

Intellia Therapeutics Announces Third Quarter 2021 Financial Results and Highlights Recent Company Progress

November 4, 2021

- Initiated dosing of Cohort 4 in the ongoing Phase 1 study of NTLA-2001; data update from completed dose-escalation (Part 1) and initiation of dose-expansion (Part 2) expected in Q1 2022
- Accelerated plans to evaluate NTLA-2001 for transthyretin amyloidosis with cardiomyopathy (ATTR-CM)
- On track to initiate first-in-human studies of NTLA-2002 for hereditary angioedema (HAE) and NTLA-5001 for acute myeloid leukemia (AML) by year-end
- Announces nomination of NTLA-3001, its first gene insertion development candidate, for alpha-1 antitrypsin deficiency (AATD); concurrently, Regeneron and Intellia have selected a Factor 9 gene insertion development candidate to advance for hemophilia B (Hem B)
- Presented new data on its proprietary and differentiated allogeneic cell engineering platform and ex vivo LNP-based delivery of CRISPR/Cas9 to T cells at ESGCT Annual Congress
- Ended the quarter with strong cash position of \$1.1 billion

CAMBRIDGE, Mass., Nov. 04, 2021 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, today reported financial results for the third quarter ended September 30, 2021, and recent operational highlights.

"Intellia continues to make important progress toward advancing its full-spectrum genome editing pipeline. Today, we are pleased to share that we remain highly encouraged by the safety profile, consistency of TTR reduction and ongoing effect of NTLA-2001 in the Phase 1 study. We believe we are closing in on identifying the recommended therapeutic dose for NTLA-2001 in patients with ATTR amyloidosis with polyneuropathy for further evaluation in Part 2, a single-dose expansion cohort. Additionally, while it has long been our plan to develop NTLA-2001 for all forms of ATTR amyloidosis, we have decided to accelerate the evaluation of NTLA-2001 in patients with ATTR amyloidosis whose primary clinical manifestation is cardiomyopathy. Based on the strength of our interim dataset, we are now seeking regulatory feedback for inclusion of the ATTR-CM patient population in our current Phase 1 study. We look forward to sharing updates from this program in Q1 2022," said Intellia President and Chief Executive Officer John Leonard, M.D. "Beyond our lead program, we remain focused on advancing NTLA-2002 for HAE and NTLA-5001 for AML. We received regulatory clearance for both programs to initiate first-in-human studies and we expect those studies to begin later this year. Finally, we nominated two gene insertion development candidates – NTLA-3001, our wholly owned AATD program, and a Factor 9 gene insertion candidate for Hem B in collaboration with Regeneron. Our rapidly expanding pipeline is yet one more example of the benefits of our modular platform in generating novel and potentially curative treatment options for the patients we aim to serve."

