Intellia Therapeutics to Present New Preclinical Data from Its CRISPR/Cas9 Programs at the 23rd Annual Meeting of the American Society of Gene and Cell Therapy

April 28, 2020

Company to Present Three Oral and Two Poster Presentations on In Vivo and Engineered Cell Therapy Pipeline Programs

CAMBRIDGE, Mass., April 28, 2020 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, announced that five abstracts were accepted for the 23rd Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), taking place virtually from May 12-15, 2020. Abstracts are available on the ASGCT website.

Intellia's data include important updates about the company's progress and platform development activities:

Oral Presentations

"Enhanced tgTCR T Cell Product Attributes Through Process Improvement of CRISPR/Cas9 Engineering"
As a follow-on to data presented at Keystone Symposia's Engineering the Genome Conference this past February, Intellia's ASGCT presentation will focus on process improvements in its CRISPR/Cas9-based engineering to deliver T cell therapies with high levels of editing, achieving robust levels of expansion, desirable memory phenotypes, improved function and reduced translocations. These platform advances support NTLA-5001, which is Intellia's first wholly owned engineered T cell therapy development candidate. NTLA-5001 utilizes a T cell receptor (TCR)-directed approach to target the Wilms' Tumor 1 (WT1) antigen, obtained through the ongoing research collaboration with IRCCS Ospedale San Raffaele, which is over-expressed in many hematologic and solid tumors. Intellia will focus its initial clinical investigation of NLTA-5001 on acute myeloid leukemia (AML), while exploring in ongoing preclinical studies its application in solid tumors. Intellia plans to submit an IND for NTLA-5001 for the treatment of AML in the first half of 2021.

Presenter: Aaron Prodeus, Ph.D., senior scientist, Cell Therapy
Abstract number: 35
Session: CAR T-Cell Therapies I
Presentation date/time: Tuesday, May 12, 2020, from 11:45 a.m.-12 p.m. ET

"Exploiting CRISPR-Genome Editing and WT1-Specific T Cell Receptors to Redirect T Lymphocytes Against Acute Myeloid Leukemia"
This presentation will focus on Intellia's ongoing research collaboration with IRCCS Ospedale San Raffaele to apply CRISPR/Cas9 technology to develop engineered T cell therapies to address intractable cancers, such as AML. Researchers will also present data showing a significant decrease in AML tumor burden in mice treated with the WT1-specific TCRs, which are incorporated into Intellia's lead NTLA-5001 candidate for the treatment of AML.

Presenter: Eliana Ruggiero, Ph.D., Experimental Hematology Unit, Division of Immunology, Transplantation and Infectious Diseases, IRCCS Ospedale San Raffaele, Italy
Abstract number: 78
Session: CAR T and Other Engineered T Cells Targeting Hematological Malignancies
Presentation date/time: Tuesday, May 12, 2020, from 3:45-4 p.m. ET

"CRISPR/Cas9-Mediated Gene Knockout of *KLKB1* to Treat Hereditary Angioedema"
Intellia will present data updates on its potential hereditary angioedema (HAE) therapy, which uses the company's modular lipid nanoparticle (LNP)-based CRISPR/Cas9 delivery system to knock out the *prekallikrein B1* (*KLKB1*) gene and reduce kallikrein activity. HAE is a rare genetic disorder characterized by recurring and unpredictable severe swelling attacks in various parts of the body, and is significantly debilitating or even fatal in certain cases. This presentation will include results from the company's ongoing collaboration with researchers at Regeneron and also will build on initial data released at Keystone Symposia's Engineering the Genome Conference in February. Intellia expects to nominate a development candidate for HAE in the first half of 2020.

