Intellia Therapeutics Announces Third Quarter 2024 Financial Results and Highlights Recent Company Progress

November 7, 2024

- *Received IND clearance from the U.S. FDA to initiate MAGNITUDE-2 Phase 3 trial of nexiguran ziclumeran (nex-z) in patients with hereditary transthyretin (ATTR) amyloidosis with polyneuropathy; on track to initiate study by year-end*
- *Strong patient enrollment continues in the MAGNITUDE Phase 3 study of nex-z for ATTR amyloidosis with cardiomyopathy, tracking ahead of plans*
- *Plan to present new clinical data from the ongoing nex-z Phase 1 study at upcoming 2024 American Heart Association Scientific Sessions*
- *Actively screening patients in the HAELO Phase 3 study of NTLA-2002 for hereditary angioedema (HAE)*
- *Reported positive results from the Phase 2 study supporting NTLA-2002's potential to be a functional cure for HAE*
- *On track to dose the first patient by year-end in the Phase 1/2 study of NTLA-3001, an* in vivo *gene insertion candidate for the treatment of alpha-1 antitrypsin deficiency (AATD)*
- *Ended the third quarter of 2024 with approximately $945 million in cash, cash equivalents and marketable securities*

CAMBRIDGE, Mass., Nov. 07, 2024 (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 third quarter ended September 30, 2024.

"Our third quarter and recent period have been marked by outstanding progress in advancing our pipeline of CRISPR-based gene editing therapies," said Intellia President and Chief Executive Officer John Leonard, M.D. "A key achievement was the initiation of the HAELO Phase 3 study, which we believe will demonstrate what we've seen in the first-in-human study — a one-time treatment of NTLA-2002 has the potential to be a functional cure for hereditary angioedema. In addition, we are very pleased the strong pace of enrollment has continued in the ongoing MAGNITUDE Phase 3 study of nex-z for people living with ATTR amyloidosis with cardiomyopathy. Following the recent IND clearance from the FDA for the MAGNITUDE-2 Phase 3 study of nex-z in polyneuropathy patients, we are on track to initiate our third Phase 3 study in the weeks ahead. We look forward to the AHA annual meeting next week, where we will be sharing the first biomarker and functional capacity data from the Phase 1 study of nex-z, and further build upon Intellia's industry-leading position in ushering in a new era of medicine."

Third Quarter 2024 and Recent Operational Highlights

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.
  - **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
    - Enrollment in the pivotal Phase 3 MAGNITUDE trial is progressing swiftly and continuing to track ahead of the Company's target enrollment projections.
  - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
    - Intellia announced today that the U.S. Food and Drug Administration (FDA) has cleared its nex-z Investigational New Drug (IND) application to initiate the MAGNITUDE-2 pivotal Phase 3 trial for ATTRv-PN. MAGNITUDE-2 is an international, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of nex-z in 50 adults with ATTRv-PN. Patients will be randomized 1:1 to receive a single 55 mg infusion of nex-z or placebo. Patients randomized to the placebo arm will be eligible for optional crossover to receive nex-z. The primary endpoints are the change from baseline in modified Neuropathy Impairment Score +7 (mNIS+7) at month 18 and serum TTR at day 29. The mNIS+7 scale is a validated measure specifically designed to assess and quantify polyneuropathy impairment, including muscle weakness, muscle stretch reflexes, sensory loss and autonomic impairment. The Company expects to initiate patient enrollment in the MAGNITUDE-2 study at ex-U.S. sites by year-end.
  - **Upcoming Clinical Data Update:**

- Intellia will be presenting new data from the ongoing Phase 1 study in a late-breaking oral presentation at the 2024 American Heart Association (AHA) Scientific Sessions on Saturday, November 16. The presentation will include safety, reduction in serum TTR and biomarkers of disease progression and functional capacity data in patients with ATTR-CM. The Company will host an investor webcast to review these data along with data from the ATTRv-PN arm on Saturday, November 16.

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 is actively screening patients in the HAELO Phase 3 study. In October, Intellia announced the initiation of HAELO, a global, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of NTLA-2002 in 60 adults with Type I or Type II HAE. Patients will be randomized 2:1 to receive a single 50 mg infusion of NTLA-2002 or placebo. Patients randomized to the placebo arm will be eligible for optional crossover to NTLA-2002 at week 28. The primary endpoint is the change in number of HAE attacks from week 5 through week 28.

  - In October, the Company presented positive Phase 2 data from the ongoing Phase 1/2 study, with results continuing to support the potential of NTLA-2002 to be a functional cure for HAE. Eight of 11 patients in the 50 mg arm ceased having any attacks during the 16-week primary observation period after a single dose of NTLA-2002. These eight patients continued to be attack-free through the reported follow-up and no further treatment has been required. NTLA-2002 was well tolerated. The most frequent adverse events (AEs) were headache, fatigue and nasopharyngitis. There have been no serious AEs, and all AEs were either Grade 1 or 2. These interim data were published in the *New England Journal of Medicine* and presented at the 2024 American College of Allergy, Asthma & Immunology (ACAAI) Scientific Meeting in Boston, Massachusetts.

Alpha-1 Antitrypsin Deficiency (AATD)-Associated Lung Disease

- **NTLA-3001:** NTLA-3001 is a first-in-class CRISPR-mediated *in vivo* targeted gene insertion development candidate for the treatment of AATD-associated lung disease. It is designed to precisely insert the wild-type *SERPINA1* gene, which encodes the alpha-1 antitrypsin (AAT) protein, with the potential to restore permanent expression of fully functional AAT protein to normal levels after a single dose. This is Intellia's first wholly owned gene insertion program.
  - Intellia expects to dose the first patient in the Phase 1/2 study of NTLA-3001 by year-end.

