Intellia Therapeutics Announces Third Quarter 2020 Financial Results

November 5, 2020

- *On track to dose first patient by year-end with NTLA-2001 for the treatment of transthyretin amyloidosis (ATTR), following regulatory authorization to initiate Phase 1 clinical trial*
- *Anticipates submitting an IND or IND-equivalent for lead TCR-T cell therapy, NTLA-5001 for the treatment of acute myeloid leukemia (AML), in 1H 2021*
- *Expects to submit an IND or IND-equivalent for NTLA-2002 for the treatment of hereditary angioedema (HAE) in 2H 2021*
- *Ended quarter with a strong cash position of $407.9 million*

CAMBRIDGE, Mass., Nov. 05, 2020 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, today reported operational highlights and financial results for the third quarter ended September 30, 2020.

"We are very pleased with the recent regulatory authorization to begin our Phase 1 study of NTLA-2001, which keeps us on track to dose our first patient by year-end. This is an important step toward improving the lives of ATTR patients with a potentially curative treatment, and marks our transition into a clinical-stage company. Further, advancing NTLA-2001 is a major milestone for the field of genome editing, as this is the first clinical trial of a systemically delivered CRISPR/Cas9-based therapy," said Intellia President and Chief Executive Officer John Leonard, M.D. "In parallel, we are progressing NTLA-5001 and NTLA-2002 for the treatment of AML and HAE, respectively, each to an IND or equivalent regulatory submission next year. We also continue to develop innovative capabilities across our platform, based on the Nobel Prize-winning CRISPR/Cas9 technology, to generate our next wave of therapeutic candidates."

Third Quarter 2020 and Recent Operational Highlights

- **ATTR Program:** Intellia recently announced the authorization of its Clinical Trial Application (CTA) by the United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA) to initiate a first-in-human clinical trial of NTLA-2001, an investigational therapy in development for the treatment of all clinical manifestations of ATTR. By applying the Company's *in vivo* liver gene knockout technology, NTLA-2001 allows for the possibility of lifelong transthyretin (TTR) protein reduction after a single course of treatment. Intellia's first-in-human study will evaluate NTLA-2001 in adults with hereditary ATTR with polyneuropathy (hATTR-PN). The Phase 1 study will be a two-part, open label, multi-center study to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of NTLA-2001, which will include the measurement of serum TTR levels following a single intravenous infusion. Intellia is on track to dose its first patient by the end of 2020, subject to the impact of the COVID-19 pandemic, and is submitting additional regulatory applications in other countries as part of its ongoing, global development strategy. Once safety and an optimal dose have been determined in the first-in-human study, Intellia intends to further evaluate NTLA-2001 in a broader ATTR patient population of both polyneuropathy and cardiomyopathy patients. NTLA-2001 is part of a co-development/co-promotion agreement between Intellia, the lead development and commercialization party, and Regeneron Pharmaceuticals, Inc. (Regeneron).

- **AML Program:** NTLA-5001 is a wholly owned, T cell receptor (TCR)-T cell therapy development candidate targeting the Wilms' Tumor 1 (WT1) antigen for the treatment of AML. The Company seeks to develop NTLA-5001 as a broadly applicable treatment for AML patients, regardless of the mutational subtypes of the cancer. Intellia continues to advance Investigational New Drug application (IND)-enabling activities and remains on track to submit an IND or IND-equivalent for NTLA-5001 in the first half of 2021. At the upcoming 62nd American Society of Hematology (ASH) Annual Meeting, taking place virtually from December 5 – 8, 2020, the Company will present new preclinical results in support of NTLA-5001, showing high anti-tumor activity of its lead WT1-directed TCR-T therapy in a mouse model of AML. The preclinical data will also highlight the advantages of its proprietary T cell engineering process to produce multiple, highly efficient sequential edits in T cells that have superior function and minimal translocations compared to results from standard T cell engineering approaches. Additional efforts are underway to evaluate the potential use of NTLA-5001 to treat WT1-positive solid tumors.

- **HAE Program:** NTLA-2002 is a wholly owned, *in vivo* development candidate for the treatment of HAE. Today, Intellia announced results from its completed non-human primate (NHP) study of its lead lipid nanoparticle (LNP) formulation for NTLA-2002. Following a single dose, the knockout of the *prekallikrein B1 (KLKB1)* gene resulted in a year-long

therapeutically relevant reduction of serum kallikrein protein levels and activity. Building on Intellia's modular LNP delivery system, NTLA-2002 is designed to knock out the *KLKB1* gene in the liver after a single course of treatment. This approach is expected to prevent improperly regulated bradykinin production and therefore reduce HAE attacks. During the third quarter, the Company initiated Good Laboratory Practices (GLP) toxicology studies in preparation for an IND or IND-equivalent submission for NTLA-2002, which remains on track for the second half of 2021.

