Intellia Therapeutics Announces Second Quarter 2025 Financial Results and Highlights Recent Company Progress

August 7, 2025

- *Enrollment in the global Phase 3 MAGNITUDE trial of nexiguran ziclumeran (nex-z) in ATTR with cardiomyopathy (ATTR-CM) continues to track ahead of projections; Tracking to enroll at least 650 patients cumulatively by year-end*
- *Expanding total enrollment of the MAGNITUDE study to approximately 1,200 patients, subject to health authority review, with no expected impact on previous projected enrollment or financial runway*
- *Expect to complete enrollment by first half 2026 in the global Phase 3 MAGNITUDE-2 study evaluating nex-z in hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN)*
- *Expect to complete randomization in the global Phase 3 HAELO study of lonvoguran ziclumeran (lonvo-z) in hereditary angioedema (HAE) during the third quarter*
- *Additional data from the Phase 1/2 study evaluating lonvo-z in HAE and longer-term data from the Phase 1 study evaluating nex-z in ATTR-CM and ATTRv-PN expected in the second half of 2025*
- *Ended the second quarter with approximately $630.5 million in cash, cash equivalents and marketable securities; Expected to fund operations into the first half of 2027 and into the anticipated first commercial launch*

CAMBRIDGE, Mass., Aug. 07, 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 second quarter ended June 30, 2025.

“We are exceeding many of our internal expectations,” said Intellia President and Chief Executive Officer John Leonard, M.D. “The enthusiasm from both patients and physicians for Intellia’s late-stage programs has resulted in strong enrollment numbers that allow us to plan to enhance the Phase 3 MAGNITUDE trial in ATTR-CM and accelerate completion of the Phase 3 HAELO study in HAE ahead of our original plans. We are full steam ahead in achieving our mission of getting one-time therapies to more patients.”

Second Quarter 2025 and Recent Operational Highlights

Hereditary Angioedema (HAE)

- **Lonvoguran ziclumeran (lonvo-z, also known as NTLA-2002):** Lonvo-z 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.
  - Recruitment ended earlier than expected during the second quarter and the Company now expects to complete randomization in the global Phase 3 HAELO study during the third quarter 2025.
  - Intellia presented three-year follow-up data from the Phase 1 portion of the ongoing Phase 1/2 study after receiving a single dose of lonvo-z. Results were shared in an oral presentation at the European Academy of Allergy and Clinical Immunology (EAACI) Congress 2025 on June 15 in Glasgow, United Kingdom. In the Phase 1 portion of the study, a one-time dose of 25 mg (N=3), 50 mg (N=4) or 75 mg (N=3) of lonvo-z was administered via intravenous infusion and plasma kallikrein protein levels were measured along with HAE attacks. At the time of the February 12, 2025 data cutoff, all 10 patients were attack-free and treatment-free for a median of nearly two years. With up to three years of follow-up, a single dose of lonvo-z led to a mean reduction in monthly HAE attack rate of 98% over the study period, compared to pre-treatment baseline. For all 10 patients, deep, dose-dependent and durable reductions in plasma kallikrein protein continued to be observed through the latest assessment. Across all three dose levels, lonvo-z was generally well tolerated and showed a safety profile consistent with earlier data presented at EAACI in 2024. The most frequent adverse events during the study period were infusion-related reactions (IRRs). IRRs were mostly Grade 1 and resolved with all patients receiving the full dose. With up to three years of follow-up, no treatment-emergent serious adverse events were observed, and no treatment-related adverse events were observed during the period following 28 days after dosing.
  - Intellia expects to present additional data from the ongoing Phase 1/2 study in the second half of 2025.
  - The Company is on track to submit a Biologics License Application (BLA) in the second half of 2026.

