# Intellia Therapeutics Announces Second Quarter 2022 Financial Results and Highlights Recent Company Progress

August 4, 2022

- *Completed dose-escalation portion of the ongoing Phase 1 study of NTLA-2001 in patients with transthyretin (ATTR) amyloidosis with cardiomyopathy; expects to present interim safety and serum TTR reduction data in 2H 2022*
- *Presented updated interim data from the dose-escalation portion of the polyneuropathy arm, establishing deep reductions of disease-causing protein were sustained through 12 months following a single dose of NTLA-2001*
- *Plans to present interim data for second* in vivo *CRISPR clinical candidate, NTLA-2002 for hereditary angioedema (HAE), in 2H 2022*
- *Moves to exclusively develop cell therapies leveraging its proprietary allogeneic platform; pivoting to an allogeneic version of NTLA-5001*
- *Ended the second quarter of 2022 with strong cash position of $907 million*

CAMBRIDGE, Mass., Aug. 04, 2022 (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, 2022.

“We continue to make excellent progress in both the cardiomyopathy and polyneuropathy arms of the landmark Phase 1 study of NTLA-2001,” said Intellia President and Chief Executive Officer John Leonard, M.D. “In June, we presented durability data demonstrating deep reductions in a disease-causing protein were sustained over time following a single-dose treatment. Additionally, we’ve completed the dose-escalation portion of the cardiomyopathy arm and look forward to starting the dose-expansion portion soon.”

Dr. Leonard continued, “As the leading full-spectrum genome editing company, our *ex vivo* capabilities and platform are also advancing. We believe our proprietary, differentiated cell engineering platform can solve many of the known challenges faced by both autologous and current allogeneic approaches. As a result, we plan to focus exclusively on developing allogeneic cell therapies, including an allogeneic version of NTLA-5001. Finally, we expect several important milestones later this year, which will include interim clinical data updates from the NTLA-2001 and NTLA-2002 programs.”

**Second Quarter 2022 and Recent Operational Highlights**

***In Vivo* Program Updates**

**Transthyretin (ATTR) Amyloidosis**

- **NTLA-2001:** NTLA-2001 is the first investigational CRISPR-based therapy to be systemically delivered to edit genes inside the human body and has the potential to be the first single-dose treatment for ATTR amyloidosis. Delivered with the Company’s *in vivo* lipid nanoparticle (LNP) technology, NTLA-2001 offers the possibility of halting and reversing the disease by driving a deep, potentially lifelong reduction in transthyretin (TTR) protein after a single dose. NTLA-2001 is being evaluated in a Phase 1, two-part, open-label study in adults with hereditary transthyretin amyloidosis with polyneuropathy (ATTRv-PN) or transthyretin amyloidosis with cardiomyopathy (ATTR-CM). NTLA-2001 is subject to a co-development/co-promotion agreement between Intellia, the lead party for this program, and Regeneron Pharmaceuticals, Inc.
    - To date, over 30 patients have been dosed across the polyneuropathy and cardiomyopathy arms. The growing body of data, particularly at therapeutically relevant doses, demonstrated treatment with NTLA-2001 resulted in rapid, deep and consistent reductions of serum TTR.
    - **ATTR-CM arm:** Intellia announced today the completion of the dose-escalation portion of the cardiomyopathy arm. The Company is finalizing selection of a fixed dose, at or near the fixed-dose equivalent of the 0.7 mg/kg dose, for evaluation in the dose-expansion portion of the study, subject to regulatory approval. The selection is based on clinical data from patients with ATTR-CM dosed at the 0.7 mg/kg and 1.0 mg/kg doses, which yielded similar TTR reductions. Additionally, both doses were generally well-tolerated. The Company remains on track to present interim data from the cardiomyopathy arm later this year.
    - **ATTRv-PN arm:** The Company announced today, subject to regulatory approval, plans to add a second cohort to the dose-expansion portion of the polyneuropathy arm, which will evaluate the same fixed dose selected for the

dose-expansion portion of the cardiomyopathy arm. The decision to study a second dose is based on the following: (1) the emerging data from the dose-escalation portion of the cardiomyopathy arm showed similar serum TTR reduction at both the 0.7 mg/kg and 1.0 mg/kg doses, (2) the comparability of performance at the 0.7 mg/kg and 1.0 mg/kg doses in the dose-escalation portion of the polyneuropathy arm, which led to an 86% and 93% mean and 97% and 98% maximum TTR reduction at day 28, respectively, and (3) a significant elevation in liver enzymes, which normalized without medical intervention, observed at day 28 in a patient treated in the dose-expansion portion of the polyneuropathy arm at the 80 mg dose (the fixed dose corresponding to 1.0 mg/kg). While the adverse event is considered possibly related to study drug, this patient was asymptomatic, had no increase in bilirubin and the event was deemed nonserious by the investigator.

