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

August 3, 2023

- *Completed identification of all patients for the Phase 2 study of NTLA-2002 for the treatment of hereditary angioedema (HAE)*
- *Plans to initiate a global pivotal Phase 3 study of NTLA-2002 as early as Q3 2024, subject to regulatory feedback*
- *On track to submit IND application in September 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*
- *Plans to present additional clinical data in 2023 from the ongoing NTLA-2001 first-in-human study by year-end*
- *Expects to submit a Clinical Trial Application (CTA) by year-end for NTLA-3001, Intellia's first* in vivo *insertion candidate, in development for the treatment of alpha-1 antitrypsin deficiency (AATD)-associated lung disease*
- *Ended the second quarter of 2023 in a strong financial position with approximately $1.1 billion in cash*

CAMBRIDGE, Mass., Aug. 03, 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 second quarter ended June 30, 2023.

"We continue to make significant and rapid progress in the development of our first two *in vivo*, CRISPR-based therapeutic candidates," said Intellia President and Chief Executive Officer John Leonard, M.D. "For NTLA-2002, we are thrilled to see the excitement for this investigational therapy from the HAE community. In just a handful of months, we were able to identify all patients required to fully enroll the ongoing Phase 2 study and now expect to initiate the pivotal Phase 3 program next year. For NTLA-2001, our team has been working diligently to prepare for an IND submission in September and to begin the Phase 3 study for the cardiomyopathy manifestation of ATTR amyloidosis before the end of this year. Overall, we continue to believe that NTLA-2001 and NTLA-2002 represent only the beginning for our pipeline and platform as additional *in vivo* and *ex vivo* candidates advance toward the clinic."

Second 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 an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) in September. Subject to regulatory feedback, the Company anticipates initiating a global pivotal study for ATTR-CM by year-end 2023.
    - The Company expects to present additional data from the ATTR-CM arm of the Phase 1 study by year-end 2023, including longer-term safety and durability data, as well as emerging clinical endpoints.
  - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
    - The Company is actively preparing for a global pivotal Phase 3 study, including discussions with regulatory authorities.
    - The Company plans to present additional data from the ATTRv-PN arm of the Phase 1 study by year-end 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 due to the substantial interest from physicians and patients to participate in the NTLA-2002 clinical program, all patients have been identified for the global Phase 2 portion of the study with enrollment expected to be completed in the second half of 2023.

- Based on the strong momentum of the program, the Company plans to complete enrollment at ex-U.S. sites in the ongoing Phase 2 study. Further, following the March 2023 IND clearance, the FDA requested supplemental preclinical data related to the inclusion of female patients of child-bearing potential. Intellia expects to submit these data in advance of the planned Phase 3 trial, which will complement the clinical data collected from female patients of child-bearing potential dosed in the ongoing Phase 1/2 study.

- 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.

- In June, Intellia announced additional positive interim results from the Phase 1 portion of the ongoing Phase 1/2 study of NTLA-2002. Across all 10 patients, a 95% mean reduction in monthly attack rate was observed after a single dose of NTLA-2002 through the latest follow-up. The median duration of follow-up was 9.0 months (range of 5.6 - 14.1 months). At all three dose levels evaluated, NTLA-2002 has been well tolerated, and the majority of adverse events were mild in severity. These interim data were presented at the European Academy of Allergy and Clinical Immunology (EAACI) Hybrid Congress 2023, held in Hamburg, Germany, and virtually.

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 a Clinical Trial Application (CTA) by year-end 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 became chair of the audit committee on June 15, 2023. In June, Intellia announced the retirement of Jean-François Formela, M.D., from its board of directors, effective June 15, 2023.

Upcoming Events

The Company will participate in the following events during the third quarter of 2023:

- Wells Fargo Healthcare Conference, September 6, Everett, Massachusetts

- BMO Gene Editing Event, September 26, virtual

- Cantor Global Healthcare Conference, September 27, New York City

Upcoming Milestones

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

- **NTLA-2001 for ATTR amyloidosis**:
  - Submit an IND application in September 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 by year-end 2023.
  - Initiate a global pivotal study for NTLA-2001 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**:
  - Complete enrollment in the Phase 2 portion of the Phase 1/2 study in 2H 2023.
- **AATD Franchise:**
  - Submit a CTA application for NTLA-3001 for AATD-associated lung disease by year-end 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.

Second Quarter 2023 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $1.1 billion as of June 30, 2023, compared to $1.3 billion as of December 31, 2022. The decrease was driven by cash used to fund operations of approximately $227.3 million. The decrease was offset in part by $24.6 million of interest income, $8.0 million of reimbursement from its collaborators, $1.5 million of net equity proceeds from the Company's "At the Market" (ATM) program and $3.3 million in proceeds from employee-based stock plans.
- **Collaboration Revenue:** Collaboration revenue decreased by $0.4 million to $13.6 million during the second quarter of 2023, compared to $14.0 million during the second quarter of 2022.
- **R&D Expenses:** Research and development expenses increased by approximately $25.1 million to $115.3 million during the second quarter of 2023, compared to $90.2 million during the second 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 $22.4 million for the second quarter of 2023.
- **G&A Expenses:** General and administrative expenses increased by $8.5 million to $30.7 million during the second quarter of 2023, compared to $22.1 million during the second quarter of 2022. This increase was primarily related to an increase in stock-based compensation of $5.1 million. Stock-based compensation expense included in general and administrative expenses was $14.0 million for the second quarter of 2023.
- **Net Loss:** The Company's net loss was $123.7 million for the second quarter of 2023, compared to $100.7 million during the second quarter of 2022.

Conference Call to Discuss Second Quarter 2023 Results

The Company will discuss these results on a conference call today, Thursday, August 3 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 August 3 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 application (“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 planned submission of an IND application for NTLA-2001 in September 2023 and planned initiation of a global pivotal 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 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 planned CTA submission for NTLA-3001 by the end of 2023; 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; 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 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. 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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Collaboration revenue	$ 13,594	$ 14,030	$ 26,200	$ 25,282
Operating expenses:				
Research and development	115,276	90,199	212,392	223,294
General and administrative	30,652	22,132	58,100	44,535
Total operating expenses	145,928	112,331	270,492	267,829
Operating loss	(132,334)	(98,301)	(244,292)	(242,547)
Other income (expense), net:				
Interest income	12,653	703	24,633	1,243
Loss from equity method investment	(4,000)	(3,252)	(7,048)	(5,997)
Change in fair value of contingent consideration	-	172	(100)	(249)
Total other income (expense), net	8,653	(2,377)	17,485	(5,003)
Net loss	$ (123,681)	$ (100,678)	$ (226,807)	$ (247,550)
Net loss per share, basic and diluted	$ (1.40)	$ (1.33)	$ (2.58)	$ (3.29)
Weighted average shares outstanding, basic and diluted	88,185	75,823	87,979	75,282

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

	June 30, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 1,072,087	$ 1,261,960
Total assets	1,323,240	1,520,114
Total liabilities	218,352	284,530
Total stockholders' equity	1,104,888	1,235,584

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Source: Intellia Therapeutics, Inc.