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

August 8, 2024

- *Phase 2 study of NTLA-2002 for hereditary angioedema (HAE) met its primary and all secondary endpoints; plan to present detailed results at an upcoming medical meeting in the fourth quarter*
- *Selected the 50 mg dose of NTLA-2002 for the pivotal Phase 3 trial on track to begin in 2H 2024*
- *Rapid enrollment continues in the Phase 3 MAGNITUDE trial of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis with cardiomyopathy*
- *On track to initiate the Phase 3 study of NTLA-2001 for the treatment of hereditary ATTR amyloidosis with polyneuropathy by year-end*
- *Plan to present new clinical data from the ongoing NTLA-2001 Phase 1 in 2H 2024*
- *Expect to dose the first patient in the Phase 1/2 study of NTLA-3001, an* in vivo *gene insertion candidate for the treatment of alpha-1 antitrypsin deficiency (AATD) in 2H 2024*
- *Ended the second quarter of 2024 in a strong financial position with approximately $940 million in cash*

CAMBRIDGE, Mass., Aug. 08, 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 second quarter ended June 30, 2024.

"We are delighted to report the Phase 2 study of NTLA-2002 met its primary efficacy and all secondary endpoints at both dose levels and, importantly, provided clear support for advancing the 50 mg dose into the pivotal Phase 3 trial in patients with hereditary angioedema," said Intellia President and Chief Executive Officer John Leonard, M.D. "Based on these positive results and our recent successful end-of-Phase 2 meeting with the FDA, we see a clear path to initiating the Phase 3 trial in the coming months. We look forward to presenting the detailed Phase 2 results at a medical meeting in the fourth quarter as we continue to advance what we believe could be a functional cure for hereditary angioedema. With three pivotal Phase 3 trials and our first gene insertion trial expected to be active by year-end, Intellia is closer than ever to transforming the future of medicine with our one-time, *in vivo* gene editing therapies."

Second Quarter 2024 and Recent Operational Highlights

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 announced today positive topline results from the Phase 2, randomized, double-blind, placebo-controlled study of NTLA-2002 in patients with HAE. The clinical trial met its primary efficacy and all secondary endpoints in the 16-week primary observation period, with a single 25 mg or 50 mg dose leading to deep reductions in attacks. No new safety findings were observed. Intellia has selected the 50 mg dose for further evaluation in the global pivotal Phase 3 trial based upon the greater number of patients with complete attack elimination and greater kallikrein protein reduction compared to the 25 mg dose observed in the Phase 2 study, which is consistent with the previously reported Phase 1 results. The Company plans to present the detailed Phase 2 data at an upcoming medical meeting in the fourth quarter of this year.
  - The Company announced today the successful completion of an end-of-Phase 2 meeting with the U.S. Food and Drug Administration (FDA) supporting its Phase 3 plans for NTLA-2002. The Phase 3 study is on track to initiate in the second half of 2024, subject to regulatory feedback.
  - In June, Intellia presented positive long-term data from the ongoing Phase 1 study. Eight of 10 patients remained completely attack-free following the 16-week primary observation period. These patients have experienced ongoing attack-free durations of greater than 18 months after a single-dose treatment, with the longest ongoing individual attack-free duration reaching over 26 months. Across all patients, NTLA-2002 led to a 98% mean reduction in monthly HAE attack rate. Consistent with previously reported results, NTLA-2002 was well-tolerated, with the majority of adverse events being mild in severity through the latest follow-up. These interim data were presented at the European Academy of Allergy and Clinical Immunology (EAACI) Congress 2024 in Valencia, Spain.

Transthyretin (ATTR) Amyloidosis

- **NTLA-2001**: NTLA-2001, now known as nexiguran ziclumeran (nex-z), is an investigational *in vivo* CRISPR-based therapy designed to inactivate the *TTR* gene in liver cells, thereby preventing 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 enrolling rapidly and continues to track ahead of the Company's initial projections. During the second quarter, the Company received approval for its application under the new European Union Clinical Trials Regulation, which enables the Phase 3 trial to proceed in Denmark, Germany, France, Italy, Spain and Sweden. The MAGNITUDE trial of NTLA-2001 is now cleared by regulatory agencies in over 12 countries and actively enrolling at over 35 sites globally.
    - **Hereditary ATTR Amyloidosis with Polyneuropathy (ATTRv-PN):**
        - The Company plans to initiate a pivotal Phase 3 trial of NTLA-2001 as a single-dose treatment for ATTRv-PN by year-end. As previously announced, the study is expected to be a small, placebo-controlled trial conducted at ex-U.S. sites with approximately 50 ATTRv-PN patients.
    - Intellia plans to present updated data from the ongoing Phase 1 study in the second half of 2024.

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.
    - In July, Intellia announced the authorization of its Clinical Trial Application by the United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA) to initiate a first-in-human study of NTLA-3001. Intellia expects to dose the first patient in the Phase 1/2 study of NTLA-3001 in the second half of 2024.

