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

May 4, 2023

- *Dosed first patient in the global Phase 2 study of NTLA-2002 for the treatment of hereditary angioedema (HAE)*
- *Expects to complete enrollment in the Phase 2 study of NTLA-2002 in 2H 2023*
- *Plans to submit IND application in mid-2023 for a global pivotal study of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy; study initiation anticipated by year-end 2023, subject to regulatory feedback*
- *On track to present additional clinical data in 2023 from both ongoing NTLA-2001 and NTLA-2002 first-in-human studies*
- *Progressing next wave of clinical candidates, including NTLA-3001 and NTLA-2003, and advancing novel gene editing capabilities*
- *Ended the first quarter of 2023 in a strong financial position with approximately $1.2 billion in cash*

CAMBRIDGE, Mass., May 04, 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 first quarter ended March 31, 2023.

“We’ve had a productive start to the year with the successful clearance of our first IND application for an investigational *in vivo* CRISPR-based therapy alongside broad pipeline and platform progress,” said Intellia President and Chief Executive Officer John Leonard, M.D. “Today, we are pleased to announce dosing has begun in the Phase 2 study of NTLA-2002, and based on strong interest from investigators and patients, we expect to complete enrollment in the second half of this year. Our rapid progression of the clinical development of NTLA-2002 supports our goal to bring forth a potential functional cure for the treatment of hereditary angioedema. Simultaneously, we are working toward submitting our second *in vivo* IND application and initiating a global pivotal trial for NTLA-2001. We look forward to sharing new interim data from both the NTLA-2001 and NTLA-2002 first-in-human studies in the months to come.”

First 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 Pharmaceuticals, Inc.
  - **ATTR Amyloidosis with Cardiomyopathy (ATTR-CM):**
    - Intellia plans to submit a U.S. Investigational New Drug (IND) application in mid-2023. Subject to regulatory feedback, the Company anticipates initiating a global pivotal trial for ATTR-CM by year-end 2023.
    - The Company expects to present additional data from the ATTR-CM arm of the Phase 1 study in 2023, including longer-term safety and durability data, as well as emerging clinical endpoints.
  - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
    - As previously announced, during the first quarter of 2023, the planned enrollment of the dose-expansion portion of the ATTRv-PN arm in the Phase 1 study was completed to inform a pivotal study. The Company is actively preparing for a global pivotal study, which includes discussions with regulatory authorities.
    - Intellia recently began redosing patients in the 0.1 mg/kg cohort (n=3), the initial cohort and lowest dose tested in the dose-escalation portion of the Phase 1 study, with the 55 mg dose selected for the dose-expansion cohort.
    - The Company plans to present additional clinical data from the ATTRv-PN arm of the Phase 1 study in 2023.

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.
    - Intellia announced today that the first patient has been dosed in the global Phase 2 portion of its Phase 1/2 clinical trial of NTLA-2002. Based on encouraging study interest from both investigators and patients, the Company expects to complete enrollment (n=25) in 2H 2023.
    - The Company announced in March that the U.S. Food and Drug Administration (FDA) cleared the NTLA-2002 Phase 2 IND application. Additionally, the FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to NTLA-2002 for the treatment of HAE.
    - In January 2023, Intellia was awarded the Innovation Passport for NTLA-2002 by the U.K. Medicines and Healthcare products Regulatory Agency (MHRA). The Innovation Passport is the point of entry into the U.K.'s Innovative Licensing and Access Pathway (ILAP), which is designed to accelerate time to market and facilitate patient access to innovative medicines.
    - The Company plans to present additional clinical data from the Phase 1 portion of the first-in-human study in 2023. Data expected to be presented include updated safety, durability of pharmacodynamic effect and attack-rate measures from all three patient cohorts.

Alpha-1 Antitrypsin Deficiency (AATD)

- **NTLA-3001 for 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 is conducting IND-enabling activities for NTLA-3001 and plans to submit an IND or IND-equivalent filing in 2H 2023.
- **NTLA-2003 for 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. This approach aims to halt the progression of liver disease and eliminate the need for liver transplant in severe cases.
    - Intellia is conducting IND-enabling activities for NTLA-2003, with the expectation of completing these activities by year-end 2023.

***Ex Vivo* Program Updates**

Immuno-oncology and Autoimmune Diseases

- 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.

Research and Corporate Updates

- **Modular Platform and Pipeline Expansion:** Intellia is expanding its industry-leading genome editing platform and scientific leadership through editing, delivery and cell engineering innovations that may enable broader *in vivo* and *ex vivo* applications.
- **Board of Directors Update:** In April, Intellia announced the appointment of Bill Chase to its board of directors. Mr. Chase will be a member of the audit committee and will succeed Caroline Dorsa as chair of the audit committee upon her retirement from the board on June 15, 2023.
- **Corporate Responsibility Report:** In May, Intellia published its 2023 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 2023:

- Bank of America Securities 2023 Health Care Conference, May 9, Las Vegas
- 2023 RBC Capital Markets Global Healthcare Conference, May 16, New York City
- Barclays Gene Editing & Therapy Summit, May 24, New York City
- Stifel Genetic Medicines Day, May 30, virtual

