Intellia Therapeutics Highlights Strategic Priorities and Anticipated Development Milestones for 2022

January 6, 2022

- *Advance clinical development of NTLA-2001, a potential single-dose therapy for transthyretin (ATTR) amyloidosis; on track to present additional data from Phase 1 study in Q1 2022*
- *Achieve preliminary proof-of-concept for NTLA-2002 in patients with hereditary angioedema (HAE) based on ongoing first-in-human study; anticipate presenting interim data in 2H 2022*
- *Establish initial safety and activity profile of NTLA-5001 for acute myeloid leukemia (AML) in Phase 1/2a study*
- *Progress* in vivo *and* ex vivo *research programs and integrate novel platform technologies; expect to nominate multiple new development candidates in 2022*
- *Ended 2021 in strong financial position with $1.1 billion in cash*

CAMBRIDGE, Mass., Jan. 06, 2022 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapies leveraging CRISPR-based technologies, today outlined its expected milestones and the following strategic priorities for 2022:

- **Accelerating clinical validation of *in vivo* pipeline**: Further characterize the safety and efficacy of NTLA-2001, including in patients with cardiomyopathy and complete enrollment of the Phase 1 study; establish the initial clinical profile of NTLA-2002 as a single-dose therapy for the treatment of hereditary angioedema (HAE).
- **Strategic pipeline expansion**: Significantly progress *in vivo* and *ex vivo* pipeline, including determining the initial safety profile of NTLA-5001 for acute myeloid leukemia (AML), advancing *in vivo* insertion candidates and nominating multiple new development candidates.
- **Platform innovation**: Broaden the Company's industry-leading platform through expansion of Intellia's genome editing, delivery and cell engineering capabilities.

"Unequivocally, 2021 was a landmark year for Intellia. We demonstrated that our proprietary CRISPR-based platform and LNP technology can turn revolutionary science into potentially transformational medicines. Our platform enables us to advance genome editing approaches, which maximizes our ability to target a multitude of life-threatening diseases," said Intellia President and Chief Executive Officer John Leonard, M.D. "As we begin 2022 with great momentum, we are poised to significantly expand our full-spectrum pipeline of potentially curative therapies with the nomination of at least two new *in vivo* candidates and our first allogeneic development candidate during the year. Importantly, we look forward to sharing additional data from the ongoing study of NTLA-2001 and interim results from the Phase 1/2 study of NTLA-2002, which we expect will further demonstrate the modularity of our genome editing platform."

Anticipated 2022 Milestones:

***In Vivo* Programs**

- **NTLA-2001 for ATTR amyloidosis**: NTLA-2001 is the first investigational CRISPR-based therapy to be systemically delivered to edit genes inside the human body, and has the potential to be the first single-dose treatment for transthyretin (ATTR) amyloidosis. Delivered with the Company's *in vivo* lipid nanoparticle (LNP) technology, NTLA-2001 offers the possibility of halting and reversing the disease by driving a deep, lifelong reduction in transthyretin (TTR) protein after a single dose. NTLA-2001 is part of a co-development/co-promotion agreement between Intellia, the lead party for this program, and Regeneron Pharmaceuticals, Inc. (Regeneron).
  - Today, Intellia announced that the first patient in the cardiomyopathy arm of the Phase 1 study has been dosed with NTLA-2001. This follows the Company's recent announcement that the United Kingdom Medicines and Healthcare products Regulatory Agency (MHRA) approved a protocol amendment for the Company's ongoing Phase 1 study of NTLA-2001 to include patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM). The study now includes patients with ATTR-CM enrolled in new dose-escalation and expansion cohorts. The inclusion of the ATTR-CM patient population is in addition to the original Phase 1 study, which continues to evaluate NTLA-2001 in patients with hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN). Intellia expects to complete enrollment of the Phase 1 study for both ATTRv-PN and ATTR-CM subjects in 2022.
  - Intellia intends to present additional interim clinical data of NTLA-2001 in ATTRv-PN patients from Part 1, the single-ascending dose portion, and to initiate Part 2, a single-cohort expansion, in the first quarter of 2022. Data to be presented at a company-sponsored event will be from all four ATTRv-PN dose cohorts in Part 1 and include

safety and serum TTR knockdown for Cohorts 3 and 4, as well as an early look at durability across all cohorts.

- **NTLA-2002 for HAE**: NTLA-2002 leverages Intellia's proprietary *in vivo* LNP delivery technology to knock out the *KLKB1* gene in the liver with the potential to permanently reduce total plasma kallikrein protein and activity, a key mediator of HAE. This investigational approach aims to prevent attacks for people living with HAE by providing continuous suppression of plasma kallikrein activity following a single dose and to eliminate the significant treatment burden associated with currently available HAE therapies.
    - In December 2021, Intellia dosed the first patient in its second clinical study of a CRISPR-based therapeutic candidate evaluating NTLA-2002 for HAE. The first-in-human Phase 1/2 trial is expected to evaluate the safety, tolerability and activity of NTLA-2002 in adults with Type I or Type II HAE, and will continue to leverage insights gained from the development of NTLA-2001.
    - The Company expects to present interim data from the Phase 1/2 study in the second half of 2022. These results are expected to characterize the emerging safety and activity profile of NTLA-2002 and demonstrate preliminary proof-of-concept.
- **NTLA-3001 for AATD-associated lung disease**: NTLA-3001 is Intellia's wholly owned CRISPR-mediated *in vivo* targeted gene insertion development candidate. It is designed with the aim to precisely insert a healthy copy of the *SERPINA1* gene, which encodes the alpha-1 antitrypsin (A1AT) protein, with the potential to restore permanent expression of functional A1AT protein to therapeutic levels after a single dose. This approach seeks to address alpha-1 antitrypsin deficiency (AATD)-associated lung disease and eliminate the need for sub-optimal weekly IV infusions of A1AT augmentation therapy or lung transplant in severe cases.
    - Intellia is conducting Investigational New Drug (IND)-enabling activities for NTLA-3001 with plans to file an IND or IND-equivalent in 2023. The Company also continues to explore additional editing strategies for AATD.