- Intellia plans to submit a protocol amendment to evaluate a fixed dose corresponding to 0.7 mg/kg in the dose-expansion portion, with enrollment across both arms expected to be completed by the end of 2022, subject to regulatory feedback.

- In June, Intellia presented updated interim data from its ongoing Phase 1 study of NTLA-2001 in patients with ATTRv-PN at the European Association for the Study of the Liver International Liver Congress™ 2022. Extended follow-up data from 15 ATTRv-PN patients, treated across all four single-ascending dose cohorts, showed deep, dose-dependent reductions in serum TTR observed with prior readouts were sustained through the last measured timepoint of follow-up, reaching 12 months in the 0.1 mg/kg and 0.3 mg/kg cohorts and six months in the 0.7 mg/kg and 1.0 mg/kg cohorts. Both 0.7 mg/kg and 1.0 mg/kg doses led to greater than 85% mean TTR reduction at day 28. The durability and persistence of effect continue to support NTLA-2001 as a potential one-time treatment to permanently inactivate the *TTR* gene and reduce the disease-causing protein.

**Hereditary Angioedema (HAE)**

- **NTLA-2002:** NTLA-2002 leverages Intellia's proprietary *in vivo* LNP delivery technology 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, and 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 is progressing the single-ascending dose portion of its first-in-human study. The Company anticipates presenting interim data in the second half of 2022, including safety, kallikrein reduction and HAE attack rate data. These initial results are expected to characterize the emerging safety and activity profile of NTLA-2002 and potentially demonstrate the modularity of Intellia's proprietary CRISPR-based, LNP platform.

**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 IV infusions of A1AT augmentation therapy or lung transplant in severe cases.

    - Intellia is conducting Investigational New Drug (IND)-enabling activities for NTLA-3001, with plans to file an IND or IND-equivalent in 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.

***Ex Vivo* Program Updates**

**Acute Myeloid Leukemia (AML)**

- **NTLA-5001:** NTLA-5001 is an investigational autologous T cell receptor (TCR)-T cell therapy engineered to target the Wilms' Tumor 1 (WT1) antigen for the treatment of all genetic subtypes of AML. In March, Intellia announced that the first patient was dosed in the Phase 1/2a first-in-human trial of NTLA-5001.

- Intellia has decided to concentrate its *ex vivo* development efforts exclusively on allogeneic cell therapies manufactured from healthy donors. The Company's proprietary technologies, including its LNP-based cell engineering platform and novel allogeneic solution, offer significant advantages over both autologous and current investigational allogeneic approaches. Preclinical data presented on its differentiated allogeneic engineering platform showed allogeneic T cells were shielded from immune rejection, both host T and natural killer (NK) cell attack.

- Intellia announced today plans to discontinue its first-in-human study of NTLA-5001, an investigational autologous TCR-T cell therapy, and is pivoting to an allogeneic version of this program currently in preclinical development. This decision is not due to any safety or efficacy data emerging from the trial. It is instead based on the potential of Intellia's allogeneic platform to consistently deliver a high-quality, readily available and persistent cell product for treatment of aggressive cancers.

- Preclinical data supporting the development of a WT1-directed allogeneic TCR-T cell candidate will be presented at a future scientific conference in 2022.

**CD30+ Lymphomas**

- **NTLA-6001:** NTLA-6001 is a wholly owned, allogeneic CAR-T development candidate targeting CD30 for the treatment of CD30-expressing hematologic cancers, including relapsed or refractory classical Hodgkin lymphoma (cHL). NTLA-6001 is the first candidate developed using Intellia's proprietary allogeneic cell engineering platform.

    - Intellia is conducting IND-enabling activities for NTLA-6001.

**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.

    - Intellia plans to advance at least one additional new *in vivo* development candidate by the end of 2022.

    - The Company plans to highlight additional advances to its proprietary technology capabilities, including both genome editing and delivery tools, at upcoming scientific conferences in 2022.

