Intellia Therapeutics Announces Fourth Quarter and Full-Year 2024 Financial Results and Highlights Recent Company Progress

February 27, 2025

- *Dosed first patient in global Phase 3 HAELO study evaluating NTLA-2002 for hereditary angioedema (HAE)*
- *Expect to complete enrollment of the HAELO study in the second half of 2025 and submit a Biologics License Application in the second half of 2026 to support plans for U.S. launch in 2027*
- *Enrollment in the pivotal Phase 3 MAGNITUDE trial of nexiguran ziclumeran (nex-z) in patients with transthyretin amyloidosis (ATTR) with cardiomyopathy continues to track ahead of projections; more than 550 total patients expected to be enrolled by year end*
- *Actively screening for the Phase 3 MAGNITUDE-2 trial for nex-z in hereditary ATTR amyloidosis with polyneuropathy; on track to dose first patient in 1Q25*
- *Ended 2024 with approximately $862 million in cash, cash equivalents and marketable securities*

CAMBRIDGE, Mass., Feb. 27, 2025 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies, today reported operational highlights and financial results for the fourth quarter and year ended December 31, 2024.

"We are off to an excellent start in 2025 with renewed focus and strong operational execution across our three, pivotal Phase 3 studies," said Intellia President and Chief Executive Officer John Leonard, M.D. "We are excited by the clinical data presented during the fourth quarter. Our Phase 1/2 results in HAE suggest that NTLA-2002 could represent a functional cure for patients with HAE – for the first time a patient has the potential to be both free from attacks and free from chronic therapy. Similarly, the rapid, deep and durable reductions in serum TTR demonstrated to date in our Phase 1 study of nex-z in ATTR represent a highly differentiated profile that may offer patients an opportunity to stabilize or improve their clinical readouts in an otherwise unrelenting, progressive disease."

Fourth Quarter 2024 and Recent Operational Highlights

Hereditary Angioedema (HAE)

- **NTLA-2002:** NTLA-2002 is a wholly owned, investigational *in vivo* CRISPR-based therapy designed to knock out the *KLKB1* gene in the liver, with the goal of lifelong control of HAE attacks after a single dose.
  - In January, Intellia announced the first patient was dosed with NTLA-2002 in the global Phase 3 HAELO study. The Company expects to complete enrollment in the second half of 2025.
  - The Company plans to submit a Biologics License Application in the second half of 2026 to support plans for a U.S. launch in 2027.
  - Intellia expects to present longer-term data from the ongoing Phase 1/2 study in 2025. The 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.

Transthyretin (ATTR) Amyloidosis

- **Nexiguran ziclumeran (nex-z, also known as NTLA-2001)**: Nex-z is an investigational *in vivo* CRISPR-based therapy designed to inactivate the *TTR* gene in liver cells, thereby preventing the production of transthyretin (TTR) protein for the treatment of ATTR amyloidosis. Nex-z offers the possibility of halting and reversing the disease by driving a deep, consistent and potentially lifelong reduction in TTR protein after a single dose. Nex-z has been generally well tolerated across all patients and at all dose levels tested. The most common treatment-related adverse event was an infusion reaction, which were mild or moderate; all patients were able to receive the intended dose of nex-z. Intellia leads development and commercialization of nex-z in collaboration with Regeneron.
  - **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
    - Enrollment in the pivotal Phase 3 MAGNITUDE trial is progressing ahead of our target projections and we anticipate enrollment to exceed 550 total patients by year end.
    - Intellia presented data from the ongoing Phase 1 study at the 2024 American Heart Association (AHA) Scientific Sessions and published the findings online in the *New England Journal of Medicine*. Across all

patients (n=36), a single dose of nex-z led to consistently rapid, deep and sustained serum TTR reduction, regardless of baseline levels, through the latest follow-up. At month 12, the mean serum TTR reduction was 90%, and the mean absolute residual serum TTR concentration was 17 µg/mL. With 11 patients who have reached 24 months of follow-up, all patients continued to show a sustained response with no evidence of a waning effect over time. The consistently low levels of serum TTR are anticipated to reduce the rate of ongoing amyloid formation and potentially allow for amyloid clearance and improvement in cardiac function. Nex-z was generally well tolerated across all patients.

