Intellia Therapeutics Announces Fourth Quarter and Full-Year 2025 Financial Results and Business Updates

February 26, 2026

- *HAELO Phase 3 clinical data for lonvo-z in HAE expected by mid-2026; BLA submission in second half of 2026; anticipated U.S. launch in first half of 2027*
- *Process underway to reactivate global sites for MAGNITUDE-2 Phase 3 clinical trial of nex-z in ATTRv-PN; enrollment completion expected in second half of 2026*
- *FDA engagement ongoing to resolve clinical hold on MAGNITUDE Phase 3 clinical trial of nex-z in ATTR-CM*
- *Ended 2025 with approximately $605 million in cash, cash equivalents and marketable securities; expected to fund operations into the second half of 2027*

CAMBRIDGE, Mass., Feb. 26, 2026 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (Nasdaq: NTLA), a leading biopharmaceutical company focused on revolutionizing medicine leveraging CRISPR gene editing and other core technologies, today reported business updates and financial results for the fourth quarter and year ended December 31, 2025.

“2025 was a time of accomplishment and resiliency for Intellia as we presented encouraging longer term Phase 1/2 clinical data for both lonvo-z and nex-z, rapidly enrolled patients in our three Phase 3 trials, commenced activities to prepare for a potential lonvo-z launch in HAE and responded to the clinical holds on our nex-z Phase 3 trials late in the year,” said Intellia President and Chief Executive Officer John Leonard, M.D. “We expect the year ahead to be a pivotal one, highlighted by our topline Phase 3 data and planned BLA submission for lonvo-z, which has the potential to transform the HAE treatment paradigm by freeing most patients from both their attacks and chronic therapy. Additionally, we are focused on resuming our forward momentum with nex-z by completing patient enrollment in MAGNITUDE-2 and resolving the clinical hold on MAGNITUDE.”

Lonvoguran Ziclumeran (Lonvo-z) for Hereditary Angioedema (HAE)

Lonvo-z is a wholly owned, investigational *in vivo* CRISPR-based therapeutic candidate designed to inactivate the *KLKB1* gene in the liver, drive consistent, deep and potentially lifelong reduction in kallikrein levels, and dramatically reduce or eliminate HAE attacks via a one-time treatment.

- In the fourth quarter at the American College of Allergy, Asthma & Immunology (ACAAI) 2025 Annual Scientific Meeting, Intellia presented positive clinical data from a pooled analysis of all patients who received a 50 milligram (mg) dose of lonvo-z in the company’s ongoing Phase 1/2 clinical trial in patients with HAE. Observations from the analysis included durable reductions in plasma kallikrein in all patients at month 24, a high percentage of patients achieving prolonged attack-free status (for at least seven months and up to 32 months for patients with the longest follow-up) and a well-tolerated safety profile for lonvo-z.
- Intellia sponsored a blinded market research study in late 2025 with 104 U.S. HAE patients and 151 U.S. HAE treating physicians. After reviewing a blinded target product profile aligned with lonvo-z’s Phase 1/2 clinical data, 99% of patients said they would be at least somewhat likely – and 64% said they would be extremely or very likely – to take lonvo-z if prescribed. Additionally, 92% of healthcare providers indicated they would prescribe a product with this profile, estimating they would prescribe it to 54% of the approximately 4,000 patients with HAE under their care.
- This weekend, the company will present four posters at the 2026 American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting taking place February 27 – March 2 in Philadelphia, Pennsylvania (poster numbers 003, 005, 061 and 716). The presentations include three-year follow-up data from patients receiving a one-time 50 mg dose of lonvo-z and new survey findings assessing the chronic treatment burden and unmet needs among patients living with HAE.
- Dosing in the global HAELO Phase 3 clinical trial was initiated in January 2025 and was completed in September 2025, with 80 patients enrolled. Intellia expects to report HAELO topline data by mid-2026 and, if the data are supportive, submit a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) in the second half of 2026.
- To prepare for a planned launch in the first half of 2027, the company has expanded its field medical team, strengthened engagement with treating physicians and patient advocacy groups, initiated its payer engagements and advanced its launch strategy. In 2026, the company intends to build its field sales and reimbursement teams, finalize its distribution models, identify U.S. treatment centers and advance its pricing and access planning strategy.

Nexiguran Ziclumeran (Nex-z) for Transthyretin (ATTR) Amyloidosis

Nex-z is an investigational *in vivo* CRISPR-based therapeutic candidate designed to inactivate the *TTR* gene in the liver, thereby preventing the production of transthyretin (TTR) protein. Nex-z offers the possibility of halting and reversing disease by driving a deep, consistent and potentially lifelong reduction in TTR protein after a one-time treatment. Intellia leads the development and commercialization of nex-z in collaboration with Regeneron Pharmaceuticals, Inc.

- In the fourth quarter at the American Heart Association (AHA) Scientific Sessions, Intellia presented positive follow-up data from the ongoing Phase 1 clinical trial of nex-z in patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM). Observations from these longer-term data included consistent and durable reductions in serum TTR through up to three years of follow up, stability or improvement in multiple markers of cardiomyopathy for most patients and encouraging mortality data.
- As previously reported, on October 29, 2025, the FDA placed a clinical hold on the Investigational New Drug (IND) applications for the MAGNITUDE and MAGNITUDE-2 Phase 3 clinical trials for patients with ATTR-CM and hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN), respectively.
    - On January 27, 2026, the company announced the FDA lifted the clinical hold on the IND for MAGNITUDE-2. The company is in the process of resuming MAGNITUDE-2 enrollment.
    - Intellia's engagement with FDA is ongoing regarding the clinical hold on the IND for the MAGNITUDE Phase 3 clinical trial of nex-z for patients with ATTR-CM. The company plans to provide an update once alignment has been achieved on the path forward for this program.

