Intellia Therapeutics Announces Anticipated 2025 Milestones and Strategic Reorganization to Prioritize the Advancement of its Late-Stage Programs, NTLA-2002 and Nexiguran Ziclumeran (nex-z)

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- *Priority programs – NTLA-2002 for hereditary angioedema (HAE) and nexiguran ziclumeran (nex-z) for transthyretin (ATTR) amyloidosis – set foundation for significant, near-term value creation*
- *Phase 3 HAELO study evaluating NTLA-2002 for HAE to complete enrollment in the second half of 2025; Company plans to submit a Biologics License Application in the second half of 2026*
- *More than 550 patients expected to be enrolled by year end within the ongoing MAGNITUDE study for nex-z in ATTR-CM – the program remains ahead of internal enrollment estimates*
- *Pipeline priorities result in NTLA-3001 discontinuation and select, research-focused investment*
- *Anticipated cost savings, including a net workforce reduction of approximately 27% in 2025, support company operations into 1H 2027 and through anticipated, first commercial launch in the U.S.*

CAMBRIDGE, Mass., Jan. 09, 2025 (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 and key anticipated 2025 milestones that support the Company's mission to transform the lives of patients and bring forth a new era in medicine.

"We have made significant progress and built strong momentum in 2024 with three actively enrolling, Phase 3, pivotal studies. Our early clinical data for both NTLA-2002 and nex-z support novel, highly differentiated product profiles that directly address the significant unmet needs of patients and prescribers in HAE and ATTR," said John Leonard, M.D., President and Chief Executive Officer of Intellia. "We understand the significant potential of our late-stage programs, and within a challenging market environment, have made a difficult decision to focus our resources predominantly on NTLA-2002 and nex-z where we have the greatest opportunity to create significant, near-term value."

Recent Pipeline Advancement and Corporate Updates

- **Strategic Reorganization Focused on Key Value Drivers:**
  - The pipeline prioritization is intended to focus resources on high value programs - NTLA-2002 and nex-z - to ensure efficient execution, achieve near-term clinical milestones, and prepare Intellia for commercial launch. As part of this prioritization, the Company discontinued development of NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency-associated lung disease and select research-stage programs. Over the course of 2025, the strategic reorganization will result in a net workforce reduction of approximately 27%.
  - The Company expects to incur charges of approximately $8 million associated with the reorganization, which are anticipated to be incurred in the first quarter of 2025.
  - Intellia ended the fourth quarter of 2024 with approximately $862 million in cash, cash equivalents and investments. The combination of its cash balance and the anticipated cost savings are expected to provide the Company with cash runway into the first half of 2027.
  - Laura Sepp-Lorenzino, Ph.D., Intellia's Chief Scientific Officer, has announced her retirement effective December 31, 2025 after more than 30 years of service in the biopharmaceutical industry and academia. Dr. Sepp-Lorenzino will transition from her role as CSO and continue as a Senior Scientific Advisor for the remainder of the year. "I would like to thank Laura for her service to Intellia. She has been a tireless innovator and advocate for the promise of gene editing, and we look forward to continuing the work and vision she championed," said John Leonard M.D., President and Chief Executive Officer of Intellia.
  - The Company also announced that Birgit Schultes, Ph.D., who has been leading Immunology and Cell Therapy since 2017, will be promoted to Executive Vice President and Chief Scientific Officer, effective January 13, 2025. Dr. Schultes has over 20 years of experience in drug development and biotechnology, including the clinical development of cell therapies and complex biologic products. Dr. Schultes received her M.S. in biology and Ph.D. in immunology from the University of Bonn, Germany, and completed the Advanced Management Development Program at the Boston University Questrom School of Business.
- **NTLA-2002 for Hereditary Angioedema (HAE):**
  - Patients are actively enrolling in the pivotal Phase 3 HAELO study.
  - Presented clinical data, in October at the 2024 American College of Allergy, Asthma & Immunology (ACAAI) Scientific Meeting, from the Phase 2 study that demonstrated the potential of NTLA-2002 to end chronic,

prophylaxis treatment and provide freedom from attacks following a one-time infusion.

