Intellia Therapeutics Announces Third Quarter 2025 Financial Results and Recent Updates

November 6, 2025

- *Awaiting FDA clinical hold letter on MAGNITUDE and MAGNITUDE-2 clinical trials of nex-z*
- *Presenting longer-term Phase 1 clinical data of nex-z for ATTR-CM on November 10 at AHA 2025; previously published longer-term Phase 1 clinical data for nex-z for ATTRv-PN in* New England Journal of Medicine
- *Completed enrollment in Phase 3 HAELO clinical trial of lonvo-z for HAE; topline data expected by mid-2026 with potential U.S. commercial launch in 1H27*
- *Presenting longer-term Phase 1/2 clinical data of lonvo-z on November 8 at ACAAI 2025*
- *Ended third quarter with approximately $670 million in cash, cash equivalents and marketable securities; expected to fund operations into mid-2027*
- *Conference call today at 6:00 p.m. ET*

CAMBRIDGE, Mass., Nov. 06, 2025 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage gene editing company focused on revolutionizing medicine with CRISPR-based therapies, today provided an update on the MAGNITUDE and MAGNITUDE-2 Phase 3 clinical trials of nexiguran ziclumeran (nex-z) and reported other business updates and financial results for the third quarter ended September 30, 2025.

“We were deeply saddened to learn that the patient who experienced Grade 4 liver transaminase elevations and increased total bilirubin following a dose of nex-z in the MAGNITUDE Phase 3 clinical trial, as reported on October 27, 2025, passed away last night,” said Intellia President and Chief Executive Officer John Leonard, M.D. “We have been advised by the treating physician that this is a case with complicating comorbidities, and it is being further evaluated. As we await the FDA’s clinical hold letter, we are working with clinical investigators and external experts to better understand the liver-related events that have been observed within MAGNITUDE and to develop our risk mitigation plan.”

“We continue to believe in nex-z’s potential to address important unmet needs for patients with ATTR amyloidosis,” Dr. Leonard continued. “Regarding our other late-stage investigational product for the treatment of HAE, lonvo-z, we have made considerable progress over the course of 2025. Enrollment was completed in the HAELO Phase 3 clinical trial of lonvo-z in September, less than nine months after we dosed our first patient in the trial, putting us on track to share topline data by mid-2026. We look forward to presenting longer-term data from our Phase 1/2 clinical trial of lonvo-z on Saturday at ACAAI.”

Recent Updates About Nexiguran Ziclumeran (nex-z) for Transthyretin (ATTR) Amyloidosis

Nex-z is an investigational *in vivo* CRISPR-based therapy 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 single dose. Intellia leads the development and commercialization of nex-z in collaboration with Regeneron Pharmaceuticals, Inc.

On October 29, 2025, the U.S. Food and Drug Administration (FDA) placed a clinical hold on the Investigational New Drug applications for the MAGNITUDE and MAGNITUDE-2 Phase 3 clinical trials for patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM) and hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN), respectively.

More than 650 patients with ATTR-CM are currently enrolled in MAGNITUDE, and 47 patients with ATTRv-PN are enrolled in MAGNITUDE-2. To date, Grade 4 liver transaminase elevations have been reported in less than one percent of all patients enrolled in MAGNITUDE and no Grade 4 liver transaminase elevations have been reported in MAGNITUDE-2.

Intellia recently mandated that all MAGNITUDE and MAGNITUDE-2 clinical sites increase their monitoring of patient laboratory values in the weeks after dosing. The company continues to consult with clinical investigators and other experts to investigate the transaminase elevations and consider potential additional risk mitigation strategies while awaiting the FDA’s formal clinical hold letter. Given the clinical hold, the company has suspended its milestone guidance for nex-z pending regulatory alignment. Intellia plans to provide an update after it has finalized a plan with regulators on the path forward.

- **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
  - On November 10, 2025 at the 2025 American Heart Association (AHA) Scientific Sessions, longer-term data will be presented in a late-breaker oral session from the company’s ongoing Phase 1 clinical trial in patients with ATTR-CM. Details about this session are as follows:
    - Title: Updated Phase 1 Clinical Trial Outcomes of CRISPR Gene Editing with Nexiguran Ziclumeran (Nex-z, NTLA-2001) in Patients with Transthyretin Amyloidosis with Cardiomyopathy
      Session: Rewriting the Code for Cardiac Amyloid: Novel Identification, Treatment, and Cure
      Date and Time: Monday, November 10, 2025, at 3:17 p.m. ET
      Presenter: Julian Gillmore, M.D., Ph.D., Professor of Medicine, National Amyloidosis Centre, UCL Division of Medicine, Royal Free Hospital, U.K.
- **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
  - In September 2025, the company presented positive longer-term follow-up data from its Phase 1 clinical trial in an oral presentation at the 5th International ATTR Amyloidosis Meeting for Patients and Doctors in Baveno, Italy. The

results were simultaneously published in the *New England Journal of Medicine*.

