



Intellia Therapeutics Announces WT1 as Its First Cell Therapy Target, Following Presentation of Early Data at the American Society of Gene and Cell Therapy 21st Annual Meeting

May 17, 2018

CAMBRIDGE, Mass., May 17, 2018 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology, and its [research collaborator](#), Ospedale San Raffaele (OSR), presented at the 21st Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) the first update on their joint discovery and development efforts of Wilms' Tumor 1 (WT1)-specific transgenic T cells. In conjunction with this presentation, Intellia announced that its first cell therapy target is WT1 for the treatment of acute myeloid leukemia and other potential hematological malignancies, as well as for solid tumors.

"As Intellia expands its wholly owned T cell therapy *ex vivo* efforts, we are pleased to present our first set of data in this cell therapy area," said Intellia President and Chief Executive Officer John Leonard, M.D. "WT1 is over-expressed in many tumor types, including both leukemias and solid tumors, and is an ideal target for immuno-oncological therapies seeking to treat these malignancies. This early data being presented is part of Intellia's broader strategy to develop next-generation solutions for immuno-oncology and autoimmune disorders, with novel, modular platforms based on genome editing."

Intellia and OSR are collaborating to develop best-in-class CRISPR-edited T cells directed to WT1, a tumor-associated antigen expressed across a wide range of different tumor types and a known driver of leukocyte blasts in hematological cancers. At this year's ASGCT Annual Meeting, OSR researchers, led by Chiara Bonini, M.D., Ph.D., shared findings showing the generation, characterization and advancement of WT1-specific, transgenic T cells against multiple WT1 epitopes presented on *HLA-A*02:01* and other Class I alleles. Initial data demonstrating both recognition and killing of acute myeloid leukemia cells also was presented.

OSR researchers and Intellia presented the poster, entitled "Hunting WT1-Specific T Cell Receptors for TCR Gene Editing for Acute Myeloid Leukemia." The abstract is available on the ASGCT website [here](#).

"Our collaboration with Intellia represents our shared belief that the characteristics of T cell receptors as a targeting moiety may be an excellent approach to broadening the field of oncology cell therapy, opening the door to many more potential antigen targets in liquid and solid tumors," added Bonini, full professor at Università Vita-Salute San Raffaele; deputy director, Division of Immunology, Transplantation and Infectious Diseases; and head, Experimental Hematology Unit, Ospedale San Raffaele, Italy.

This *ex vivo* research effort is part of an integrated strategy to develop CRISPR-edited T cell therapies to address hard-to-treat cancers and overcome certain limitations of current-generation cell therapies, including widely reported immune cell suppression in tumor microenvironments.

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 optimized cell therapies that can treat cancer and immunological diseases by replacing 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 intelliatax.com and follow us on Twitter @intelliatweets.

About Ospedale San Raffaele

Ospedale San Raffaele is a university and research hospital established in 1971 to provide international-level specialised care for the most complex and difficult health conditions. Since 2012, it has been part of Gruppo ospedaliero San Donato, the leading hospital group in Italy. San Raffaele is a multi-speciality center with over 50 clinical specialties and over 1,300 beds. Research focuses on integrating basic, translational and clinical activities to provide the most advanced care to its patients. For further information, visit hsr.it.

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; the potential development of *ex vivo* cell therapies of all types, including immuno-oncology and autoimmunity therapies, and those targeting WT1 in particular, using CRISPR/Cas9 technology; our intent to present additional data for WT1, other *ex vivo* programs (including programs developing cell therapies for other immuno-oncology and autoimmune/inflammation indications), *in vivo* therapies (including those focused on the liver and other organs), and additional insertion/repair data during 2018; and the impact of our collaborations on our development programs. 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 IRCCS Ospedale San Raffaele, or Intellia's other *ex vivo* or *in 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 and quarterly reports on Form 10-Q 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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Source: Intellia Therapeutics, Inc.