# Intellia Therapeutics Announces Fourth Quarter and Full-Year 2023 Financial Results and Highlights Recent Company Progress

February 22, 2024

- *On track to dose the first patient in the Phase 3 MAGNITUDE trial of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy in Q1 2024*
- *Expect to initiate the Phase 3 study of NTLA-2002 for the treatment of hereditary angioedema (HAE) in 2H24*
- *Plan to present new clinical data in 2024 from both ongoing NTLA-2001 and NTLA-2002 first-in-human studies, including NTLA-2002 Phase 2 results*
- *Published positive interim results from the Phase 1 study of NTLA-2002 in the* New England Journal of Medicine
- *On track to dose the first patient in the Phase 1 study of NTLA-3001, an* in vivo *gene insertion candidate for the treatment of alpha-1 antitrypsin deficiency (AATD)-associated lung disease, in 2024*
- *Expanding development of* in vivo *CRISPR-based therapies to target tissues outside of the liver; announced strategic collaboration with ReCode to develop novel gene editing therapies for cystic fibrosis*
- *Ended 2023 in a strong financial position with approximately $1.0 billion in cash*

CAMBRIDGE, Mass., Feb. 22, 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 fourth quarter and year ended December 31, 2023.

"We're off to a very strong start in 2024 as we execute against our strategic priorities to realize the full potential of CRISPR-based gene editing," said Intellia President and Chief Executive Officer John Leonard, M.D. "We are focused on rapidly enrolling patients in the pivotal Phase 3 MAGNITUDE trial of NTLA-2001 for the treatment of ATTR amyloidosis with cardiomyopathy and expect to dose the first patient in the first quarter of this year. We also remain on track to begin the Phase 3 trial for our second *in vivo* CRISPR-based therapy, NTLA-2002 for hereditary angioedema, later in the year. At the same time, we continue to expand both the technical approaches for CRISPR-based therapies, as well as the range of diseases they can potentially address. We plan to initiate two first-in-human studies for product candidates leveraging our modular gene insertion platform to produce a deficient protein – one wholly owned program focused on alpha-1 antitrypsin deficiency-associated lung disease and, together with Regeneron, a second program focused on hemophilia B. Finally, we are progressing our editing capabilities, including DNA writing, and applying them to diseases that originate outside of the liver. We announced last week a new collaboration with ReCode Therapeutics to advance novel gene editing treatments directly to the lung in patients with cystic fibrosis. These pipeline and platform efforts move us closer to setting a new standard of care for people living with serious diseases and expanding Intellia's impact as the leading gene editing company."

**Fourth Quarter 2023 and Recent Operational Highlights**

**Transthyretin (ATTR) Amyloidosis**

- **NTLA-2001:** NTLA-2001 is an investigational *in vivo* CRISPR-based therapy designed to inactivate the *TTR* gene in the liver 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. Intellia leads development and commercialization of NTLA-2001 in collaboration with Regeneron.
    - **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
        - Intellia is actively enrolling patients, including in the U.S., in the pivotal Phase 3 MAGNITUDE trial. The Company is on track to dose the first patient in Q1 2024 and continues to open new clinical sites.
    - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
        - Intellia is actively preparing for a global pivotal Phase 3 study of NTLA-2001 for the treatment of ATTRv-PN.
    - The Company plans to present updated data from the ongoing Phase 1 study in 2024.

**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 plans to initiate the global pivotal Phase 3 study, including U.S. patients, in the second half of 2024, subject to regulatory feedback.
    - As previously announced in January, Intellia completed enrollment and dosing in the Phase 2 portion of the Phase 1/2 study in adults with HAE. The Company plans to present updated data from the Phase 1 and new data from the Phase 2 portion in 2024.
    - In January, the Company announced that positive interim results from the Phase 1 portion of the Phase 1/2 study

of NTLA-2002 were published in the *New England Journal of Medicine* (NEJM). The reported data showed that a single dose of NTLA-2002 led to a 95% mean reduction in monthly HAE attack rate across all 10 patients in the Phase 1 portion. NTLA-2002 was well tolerated at all dose levels. The most frequent adverse events reported were mild, transient infusion-related reactions and fatigue.

- During the fourth quarter of 2023, Intellia received Priority Medicines (PRIME) designation from the European Medicines Agency and orphan drug designation from the European Commission for NTLA-2002.

**_In Vivo_ Targeted Gene Insertion**

- **NTLA-3001 for Alpha-1 Antitrypsin Deficiency (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.
  - In December 2023, Intellia submitted a Clinical Trial Application (CTA) to initiate a first-in-human, Phase 1 study of NTLA-3001. The Company plans to dose the first patient in 2024.

- **Hemophilia B:** In February, Regeneron and Intellia announced the clearance by the U.S. Food and Drug Administration of its investigational new drug application to initiate a clinical trial of its investigational *in vivo* CRISPR-based *Factor 9* gene insertion program for people living with hemophilia B. A Phase 1, first-in-human study is expected to begin in mid-2024. Regeneron leads development and commercialization of hemophilia A and B programs in collaboration with Intellia.

**_In Vivo_ Platform Expansion Including to Tissues Outside of the Liver**

- In February, Intellia and ReCode announced a strategic collaboration to develop novel genomic medicines for the treatment of cystic fibrosis (CF). The collaboration will leverage Intellia's proprietary CRISPR-based gene editing platform, including its DNA writing technology, and ReCode's proprietary Selective Organ Targeting (SORT) lipid nanoparticle delivery platform to precisely correct one or more CF disease-causing gene mutations.

