

# Intellia Therapeutics and Novartis Expand Cell Therapy Collaboration to Pursue CRISPR/Cas9-based Genome Editing in Additional Stem Cell Population

December 6, 2018

- Intellia's Right to Use Proprietary Lipid Nanoparticle Technology Extended to All Settings
- Intellia Will Receive a One-time \$10 Million Payment

CAMBRIDGE, Mass., Dec. 06, 2018 (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 an expansion of its existing cell therapy collaboration with Novartis, to include the *ex vivo* development of innovative cell therapies using certain ocular stem cells. As part of the updated collaboration terms, Novartis will have the right to develop CRISPR/Cas9-based products for one or more targets using these stem cells. Intellia will receive a one-time \$10 million cash payment and, consistent with the original collaboration agreement, Intellia also is eligible to receive downstream success-based milestones and royalties.

With the collaboration expansion announced today, Intellia will gain expanded rights to Novartis' lipid nanoparticle (LNP) technology for all genome editing applications in both *in vivo* and *ex vivo* settings. This licensed LNP technology is the foundation of Intellia's proprietary modular delivery system of CRISPR/Cas9 for its *in vivo* product pipeline. Intellia retains rights to all other *in vivo* and *ex vivo* applications of CRISPR/Cas9, including for eye disorders, subject to certain *in vivo* target selection options by Novartis set forth in the original agreement.

"Genome editing enhancements made by CRISPR/Cas9 will enable the next generation of cell therapies. With our collaborator, Novartis, we are broadening the *ex vivo* application of our CRISPR/Cas9 technology from hematopoietic stem cells, or HSCs, to ocular stem cells. We are pleased to expand our relationship with Novartis, and to continue to work together to develop cell therapies," said Intellia President and Chief Executive Officer John Leonard, M.D. "Broader rights to Novartis' LNP technology will assist our efforts to apply this technology in *ex vivo* settings for the development of proprietary cell therapies, just as we have done to develop our proprietary modular delivery system for *in vivo* products in the liver and other organs."

# About the Novartis/Intellia Agreement

Under the terms of the original agreement, Novartis received exclusive rights to develop all collaboration programs focused on engineered chimeric antigen receptor T cells (CARTs), while both companies committed to advancing their respective hematopoietic stem cell (HSC) programs. The work of these preclinical programs, including for sickle cell disease, is ongoing. The discovery and development collaboration, including the updates announced today, is set to expire in December 2019.

#### About Intellia's Ex Vivo Programs

Independent from its *ex vivo* collaboration with Novartis, Intellia also is advancing its wholly owned *ex vivo* pipeline of immuno-oncology and autoimmune cell therapies. Intellia's proprietary *ex vivo* programs include its acute myeloid leukemia (AML) program utilizing transgenic T cell receptors (TCRs) against Wilms' Tumor 1 (WT1), a target identified in collaboration with Ospedale San Raffaele (OSR).

#### **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 intelliats.com and follow us on Twitter @intelliatweets.

### **Forward-Looking Statements**

This press release contains "forward-looking statements" of Intellia Therapeutics, Inc. ("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; our CRISPR/Cas9 intellectual property portfolio; the potential timing and advancement of our preclinical studies, including for our internal or partnered programs; the potential development of our proprietary lipid nanoparticle ("LNP") delivery system; the potential development of our in vivo or ex vivo cell therapeutics of all types using CRISPR/Cas9 technology, including therapies targeting Wilms' Tumor 1 ("WT1") in particular; the intellectual property position and strategy of Intellia's licensors or other parties from which it derives rights, as well as third-parties and competitors; and the potential timing and receipt of future milestones and royalties based on Intellia's collaboration with Novartis.

Any forward-looking statements in this presentation 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 not be predictive of future results

in connection with future studies; the risk that Intellia's collaborations with Novartis will not continue or will not be successful; and risks related to the competitive landscape. 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 presentation 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.