- **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
  - We are actively screening patients for the Phase 3 MAGNITUDE-2 study and are on track to dose the first patient in the first quarter of 2025.
  - Intellia presented data from the ongoing Phase 1 study in November. At month 12, patients who received a dose of 0.3 mg/kg or higher (n=33) had a mean serum TTR reduction of 91% and mean absolute residual serum TTR concentration of 20 µg/mL. For the 16 patients who reached 24 months of follow-up, there was no change to their post-dose TTR levels. It is anticipated that greater TTR reduction may lead to a greater clinical benefit in patients with ATTRv-PN. Nex-z was generally well tolerated across all patients and at all dose levels tested.
  - In November, Intellia announced the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) to nex-z for the treatment of ATTRv-PN.
- Intellia expects to present longer-term data from both ATTR-CM and ATTRv-PN patients in the Phase 1 study in 2025. The data will include updated measures of clinical efficacy and safety.

Platform Update

- Intellia continues to apply novel technologies, such as CRISPR-based gene editing technologies and lipid nanoparticle (LNP) delivery technologies, to develop *in vivo* and *ex vivo* product candidates. Treating—and potentially curing—a broad range of severe diseases requires the application of multiple technologies. With Intellia's proprietary technology at the core of the platform, the Company continues to research and develop new gene editing and delivery technologies to expand the therapeutic opportunities, furthering progress on the frontier of genetic medicine.

Corporate Update

- On January 9, 2025, the Company announced that, after a strategic review of its business, it elected to prioritize late-stage programs – NTLA-2002 for HAE and nex-z for ATTR amyloidosis – and select research investments to focus on near-term value creation. As a result, the Company discontinued NTLA-3001 and other, undisclosed programs, and is reducing its workforce by approximately 27% in 2025. The Company expects to incur charges of approximately $8.0 million for severance and other employee termination-related costs in the first quarter of 2025.

Upcoming Events

The Company will participate in the following events during the first quarter of 2025:

- AAAAI/WAO Joint Congress, March 1, San Diego
- TD Cowen 45th Annual Health Care Conference, March 4, Boston
- Leerink 2025 Global Biopharma Conference, March 10, Miami
- Barclays 27th Annual Global Healthcare Conference, March 11, Miami
- Jefferies Biotech on the Beach Summit, March 12, Miami

Fourth Quarter and Full-Year 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $861.7 million as of December 31, 2024, compared to $1.0 billion as of December 31, 2023. The Company's cash position as of December 31, 2024, is expected to fund operations into the first half of 2027.
- **Collaboration Revenue:** Collaboration revenue was $12.9 million during the fourth quarter of 2024, compared to negative $1.9 million during the fourth quarter of 2023. The $14.8 million increase was mainly driven by collaboration revenue received under the Regeneron agreements.
- **R&D Expenses:** Research and development (R&D) expenses were $116.9 million during the fourth quarter of 2024, compared to $109.0 million during the fourth quarter of 2023. The $7.9 million increase was primarily driven by the advancement of our lead programs. Stock-based compensation expense included in R&D expenses was $24.4 million for the fourth quarter of 2024.

- **G&A Expenses:** General and administrative (G&A) expenses were $32.4 million during the fourth quarter of 2024, compared to $29.0 million during the fourth quarter of 2023. The $3.4 million increase was primarily related to stock-based compensation. Stock-based compensation expense included in G&A expenses was $15.2 million for the fourth quarter of 2024.

- **Net Loss:** Net loss was $128.9 million for the fourth quarter of 2024, compared to $132.2 million during the fourth quarter of 2023.