***In Vivo* Platform Expansion**

- Intellia is expanding the range of diseases that can be targeted with its CRISPR-based technologies by deploying new editing and delivery innovations. This includes advancing gene editing programs in five different tissues outside the liver, either independently or in collaboration with partners. These research and preclinical programs are targeting diseases that originate in the bone marrow, brain, muscle, lung and eye, which, if successful, could dramatically expand the opportunities for CRISPR-based treatments.

***Ex Vivo* Program Updates**

- Intellia is advancing multiple programs, wholly owned and in collaboration with partners, utilizing its allogeneic platform for the treatment of immuno-oncology and autoimmune diseases. The Company's proprietary allogeneic cell engineering platform avoids both T cell- and NK cell-mediated rejection in preclinical models, a key unsolved challenge with other investigational allogeneic approaches. Cell therapies engineered with Intellia's allogeneic platform, combined with edits to enhance cell function, offer a new approach to target both hematological and solid tumors.

Upcoming Events

The Company will participate in the following events during the fourth quarter of 2024:

- 2024 American Heart Association (AHA) Scientific Sessions, November 16 – 18, Chicago

Third Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $944.7 million as of September 30, 2024, compared to $1.0 billion as of December 31, 2023. The decrease was driven by cash used to fund operations of $335.0 million. The Company's investments were offset in part by $176.9 million of net equity proceeds from the Company's "At the Market" (ATM) program, $47.0 million of reimbursement from collaborators, including a one-time $30.0 million payment received in April 2024 related to the Company's technology collaboration with Regeneron, $37.2 million of interest income and $6.5 million in proceeds from employee-based stock plans. The cash position is expected to fund operations into late 2026.

- **Collaboration Revenue:** Collaboration revenue was $9.1 million during the third quarter of 2024, compared to $12.0 million during the third quarter of 2023. The $2.9 million decrease was mainly driven by a reduction in revenue related to the AvenCell license and collaboration agreement.

- **R&D Expenses:** Research and development (R&D) expenses were $123.4 million during the third quarter of 2024, compared to $113.7 million during the third quarter of 2023. The $9.7 million increase was primarily driven by the advancement of our lead programs. Stock-based compensation expense included in R&D expenses was $24.2 million for the third quarter of 2024.

- **G&A Expenses:** General and administrative (G&A) expenses were $30.5 million during the third quarter of 2024, compared to $29.4 million during the third quarter of 2023. The $1.1 million increase was primarily related to stock-based compensation. Stock-based compensation expense included in G&A expenses was $15.4 million for the third quarter of 2024.

- **Net Loss:** Net loss was $135.7 million for the third quarter of 2024, compared to $122.2 million during the third quarter of 2023.

Conference Call to Discuss Third Quarter 2024 Results

The Company will discuss these results on a conference call today, Thursday, November 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 November 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. The company's *in vivo* programs use CRISPR to enable precise editing of disease-causing genes directly inside the human body. Intellia's *ex vivo* programs use CRISPR to engineer human cells outside the body for the treatment of cancer and autoimmune diseases. 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, NTLA-2002 for the treatment of hereditary angioedema ("HAE"), and NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency ("AATD")-associated lung disease, 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 presentation of additional data from the Phase 1 clinical trial of nex-z in 2024, 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 study of NTLA-2002 in 2024, its ability to dose the first patient in its NTLA-3001 Phase 1 study in 2024, 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, the potential of NTLA-2002 to be a functional cure for patients with HAE and to demonstrate lifelong control of HAE attacks after a single dose, and the potential of NTLA-3001 to restore permanent expression of functional alpha-1 antitrypsin protein to normal levels after a single dose; the expansion of its CRISPR/Cas9 technology and related novel technologies, including advancing gene editing programs in tissues outside of the liver, such as bone marrow, brain, muscle, lung and eye, and the potential of those programs to dramatically expand the opportunities for CRISPR-based treatments; its ability to advance multiple *ex vivo* programs utilizing its allogeneic platform, which is designed to avoid both T cell- and NK cell-mediated rejection, for the treatment of immuno-oncology and autoimmune diseases, and to combine its allogeneic platform with edits to enhance cell function and offer a new approach to target both hematological and solid tumors; its ability to advance additional *in vivo* and *ex vivo* development candidates and timing expectations of advancing such development candidates and releasing data related to such technologies and development 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 late 2026.

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 September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Collaboration revenue	$ 9,111	$ 11,992	$ 45,003	$ 38,192
Operating expenses:				
Research and development	123,380	113,696	349,434	326,088
General and administrative	30,501	29,403	93,385	87,503
Total operating expenses	153,881	143,099	442,819	413,591
Operating loss	(144,770)	(131,107)	(397,816)	(375,399)
Other income (expense), net:				
Interest income	12,122	12,740	37,176	37,373
Change in fair value of investments, net	(3,064)	-	(29,483)	-
Loss from equity method investment	-	(3,857)	-	(10,905)
Change in fair value of contingent consideration	-	-	-	(100)
Total other income, net	9,058	8,883	7,693	26,368
Net loss	$ (135,712)	$ (122,224)	$ (390,123)	$ (349,031)
Net loss per share, basic and diluted	$ (1.34)	$ (1.38)	$ (3.99)	$ (3.96)
Weighted average shares outstanding, basic and diluted	101,002	88,645	97,842	88,204

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

	September 30, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 944,681	$ 1,012,087
Total assets	1,173,351	1,300,977
Total liabilities	210,736	250,808
Total stockholders' equity	962,615	1,050,169

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