to commence IND-enabling studies of a lead TTR development candidate in 2019 and subsequently submitting an Investigational New Drug application; our intent to present additional data for our liver programs, organs beyond the liver, additional insertion/repair data, and preclinical data in support of our *in vivo* programs, including TTR and PH1, as well as our *ex vivo* programs on immuno-oncology, including WT1, during 2019; our ability to nominate a development candidate for an *ex vivo* program, as well as a second *in vivo* indication, in 2019; the intellectual property position and strategy of Intellia, Intellia's licensors and other third parties from which Intellia derived rights; actions by government agencies; the impact of our collaborations on our development programs; the potential timing of regulatory filings regarding our development programs; the potential commercialization opportunities, including value and market, for product candidates; our expectations regarding our uses of capital, expenses, future accumulated deficit and other 2019 financial results; and our ability to fund operations into the first 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; 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, Regeneron, IRCCS Ospedale San Raffaele or other 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 filed with the Securities and Exchange Commission, 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 Therapeutics undertakes no duty to update this information unless required by law.

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