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

May 8, 2025

- *On track to complete enrollment of the global Phase 3 HAELO study in hereditary angioedema (HAE) in the third quarter of 2025*
- *Dosed first patient in the global Phase 3 MAGNITUDE-2 study evaluating nexiguran ziclumeran (nex-z) in patients with hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN)*
- *Enrollment in the global Phase 3 MAGNITUDE trial of nex-z in patients with ATTR with cardiomyopathy (ATTR-CM) continues to track ahead of projections*
- *Presenting additional data from the Phase 1 portion of the ongoing Phase 1/2 study of NTLA-2002 in patients with HAE at EAACI Congress in June 2025*
- *Expect to present longer-term data from both ATTR-CM and ATTRv-PN patients in the Phase 1 study of nex-z in the second half of 2025*
- *Ended the first quarter with approximately $707.1 million in cash, cash equivalents and marketable securities; Expected to fund operations into the first half of 2027*

CAMBRIDGE, Mass., May 08, 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 first quarter ended March 31, 2025.

“Intellia is full steam ahead and making excellent progress across its clinical programs,” said Intellia President and Chief Executive Officer John Leonard, M.D. “Two key achievements in the first quarter were dosing the first patients in two of our Phase 3 studies: the HAELO study for hereditary angioedema and the MAGNITUDE-2 study for hereditary ATTR amyloidosis with polyneuropathy. Additionally, our Phase 3 MAGNITUDE study for ATTR with cardiomyopathy continues to enroll rapidly. Upcoming catalysts include longer-term data from the ongoing Phase 1 study of NTLA-2002 at the upcoming EAACI Congress in addition to updated data from the Phase 2 study of NTLA-2002 and longer-term Phase 1 data of nex-z in the second half of 2025.”

First Quarter 2025 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.
  - Intellia will present additional data from the ongoing Phase 1/2 study in an oral presentation at the European Academy of Allergy and Clinical Immunology (EAACI) Congress 2025 on Sunday, June 15 in Glasgow, United Kingdom. The presentation will include longer-term durability data from patients in the Phase 1 portion of the Phase 1/2 study.
  - Enrollment is progressing in the global Phase 3 HAELO study and the Company expects to complete enrollment in the third quarter of 2025.
  - Intellia expects to present new and longer-term data from the Phase 2 portion of the ongoing Phase 1/2 study in the second half of 2025. The data will include patients 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.
  - 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. Nex-z has been generally well tolerated across all patients and at all dose levels tested. The most common treatment-related adverse events have been mild or moderate infusion reactions; all patients were able to receive the intended dose of nex-z. Intellia leads development and commercialization of nex-z in collaboration with Regeneron Pharmaceuticals, Inc.
  - **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
    - In March, Intellia announced the U.S. Food and Drug Administration (FDA) granted Regenerative Medicine Advanced Therapy (RMAT) to nex-z for the treatment of ATTR-CM.

- Enrollment in the global Phase 3 MAGNITUDE trial is progressing ahead of the Company's target projections and anticipates enrollment to exceed 550 total patients by year-end.

- **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
  - In April, the Company announced the first patient was randomized and dosed with nex-z in the global Phase 3 MAGNITUDE-2 study. Intellia expects enrollment to be completed in 2026.

- In May, the Company will present data at the European Society of Cardiology Heart Failure (ESC-HF) Congress and Peripheral Nerve Society (PNS) Annual Meeting. At ESC-HF, Intellia will show wildtype vs. variant data in patients with ATTR-CM. At PNS, the Company will present interim Phase 1 extended data in patients with ATTRv-PN.

- Intellia expects to present longer-term data from both ATTR-CM and ATTRv-PN patients in the Phase 1 study in the second half of 2025. The data will include updated measures of clinical efficacy and safety.

Platform Update

- Intellia is pioneering novel technologies, such as CRISPR-based gene editing technologies and lipid nanoparticle (LNP) delivery technologies, to create highly differentiated, future *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.

Upcoming Events

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

- Bank of America Securities Health Care Conference, May 13, Las Vegas
- ESC Heart Failure Congress, May 17, Belgrade
- PNS Annual Meeting, May 18, Edinburgh
- RBC Capital Markets Global Healthcare Conference, May 21, New York
- EAACI Congress, June 15, Glasgow

First Quarter 2025 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $707.1 million as of March 31, 2025, compared to $861.7 million as of December 31, 2024. The decrease in cash, cash equivalents and marketable securities was primarily driven by first quarter operations and approximately $51 million of non-recurring cash payments 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 March 31, 2025 are expected to fund operations into the first half of 2027.

- **Collaboration Revenue:** Collaboration revenue was $16.6 million during the first quarter of 2025, compared to $28.9 million during the first quarter of 2024. The $12.3 million decrease was mainly driven by a decrease in collaboration revenue under the AvenCell license and collaboration agreement.

- **R&D Expenses:** Research and development (R&D) expenses were $108.4 million during the first quarter of 2025, compared to $111.8 million during the first quarter of 2024. The $3.4 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 $12.6 million for the first quarter of 2025.

- **G&A Expenses:** General and administrative (G&A) expenses were $29.0 million during the first quarter of 2025, compared to $31.1 million during the first quarter of 2024. The $2.1 million decrease was primarily related to lower employee-related expenses due to a workforce reduction in January 2025 and lower stock-based compensation, partially offset by increases related to severance expenses recorded in the first quarter. Stock-based compensation expense included in G&A expenses was $9.2 million for the first quarter of 2025.

- **Net Loss:** Net loss was $114.3 million for the first quarter of 2025, compared to $107.4 million during the first quarter of 2024.

Conference Call to Discuss First Quarter 2025 Results

The Company will discuss these results on a conference call today, Thursday, May 8 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 May 8 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 application ("IND") submissions, including the expected timing of data releases from its ongoing clinical trials of nex-z and NTLA-2002, 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 third quarter of 2025 and the submission of a biologics license application ("BLA") in the second half of 2026, its ability to enroll the Phase 3 MAGNITUDE study and exceed 550 total enrolled patients by the end of 2025, its ability to enroll the Phase 3 MAGNITUDE-2 study and complete enrollment in 2026, its plans to present new data from the Phase 2 portion of the Phase 1/2 study of NTLA-2002 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 NTLA-2002 to provide lifelong control of HAE attacks after a single dose; its ability to apply novel technologies, such as CRISPR-based gene editing technologies and lipid nanoparticle 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 March 31,	
	2025	**2024**
Collaboration revenue	$ 16,627	$ 28,935
Operating expenses:		
Research and development	108,427	111,847
General and administrative	29,007	31,091
Total operating expenses	137,434	142,938
Operating loss	(120,807)	(114,003)
Other income (expense), net:		
Interest income	8,603	12,632
Change in fair value of investments, net	(2,125)	(6,065)
Total other income, net	6,478	6,567
Net loss	$ (114,329)	$ (107,436)
Net loss per share, basic and diluted	$ (1.10)	$ (1.12)
Weighted average shares outstanding, basic and diluted	103,500	95,502

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

	March 31, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 707,100	$ 861,730
Total assets	986,163	1,191,015
Total liabilities	206,244	319,059
Total stockholders' equity	779,919	871,956

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