Intellia Therapeutics Highlights Strategic Priorities and Anticipated Development Milestones for 2021

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- *Continued advancement of global Phase 1 study of NTLA-2001, a potentially curative single-course therapy for transthyretin amyloidosis (ATTR)*
- *Anticipates submitting an IND or IND-equivalent in mid-2021 for NTLA-5001 for the treatment of acute myeloid leukemia (AML)*
- *Expects to submit an IND or IND-equivalent in 2H 2021 for NTLA-2002 for the treatment of hereditary angioedema (HAE)*
- *Plans to nominate at least one new development candidate in 2021 from broad research efforts and continued platform innovation*
- *Ended 2020 in strong financial position with $597M in cash*

CAMBRIDGE, Mass., Jan. 07, 2021 (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*, today outlined its expected 2021 milestones and the following strategic priorities:

- **Clinical validation:** Evaluate the clinical profile of NTLA-2001 as a single-course therapy for transthyretin amyloidosis (ATTR) and Intellia's *in vivo* non-viral, lipid nanoparticle (LNP)-based CRISPR/Cas9 delivery system as a platform for achieving clinically-relevant protein reduction for patients;
- **Full-spectrum pipeline advancement:** Rapidly progress *in vivo* and engineered cell therapy candidates for genetic diseases and cancers towards the clinic; and
- **Platform innovation:** Extend Intellia's continued scientific leadership across genome editing, delivery and cell engineering capabilities.

"Since our founding, we set out to develop modular platform components that could serve as the engine powering an expansive portfolio of curative therapeutics. We have paved a rapid and reproducible development path for both *in vivo* and engineered cell therapies to address serious genetic diseases and cancers," said Intellia President and Chief Executive Officer John Leonard, M.D. "Over the next 12 months, we will evaluate the clinical profile of NTLA-2001, both as a one-time treatment option for ATTR patients and as a validation of our non-viral approach to *in vivo* delivery. In addition, we anticipate first-in-human regulatory submissions for NTLA-5001 and NTLA-2002, at least one new development candidate and new platform innovations to create the next wave of genomic medicines. These priorities for 2021 reflect our long-term vision for Intellia: to unlock genome editing's full therapeutic potential."

Anticipated 2021 Milestones:

- **NTLA-2001 for ATTR:** NTLA-2001 is the first systemically delivered CRISPR-based therapy dosed in a patient, and could potentially be the first curative treatment for ATTR. By applying the Company's *in vivo* LNP technology, NTLA-2001 offers the possibility of halting and reversing the disease with potent, lifelong transthyretin (TTR) protein reduction after a single course of treatment. NTLA-2001 is part of a co-development/co-promotion agreement between Intellia, the lead party, and Regeneron Pharmaceuticals, Inc. (Regeneron).
    - Intellia is continuing to enroll patients in the global Phase 1 study of NTLA-2001 in adults with hereditary ATTR with polyneuropathy (hATTR-PN) in order to establish an optimal dose. Later this year, the Company plans to provide guidance around timing of the first expected data readout, with the goal of demonstrating clinical proof-of-concept for its modular LNP delivery platform.
    - Intellia intends to evaluate NTLA-2001 in a broader ATTR population of both polyneuropathy and cardiomyopathy patients following its Phase 1 safety assessment and dose optimization.

- **NTLA-5001 for AML:** NTLA-5001 is a potential best-in-class engineered T cell therapy designed to treat all genetic subtypes of acute myeloid leukemia (AML). This investigational candidate is a T cell receptor (TCR)-T cell therapy targeting the Wilms' Tumor 1 (WT1) antigen utilizing Intellia's proprietary cell engineering process.
    - Intellia plans to submit an Investigational New Drug (IND) or equivalent regulatory application for NTLA-5001 in mid-2021. The first-in-human trial is expected to evaluate the safety and activity of NTLA-5001 in patients with persistent or recurrent AML who have previously received first-line therapies.
    - The Company is also evaluating the potential use of NTLA-5001 to treat WT1-positive solid tumors in preclinical studies.

- **NTLA-2002 for HAE:** NTLA-2002 aims to prevent attacks and eliminate the current, significant treatment burden for people living with hereditary angioedema (HAE) after a single course. Intellia is applying its modular LNP delivery system to

develop NTLA-2002 to knock out the *KLKB1* gene in the liver to permanently reduce plasma kallikrein activity.
    - Intellia plans to submit an IND or equivalent regulatory application in the second half of 2021.
    - The Company is applying insights gained from NTLA-2001 to expedite clinical development of NTLA-2002.