Recent Updates About Lonvoguran Ziclumeran (lonvo-z) for Hereditary Angioedema (HAE)

Lonvo-z is a wholly owned, investigational *in vivo* CRISPR-based therapy designed to inactivate the *KLKB1* gene in the liver that offers the possibility of dramatically reducing or eliminating HAE attacks by driving consistent, deep and potentially lifelong reduction in kallikrein levels after a one-time treatment.

- Dosing in the global Phase 3 HAELO clinical trial was initiated in January 2025 and enrollment was completed in September 2025. Patients in this trial are receiving a 50 milligram (mg) dose of lonvo-z. Intellia expects to:
  - Report HAELO topline data by mid-2026;
  - Submit a Biologics License Application (BLA) to the FDA in the second half of 2026; and
  - Continue preparing for an anticipated U.S. commercial launch in the first half of 2027.
- On November 8, 2025 at the American College of Allergy, Asthma & Immunology Annual Scientific Meeting (ACAAI), longer-term clinical data will be presented in an oral session from all patients who received a 50 mg dose of lonvo-z in Intellia's ongoing Phase 1/2 clinical trial. Details about this session are as follows:
  - Title: Two-Year Durability/Safety of One-time Lonvoguran Ziclumeran (Lonvo-z, NTLA-2002) 50 mg in Patients with Hereditary Angioedema
    Session: Distinguished Industry & Late-Breaking Oral Abstracts – Session 1
    Date and Time: Saturday, November 8, 2025, at 5:13 p.m. ET
    Presenter: Danny Cohn, M.D., Ph.D., Internist, Department of Vascular Medicine, Amsterdam University Medical Center

Third Quarter 2025 Financial Results

- Cash Position: Cash, cash equivalents and marketable securities were $669.9 million as of September 30, 2025, compared to $861.7 million as of December 31, 2024. In the third quarter of 2025, the company raised $114.5 million of net equity proceeds from its "At the Market" (ATM) program. The company's cash, cash equivalents and marketable securities as of September 30, 2025, are now expected to fund operations into mid-2027 and through lonvo-z's anticipated U.S. commercial launch for HAE.
- Collaboration Revenue: Collaboration revenue was $13.8 million for the third quarter of 2025, compared to $9.1 million for the third quarter of 2024. The $4.7 million increase was mainly driven by cost reimbursements related to the company's collaboration with Regeneron Pharmaceuticals, Inc.
- R&D Expenses: Research and development (R&D) expenses were $94.7 million for the third quarter of 2025, compared to $123.4 million for the third quarter of 2024. The $28.7 million decrease was primarily driven by employee-related expenses, stock-based compensation, research materials and contracted services, partially offset by an increase in clinical trial expenses related to lonvo-z. Stock-based compensation expense included in R&D expenses was $12.2 million for the third quarter of 2025.
- G&A Expenses: General and administrative (G&A) expenses were $30.5 million for the third quarter of 2025, compared to $30.5 million for the third quarter of 2024. Stock-based compensation expense included in G&A expenses was $7.4 million for the third quarter of 2025.
- Net Loss: Net loss was $101.3 million for the third quarter of 2025, compared to $135.7 million for the third quarter of 2024.

Conference Call Information

The company will host a conference call and webcast today at 6:00 p.m. ET to discuss recent updates and the company's third quarter 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 at intelliatx.com for approximately 90 days.

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 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 placed on the investigational new drug ("IND") applications for the MAGNITUDE and MAGNITUDE-2 Phase 3 studies of nex-z, resume dosing and screening in these studies, 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; its plans to present longer-term data from the ongoing Phase 1/2 study of lonvo-z and updated longer-term data from the ongoing Phase 1 study of nex-z in November 2025; 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 single dose; the potential of lonvo-z to provide lifelong control of HAE attacks after a single dose; 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 mid-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 may be resolved, including the actions or studies 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, the implications of the clinical hold on the safety and efficacy of nex-z, 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; 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 September 30,		Nine Months ended September 30,	
	2025	2024	2025	2024
Collaboration revenue	$ 13,782	$ 9,111	$ 44,654	$ 45,003
Operating expenses:				
Research and development	94,747	123,380	300,209	349,434
General and administrative	30,512	30,501	86,725	93,385
Total operating expenses	125,259	153,881	386,934	442,819
Operating loss	(111,477)	(144,770)	(342,280)	(397,816)
Other income, net:				
Interest income	6,714	12,122	22,719	37,176
Change in fair value of investments, net	3,439	(3,064)	2,653	(29,483)
Total other income, net	10,153	9,058	25,372	7,693
Net loss	$ (101,324)	$ (135,712)	$ (316,908)	$ (390,123)
Net loss per share, basic and diluted	$ (0.92)	$ (1.34)	$ (2.99)	$ (3.99)
Weighted average shares outstanding, basic and diluted	110,188	101,002	105,841	97,842

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

	September 30, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 669,858	$ 861,730
Total assets	925,275	1,191,015
Total liabilities	176,853	319,059
Total stockholders' equity	748,422	871,956

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