



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

November 3, 2022

- Presented interim data from the cardiomyopathy arm of NTLA-2001 Phase 1 study demonstrating deep and sustained mean serum TTR reductions of 93% and 92% at 0.7 mg/kg and 1.0 mg/kg doses, respectively, at day 28; additional data to be presented in late-breaking, oral presentation at AHA on November 5
- Initiated dosing at 55 mg in Part 2 of the Phase 1 study of NTLA-2001 for the treatment of transthyretin (ATTR) amyloidosis
- Presented interim data from the ongoing Phase 1/2 study of NTLA-2002, demonstrating that a single dose of NTLA-2002 resulted in significant, dose-dependent and durable reductions in plasma kallikrein and a 91% reduction in attack rate in the 25 mg cohort through week 16; additional interim data to be presented at ACAAI on November 12
- Expects to initiate the Phase 2 portion of NTLA-2002 study for the treatment of hereditary angioedema (HAE) in 1H 2023
- Continues to advance full-spectrum pipeline and platform innovations, including NTLA-3001 and NTLA-2003 for the treatment of alpha-1 antitrypsin deficiency (AATD) and its proprietary allogeneic technology
- Ended the third quarter of 2022 with strong cash position of \$848.7 million

CAMBRIDGE, Mass., Nov. 03, 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 third quarter ended September 30, 2022.

"During the most recent quarter, Intellia achieved significant clinical milestones across two landmark first-in-human studies," said Intellia President and Chief Executive Officer John Leonard, M.D. "We believe the latest data from our lead *in vivo* program, NTLA-2001, indicate it has the potential to set a new standard for how people living with ATTR amyloidosis are treated for this life-threatening disease. We are rapidly completing the dose-expansion portion of the study and are engaging with regulatory agencies, including in the U.S., as we move closer to a global, pivotal trial. Interim data from our second *in vivo* program, NTLA-2002, demonstrated robust reductions in both plasma kallikrein levels and the rate of swelling attacks in patients with hereditary angioedema. These initial data support the potential for a single dose of NTLA-2002 to permanently prevent the debilitating and potentially fatal HAE attacks that characterize this lifelong genetic disease. We look forward to presenting additional data from both NTLA-2001 and NTLA-2002 later this month."

Dr. Leonard continued, "Together, these interim datasets demonstrate the modularity of our CRISPR-based platform, providing powerful evidence of our ability to apply this technology broadly to address a wide range of diseases. Beyond gene knockout programs, we are advancing our first *in vivo* insertion candidate, NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency, with plans to submit a regulatory filing next year."

### Third 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.
  - **ATTR-CM arm:** Intellia [announced](#) in September positive interim results from the cardiomyopathy arm of the ongoing Phase 1 clinical trial of NTLA-2001. The interim data were from 12 adult patients with ATTR-CM with New York Heart Association (NYHA) Class I – III heart failure. Single doses of 0.7 mg/kg and 1.0 mg/kg of NTLA-2001 were administered via intravenous infusion, and the change from baseline in serum TTR protein concentration was

measured for each patient. These data showed deep and sustained mean serum TTR reductions of 93% and 92% at the 0.7 mg/kg and 1.0 mg/kg doses, respectively, at day 28. At both dose levels, NTLA-2001 was generally well-tolerated. Two of 12 patients reported transient infusion reactions, which was the only observed treatment-related adverse event. One patient in the 0.7 mg/kg dose NYHA Class III cohort experienced a Grade 3 infusion-related reaction, which resolved without clinical consequence. No clinically significant laboratory abnormalities were observed at either dose level. These data support NTLA-2001's potential as a one-time treatment to permanently inactivate the *TTR* gene and reduce the disease-causing protein in people with ATTR-CM.

- Intellia initiated dosing at a 55 mg dose, the fixed dose corresponding to 0.7 mg/kg, in Part 2, the dose-expansion portion of the study. The Company remains on track to complete, by the end of this year, planned enrollment of both arms of the Phase 1 study that will inform the dose selection for subsequent pivotal studies.
- Intellia will be presenting interim clinical data from the cardiomyopathy arm in a Late-Breaking Science session on November 5, 2022, at this year's American Heart Association (AHA) Scientific Sessions, taking place in Chicago, Illinois.

#### 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 [announced](#) in September positive interim results from an ongoing Phase 1/2 clinical study of NTLA-2002, its second *in vivo* genome editing candidate, at the 2022 Bradykinin Symposium held in Berlin, Germany. Administration of single doses of NTLA-2002 led to dose-dependent reductions in plasma kallikrein, with mean reductions of 65% and 92% in the 25 mg (n=3) and 75 mg (n=3) dose cohorts, respectively, by week eight. In addition to plasma kallikrein levels, HAE attack rates are also being measured in the study, with the first analysis occurring at the end of the pre-specified 16-week primary observation period. A single dose of 25 mg of NTLA-2002 resulted in a 91% mean reduction in HAE attacks through the 16-week observation period. Additionally, two of the three patients have not had a single HAE attack since treatment, and all three patients have been attack-free since week 10 (based on follow-up through weeks 24 – 32). Patients in the 75 mg cohort had not completed the primary 16-week observation period. At both dose levels, NTLA-2002 was generally well-tolerated, and the majority of adverse events were mild in severity.
  - Intellia will be presenting additional data at the American College of Allergy, Asthma & Immunology (ACAAI) Annual Scientific Meeting, taking place November 10 – 14 in Louisville, Kentucky. The new presentation is expected to include interim safety and kallikrein reduction data from the 50 mg dose cohort, and additional safety, kallikrein reduction and attack rate data from the 25 mg and 75 mg dose cohorts from the dose-escalation portion of the study.
  - Intellia expects to select up to two doses to further evaluate in the Phase 2, placebo-controlled, dose-expansion portion of the study, slated to begin in the first half of 2023. Intellia anticipates expanding country and site participation, including U.S. clinical sites, as part of the Phase 2 study.