Presenter: Jessica Seitzer, director, Genomics
Abstract number: 1320
Session: Gene Regulation and Delivery Technologies
Presentation date/time: Friday, May 15, 2020, from 10:30-10:45 a.m. ET

Poster Presentations

"Validation of CRISPR/Cas9 Off-Target Discovery Profiles from *In Silico* Prediction, Cell-Based and Biochemical-Based Assays with Targeted Off-Target Sequencing"
This 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: Nishit Patel, senior associate scientist, Informatics, Computational Biology and Genomics

Abstract number: 203
Session date/time: Tuesday, May 12, 2020, from 5:30-6:30 p.m. ET

"***In Vivo* Model Development for Genome-Edited T Cell Therapeutics"**
Researchers will present improved murine models used to assess safety, efficacy and persistence of therapeutic modalities in cell-based therapies. Together with biotechnology company Taconic Biosciences, Intellia developed two *in vivo* mouse models, which allow for enhanced monitoring of graft-versus-host disease and human natural killer cytotoxicity.

Presenter: Yong Zhang, Ph.D., principal scientist, Cell Therapy
Abstract number: 1174
Session date/time: Thursday, May 14, 2020, from 5:30-6:30 p.m. ET

Workshop and Symposia

"Building a Modular CRISPR/Cas9 Platform for Human Therapeutic Applications"
Intellia will participate in the ASGCT Translational Science Genome Editing Pre-Meeting Workshop, which will include an overview on important factors to consider in building a modular CRISPR/Cas9 platform for human therapeutic applications.

Presenter: Jessica Seitzer, director, Genomics
Session: Gene Editing Workshop
Presentation date/time: Monday, May 11, 2020, from 4:10-4:30 p.m. ET

Industry Sponsored Symposia

Intellia will participate in an industry lunchtime panel discussion during ASGCT.

Panelist: Laura Sepp-Lorenzino, Ph.D., chief scientific officer
Panel date/time: Wednesday, May 13, 2020, from 12-1:30 p.m. ET

About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing proprietary, 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 ability to advance and expand the CRISPR/Cas9 technology to develop human therapeutic products that meet the regulatory requirements in the relevant jurisdictions, as well as our CRISPR/Cas9 intellectual property portfolio; our ability to achieve stable or effective genome editing; our ability to effectively administer one dose or multiple doses of our CRISPR/Cas9 product candidates; the potential timing and advancement of our preclinical studies, including non-human primate studies for our transthyretin amyloidosis ("ATTR") program ("NTLA-2001") and our hereditary angioedema ("HAE") program, and other studies for these and other programs, including preclinical and human clinical trials; the ability to demonstrate our platform's modularity and replicate or apply results achieved in our preclinical studies, including those in our ATTR, HAE and Wilms' Tumor 1 ("WT1")/acute myeloid leukemia ("AML") programs or research projects, in any future studies, including human clinical trials; our ability to generate data and replicate results relating to enhancements to our proprietary lipid nanoparticle ("LNP") technology, including its formulation and components, in preclinical or clinical studies, or that any enhancements will result in an improved product candidate profile; the potential development of other *in vivo* or *ex vivo* cell therapeutics of all types, and those targeting WT1 in particular, using CRISPR/Cas9 technology; our plans to submit an IND application for NTLA-2001 for ATTR in the second half of 2020, as well as for NTLA-5001, our first T cell receptor ("TCR")-directed engineered cell therapy development candidate for our AML program in the first half of 2021; our plans to nominate a development candidate for our HAE program in the first half of 2020; our expectations regarding potential patient populations that may be addressed by each of our programs; actions by government agencies; our growth as a company and the anticipated contribution of the members of our board of directors and our executives to our operations and progress; the impact of our collaborations on our research and development programs; the potential timing of regulatory filings regarding our development programs; the potential commercial opportunities, including value and market, for our product candidates; our expectations regarding our use of capital and other financial results during 2020; and our ability to fund operations through the end 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 its intellectual property position; risks related to Intellia's relationship with third parties, including its licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; uncertainties related to the initiation and conduct of studies and other development requirements for its 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 or clinical studies will not be predictive of future results in connection with future studies; and the risk that Intellia's collaborations with Regeneron, OSR or its 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 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.

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