***Ex Vivo* Programs**

- **NTLA-5001 for AML**: NTLA-5001 is an investigational autologous T cell receptor (TCR)-T cell therapy engineered to target the Wilms' Tumor 1 (WT1) antigen for the treatment of all genetic subtypes of acute myeloid leukemia (AML).
    - In the fourth quarter of 2021, Intellia initiated screening of patients in the Phase 1/2a study of NTLA-5001 for patients with AML. The Company expects to dose its first patient in the coming weeks and enroll patients throughout the year. 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 TCR-based platform.

Modular Platform and Pipeline Expansion

- **Platform Innovation:** Intellia is expanding its industry-leading genome editing platform and scientific leadership through editing, delivery and cell engineering innovations that will enable broader *in vivo* and *ex vivo* applications.
    - Intellia plans to advance at least two new *in vivo* development candidates by the end of 2022.
    - The Company expects to nominate its first allogeneic *ex vivo* development candidate by the first half of 2022.
    - The Company plans to highlight additional advances to its proprietary technology capabilities, including both genome editing and delivery tools, at upcoming scientific conferences in 2022.

Corporate Updates:

- In January, Intellia and Kyverna Therapeutics announced a licensing and collaboration agreement for the development of KYV-201, an allogeneic CD19 CART-cell therapy for the treatment of a variety of B cell-mediated autoimmune diseases. Intellia may exercise an option to lead U.S. commercialization for KYV-201 under a co-development and co-commercialization agreement.
- In December 2021, Intellia appointed Derek Hicks as Executive Vice President, Chief Business Officer. Mr. Hicks joins Intellia with more than 25 years of combined business, leadership and biotechnology experience, having most recently served as Head of Business Development at Spark Therapeutics.

Cash Position

- Intellia ended the fourth quarter of 2021 with approximately $1.1 billion in cash, cash equivalents and marketable securities.

Intellia's Presentation at the 40 th Annual J.P. Morgan Healthcare Conference

Intellia is scheduled to present virtually at the 40th Annual J.P. Morgan Healthcare Conference on Wednesday, January 12, at 2:15 p.m. ET. A live audio webcast of Intellia's presentation can be accessed under the Events and Presentations page of the Investors & Media section on the company's website at www.intelliatx.com. A replay of the webcast will be available on Intellia's website for at least two weeks following the presentation.

About Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics leveraging CRISPR-based technologies. To fully realize the transformative potential of CRISPR-based technologies, Intellia is pursuing two primary approaches. The company's *in vivo* programs use intravenously administered CRISPR as the therapy, in which proprietary delivery technology enables highly precise editing of disease-causing genes directly within specific target tissues. Intellia's *ex vivo* programs use CRISPR to create the therapy by using engineered human cells to treat cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its robust intellectual property portfolio, have enabled the company to take a leadership role in harnessing the full potential of genome editing to create new classes of genetic medicine. Learn more at intelliatx.com. 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 beliefs and expectations regarding: the safety, efficacy and advancement of its clinical programs for NTLA-2001 for the treatment of transthyretin amyloidosis, NTLA-2002 for the treatment of hereditary angioedema, and NTLA-5001 for the treatment of acute myeloid leukemia pursuant to its clinical trial applications ("CTA") and IND submissions, including the expected timing of data releases, regulatory filings, and the initiation and completion of clinical trials; the advancement of development candidates including NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency (AATD)-associated lung disease; the ability to generate data to initiate clinical trials and the timing of CTA and IND submissions; the expansion of its CRISPR/Cas9 technology and related technologies to advance additional development candidates, as well as its ability to maintain and expand its related intellectual property portfolio; expectations of the potential impact of the coronavirus disease 2019 pandemic on strategy, future operations and timing of its clinical trials; and the ability to optimize the impact of its collaborations on its development programs, including but not limited to its collaborations with Regeneron, including its co-development program for ATTR amyloidosis, and with Kyverna for the development of KYV-201.

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 authorization, initiation and conduct of studies and other development requirements for its product candidates, including uncertainties related to regulatory approvals to conduct clinical trials; 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; the risk that clinical study results will not be positive; and the risk that Intellia's collaborations with Regeneron or Kyverna 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 and quarterly report on Form 10-Q, 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.

Intellia Contacts:

Investors:
Ian Karp
Senior Vice President, Investor Relations and Corporate Communications
+1-857-449-4175
ian.karp@intelliatx.com

Lina Li
Director, Investor Relations
+1-857-706-1612
lina.li@intelliatx.com

Media:
Matt Crenson
Ten Bridge Communications
+1-917-640-7930
media@intelliatx.com
mcrenson@tenbridgecommunications.com

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