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

May 9, 2024

- *Rapidly enrolling patients in the Phase 3 MAGNITUDE trial of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy*
- *Aligned with FDA on design for a new Phase 3 trial of NTLA-2001 for the treatment of hereditary ATTR amyloidosis with polyneuropathy, subject to review of IND application; expect to initiate the study by year-end*
- *Plan to present new clinical data from the ongoing NTLA-2001 Phase 1 in 2H 2024*
- *On track to initiate the Phase 3 study of NTLA-2002 for the treatment of hereditary angioedema (HAE) in 2H 2024*
- *Will present long-term follow-up data from the NTLA-2002 Phase 1 study on June 2 and expect to report topline results from Phase 2 in mid-2024*
- *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), in 2024*
- *Ended the first quarter of 2024 in a strong financial position with approximately $953 million in cash*

CAMBRIDGE, Mass., May 09, 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 first quarter ended March 31, 2024.

“Intellia continues to make outstanding progress across our pipeline of *in vivo* and *ex vivo* single-dose CRISPR-based therapies. With one ongoing and two soon-to-be-initiated pivotal Phase 3 trials, Intellia is undoubtedly at the forefront of a new era in medicine,” said Intellia President and Chief Executive Officer John Leonard, M.D. “We have been extremely pleased with the speed of enrollment in the Phase 3 MAGNITUDE trial for patients with ATTR amyloidosis with cardiomyopathy, which is tracking ahead of our initial projections. In addition, we now expect to start a pivotal Phase 3 trial of NTLA-2001 for patients with polyneuropathy by year-end, based on productive discussions with the FDA. Moving to NTLA-2002, we expect to report key data readouts from the Phase 1/2 study this year. These data will support the dose selection for the Phase 3 trial and highlight what we believe is the potential for NTLA-2002 to dramatically change the HAE treatment paradigm. Building on our success with *in vivo* gene inactivation, we are excited to initiate the first-in-human study of NTLA-3001 for AATD this year, positioning us to be the first to clinically validate CRISPR-based gene insertion. We look forward to continuing our strong execution, with many notable milestones to mark the progress against our strategic priorities.”

First Quarter 2024 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):**
    - The pivotal Phase 3 MAGNITUDE trial is rapidly enrolling. In March, the first patients in the U.S. and globally were dosed. Enrollment is currently tracking well ahead of the Company’s initial projections, with over 30 patients dosed to date and more than 40 additional patients in screening. Many additional sites are expected to open in the weeks and months ahead to further accelerate enrollment.
  - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
    - Intellia announced today alignment with the U.S. Food and Drug Administration (FDA) on a pivotal Phase 3 trial design to support a biologics license application (BLA) filing for NTLA-2001 as a single-dose treatment for people living with ATTRv-PN, subject to review of its investigational new drug (IND) application. The study is expected to be a small, placebo-controlled trial conducted at ex-U.S. sites with approximately 50 ATTRv-PN patients. The Company plans to initiate the study by year-end.
  - The Company plans to present updated data from the ongoing Phase 1 study in the second half of 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.
    - The Company will present updated data from the Phase 1 study at the European Academy of Allergy and Clinical Immunology (EAACI) Congress 2024, taking place May 31 – June 3 in Valencia, Spain. Long-term data from the Phase 1 portion of the Phase 1/2 study will include safety, kallikrein reduction and attack rate data, including number of patients who continue to be completely attack free through the latest follow-up. Additionally, Intellia plans to report topline results from the Phase 2 portion in mid-2024 and present full results at a medical meeting in the second half of 2024.
    - In January, landmark findings from the Phase 1 portion of the Phase 1/2 study of NTLA-2002 were published in the *New England Journal of Medicine* (NEJM).

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 a Phase 1 study of NTLA-3001 in 2024.

***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.
- 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.

***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 solid tumors.

Corporate Updates

- Corporate Responsibility Report: In April, Intellia published its 2024 Corporate Responsibility Report. The report highlights the Company's Environmental, Social and Governance (ESG) principles and practices as part of its objective to build a sustainable company, while delivering on its commitments to patients, employees and shareholders.