Third Quarter 2021 and Recent Operational Highlights

In Vivo Program Updates

- **NTLA-2001 for ATTR amyloidosis:** NTLA-2001 is the first systemically delivered CRISPR-based therapy to be dosed in a patient and has the potential to be a curative treatment for transthyretin (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, lifelong reduction in transthyretin (TTR) protein after a single dose. NTLA-2001 is part of a co-development/co-promotion agreement between Intellia, the lead party for this program, and Regeneron Pharmaceuticals, Inc. (Regeneron).
 - NTLA-2001 is completing the dose-escalation portion of the Phase 1 study to determine the recommended dose for evaluation in Part 2 of the study, a single-dose expansion cohort. During the third quarter, to more fully elucidate the dose-response relationship, Intellia began dosing of subjects in Cohort 4, evaluating NTLA-2001 in patients with hereditary transthyretin amyloidosis with polyneuropathy (ATTRv-PN) at the 0.7 mg/kg dose level.
 - Intellia accelerated the evaluation of NTLA-2001 for the treatment of patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM). The Company is in discussions with regulatory authorities on a protocol amendment to expand the Phase 1 trial population to include patients with ATTR-CM.
 - Intellia now plans to present interim data from Part 1, the single-ascending dose portion, and to initiate Part 2, a single-cohort expansion, in the first quarter of 2022. Data to be presented at a company-sponsored event will be from all four cohorts in Part 1 and include safety and serum TTR knockdown for Cohorts 3 and 4, as well as an early look at durability across all cohorts.
 - In October, Intellia [announced](#) that NTLA-2001 received Orphan Drug Designation (ODD) from the U.S. Food and Drug Administration (FDA) for the treatment of ATTR amyloidosis.
- **NTLA-2002 for HAE:** 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 hereditary angioedema (HAE). This approach aims to prevent attacks for people living with HAE by providing continuous suppression of plasma kallikrein activity following a single dose and to eliminate the significant treatment burden associated with currently available HAE therapies.
 - In October, Intellia [announced](#) the authorization of its Clinical Trial Application (CTA) by the New Zealand Medicines and Medical Devices Safety Authority (MEDSAFE) to initiate a Phase 1/2 study evaluating NTLA-2002. Additionally, a CTA has been subsequently authorized by the United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA) for the first-in-human study of NTLA-2002.
 - Intellia intends to enroll the first patient in the first-in-human study by year-end. The Phase 1/2 study will evaluate safety, tolerability, pharmacokinetics and pharmacodynamics of NTLA-2002 in adults with Type I or Type II HAE, and will leverage insights gained from the development of NTLA-2001 enabling this study to begin evaluating NTLA-2002 at a higher initial dose.
- **NTLA-3001 for AATD-associated lung disease:** NTLA-3001 is Intellia's first and wholly owned CRISPR/Cas9-mediated *in vivo* targeted gene insertion development candidate. It is designed with the aim to precisely insert a healthy copy of the *SERPINA1* gene, which encodes the alpha-1 antitrypsin (A1AT) protein, with the potential to permanently restore expression of functional A1AT protein to therapeutic levels after a single dose. This approach seeks to address alpha-1 antitrypsin deficiency (AATD)-associated lung disease and eliminate the need for sub-optimal weekly IV infusions of A1AT augmentation therapy or transplant in severe cases.
 - Today, Intellia announced the nomination of a new development candidate, NTLA-3001 for treatment of AATD. At the 29th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT), Intellia [presented](#) data showing that insertion of a healthy form of the *SERPINA1* gene led to normal human A1AT levels in non-human primates (NHPs) which were durable through 52 weeks in an ongoing study. The Company is advancing towards Investigational New Drug (IND)-enabling activities for this program and continues to explore additional editing strategies for AATD.

Ex Vivo Program Updates

- **NTLA-5001 for AML:** NTLA-5001 is an 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 acute myeloid leukemia (AML).
 - In September, Intellia [announced](#) that the U.S. FDA accepted its IND application for NTLA-5001.
 - Intellia intends to initiate patient screening by year-end for a Phase 1/2a study evaluating NTLA-5001 in adults with persistent or recurrent AML who have previously received first-line therapy.

