Intellia Therapeutics Highlights its Three-Year Strategic Priorities and Anticipated 2024 Key Milestones

January 4, 2024

- *Core priorities through 2026 include completion of Phase 3 study and BLA submission for NTLA-2002, completion of patient enrollment in the pivotal study of NTLA-2001, clinical validation of modular* in vivo *gene insertion technology and advancement of earlier-stage pipeline and platform innovations*
- *On track to dose the first patient in the pivotal Phase 3 MAGNITUDE trial of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy in Q1 2024*
- *Expect to present results from the Phase 2 study of NTLA-2002 for the treatment of hereditary angioedema (HAE) and to initiate the Phase 3 study in 2024*
- *On track to dose the first patient in the Phase 1 study of NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency (AATD)-associated lung disease in 2024*
- *Ended 2023 in a strong financial position with approximately $1.0 billion in cash; cash runway extended into mid-2026*

CAMBRIDGE, Mass., Jan. 04, 2024 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies, today announced its strategic priorities through 2026 and key anticipated 2024 milestones that support the company's mission to transform the lives of patients and bring forth a new era in medicine.

"2023 was a momentous year for Intellia as our lead investigational therapy, NTLA-2001, advanced into the final stage of clinical development for the treatment of transthyretin amyloidosis with cardiomyopathy," said Intellia President and Chief Executive Officer John Leonard, M.D. "Looking ahead, we will continue to turn groundbreaking science into real-world medicines by capitalizing on our extensive experience and capabilities to edit disease-causing genes. We are rapidly expanding the potential to treat an even larger range of diseases, including those that originate outside of the liver, by deploying our novel delivery and editing technologies. With our comprehensive gene editing toolbox, we are well-positioned to harness the full potential of CRISPR-based medicines for patients."

Recent Pipeline Advancement and Corporate Updates

- **NTLA-2001 for Transthyretin (ATTR) Amyloidosis:** Initiated and actively enrolling the pivotal Phase 3 MAGNITUDE trial in adults with ATTR amyloidosis with cardiomyopathy (ATTR-CM).
- **NTLA-2002 for Hereditary Angioedema (HAE):** Completed enrollment and dosing in the Phase 2 portion of the Phase 1/2 study in adults with HAE.
- **NTLA-3001 for Alpha-1 Antitrypsin Deficiency (AATD)-Associated Lung Disease:** Submitted a clinical trial application to initiate a first-in-human Phase 1 study of NTLA-3001 ahead of previous Q1 2024 expectation.
- **Organizational Streamlining:** Following an internal strategic review, Intellia will be streamlining company-wide operations to further focus resources on key strategic priorities and programs. These changes will result in a pause of select exploratory research-stage programs and a workforce reduction of approximately 15%.
- **Cash Runway:** Intellia ended the fourth quarter of 2023 with approximately $1.0 billion in cash, cash equivalents and marketable securities. The cash position is expected to fund operations into mid-2026.
- **Board Update:** In December, Intellia announced the departure of John Crowley from its board of directors following his appointment as Chief Executive Officer of the Biotechnology Innovation Organization (BIO), the life sciences industry's leading trade and advocacy association.

2024 – 2026 Strategic Priorities

1. **Complete patient enrollment for pivotal studies of NTLA-2001 and NTLA-2002**, including planned biologics license application (BLA) submission for NTLA-2002 in 2026;
2. **Launch clinical trials for next wave of *in vivo* and *ex vivo* programs**, validating CRISPR-based *in vivo* targeted gene insertion and an allogeneic cell engineering solution designed to avoid NK cell-mediated rejection; and
3. **Expand the range of diseases that can be targeted by deploying new editing and delivery innovations**, including advancing gene editing in tissues outside the liver and DNA writing technology.