Fourth Quarter and Full-Year 2025 Financial Results

- Cash Position: Cash, cash equivalents and marketable securities were $605.1 million as of December 31, 2025, compared to $861.7 million as of December 31, 2024. The company's cash, cash equivalents and marketable securities as of December 31, 2025 are expected to fund operations into the second half of 2027 and through lonvo-z's anticipated U.S. commercial launch for HAE.
- Collaboration Revenue: Collaboration revenue was $23.0 million for the fourth quarter of 2025, compared to $12.9 million for the fourth quarter of 2024. The increase was primarily driven by the recognition of $9.0 million in revenue related to the termination of the license and collaboration agreement with SparingVision SAS and an increase in cost reimbursements related to the company's collaboration with Regeneron.
- R&D Expenses: Research and development (R&D) expenses were $88.7 million for the fourth quarter of 2025, compared to $116.9 million for the fourth quarter of 2024. The $28.2 million decrease was primarily driven by employee-related expenses, stock-based compensation, research materials and contracted services, partially offset by an increase in facility-related expenses as well as clinical trial expenses related to nex-z. Stock-based compensation expense included in R&D expenses was $10.5 million for the fourth quarter of 2025.
- G&A Expenses: General and administrative (G&A) expenses were $33.1 million for the fourth quarter of 2025, compared to $32.4 million for the fourth quarter of 2024. Stock-based compensation expense included in G&A expenses was $6.2 million for the fourth quarter of 2025.
- Net Loss: Net loss was $95.8 million for the fourth quarter of 2025, compared to $128.9 million for the fourth quarter of 2024.

Conference Call Information
The company will host a conference call and webcast today at 8:00 a.m. ET to discuss recent updates and the company's fourth quarter and full-year 2025 financial results. To join the webcast, please visit the Events and Presentations page of the Investors & Media section on Intellia's website at intelliatx.com. To join by phone, 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. A replay of the webcast will be available for approximately 90 days.

About Intellia Therapeutics
Intellia Therapeutics, Inc. (Nasdaq: NTLA) is a leading clinical-stage biopharmaceutical company focused on revolutionizing medicine leveraging CRISPR gene editing and other core technologies. The company's mission is to transform the lives of people with severe diseases by developing and commercializing potentially curative treatments. With deep scientific, technical and clinical development experience, Intellia aims to reset the standard for medicine by durably treating the root causes of disease. 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 nexiguran ziclumeran or "nex-z" (also known as NTLA-2001) for transthyretin ("ATTR") amyloidosis, including the ability to resolve the clinical hold that the United States Food and Drug Administration ("FDA") placed on the investigational new drug ("IND") application for the MAGNITUDE Phase 3 study of nex-z for ATTR amyloidosis with cardiomyopathy ("ATTR-CM"), resume dosing and screening in the MAGNITUDE study and the MAGNITUDE-2 Phase 3 study of nex-z for hereditary ATTR amyloidosis with polyneuropathy ("ATTRv-PN"), and provide an update on its guidance for nex-z; the safety, efficacy, success and advancement of its clinical programs for lonvoguran ziclumeran or "lonvo-z" (also known as NTLA-2002) for the treatment of hereditary angioedema ("HAE") pursuant to its clinical trial applications ("CTA") and IND application submissions, including the expected timing of data releases from its ongoing clinical trials of nex-z and lonvo-z, regulatory feedback, regulatory filings, and the enrollment, dosing and completion of clinical trials, such as sharing topline data from the HAELO Phase 3 study of lonvo-z by mid-2026; its ability to submit a biologics license application ("BLA") for lonvo-z in the second half of 2026; the potential of nex-z to address important unmet needs for patients with ATTR amyloidosis and to halt and reverse disease by driving a deep, consistent and potentially lifelong reduction in TTR protein after a one-time treatment; the potential of lonvo-z to inactivate the *KLKB1* gene in the liver, drive consistent, deep and potentially lifelong reduction in kallikrein levels and to dramatically reduce or eliminate HAE attacks via a one-time treatment; 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 program 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 second half of 2027 and lonvo-z's anticipated U.S. commercial launch for HAE.

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: uncertainty as to when the clinical hold on MAGNITUDE may be resolved, including the actions that the company may be required to take or conduct in order to resolve the clinical hold; uncertainties related to Intellia's ability to resume the MAGNITUDE and MAGNITUDE-2 trials, further development of nex-z, and provide an update on its guidance for nex-z; 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, including lonvo-z for HAE; 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,	
	2025	2024	2025	2024
Collaboration revenue	$ 23,017	$ 12,874	$ 67,671	$ 57,877
Operating expenses:				
Research and development	88,652	116,877	388,861	466,311
General and administrative	33,075	32,444	119,800	125,829
Total operating expenses	121,727	149,321	508,661	592,140
Operating loss	(98,710)	(136,447)	(440,990)	(534,263)
Other income, net:				
Interest income	6,476	10,631	29,195	47,807
Change in fair value of investments, net	(3,552)	(3,082)	(899)	(32,565)
Total other income, net	2,924	7,549	28,296	15,242
Net loss	$ (95,786)	$ (128,898)	$ (412,694)	$ (519,021)
Net loss per share, basic and diluted	$ (0.83)	$ (1.27)	$ (3.81)	$ (5.25)
Weighted average shares outstanding, basic and diluted	115,921	101,855	108,376	98,849

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

	December 31, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 605,134	$ 861,730
Total assets	842,127	1,191,015
Total liabilities	170,733	319,059
Total stockholders' equity	671,394	871,956

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Source: Intellia Therapeutics, Inc.