Research and Corporate Updates

- **Modular Platform and Pipeline Expansion:** Intellia is advancing its modular platform technologies to broaden the *in vivo* and *ex vivo* applications of genome editing. This includes progressing capabilities for innovative CRISPR/Cas9-mediated targeted transgene insertion, *in vivo* editing in multiple tissue types and an allogeneic approach for the development of "off-the-shelf" T cell therapies. These efforts support new therapeutic candidates and strategic business development partnerships for advancing treatments for genetic diseases, cancers and autoimmune diseases.
 - **ESGCT Annual Congress:** In October, Intellia [presented](#) new preclinical data demonstrating key platform capabilities.
 - Intellia shared the first data highlighting its proprietary allogeneic cell engineering platform, demonstrating it can prevent immune rejection of allogeneic T cells for application in TCR-T and CAR-T cell therapy. Intellia's proprietary approach leverages a novel combination of sequential gene edits to shield the engineered cell therapy from host T and NK cell attack. It does not rely on long-term, aggressive immune suppression of patients or the knockout of a protein required for HLA class I expression, which are approaches currently employed by others to address the challenge of immune rejection of the allogeneic cell product. Intellia intends to nominate its first allogeneic cell therapy development candidate by the first half of 2022.
 - Intellia shared new data on its proprietary cell engineering process, demonstrating LNP-based delivery of CRISPR/Cas9 *ex vivo* allows for sequential editing of T cells with high efficiency, faster expansion and minimal translocations as compared to electroporation. The data support the ability of this platform to be used for a variety of targeting modalities, including CAR and TCRs, and to support both autologous and allogeneic T cell candidates. This LNP-based approach is being used for NTLA-5001.
 - **Collaboration Updates:**
 - In the third quarter, Intellia and Regeneron nominated a Factor 9 (F9) gene insertion development candidate for its Hemophilia B (Hem B) program, leveraging their jointly developed targeted transgene insertion capabilities to insert F9. This candidate is part of a co-development/co-funding agreement between Intellia and Regeneron, the lead party for this program. F9 is a gene that encodes for Factor IX (FIX), a blood-clotting protein that is missing or defective in Hem B patients. In preclinical studies, the companies demonstrated the first CRISPR/Cas9-mediated targeted transgene insertion in the liver of NHPs, which resulted in circulating FIX levels at or above those found in normal human plasma.
 - In October, the Company [announced](#) a strategic collaboration with SparingVision to develop novel genomic medicines utilizing Intellia's proprietary CRISPR/Cas9 technology for the treatment of ocular diseases. As part of the collaboration, Intellia will receive an equity stake in SparingVision, option for exclusive U.S. commercialization rights for product candidates arising from two of three collaboration targets, and eligibility for development and commercial milestone payments as well as royalties on potential future sales of products arising from the collaboration. The companies will additionally research and develop novel self-inactivating AAV vectors and LNP-based approaches to address delivery of CRISPR/Cas9 genome reagents to the retina.

- In July, the Company completed the formation of a new universal CAR-T cell therapy company, which Intellia launched in collaboration with Blackstone Life Sciences and Cellex Cell Professionals GmbH. The new company will combine clinical-stage universal CAR-T platforms with Intellia's differentiated allogeneic cell engineering platform to develop therapies for immuno-oncology and autoimmune diseases.

Upcoming Events

The Company will participate in the following events during the fourth quarter of 2021:

- Barclays Gene Editing & Gene Therapy Summit, November 15, Virtual
- Evercore ISI 4th Annual HealthCONx Conference, December 1, Virtual
- 63rd ASH Annual Meeting & Exposition, December 11-14, Atlanta, GA and Virtual

Upcoming Milestones

The Company has set forth the following for pipeline progression:

- ATTR:
 - Report additional interim clinical data from Phase 1 study of NTLA-2001 in Q1 2022
 - Initiate Part 2, a single-dose expansion cohort, of Phase 1 study of NTLA-2001 in Q1 2022
- HAE: Initiate enrollment in the first-in-human study of NTLA-2002 by year-end
- AML: Initiate patient screening in the first-in-human study of NTLA-5001 by year-end
- Pipeline Expansion: Nominate the Company's first allogeneic development candidate by 1H 2022

Third Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$1,148.7 million as of September 30, 2021, compared to \$597.4 million as of December 31, 2020. The increase was driven by net proceeds of \$648.3 million from a follow-on offering in Q3 2021, \$45.3 million of net proceeds from the Company's "At the Market" (ATM) agreement, \$40.8 million in proceeds from employee-based stock plans, and \$4.2 million of funding for a cost-sharing agreement received from Regeneron. These increases were offset in part by cash used to fund operations of approximately \$187.3 million.
- **Collaboration Revenue:** Collaboration revenue decreased by \$15.0 million to \$7.2 million during the third quarter of 2021, compared to \$22.2 million during the third quarter of 2020. The decrease in collaboration revenue in 2021 was primarily driven by \$15.3 million related to the transfer of control of the license to develop the Factor VIII target for hemophilia A that was recorded during Q3 2020.
- **R&D Expenses:** Research and development expenses increased by \$20.7 million to \$60.5 million during the third quarter of 2021, compared to \$39.8 million during the third quarter of 2020. 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 \$8.1 million to \$18.7 million during the third quarter of 2021, compared to \$10.6 million during the third quarter of 2020. This increase was primarily related to employee related expenses, including stock-based compensation of \$3.8 million.
- **Net Loss:** The Company's net loss was \$71.6 million for the third quarter of 2021, compared to \$27.8 million during the third quarter of 2020.