- **Pipeline Expansion:** Intellia is focused on advancing its differentiated genome editing, delivery and cell engineering strategies to broaden *in vivo* and *ex vivo* applications for wholly owned and partnered programs. The Company continues to progress its robust research efforts and modular platform to develop new therapeutic candidates for genetic diseases requiring removal and/or restoration of a protein, as well as the next generation of engineered cell therapies for cancers.
    - Intellia today announced plans to nominate at least one additional development candidate in 2021.
    - Intellia, in partnership with lead party Regeneron, is also continuing to advance hemophilia A and B therapeutic programs toward IND-enabling studies using their jointly-developed *in vivo* targeted insertion technology.
    - The Company plans to present at upcoming scientific conferences, with updates on multiple *in vivo* targets in the liver and other tissues and an allogeneic solution that enables the next generation of engineered cell therapies.

Cash Position and Financial Guidance

- Intellia ended the fourth quarter of 2020 with approximately $597 million in cash, cash equivalents and marketable securities. Intellia expects that its cash, cash equivalents and marketable securities as of December 31, 2020 will enable the Company to fund its anticipated operating expenses and capital expenditure requirements for at least the next 24 months. This expectation excludes any strategic use of capital not currently in the Company's base-case planning assumptions.

About Intellia Therapeutics
Intellia Therapeutics is a leading genome editing company, focused on the development of proprietary, potentially curative therapeutics using the CRISPR/Cas9 system. Intellia believes the CRISPR/Cas9 technology has the potential to transform medicine by both producing therapeutics that permanently edit and/or correct disease-associated genes in the human body with a single treatment course, and creating enhanced engineered cells that can treat oncological and immunological diseases. Intellia's combination of deep scientific, technical and clinical development experience, along with its leading intellectual property portfolio, puts it in a unique position to unlock broad therapeutic applications of the CRISPR/Cas9 technology and create new classes of therapeutic products. Learn more about Intellia and CRISPR/Cas9 at intelliatx.com. Follow us on Twitter @intelliatweets.

Forward-Looking Statements
This press release contains "forward-looking statements" of Intellia Therapeutics, Inc. ("Intellia", "we" or "our") 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 our: being able to enroll and dose the necessary subjects in our clinical studies for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR"), provide timing on the first expected data readout, and successfully secure additional clinical studies authorizations, such as clinical trial applications ("CTA"), in other countries; ability to evaluate NTLA-2001 in a broader ATTR population; plans to submit an investigational new drug ("IND") application or similar clinical trial application for NTLA-5001, our first T cell receptor ("TCR")-directed engineered cell therapy development candidate in our acute myeloid leukemia ("AML") program in mid-2021; expectations of evaluating the safety and activity of NTLA-5001 in patients with persistent or recurrent AML who have previously received first-line therapies; plans to submit an IND or similar clinical trial application for our hereditary angioedema ("HAE") program in the second half of 2021; plans to nominate at least one additional development candidate in 2021; plans to advance and complete preclinical studies for our programs; development of our modular platform to advance our complex genome editing capabilities, such as gene insertion; further development of our proprietary cell engineering process for multiple sequential editing; presentation of additional data at upcoming scientific conferences, and other preclinical data in 2021; advancement and expansion of our CRISPR/Cas9 technology to develop human therapeutic products, as well as our ability to maintain and expand our related intellectual property portfolio; ability to demonstrate our platform's modularity and replicate or apply results achieved in preclinical studies, including those in our ATTR, AML, and HAE programs, in any future studies, including human clinical trials; ability to develop other *in vivo* or *ex vivo* cell therapeutics of all types, and those targeting Wilms' Tumor 1 (WT1) in AML in particular, using CRISPR/Cas9 technology; ability to optimize the impact of our collaborations on our development programs, including but not limited to our collaboration with Regeneron Pharmaceuticals, Inc. ("Regeneron"), including our co-development programs for hemophilia A and hemophilia B; Regeneron's ability to successfully co-develop products in the hemophilia A and B programs, and the potential timing and receipt of future milestones and royalties, or profits, as applicable, based on our license, collaboration and, if applicable, co-development agreements with Regeneron and Novartis; statements regarding the timing of regulatory filings and clinical trial execution, including dosing of patients, regarding our development programs; potential commercial opportunities, including value and market, for our product candidates; our expectations regarding our use of capital and other financial results during 2021; and our ability to fund operations for at least the next 24 months.

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 our ability to protect and maintain our intellectual property position; risks related to our relationship with third parties, including our licensors and licensees; risks related to the ability of our licensors to protect and maintain their intellectual property position; uncertainties related to regulatory agencies' evaluation of regulatory filings and other information related to our product candidates; uncertainties related to the authorization, initiation and conduct of studies and other development requirements for our product candidates; the risk that any one or more of our product candidates, including those that are co-developed, 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 our collaborations with Regeneron or our 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 ("SEC"). 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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