# Intellia Therapeutics Announces Third Quarter 2023 Financial Results and Highlights Recent Company Progress

November 9, 2023

- *FDA cleared NTLA-2001 IND application for first* in vivo *CRISPR candidate to enter late-stage clinical development; on track to initiate the MAGNITUDE pivotal Phase 3 trial in patients with transthyretin (ATTR) amyloidosis with cardiomyopathy by year-end*
- *Clinical data presented from over 60 patients with ATTR amyloidosis demonstrated consistent, deep and durable serum TTR reduction after a single dose of NTLA-2001; greater than 90% median serum TTR reduction*
- *On track to complete enrollment of the NTLA-2002 Phase 2 study for the treatment of hereditary angioedema (HAE) in Q4 2023*
- *Plan to submit a Clinical Trial Application (CTA) in Q1 2024 for NTLA-3001, an* in vivo *insertion candidate in development for the treatment of alpha-1 antitrypsin deficiency (AATD)-associated lung disease*
- *Intellia and Regeneron expand research collaboration to develop* in vivo *CRISPR-based gene editing therapies focused on neurological and muscular diseases*
- *Ended the third quarter of 2023 in a strong financial position with $992.5 million in cash*

CAMBRIDGE, Mass., Nov. 09, 2023 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing potentially curative therapies leveraging CRISPR-based technologies, today reported operational highlights and financial results for the third quarter ended September 30, 2023.

“2023 has been a year of remarkable progress in which Intellia received two IND clearances for investigational *in vivo* CRISPR therapies. With the imminent start of the NTLA-2001 MAGNITUDE Phase 3 trial, Intellia has now become a late-stage drug development company,” said Intellia President and Chief Executive Officer John Leonard, M.D. “Looking ahead, we expect to achieve several notable milestones in the coming weeks and months, including completing enrollment of the NTLA-2002 Phase 2 study in people with hereditary angioedema and submitting a regulatory filing to begin clinical development for NTLA-3001, our *in vivo* gene insertion program for people living with alpha-1 antitrypsin deficiency. We look forward to advancing our pipeline and platform as we move closer to realizing the potential of CRISPR-based medicines.”

**Third Quarter 2023 and Recent Operational Highlights**

***In Vivo* Program Updates**

**Transthyretin (ATTR) Amyloidosis**

- **NTLA-2001:** NTLA-2001 is an *in vivo*, systemically delivered, investigational CRISPR-based therapy designed to inactivate the *TTR* gene in liver cells and thereby prevent the production of transthyretin (TTR) protein for the treatment of ATTR amyloidosis. NTLA-2001 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. NTLA-2001 is subject to a co-development/co-promotion agreement between Intellia, the lead party for this program, and Regeneron.
  - **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
    - The Company announced in October that the U.S. Food and Drug Administration (FDA) cleared the NTLA-2001 Phase 3 Investigational New Drug (IND) application for the treatment of ATTR-CM. The MAGNITUDE pivotal Phase 3 trial is a randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of NTLA-2001 in approximately 765 patients with ATTR-CM. The primary endpoint of the study is a composite endpoint of cardiovascular (CV)-related mortality and CV-related events. Patients will be randomized 2:1 NTLA-2001:placebo, with a single 55 mg infusion of NTLA-2001 administered. The Company expects to initiate the study by year-end with patient dosing to commence early 2024.
  - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
    - The Company is actively preparing for a global pivotal Phase 3 study of NTLA-2001 for the treatment of ATTRv-PN, including discussions with regulatory authorities.
  - **Updated Data from NTLA-2001 Phase 1 Study**
    - In November, Intellia announced new positive interim results from the Phase 1 study of NTLA-2001. Updated data from over 60 patients showed consistent, deep and durable serum TTR reduction achieved

with a single dose of NTLA-2001, including 29 patients who reached 12 months or more of follow-up as of the data cut-off date of May 11, 2023. Across all patients who received a dose of 0.3 mg/kg or higher (n=62), the median serum TTR reduction was 91% and the median absolute residual serum TTR concentration was 17 µg/mL at day 28. Across all patients and at all dose levels tested, NTLA-2001 was generally well tolerated, and the majority of adverse events were mild in severity. These interim data were presented at the 4th International ATTR Amyloidosis Meeting, held in Madrid, Spain.