Conference Call to Discuss Fourth Quarter and Full-Year 2024 Results

The Company will discuss these results on a conference call today, Thursday, February 27 at 8 a.m. ET.
To join the call:

- U.S. callers should dial 1-833-316-0545 and international callers should dial 1-412-317-5726, approximately five minutes before the call. All participants should ask to be connected to the Intellia Therapeutics conference call.
- Please visit this link for a simultaneous live webcast of the call.

A replay of the call will be available through the Events and Presentations page of the Investors & Media section on Intellia's website at intelliatx.com, beginning on February 27 at 12 p.m. ET.

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: the safety, efficacy, success and advancement of its clinical programs for NTLA-2001, also known as nexiguran ziclumeran or "nex-z", for transthyretin ("ATTR") amyloidosis and NTLA-2002 for the treatment of hereditary angioedema ("HAE") pursuant to its clinical trial applications ("CTA") and investigational new drug ("IND") submissions, including the expected timing of data releases, regulatory feedback, regulatory filings, and the initiation, enrollment, dosing and completion of clinical trials, such as the completion of enrollment of the Phase 3 HAELO study in the second half of 2025 and the submission of a biologics license application in the second half of 2026, its ability to rapidly enroll the Phase 3 MAGNITUDE study, the planned initiation of the Phase 3 trial MAGNITUDE-2 by year-end, the plan to dose the first patient in the global pivotal Phase 3 MAGNITUDE-2 trial in the first quarter of 2025, the potential of NTLA-2001 to halt and reverse disease by driving a deep, consistent and potentially lifelong reduction in TTR protein after a single dose, and the potential of NTLA-2002 to be a functional cure for patients with HAE and to demonstrate lifelong control of HAE attacks and chronic therapy after a single dose; its ability to apply novel technologies, such as CRISPR-based gene editing technologies and lipid nanoparticle (LNP) delivery technologies, to develop *in vivo* and *ex vivo* product candidates, including its ability to use those technologies to expand therapeutic opportunities and the timing expectations of advancing such product candidates; its ability to optimize the impact of its collaborations on its development programs, including, but not limited to, its collaboration with Regeneron Pharmaceuticals, Inc. ("Regeneron") and their co-development programs for ATTR amyloidosis; and its growth as a company and expectations regarding its uses of capital, expenses, future accumulated deficit and financial results, including its 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, collaborators, 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 potential delay of planned clinical trials due to regulatory feedback or other developments; and risks related to Intellia's collaborations with Regeneron, 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 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, including its quarterly report on Form 10-Q. 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 THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)
(Amounts in thousands, except per share data)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2024	2023	2024	2023
Collaboration revenue	$ 12,874	$ (1,917)	$ 57,877	$ 36,275
Operating expenses:				
Research and development	116,877	108,981	466,311	435,069
General and administrative	32,444	28,994	125,829	116,497
Total operating expenses	149,321	137,975	592,140	551,566

Operating loss	(136,447)	(139,892)	(534,263)	(515,291)
Other income (expense), net:				
Interest income	10,631	12,459	47,807	49,832
Change in fair value of investments, net	(3,082)	-	(32,565)	-
Loss from equity method investment	-	(4,728)	-	(15,633)
Change in fair value of contingent consideration	-	-	-	(100)
Total other income (expense), net	7,549	7,731	15,242	34,099
Net loss	$ (128,898)	$ (132,161)	$ (519,021)	$ (481,192)
Net loss per share, basic and diluted	$ (1.27)	$ (1.46)	$ (5.25)	$ (5.42)
Weighted average shares outstanding, basic and diluted	101,855	90,461	98,849	88,770

INTELLIA THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEET DATA (UNAUDITED)
(Amounts in thousands)

	December 31, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 861,730	$ 1,012,087
Total assets	1,191,015	1,300,977
Total liabilities	319,059	250,808
Total stockholders' equity	871,956	1,050,169

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