***In Vivo* Platform Expansion**

- In June, Intellia presented positive clinical proof-of-concept data that redosing with CRISPR, utilizing the Company's proprietary non-viral, LNP-based delivery platform, enabled an additive pharmacodynamic effect. In the three patients who previously received the lowest dose of 0.1 mg/kg in the Phase 1 dose-escalation study of NTLA-2001, follow-on dosing with a 55 mg dose of NTLA-2001 led to a deeper protein reduction. Median reduction in serum TTR was 90% at day 28 after redosing. The corresponding reduction from original baseline levels was a 95% median reduction in serum TTR. NTLA-2001 was generally well tolerated across all patients after receiving the follow-on dose. While redosing is not planned for the NTLA-2001 program in ATTR amyloidosis, a redosing option could be an important advantage of Intellia's LNP-based delivery platform for future investigational therapies where a target additive effect is desired. These data were presented at the Peripheral Nerve Society (PNS) Annual Meeting in Montreal, Canada.

- 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.

***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 both hematological and solid tumors.

Corporate Updates

- In June, Intellia announced the appointment of Brian Goff to its board of directors. Mr. Goff joins the board of directors with over three decades of commercialization, operations and sales and marketing experience at leading biopharmaceutical companies.

- In June, Intellia announced the appointment of Edward Dulac as Executive Vice President, Chief Financial Officer, and Treasurer, effective July 22, 2024. Mr. Dulac succeeds Glenn Goddard, who stepped down from his role effective June 30, 2024. Mr. Dulac joins Intellia with more than 20 years of combined finance, business development and corporate strategy experience.

Upcoming Events

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

- Morgan Stanley 22nd Annual Global Healthcare Conference, September 4, New York
- Wells Fargo Healthcare Conference, September 4, Boston
- Cantor Global Healthcare Conference, September 17, New York

Second Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $939.9 million as of June 30, 2024, compared to $1.0 billion as of December 31, 2023. The decrease was driven by cash used to fund operations of $234.4 million. The Company's investments were offset in part by $96.4 million of net equity proceeds from the Company's "At the Market" (ATM) program, $35.9 million of reimbursement from collaborators, including a one-time $30.0 million payment received in April 2024 related to the Company's technology collaboration with Regeneron, $25.1 million of interest income and $4.8 million in proceeds from employee-based stock plans. The cash position is expected to fund operations into late 2026.
- **Collaboration Revenue:** Collaboration revenue was $7.0 million during the second quarter of 2024, compared to $13.6 million during the second quarter of 2023. The $6.6 million decrease was mainly driven by a reduction in revenue related to the AvenCell license and collaboration agreement.
- **R&D Expenses:** Research and development (R&D) expenses were $114.2 million during the second quarter of 2024, compared to $115.3 million during the second quarter of 2023. The $1.1 million decrease was primarily driven by a decrease in employee-related expenses. Stock-based compensation expense included in R&D expenses was $25.4 million for the second quarter of 2024.
- **G&A Expenses:** General and administrative (G&A) expenses were $31.8 million during the second quarter of 2024, compared to $30.7 million during the second quarter of 2023. The $1.1 million increase was primarily related to stock-based compensation. Stock-based compensation expense included in G&A expenses was $15.4 million for the second quarter of 2024.
- **Net Loss:** Net loss was $147.0 million for the second quarter of 2024, compared to $123.7 million during the second quarter of 2023.

Conference Call to Discuss Second Quarter 2024 Results

The Company will discuss these results on a conference call today, Thursday, August 8 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 8 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, now known as nexiguran ziclumeran or "nex-z", 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 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 be a functional cure for patients with HAE and to demonstrate 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 normal levels after a single dose; the expansion of its CRISPR/Cas9 technology and related novel technologies, including advancing gene editing programs in tissues outside of the liver, such as bone marrow, brain, muscle, lung and eye, and the potential of those programs to dramatically expand the opportunities for CRISPR-based treatments; its ability to advance multiple *ex vivo* programs utilizing its allogeneic platform, which is designed to avoid both T cell- and NK cell-mediated rejection, for the treatment of immuno-oncology and autoimmune diseases, and to combine its allogeneic platform with edits to enhance cell function and offer a new approach to target both hematological and solid tumors; 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 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, 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 June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Collaboration revenue	$ 6,957	$ 13,594	$ 35,892	$ 26,200
Operating expenses:				
Research and development	114,207	115,276	226,054	212,392
General and administrative	31,793	30,652	62,884	58,100
Total operating expenses	146,000	145,928	288,938	270,492
Operating loss	(139,043)	(132,334)	(253,046)	(244,292)
Other (expense) income, net:				
Interest income	12,422	12,653	25,054	24,633
Change in fair value of investments, net	(20,354)	-	(26,419)	-
Loss from equity method investment	-	(4,000)	-	(7,048)
Change in fair value of contingent consideration	-	-	-	(100)
Total other (expense) income, net	(7,932)	8,653	(1,365)	17,485
Net loss	$ (146,975)	$ (123,681)	$ (254,411)	$ (226,807)
Net loss per share, basic and diluted	$ (1.52)	$ (1.40)	$ (2.64)	$ (2.58)
Weighted average shares outstanding, basic and diluted	96,975	88,185	96,238	87,979

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

	June 30, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 939,873	$ 1,012,087
Total assets	1,191,536	1,300,977
Total liabilities	220,474	250,808
Total stockholders’ equity	971,062	1,050,169

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