- In October 2023, Intellia and Regeneron announced an expanded research collaboration to develop additional *in vivo* CRISPR-based gene editing therapies focused on neurological and muscular diseases.

- In October 2023, Regeneron 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 due in April 2024.

**_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.

**Upcoming Events**

The Company will participate in the following events in March:

- TD Cowen 44$^{th}$ Annual Health Care Conference, March 4, Boston

- Leerink Global Biopharma Conference, March 12, Miami

- Barclays Global Healthcare Conference, March 13, Miami

**Fourth Quarter and Full-Year 2023 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.0 billion as of December 31, 2023, compared to $1.3 billion as of December 31, 2022. The decrease was driven by cash used to fund operations of $448.8 million. The decrease was offset in part by $119.8 million of net equity proceeds from the Company's "At the Market" (ATM) program, $49.8 million of interest income, $18.7 million of reimbursement from its collaborators, and $10.5 million in proceeds from employee-based stock plans. The cash position is expected to fund operations into mid-2026.

- **Collaboration Revenue:** Collaboration revenue decreased by $15.5 million to negative $1.9 million during the fourth quarter of 2023, compared to $13.6 million during the fourth quarter of 2022. This decrease was mainly driven by a $10.3 million one-time revenue recognition adjustment related to Regeneron extending the technology collaboration to April 2026. Intellia will receive a $30.0 million payment due in April 2024 as part of the Regeneron extension.

- **R&D Expenses:** Research and development expenses increased by $9.0 million to $109.0 million during the fourth quarter of 2023, compared to $100.0 million during the fourth 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 $21.7 million for the fourth quarter of 2023.

- **G&A Expenses:** General and administrative expenses increased by $5.4 million to $29.0 million during the fourth quarter of 2023, compared to $23.6 million during the fourth quarter of 2022. This increase was primarily related to an increase in stock-based compensation of $4.3 million. Stock-based compensation expense included in general and administrative expenses was $13.3 million for the fourth quarter of 2023.

- **Net Loss:** The Company's net loss was $132.2 million for the fourth quarter of 2023, compared to $113.4 million during the fourth quarter of 2022.

**Conference Call to Discuss Fourth Quarter and Full-Year 2023 Results**

The Company will discuss these results on a conference call today, Thursday, February 22, 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 February 22, 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 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 NTLA-2001 and NTLA-2002 clinical trials in 2024, dosing of the first patient in its global pivotal Phase 3 MAGNITUDE trial for NTLA-2001 in Q1 2024 and its ability to rapidly enroll this study, the planned initiation of a global pivotal Phase 3 study of NTLA-2002 in 2H 2024 subject to regulatory feedback, its ability to dose the first patient in its NTLA-3001 Phase 1 study in 2024, and the potential of NTLA-3001 to restore permanent expression of functional alpha-1 antitrypsin protein to therapeutic levels after a single dose; 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 multiple *ex vivo* programs utilizing an allogeneic platform, which is designed to avoid both T cell- and NK cell-mediated rejection, for the treatment of immuno-oncology and autoimmune diseases; 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 Hemophilia B, as well as their expanded research collaboration to develop additional *in vivo* CRISPR-based gene editing therapies focused on neurological and muscular diseases and its collaboration with ReCode Therapeutics, Inc. ("ReCode") to develop novel genomic medicines for the treatment of cystic fibrosis utilizing CRISPR-based gene editing, including DNA writing, and Selective Organ Targeting lipid nanoparticle delivery technologies; 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, ReCode, 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. 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 December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2023 | 2022 | 2023 | 2022 |

| | | | | |
|---|---|---|---|---|
| Collaboration revenue | $ (1,917) | $ 13,573 | $ 36,275 | $ 52,121 |
| Operating expenses: | | | | |
| Research and development | 108,981 | 100,034 | 435,069 | 419,979 |
| General and administrative | 28,994 | 23,626 | 116,497 | 90,306 |
| Total operating expenses | 137,975 | 123,660 | 551,566 | 510,285 |
| Operating loss | (139,892) | (110,087) | (515,291) | (458,164) |
| Other income (expense), net: | | | | |
| Interest income | 12,459 | 5,354 | 49,832 | 8,542 |
| Loss from equity method investment | (4,728) | (3,248) | (15,633) | (11,079) |
| Change in fair value of contingent consideration | - | (5,426) | (100) | (13,485) |
| Total other income (expense), net | 7,731 | (3,320) | 34,099 | (16,022) |
| Net loss | $ (132,161) | $ (113,407) | $ (481,192) | $ (474,186) |
| Net loss per share, basic and diluted | $ (1.46) | $ (1.40) | $ (5.42) | $ (6.16) |
| Weighted average shares outstanding, basic and diluted | 90,461 | 81,223 | 88,770 | 76,972 |

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

| | December 31, 2023 | December 31, 2022 |
|---|---|---|
| Cash, cash equivalents and marketable securities | $ 1,012,087 | $ 1,261,960 |
| Total assets | 1,300,977 | 1,520,114 |
| Total liabilities | 250,808 | 284,530 |
| Total stockholders' equity | 1,050,169 | 1,235,584 |

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