#### 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

### CD30+ Lymphomas

- **NTLA-6001:** NTLA-6001 is an 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 wholly owned 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.
- **Allogeneic Platform:** In October, at the European Society of Gene & Cell Therapy 29<sup>th</sup> Congress, Intellia highlighted its proprietary allogeneic solution to create engineered T cells with high anti-tumor activity, which have the potential to be uniquely capable of persisting in the patient to maintain durable responses. Notably, a novel combination of gene edits, including knockout of HLA Class II and HLA-A while retaining HLA-B and HLA-C proteins, yielded T cells capable of avoiding rejection by host T and natural killer (NK) cells in preclinical models. By only matching for HLA-B and HLA-C with homozygous donors for a 2/2 match, Intellia's approach allows for a simplified HLA matching strategy between healthy donor T cells and recipient patients. Development of such an "off-the-shelf" therapy aims to address the majority of the patient population with only a small set of donors. Intellia's allogeneic platform is being deployed for investigational TCR-T and CAR-T cell therapies.

## Upcoming Events

The Company will participate in the following events during the fourth quarter of 2022:

- American Heart Association Scientific Sessions 2022, November 5, Chicago
- American College of Allergy, Asthma & Immunology 2022 Annual Scientific Meeting, November 12, Louisville
- Piper Sandler 34<sup>th</sup> Annual Healthcare Conference, November 29, New York

## Upcoming Milestones

The Company has set forth the following for pipeline progression:

### In Vivo

- NTLA-2001 for ATTR amyloidosis:
  - Present additional interim data from ATTR-CM arm of Phase 1 study at AHA
  - Complete planned enrollment of Phase 1 study for both ATTRv-PN and ATTR-CM subjects by the end of this year
- NTLA-2002 for HAE:
  - Present additional interim data from Phase 1/2 study at ACAAI
  - Initiate Phase 2 portion of the study in 1H 2023
- 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

## Third Quarter 2022 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$848.7 million as of September 30, 2022, compared to \$1.1 billion as of December 31, 2021. The decrease was driven by cash used to fund operations of approximately \$276.2 million as well as the acquisition of Rewrite for \$45.0 million. The decrease was offset in part by \$62.1 million in net equity proceeds raised from the Company's "At the Market" (ATM) agreement and \$15.1 million in proceeds from employee-based stock plans. Subsequent to the end of the third quarter, through October 27, 2022, the Company increased its cash position by approximately \$115 million. This included an additional \$107.2 million in net equity proceeds raised from its ATM agreement and an additional \$8.3 million received for ATM transactions with trade dates in September that were settled in October.

- **Collaboration Revenue:** Collaboration revenue increased by approximately \$6.1 million to \$13.3 million during the third quarter of 2022, compared to \$7.2 million during the third quarter of 2021. The increase was primarily driven by our collaborations with AvenCell and Kyverna.
- **R&D Expenses:** Research and development expenses increased by \$36.2 million to \$96.7 million during the third quarter of 2022, compared to \$60.5 million during the third quarter of 2021. This increase was primarily driven by the advancement of our lead programs and personnel growth to support these programs.
- **G&A Expenses:** General and administrative expenses increased by \$3.4 million to \$22.1 million during the third quarter of 2022, compared to \$18.7 million during the third quarter of 2021. This increase was primarily related to employee-related expenses, including stock-based compensation of \$2.4 million.
- **Net Loss:** The Company's net loss was \$113.2 million for the third quarter of 2022, compared to \$71.6 million during the third quarter of 2021.

#### Conference Call to Discuss Third Quarter 2022 Results

The Company will discuss these results on a conference call today, Thursday, November 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 [intelliadx.com](https://intelliadx.com), beginning on November 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 [intelliadx.com](https://intelliadx.com). Follow us on Twitter [@intelliadx](https://twitter.com/intelliadx).

#### 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 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 completion of planned enrollment of Phase 1 study for NTLA-2001; its ability to select a dose to advance to a pivotal study for NTLA-2001; 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, including the submission of an IND or IND-equivalent for NTLA-3001 in 2023; 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 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; risks related to 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 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 September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Collaboration revenue	\$ 13,266	\$ 7,204	\$ 38,548	\$ 20,199
Operating expenses:				
Research and development	96,651	60,486	319,945	158,646
General and administrative	22,145	18,711	66,680	48,988
Total operating expenses	118,796	79,197	386,625	207,634
Operating loss	(105,530)	(71,993)	(348,077)	(187,435)
Other (expense) income, net:				
Interest income	1,945	349	3,188	780
Loss from equity method investment	(1,834)	-	(7,831)	-
Change in fair value of contingent consideration	(7,810)	-	(8,059)	-
Total other (expense) income, net	(7,699)	349	(12,702)	780
Net loss	\$ (113,229)	\$ (71,644)	\$ (360,779)	\$ (186,655)
Net loss per share, basic and diluted	\$ (1.49)	\$ (0.97)	\$ (4.78)	\$ (2.68)
Weighted average shares outstanding, basic and diluted	76,047	73,706	75,543	69,720

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

	September 30, 2022	December 31, 2021
Cash, cash equivalents and marketable securities	\$ 848,727	\$ 1,086,049
Total assets	1,084,458	1,294,464
Total liabilities	259,507	254,220
Total stockholders' equity	824,951	1,040,244

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Source: Intellia Therapeutics, Inc.