Upcoming Milestones

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

- **NTLA-2001 for ATTR amyloidosis**:
  - Submit an IND application in mid-2023 to enable inclusion of U.S. sites in a pivotal study of NTLA-2001 for patients with ATTR-CM.
  - Present additional clinical data from the ongoing Phase 1 study of NTLA-2001 in 2023.
  - Initiate a global pivotal NTLA-2001 trial for ATTR-CM by year-end 2023, subject to regulatory feedback.
  - Prepare for a Phase 3 study of NTLA-2001 for the treatment of ATTRv-PN, including discussions with regulatory authorities.
- **NTLA-2002 for HAE**:
  - Present additional clinical data from the ongoing first-in-human study of NTLA-2002 in 2023.
  - Complete enrollment in the Phase 2 portion of the Phase 1/2 study in 2H 2023.
- **AATD Franchise:**
  - Submit an IND or IND-equivalent application for NTLA-3001 for AATD-associated lung disease in 2H 2023.
  - Complete IND-enabling activities for NTLA-2003 for AATD-associated liver disease by year-end 2023.
- **Platform Innovation:**
  - Advance novel gene editing technologies, including DNA writing and delivery to other tissues outside of the liver.

First Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.2 billion as of March 31, 2023, compared to $1.3 billion as of December 31, 2022. The decrease was driven by cash used to fund operations of approximately $120.9 million. The decrease was offset in part by $12.0 million of interest income, $3.4 million of reimbursement from its collaborators, $1.5 million of net equity proceeds from the Company's "At the Market" (ATM) program and $0.8 million in proceeds from employee-based stock plans.
- **Collaboration Revenue:** Collaboration revenue increased by $1.3 million to $12.6 million during the first quarter of 2023, compared to $11.3 million during the first quarter of 2022.
- **R&D Expenses:** Research and development expenses decreased by approximately $36.0 million to $97.1 million during the first quarter of 2023, compared to $133.1 million during the first quarter of 2022. This decrease was primarily driven by $56.0 million of expense related to the acquisition of Rewrite Therapeutics, Inc. during the first quarter of 2022. The decrease related to the acquisition of Rewrite Therapeutics, Inc. was offset by an increase in expenses of $20.0 million primarily driven by the advancement of its lead programs and personnel growth to support these programs. Stock-based compensation expense included in research and development expenses was $16.9 million for the first quarter of 2023.
- **G&A Expenses:** General and administrative expenses increased by $5.0 million to $27.4 million during the first quarter of 2023, compared to $22.4 million during the first quarter of 2022. This increase was primarily related to an increase in stock-based compensation of $2.1 million. Stock-based compensation expense included in general and administrative expenses was $10.3 million for the first quarter of 2023.
- **Net Loss:** The Company's net loss was $103.1 million for the first quarter of 2023, compared to $146.9 million during the first quarter of 2022.

Conference Call to Discuss First Quarter 2023 Results

The Company will discuss these results on a conference call today, Thursday, May 4, 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 4, 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 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 the treatment of ATTR amyloidosis and NTLA-2002 for the treatment of hereditary angioedema ("HAE") pursuant to its clinical trial applications ("CTA"), including the expected timing of data releases, 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 completion of planned enrollment of the Phase 2 study for NTLA-2002 in the second half of 2023; the advancement of development candidates, such as NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency ("AATD")-associated lung disease and NTLA-2003 for AATD-associated liver disease, including the success of its investigational new drug ("IND")-enabling studies and completion of IND-enabling activities for NTLA-2003 by the end of 2023; 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 submission of an IND or IND-equivalent for NTLA-3001 in the second half of 2023, submission of an IND application for NTLA-2001 in mid-2023 and initiating a global pivotal trial for NTLA-2001 by the end of 2023; its ability to leverage the designation of NTLA-2002 under the U.S. Food and Drug Administration's Regenerative Medicine Advanced Therapy and the U.K. Medicines and Healthcare products Regulatory Agency's Innovation Passport to accelerate time to market and facilitate patient access; 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 delivery to other tissues outside of the liver, to advance additional 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 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; and risks related to Intellia's collaborations with Regeneron Pharmaceuticals, Inc. 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 ("SEC"). 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,	
	2023	**2022**
Collaboration revenue	$ 12,606	$ 11,252
Operating expenses:		
Research and development	97,116	133,095

General and administrative	27,448	22,403
Total operating expenses	124,564	155,498
Operating loss	(111,958)	(144,246)
Other income (expense), net:		
Interest income	11,980	540
Loss from equity method investment	(3,048)	(2,745)
Change in fair value of contingent consideration	(100)	(421)
Total other income (expense), net	8,832	(2,626)
Net loss	$ (103,126)	$ (146,872)
Net loss per share, basic and diluted	$ (1.17)	$ (1.96)
Weighted average shares outstanding, basic and diluted	87,772	74,751

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

	March 31, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 1,158,580	$ 1,261,960
Total assets	1,417,889	1,520,114
Total liabilities	227,046	284,530
Total stockholders' equity	1,190,843	1,235,584

Intellia Contacts:

Investors:
Ian Karp
Senior Vice President, Investor Relations and Corporate Communications
+1-857-449-4175
ian.karp@intelliatx.com

Lina Li
Senior Director, Investor Relations and Corporate Communications
+1-857-706-1612
lina.li@intelliatx.com

Media:
Rebecca Spalding
Ten Bridge Communications
+1-646-509-3831
media@intelliatx.com
rebecca@tenbridgecommunications.com

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