**Upcoming Milestones**

The Company has set forth the following for pipeline progression:

***In Vivo***

- NTLA-2001 for ATTR amyloidosis:

    - Present interim data from ATTR-CM arm of Phase 1 study in 2H 2022

    - Complete enrollment of Phase 1 study for both ATTRv-PN and ATTR-CM subjects by the end of 2022

- NTLA-2002 for HAE: Present interim data from Phase 1/2 study in 2H 2022

- NTLA-3001 for AATD: File an IND or IND-equivalent in 2023

- Advance at least one additional new *in vivo* development candidate by the end of 2022

***Ex Vivo***

- Present on its proprietary allogeneic cell engineering platform at an upcoming scientific conference in 2022

**Modular Platform**

- Advance additional novel platform capabilities in 2022

**Second Quarter 2022 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities were $906.9 million as of June 30, 2022, compared to

$1.1 billion as of December 31, 2021. The decrease was driven by cash used to fund operations of approximately $191.2 million as well as the acquisition of Rewrite for $45.0 million. The decrease was offset in part by $38.9 million in net equity proceeds raised from the Company's "At the Market" (ATM) agreement and $14.3 million in proceeds from employee-based stock plans.

- **Collaboration Revenue:** Collaboration revenue increased by approximately $7.5 million to $14.0 million during the second quarter of 2022, compared to $6.6 million during the second quarter of 2021. The increase was primarily driven by our collaborations with AvenCell and Kyverna.

- **R&D Expenses:** Research and development expenses increased by $31.3 million to $90.2 million during the second quarter of 2022, compared to $58.9 million during the second quarter of 2021. This increase was primarily driven by the advancement of our lead programs, research personnel growth to support these programs and expansion of the development organization.

- **G&A Expenses:** General and administrative expenses increased by $5.4 million to $22.1 million during the second quarter of 2022, compared to $16.7 million during the second quarter of 2021. This increase was primarily related to employee-related expenses, including stock-based compensation of $4.5 million.

- **Net Loss:** The Company's net loss was $100.7 million for the second quarter of 2022, compared to $68.8 million during the second quarter of 2021.

**Conference Call to Discuss Second Quarter 2022 Results**

The Company will discuss these results on a conference call today, Thursday, August 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 August 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; the safety, efficacy, success and advancement of its clinical programs for NTLA-2001 for the treatment of ATTR amyloidosis, NTLA-2002 for the treatment of hereditary angioedema ("HAE") and NTLA-5001 for the treatment of acute myeloid leukemia ("AML") pursuant to its clinical trial applications ("CTA") and investigational new drug ("IND") submissions, including the expected timing of data releases, regulatory filings, and the initiation, enrollment, and completion of clinical trials; its ability to successfully pivot to an allogeneic version of its NTLA-5001 program for the treatment of AML, including the expected timing of data releases and regulatory filings; the advancement of development candidates, such as NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency ("AATD")-associated lung disease, NTLA-2003 for AATD-associated liver disease and NTLA-6001 for CD30+ lymphomas, including the success of its IND-enabling studies; its ability to generate data to initiate clinical trials and the timing of CTA and IND submissions; the expansion of its CRISPR/Cas9 technology and related technologies 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 maintain and expand its related intellectual property portfolio; expectations of the potential impact of the coronavirus disease pandemic, including the impact of any variants, on strategy, future operations and timing of its clinical trials; and 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.

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 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 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, | |
|---|---|---|---|---|
| | 2022 | 2021 | 2022 | 2021 |
| Collaboration revenue | $ 14,030 | $ 6,550 | $ 25,282 | $ 12,995 |
| Operating expenses: | | | | |
| Research and development | 90,199 | 58,884 | 223,294 | 98,160 |
| General and administrative | 22,132 | 16,683 | 44,535 | 30,277 |
| Total operating expenses | 112,331 | 75,567 | 267,829 | 128,437 |
| Operating loss | (98,301) | (69,017) | (242,547) | (115,442) |
| Other (expense) income, net: | | | | |
| Interest income | 703 | 211 | 1,243 | 431 |
| Loss from equity method investment | (3,252) | - | (5,997) | - |
| Change in fair value of contingent consideration | 172 | - | (249) | - |
| Total other (expense) income, net | (2,377) | 211 | (5,003) | 431 |
| Net loss | $ (100,678) | $ (68,806) | $ (247,550) | $ (115,011) |
| Net loss per share, basic and diluted | $ (1.33) | $ (1.01) | $ (3.29) | $ (1.70) |
| Weighted average shares outstanding, basic and diluted | 75,823 | 68,164 | 75,282 | 67,675 |

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

| | June 30, 2022 | December 31, 2021 |
|---|---|---|
| Cash, cash equivalents and marketable securities | $ 906,879 | $ 1,086,049 |
| Total assets | 1,110,424 | 1,294,464 |
| Total liabilities | 229,877 | 254,220 |
| Total stockholders' equity | 880,547 | 1,040,244 |

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