- **Nex-z for Transthyretin (ATTR) Amyloidosis:**
  - Strong enrollment and momentum continue in the Phase 3 MAGNITUDE study in patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM), tracking ahead of the Company's target enrollment projections.
  - Patients with hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN) are actively screening in the Phase 3 MAGNITUDE-2 study.
  - Presented first clinical evidence, in November at the 2024 American Heart Association (AHA) Scientific Sessions, that consistent, rapid, deep and durable reductions in serum TTR achieved after a one-time treatment of nex-z may halt, and potentially reverse, disease progression.

Strategic Priorities and Anticipated 2025 Milestones

Intellia's strategic priorities reflect the Company's ongoing evolution from a late-stage development company to a commercial-ready organization by the end of 2026.

1. Drive focused clinical execution to complete or accelerate enrollment in the pivotal studies of NTLA-2002 and nex-z

- NTLA-2002 for HAE:
  - Dose the first patient in the pivotal Phase 3 HAELO trial in 1Q25.
  - Complete HAELO enrollment in second half of 2025.
  - Present longer-term data from the Phase 1/2 study – data will include patients in the Phase 2 portion who initially received a 25 mg dose or placebo and were subsequently given the 50 mg dose of NTLA-2002 selected for the Phase 3 study
- Nex-z for ATTR amyloidosis:
  - Dose the first patient in the pivotal Phase 3 MAGNITUDE-2 trial for ATTRv-PN in 1Q25.
  - Enroll at least 550 patients cumulatively within the MAGNITUDE trial for ATTR-CM.
  - Present longer-term data from both ATTR-CM and ATTRv-PN patients in the Phase 1 study – data will include updated measures of clinical efficacy and safety.

2. Advance commercial readiness by implementing core commercialization and medical capabilities for initial launch in the U.S.

- Complete buildout of the commercial leadership team by second half of 2025.
- Expand the reach of medical education activities in HAE and ATTR amyloidosis in partnership with key medical societies and patient organizations.
- Initiate pre-approval information exchange to allow payers to begin planning for coverage and formulary decisions.

Presentation at the 43rd Annual J.P. Morgan Healthcare Conference

John Leonard, M.D., President and Chief Executive Officer of Intellia, will present a company overview at the 43rd Annual J.P. Morgan Healthcare Conference in San Francisco on Monday, January 13, at 8:15 a.m. PT (11:15 a.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. Since its inception, Intellia has focused on leveraging gene editing technology to develop novel, first-in-class medicines that address important unmet medical needs and advance the treatment paradigm for patients. 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 successfully develop and commercialize nexiguran ziclumeran ("nex-z"), formerly known as NTLA-2001, for the treatment of transthyretin ("ATTR") amyloidosis and NTLA-2002 for the treatment of hereditary angioedema ("HAE") to address the significant unmet needs of patients and prescribers in HAE and ATTR; its ability to achieve near-term clinical milestones, including dosing the first patient in the Phase 3 HAELO trial in the first quarter of 2025, completing enrollment in the the Phase 3 HAELO trial in the second half of 2025, dosing the first patient in the Phase 3 MAGNITUDE-2 trial for hereditary ATTR with polyneuropathy ("ATTRv-PN") in the first quarter of 2025, enroll at least 550 patients across the Phase 3 MAGNITUDE trial for ATTR with cardiomyopathy ("ATTR-CM") by year-end, and the expected timing of data releases from its clinical trials of nex-z and NTLA-2002, including longer-term data from the Phase 1/2 study of NTLA-2002, including data from patients that previously received the 25 mg dose or placebo and were subsequently given the 50 mg dose, and longer-term data from the Phase 1 study of nex-z, including updated measure of clinical efficacy and safety; its ability to prepare for commercial launch, including completing buildout of the commercial leadership team in the first half of 2025, expanding the reach of medical education activities in HAE and ATTR amyloidosis in 2025, and initiating pre-approval information exchange to allow payers to begin planning for potential coverage and formulary decisions in 2025; its interactions with regulatory authorities, including the potential submission of a biologics license application for NTLA-2002 for the treatment of HAE in the second half of 2026; 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 expectations regarding its uses of capital, expenses, and ability to fund operations into the first half of 2027.

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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