**Hereditary Angioedema (HAE)**

- **NTLA-2002:** NTLA-2002 is a wholly owned, *in vivo*, systemically delivered investigational CRISPR-based therapy. NTLA-2002 is designed to knock out the *KLKB1* gene in the liver, with the potential to permanently reduce total plasma kallikrein protein and activity, a key mediator of HAE. This investigational approach aims to prevent attacks for people living with HAE by providing continuous reduction of plasma kallikrein activity, following a single dose. It also aims to eliminate the significant treatment burden associated with currently available HAE therapies. NTLA-2002 is being evaluated in a Phase 1/2 study in adults with Type I or Type II HAE.
    - In October, Intellia announced that the European Medicines Agency (EMA) granted Priority Medicine (PRIME) designation to NTLA-2002 for the treatment of HAE. PRIME designation is granted by the EMA to drug candidates that may offer a major therapeutic advantage over existing treatments or that benefit patients without treatment options.
    - Following the identification of all patients, Intellia is on track to complete enrollment in the Phase 2 portion of the Phase 1/2 study in Q4 2023.
    - Intellia plans to initiate the global pivotal Phase 3 study, including U.S. patients, as early as the third quarter of 2024, subject to regulatory feedback.

**Alpha-1 Antitrypsin Deficiency (AATD)**

- **NTLA-3001 for AATD-Associated Lung Disease:** NTLA-3001 is a wholly owned, 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 a healthy copy of the *SERPINA1* gene, which encodes the alpha-1 antitrypsin (A1AT) protein, with the potential to restore permanent expression of functional A1AT protein to therapeutic levels after a single dose. This approach seeks to improve patient outcomes, including eliminating the need for weekly intravenous infusions of A1AT augmentation therapy or lung transplant in severe cases.
    - Intellia plans to submit a Clinical Trial Application (CTA) in Q1 2024 to initiate a first-in-human, Phase 1 study of NTLA-3001.
- **NTLA-2003 for AATD-Associated Liver Disease:** NTLA-2003 is a wholly owned, *in vivo* knockout development candidate for the treatment of AATD-associated liver disease. It is designed to inactivate the *SERPINA1* gene responsible for the production of abnormal A1AT protein in the liver.
    - Based on a prioritization of resources, the Company is making a strategic shift to halt further IND-enabling activities for NTLA-2003 to advance an AATD research-stage program leveraging the Company's DNA writing technology.

***Ex Vivo* Program Updates**

- Intellia is advancing multiple preclinical 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 is designed to avoid both T cell- and NK cell-mediated rejection, a key unsolved challenge with other investigational allogeneic approaches.

**Platform and Pipeline Expansion**

- As Intellia's DNA writing technology has met key internal research milestones, the Company will now prioritize the advancement of a research-stage program for the treatment of AATD.
- In October, Intellia and Regeneron announced an expanded research collaboration to develop additional *in vivo* CRISPR-based gene editing therapies focused on neurological and muscular diseases. The collaboration will leverage Regeneron's proprietary antibody-targeted adeno-associated virus (AAV) vectors and delivery systems and Intellia's proprietary Nme2 CRISPR/Cas9 (Nme2Cas9) systems adapted for viral vector delivery and designed to precisely modify a target gene.
- Additionally, Regeneron has exercised its option to extend the existing technology collaboration term with Intellia for two years. The technology collaboration term now extends to April 2026, and Intellia will receive a $30 million payment in the first half of 2024.
- In September, SparingVision announced that it had selected a second target as part of its strategic collaboration with Intellia to develop novel genomic medicines, utilizing CRISPR-based gene editing technologies for the treatment of ocular diseases.

**Upcoming Event**

The Company will participate in the Jefferies London Healthcare Conference, taking place November 14-16 in London.

**Upcoming Milestones**

The Company has set forth the following expected milestones for pipeline progression:

- **NTLA-2001 for ATTR amyloidosis**:
  - Initiate a global pivotal study for NTLA-2001 for ATTR-CM by year-end 2023.
  - Prepare for a Phase 3 study of NTLA-2001 for the treatment of ATTRv-PN, including discussions with regulatory authorities.
- **NTLA-2002 for HAE**:
  - Complete enrollment in the Phase 2 portion of the Phase 1/2 study in Q4 2023.
- **NTLA-3001 for AATD:**
  - Submit a CTA application for NTLA-3001 for AATD-associated lung disease in Q1 2024.
- **Platform Innovation:**
  - Advance novel gene editing technologies, including DNA writing and delivery to other tissues outside of the liver.

**Third Quarter 2023 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities were $992.5 million as of September 30, 2023, compared to $1.3 billion as of December 31, 2022. The decrease was driven by cash used to fund operations of $343.2 million. The decrease was offset in part by $37.4 million of interest income, $12.6 million of reimbursement from its collaborators, $16.2 million of net equity proceeds from the Company's "At the Market" (ATM) program and $7.7 million in proceeds from employee-based stock plans.
- **Collaboration Revenue:** Collaboration revenue decreased by $1.3 million to $12.0 million during the third quarter of 2023, compared to $13.3 million during the third quarter of 2022.
- **R&D Expenses:** Research and development expenses increased by $17.0 million to $113.7 million during the third quarter of 2023, compared to $96.7 million during the third quarter of 2022. This increase was primarily driven by the advancement of our lead programs and personnel growth to support these programs. Stock-based compensation expense included in research and development expenses was $21.2 million for the third quarter of 2023.
- **G&A Expenses:** General and administrative expenses increased by $7.3 million to $29.4 million during the third quarter of 2023, compared to $22.1 million during the third quarter of 2022. This increase was primarily related to an increase in stock-based compensation of $5.3 million. Stock-based compensation expense included in general and administrative expenses was $14.1 million for the third quarter of 2023.
- **Net Loss:** The Company's net loss was $122.2 million for the third quarter of 2023, compared to $113.2 million during the third quarter of 2022.