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. Intellia leads development and commercialization of nex-z in collaboration with Regeneron Pharmaceuticals, Inc.
    - **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
        - Enrollment in the global Phase 3 MAGNITUDE trial is progressing ahead of the Company's projections and the Company is tracking to enroll at least 650 patients cumulatively by year-end. Intellia is amending the MAGNITUDE study to expand enrollment to approximately 1,200 patients from 765 patients, subject to health authority review. Expanding the patient number in the study would provide a more robust dataset, particularly in the stabilizer stratum, which we believe will be very important to patients, clinicians, and payers. This change has no expected impact on previously projected enrollment timelines or the Company's projected cash runway.
        - In May 2025, the Company presented Phase 1 wild-type vs. variant ATTR-CM data at the Heart Failure 2025 Meeting in Belgrade, Serbia. The data showed that nex-z reduced TTR production and showed promise for treating both wild-type (ATTRwt) and variant (ATTRv) ATTR-CM with a favorable safety profile. Absolute TTR levels dropped from 222.4 to 16.5 µg/mL (ATTRwt) and 132.0 to 16.6 µg/mL (ATTRv). Functional capacity and clinical biomarkers were favorably impacted in both patient groups. Evidence of stability or improvement in disease progression markers were observed across both populations at similar rates. The most commonly reported treatment-related adverse events were IRR, which were mild or moderate, and did not result in any discontinuations. Observed liver enzyme abnormalities were not considered serious, were asymptomatic and resolved spontaneously without medical intervention or sequelae.
        - Intellia expects to present longer-term data from ATTR-CM patients in the Phase 1 study in the second half of 2025. The data will include updated measures of clinical efficacy and safety.
    - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
        - Enrollment is ahead of schedule in the global Phase 3 MAGNITUDE-2 study. Intellia now expects enrollment to be completed in the first half of 2026.
        - In May 2025, the Company presented positive two-year follow-up Phase 1 data in an oral presentation at the 2025 Peripheral Nerve Society (PNS) Annual Meeting in Edinburgh, United Kingdom. Across patients who received a one-time dose of 0.3 mg/kg or higher (n=33), the mean serum TTR reduction by Day 28 was 90% (corresponding mean absolute serum TTR level of 23.8 µg/mL), with levels remaining virtually unchanged through at least 24 months. Among the 18 patients with 24 month mNIS+7 endpoint assessments, 13 showed improvements of ≥ 4 points, which is considered to be a clinically meaningful threshold. Most of the patients in the cohort who had progressed on patisiran improved, and only a single patient among the 18 had a deterioration of ≥ 4. Nex-z was generally well tolerated across all patients and at all dose levels tested. Treatment-related adverse events were consistent with those described for the cardiomyopathy population.
        - In September 2025, the Company will present interim Phase 1 extended data in a symposium at the 5th International ATTR Amyloidosis Meeting for Patients and Doctors in Baveno, Italy.

Platform and Company Updates

- Intellia is pioneering novel CRISPR-based gene editing technologies, such as gene writing and extrahepatic lipid nanoparticle (LNP) delivery technologies, to create highly differentiated *in vivo* and *ex vivo* product candidates. The Company's proprietary platform technologies are being researched and developed to expand therapeutics opportunities to support the mission of transforming lives of people with severe diseases, including the possibility of curative genome editing therapeutics.
- Intellia has expanded its commercial and medical affairs teams to build a strong foundation for commercial readiness. Since the beginning of the year, the company welcomed two key leaders: Jim McNinch, Vice President, U.S. Head of Sales and Ben Newman, Vice President, Commercial Operations, as well as several additional senior leaders with responsibilities for commercial data and field operations, marketing, pricing, patient services, market access, forecasting and medical communications. The company has largely completed the buildout of the commercial and medical affairs leadership teams.

Upcoming Events

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

- Citi 2025 Biopharma Back to School Conference, Sept. 3, Boston
- Wells Fargo Health Care Conference, Sept. 4, Boston
- Bernstein Healthcare Forum, Sept. 23, New York
- 5th International ATTR Amyloidosis Meeting for Patients and Doctors, Sept. 25-26, Baveno, Italy