Upcoming Events

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

- Bank of America Health Care Conference, May 14, Las Vegas
- RBC Capital Markets Global Healthcare Conference, May 14, New York
- EAACI Congress 2024, May 31 – June 3, Valencia, Spain
- Goldman Sachs 45th Annual Global Healthcare Conference, June 10, Miami

First Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $953.4 million as of March 31, 2024, compared to $1.0 billion as of December 31, 2023. The decrease was driven by cash used to fund operations of $137.2 million. The decrease was offset in part by $58.0 million of net equity proceeds from the Company's "At the Market" (ATM) program, $12.6 million of interest income, $5.9 million of reimbursement from its collaborators, and $2.0 million in proceeds from employee-based stock plans. The cash position is expected to fund operations into late 2026.

- **Collaboration Revenue:** Collaboration revenue was $28.9 million during the first quarter of 2024, compared to $12.6 million during the first quarter of 2023. The $16.3 million increase was mainly driven by a $21.0 million non-cash revenue recognition adjustment related to the AvenCell collaboration.

- **R&D Expenses:** Research and development expenses were $111.8 million during the first quarter of 2024, compared to $97.1 million during the first quarter of 2023. The $14.7 million increase was primarily driven by the advancement of our lead programs. Stock-based compensation expense included in research and development expenses was $20.2 million for the first quarter of 2024.

- **G&A Expenses:** General and administrative expenses were $31.1 million during the first quarter of 2024, compared to $27.4 million during the first quarter of 2023. The $3.7 million increase was primarily related to stock-based compensation. Stock-based compensation expense included in general and administrative expenses was $14.0 million for the first quarter of 2024.

- **Net Loss:** Net loss was $107.4 million for the first quarter of 2024, compared to $103.1 million during the first quarter of 2023.

Conference Call to Discuss First Quarter 2024 Results

The Company will discuss these results on a conference call today, Thursday, May 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 May 9 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, its ability to rapidly enroll the Phase 3 MAGNITUDE study, the planned initiation of its Phase 3 trial of NTLA-2001 for the treatment of hereditary ATTR amyloidosis with polyneuropathy by year-end, the planned initiation of a global pivotal Phase 3 study of NTLA-2002 in 2H 2024, its expectations that data will inform the dose selection for the Phase 3 trial of NTLA-2002, 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 lead to 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 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, such as bone marrow, brain, muscle, lung and eye; 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, 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, 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, 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, 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,	
	2024	**2023**
Collaboration revenue	$ 28,935	$ 12,606
Operating expenses:		
Research and development	111,847	97,116
General and administrative	31,091	27,448
Total operating expenses	142,938	124,564
Operating loss	(114,003)	(111,958)
Other income (expense), net:		
Interest income	12,632	11,980
Change in fair value of investments, net	(6,065)	-
Loss from equity method investment	-	(3,048)
Change in fair value of contingent consideration	-	(100)
Total other income (expense), net	6,567	8,832
Net loss	$ (107,436)	$ (103,126)
Net loss per share, basic and diluted	$ (1.12)	$ (1.17)
Weighted average shares outstanding, basic and diluted	95,502	87,772

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

	March 31, 2024	**December 31, 2023**
Cash, cash equivalents and marketable securities	$ 953,384	$ 1,012,087
Total assets	1,259,589	1,300,977
Total liabilities	223,452	250,808
Total stockholders' equity	1,036,137	1,050,169

Intellia Contacts:

Investors:
Ian Karp
Senior Vice President, Investor Relations and Corporate Communications
ian.karp@intelliatx.com

Lina Li
Senior Director, Investor Relations and Corporate Communications
lina.li@intelliatx.com

Media:
Matt Crenson
Ten Bridge Communications
media@intelliatx.com
mcrenson@tenbridgecommunications.com

Source: Intellia Therapeutics, Inc.