Conference Call to Discuss Third Quarter Earnings

The Company will discuss these results on a conference call today, Thursday, November 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 www.intelliatrix.com, beginning on November 4 at 12 p.m. ET.

About Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics using CRISPR/Cas9 technology. To fully realize the transformative potential of CRISPR/Cas9, 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 CRISPR/Cas9 to create new classes of genetic medicine. Learn more at intelliatrix.com. Follow us on Twitter [@intelliatrix](https://twitter.com/intelliatrix).

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 regarding its: ability to complete clinical studies for NTLA-2001 for the treatment of transthyretin ("ATTR") amyloidosis pursuant to its clinical trial applications ("CTA"), including submitting additional regulatory applications in other countries; clinical data from the ongoing single-ascending dose portion of the Phase 1 study evaluating NTLA-2001; expectations on regulatory approval to expand the Phase 1 trial population to include patients with ATTR with cardiomyopathy ("ATTR-CM"); expectations of being able to initiate clinical trials for NTLA-2002 for the treatment of hereditary angioedema ("HAE") and NTLA-5001 for the treatment of acute myeloid leukemia ("AML"); ability to enroll patients in its Phase 1/2 study for NTLA-2002 for the treatment of hereditary angioedema ("HAE") in New Zealand and the United Kingdom; its IND-enabling activities for NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency; nomination and continued preclinical development of new development candidates; expectations for a new collaboration with Regeneron for a Factor 9 ("F9") development candidate for its Hemophilia B program; expectations for a new collaboration with SpringVision for the treatment of ocular diseases; advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, as well as its ability to maintain and expand its related intellectual property portfolio; statements regarding the timing of regulatory filings regarding its development programs; plans to attend and present data at scientific conferences later this year; and our expectations regarding our use of capital and our ability to fund operations beyond the next 24 months.

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 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; the risk that any one or more of Intellia's product candidates will not be successfully developed and commercialized; the risk that the results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies; the risk that regulatory approval to expand the Phase 1 patient population to include patients with ATTR-CM will be denied; risks related to the COVID-19 pandemic including the effects of the Delta variant; and the risk that Intellia's collaborations with Regeneron, SpringVision or its other collaborations will not continue or will not be 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,	
	2021	2020	2021	2020
Collaboration revenue	\$ 7,204	\$ 22,220	\$ 20,199	\$ 51,399
Operating expenses:				
Research and development	60,486	39,756	158,646	112,177
General and administrative	18,711	10,566	48,988	33,406
Total operating expenses	79,197	50,322	207,634	145,583
Operating loss	(71,993)	(28,102)	(187,435)	(94,184)
Interest income	349	262	780	2,145
Net loss	\$ (71,644)	\$ (27,840)	\$ (186,655)	\$ (92,039)
Net loss per share, basic and diluted	\$ (0.97)	\$ (0.47)	\$ (2.68)	\$ (1.70)
Weighted average shares outstanding, basic and diluted	73,706	58,754	69,720	54,218

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

	September 30, 2021		December 31, 2020
Cash, cash equivalents and marketable securities	\$ 1,148,718	\$	597,371
Total assets	1,334,961		676,322
Total liabilities	227,863		149,250
Total stockholders' equity	1,107,098		527,072

Intellia Contacts:

Investors:

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

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

Media:

Matt Crenson
Ten Bridge Communications
+1-917-640-7930
media@intelliatx.com
mcrenson@tenbridgecommunications.com



Source: Intellia Therapeutics, Inc.