Second Quarter 2025 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $630.5 million as of June 30, 2025, compared to $861.7 million as of December 31, 2024. The decrease in cash, cash equivalents and marketable securities includes approximately $65.0 million of non-recurring cash payments in the first half of 2025 associated with the Company's previously announced portfolio prioritization, workforce reduction, and real estate consolidation. The Company's cash, cash equivalents and marketable securities as of June 30, 2025 are expected to fund operations into the first half of 2027 and into the anticipated first commercial launch.
- **Collaboration Revenue:** Collaboration revenue was $14.2 million during the second quarter of 2025, compared to $6.9 million during the second quarter of 2024. The $7.3 million increase was mainly driven by cost reimbursements related to our collaboration with Regeneron Pharmaceuticals, Inc.
- **R&D Expenses:** Research and development (R&D) expenses were $97.0 million during the second quarter of 2025, compared to $114.2 million during the second quarter of 2024. The $17.2 million decrease was primarily driven by employee-related expenses, stock-based compensation, research materials and contracted services offset by an increase in the advancement of our lead programs. Stock-based compensation expense included in R&D expenses was $14.1 million for the second quarter of 2025.
- **G&A Expenses:** General and administrative (G&A) expenses were $27.2 million during the second quarter of 2025, compared to $31.8 million during the second quarter of 2024. The $4.6 million decrease was primarily related to lower stock-based compensation, offset in part by increased expenses related to the ongoing buildout of our commercial infrastructure. Stock-based compensation expense included in G&A expenses was $8.0 million for the second quarter of 2025.
- **Net Loss:** Net loss was $101.3 million for the second quarter of 2025, compared to $147.0 million during the second quarter of 2024.

Conference Call to Discuss Second Quarter 2025 Results

The Company will discuss these results on a conference call today, Thursday, August 7 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 August 7 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 nexiguran ziclumeran or "nex-z" (also known as NTLA-2001) for transthyretin ("ATTR") amyloidosis and 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 investigational new drug application ("IND") 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 completing randomization in the Phase 3 HAELO study in the third quarter of 2025 and submitting a biologics license application ("BLA") for lonvo-z in the second half of 2026, its ability to enroll the Phase 3 MAGNITUDE study and enroll at least 650 patients cumulatively by the end of 2025, its ability to enroll the Phase 3 MAGNITUDE-2 study and complete enrollment in the first half of 2026, its plans to present new data from the ongoing Phase 1/2 study of lonvo-z and longer-term Phase 1 data of nex-z, including updated measures of clinical efficacy and safety, in the second half of 2025, the potential of nex-z 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 lonvo-z to provide lifelong control of HAE attacks after a single dose; its expectations that expanding the patient number in the Phase 3 MAGNITUDE study would provide a more robust dataset, particularly in the

stabilizer stratum, and its belief that such dataset will be very important to patients, clinicians and payers, while having no expected impact on previously projected enrollment timelines or its projected cash runway; its ability to apply novel CRISPR-based gene editing technologies, such as gene writing, and extrahepatic lipid nanoparticle ("LNP") delivery technologies to create highly differentiated *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 build a strong foundation for commercial readiness through hiring certain senior leadership positions in its commercial and medical affairs organizations; 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 and into the first anticipated commercial launch.

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 June 30,		Six Months ended June 30,	
	2025	2024	2025	2024
Collaboration revenue	$ 14,245	$ 6,957	$ 30,872	$ 35,892
Operating expenses:				
Research and development	97,035	114,207	205,462	226,054
General and administrative	27,206	31,793	56,213	62,884
Total operating expenses	124,241	146,000	261,675	288,938
Operating loss	(109,996)	(139,043)	(230,803)	(253,046)
Other income (expense), net:				
Interest income	7,402	12,422	16,005	25,054
Change in fair value of investments, net	1,339	(20,354)	(786)	(26,419)
Total other income (expense), net	8,741	(7,932)	15,219	(1,365)
Net loss	$ (101,255)	$ (146,975)	$ (215,584)	$ (254,411)
Net loss per share, basic and diluted	$ (0.98)	$ (1.52)	$ (2.08)	$ (2.64)
Weighted average shares outstanding, basic and diluted	103,732	96,975	103,617	96,238

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

	June 30, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 630,506	$ 861,730
Total assets	898,894	1,191,015
Total liabilities	183,639	319,059
Total stockholders' equity	715,255	871,956

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