**Conference Call to Discuss Third Quarter 2023 Results**

The Company will discuss these results on a conference call today, Thursday, November 9 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 9 at 12 p.m. ET.

**About Intellia Therapeutics**
Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics leveraging CRISPR-based technologies. To fully realize the transformative potential of CRISPR-based technologies, Intellia is pursuing two primary approaches. The company's *in vivo* programs use intravenously administered CRISPR as the therapy, in which proprietary delivery technology enables highly precise editing of disease-causing genes directly within specific target tissues. Intellia's *ex vivo* programs use CRISPR to create the therapy by using engineered human cells to treat cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its robust intellectual property portfolio, have enabled the company to take a leadership role in harnessing the full potential of genome editing to create new classes of genetic medicine. Learn more at intelliatx.com. Follow us on X (formerly known as Twitter) @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: its ability to successfully extend its leadership position and harness the full potential of genomic medicines to bolster its genome editing capabilities and pipeline, including to enable broader *in vivo* and *ex vivo* applications; the safety, efficacy, success and advancement of its clinical programs for NTLA-2001 for transthyretin ("ATTR") amyloidosis and NTLA-2002 for the treatment of hereditary

angioedema (“HAE”) pursuant to its clinical trial application (“CTA”), 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, including emerging clinical endpoints, related to the NTLA-2001 and NTLA-2002 clinical trials; the planned completion of enrollment of the NTLA-2002 Phase 2 study in patients with HAE; the planned initiation of a global pivotal Phase 3 MAGNITUDE trial for NTLA-2001 by the end of 2023; the planned initiation of a global pivotal Phase 3 study of NTLA-2002 as early as Q3 2024, subject to regulatory feedback; the advancement of development candidates, such as NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency (“AATD”)-associated lung disease; its ability to generate data to initiate clinical trials and the timing of CTA and IND submissions and initiation of related clinical trials, including the planned CTA submission for NTLA-3001 for AATD in Q1 2024; its ability to advance multiple programs utilizing an allogeneic platform for the treatment of immuno-oncology and autoimmune diseases; the expansion of its CRISPR/Cas9 technology and related novel technologies, including DNA writing and related research milestones and delivery to other tissues outside of the liver; 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. and their co-development program for ATTR amyloidosis and expanded research collaboration to develop additional *in vivo* CRISPR-based gene editing therapies focused on neurological and muscular diseases and its collaboration with SparingVision to develop novel genomic medicines utilizing CRISPR-based gene editing technologies for the treatment of ocular diseases; and its growth as a Company and expectations regarding its uses of capital, expenses, future accumulated deficit and financial results.

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, 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 Pharmaceuticals, Inc., SparingVision, 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 and quarterly report on Form 10-Q, as well as discussions of potential risks, uncertainties, and other important factors in Intellia’s other filings with the Securities and Exchange Commission. 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, | |
|---|---|---|---|---|
| | 2023 | 2022 | 2023 | 2022 |
| Collaboration revenue | $ 11,992 | $ 13,266 | $ 38,192 | $ 38,548 |
| Operating expenses: | | | | |
| Research and development | 113,696 | 96,651 | 326,088 | 319,945 |
| General and administrative | 29,403 | 22,145 | 87,503 | 66,680 |
| Total operating expenses | 143,099 | 118,796 | 413,591 | 386,625 |
| Operating loss | (131,107) | (105,530) | (375,399) | (348,077) |
| Other income (expense), net: | | | | |
| Interest income | 12,740 | 1,945 | 37,373 | 3,188 |
| Loss from equity method investment | (3,857) | (1,834) | (10,905) | (7,831) |
| Change in fair value of contingent consideration | - | (7,810) | (100) | (8,059) |
| Total other income (expense), net | 8,883 | (7,699) | 26,368 | (12,702) |
| Net loss | $ (122,224) | $ (113,229) | $ (349,031) | $ (360,779) |
| Net loss per share, basic and diluted | $ (1.38) | $ (1.49) | $ (3.96) | $ (4.78) |
| Weighted average shares outstanding, basic and diluted | 88,645 | 76,047 | 88,204 | 75,543 |

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

| | September 30, 2023 | December 31, 2022 |
|---|---|---|
| Cash, cash equivalents and marketable securities | $ 992,540 | $ 1,261,960 |
| Total assets | 1,243,349 | 1,520,114 |
| Total liabilities | 205,935 | 284,530 |
| Total stockholders' equity | 1,037,414 | 1,235,584 |

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