Anticipated 2024 Milestones

- **NTLA-2001 for ATTR Amyloidosis:**
  - Dose the first patient in MAGNITUDE trial of NTLA-2001 for the treatment of ATTR-CM in Q1 2024.
  - Continue to open new sites and enroll patients, globally, in the MAGNITUDE study.
  - Prepare for the Phase 3 study of NTLA-2001 for the treatment of ATTR amyloidosis with polyneuropathy (ATTRv-PN) in 2024.
  - Present updated clinical data from the ongoing Phase 1 study of NTLA-2001 in 2024.
- **NTLA-2002 for HAE:**
  - Initiate the global pivotal Phase 3 trial of NTLA-2002 in 2H 2024, subject to regulatory feedback.
  - Present updated data from Phase 1 and new data from Phase 2 portion in 2024.
- **NTLA-3001 for AATD-Associated Lung Disease:**
  - Dose first patient in Phase 1 study of NTLA-3001 in 2024.

Presentation at the 42nd Annual J.P. Morgan Healthcare Conference

John Leonard, M.D., President and Chief Executive Officer of Intellia, will present a company overview at the 42nd Annual J.P. Morgan Healthcare Conference in San Francisco on Tuesday, January 9, at 5:15 p.m. PT (8:15 p.m. ET). A live webcast will be available through the Events and Presentations page of the Investors & Media section on Intellia's website, www.intelliatx.com. A replay of the webcast will be available on Intellia's website for a limited time following the conference.

About Intellia Therapeutics

Intellia Therapeutics, Inc. (NASDAQ:NTLA) is a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies. The company's *in vivo* programs use CRISPR to enable precise editing of disease-causing genes directly inside the human body. Intellia's *ex vivo* programs use CRISPR to engineer human cells outside the body for the treatment of cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its people, is helping set the standard for a new class of medicine. To harness the full potential of gene editing, Intellia continues to expand the capabilities of its CRISPR-based platform with novel editing and delivery technologies. Learn more at intelliatx.com and follow us @intelliatx.

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 concerning: its ability to expand and validate its industry-leading gene editing platform, including the advancement of novel gene editing technologies, such as DNA writing, and delivery to other tissues outside of the liver; the safety, efficacy, success and advancement of its clinical programs for NTLA-2001 for the treatment of transthyretin ("ATTR") amyloidosis, NTLA-2002 for the treatment of hereditary angioedema ("HAE"), and NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency ("AATD")-associated lung disease, including the expected timing of data releases, regulatory filings, and the initiation, dosing, and completion of clinical trials, such as the dosing of the first patient in the pivotal Phase 3 MAGNITUDE trial of NTLA-2001 for ATTR amyloidosis with cardiomyopathy ("ATTR-CM") in Q1 2024, the preparation for a Phase 3 study of NTLA-2001 for the treatment of ATTR amyloidosis with polyneuropathy ("ATTRv-PN") in 2024, the presentation of updated clinical data from the ongoing Phase 1 study of NTLA-2001 in 2024, the initiation of a global pivotal Phase 3 trial of NTLA-2002 in 2H 2024, the presentation of additional data from the Phase 1/2 trial of NTLA-2002 in 2024, the submission of a biologics license application for NTLA-2002 in 2026, and the dosing of the first patient in the Phase 1 study of NTLA-3001 for the treatment of AATD-associated lung disease in 2024; the advancement of additional development candidates and expectations of advancing such development candidates and releasing data related to such technologies and development candidates, including its ability to launch clinical trials for its next wave of *in vivo* and *ex vivo* programs and to validate CRISPR-based *in vivo* targeted gene insertion and an allogeneic cell engineering solution designed to avoid NK cell-mediated rejection; its ability to optimize the impact of its collaborations on its development programs, including its collaboration with Regeneron Pharmaceuticals, Inc. and their co-development program for ATTR amyloidosis, and to advance additional development candidates; and its ability to fund operations into mid-2026.

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 contract manufacturers, 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 preclinical and clinical studies and other development requirements for its product candidates, including uncertainties related to regulatory approvals to conduct clinical trials; risks related to the ability to develop and commercialize any one or more of Intellia's product candidates successfully; risks related to the results of preclinical studies or clinical studies not being predictive of future results in connection with future studies; the risk that clinical study results will not be positive; risks related to the development and advancement of novel platform capabilities, such as DNA writing technology and gene editing in tissues outside the liver; risks related to Intellia's future financial condition and its ability to fund its operations; and risks related to Intellia's collaborations with Regeneron Pharmaceuticals, Inc. or its other collaborations not continuing or not being 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 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. 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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