- **Modular Platform:** Intellia continues to make significant progress across its platform technologies, broadening the *in vivo* and *ex vivo* application of genome editing. This includes developing innovative CRISPR/Cas9-mediated targeted transgene insertion and allogeneic cell solutions. At the 16th Annual Meeting of the Oligonucleotide Therapeutics Society, held September 27-30, 2020, Intellia presented new data highlighting the potential to develop single-course therapies that may have a lifelong effect for a variety of genetic diseases. The data showed the persistence of both *in vivo* knockout and insertion CRISPR/Cas9 edits and corresponding durability of effect following a partial hepatectomy (PHx) and liver regrowth in a murine model. Unlike traditional gene therapy, for which a significant loss (over 80%) in transgene expression was observed in the insertion PHx model, Intellia's targeted gene insertion approach yielded durable edits, with no significant loss in expression in the same model. Intellia and Regeneron are co-developing potential hemophilia A and B CRISPR/Cas9-based treatments using their jointly developed insertion capabilities. Intellia is also continuing to develop its proprietary platform to advance its wholly-owned programs.

- **Board of Directors:** In October 2020, Intellia appointed John F. Crowley to its Board of Directors. Mr. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, is a well-established leader in biotech and pharmaceuticals and a visionary advocate for the advancement of treatments for people living with rare diseases.

- **Scientific Co-Founder Awarded Nobel Prize:** Jennifer Doudna, Ph.D., one of Intellia's scientific co-founders, was awarded the 2020 Nobel Prize in Chemistry for inventing the revolutionary CRISPR/Cas9 genome editing technology. Dr. Doudna shares the award with her research collaborator, Dr. Emmanuelle Charpentier.

Upcoming Events

The Company will participate in the following events during the fourth quarter of 2020:

- Credit Suisse Healthcare Conference, November 11, Virtual
- Barclays Gene Editing and Therapy Summit, November 16, Virtual
- 62nd ASH Annual Meeting, December 5-8, Virtual

Upcoming Milestones

The Company has set forth the following for pipeline progression:

- ATTR: Dose first patient in Phase 1 study by year-end
- AML: Submit an IND or IND-equivalent for NTLA-5001 in 1H 2021
- HAE: Submit an IND or IND-equivalent for NTLA-2002 in 2H 2021

Third Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $407.9 million as of September 30, 2020, compared to $284.5 million as of December 31, 2019. The increase was driven by net proceeds of $107.7 million from the June follow-on public offering, $100.0 million upfront payment from the Regeneron collaboration expansion, which included a $30.0 million equity investment, $14.7 million of net equity proceeds raised from the Company's "At the Market" (ATM) agreement, $18.2 million of funding received under the Regeneron and Novartis collaborations and $2.7 million in proceeds from employee-based stock plans. These increases were offset in part by cash used to fund operations of approximately $119.8 million.

- **Collaboration Revenue:** Collaboration revenue increased by $11.6 million to $22.2 million during the third quarter of 2020, compared to $10.6 million during the third quarter of 2019. The increase was mainly driven by a $15.3 million amount recognized for the transfer of control of the license to develop the Factor VIII target for hemophilia A associated with the extension of the Regeneron collaboration.

- **R&D Expenses:** Research and development expenses increased by approximately $12.2 million to $39.8 million during the third quarter of 2020, compared to $27.5 million during the third quarter of 2019. This increase was primarily driven by the advancement of our lead programs, research personnel growth to support these programs, and the expansion of the

development organization.

- **G&A Expenses:** General and administrative expenses increased by approximately $2.1 million to $10.6 million during the third quarter of 2020, compared to $8.4 million during the third quarter of 2019. This increase was primarily related to employee related expenses, including stock-based compensation, of $2.0 million.

- **Net Loss:** The Company's net loss was $27.8 million for the third quarter of 2020, compared to $23.6 million during the third quarter of 2019.

Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of September 30, 2020 will enable the Company to fund its anticipated operating expenses and capital expenditure requirements for at least the next 24 months. This expectation excludes any strategic use of capital not currently in the Company's base-case planning assumptions.

Conference Call to Discuss Third Quarter 2020 Earnings

The Company will discuss these results on a conference call today, November 5, 2020, at 8 a.m. ET.

To join the call:

- U.S. callers should dial 1-877-317-6789 and international callers should dial 1-412-317-6789, approximately five minutes before the call.
- All participants should ask to be connected to the Intellia Therapeutics conference 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.intelliatx.com, beginning on November 5, 2020 at 12 p.m. ET.

About Intellia Therapeutics
Intellia Therapeutics is a leading genome editing company, focused on the development of proprietary, potentially curative therapeutics using the CRISPR/Cas9 system. Intellia believes the CRISPR/Cas9 technology has the potential to transform medicine by both producing therapeutics that permanently edit and/or correct disease-associated genes in the human body with a single treatment course, and creating enhanced engineered cells that can treat oncological and immunological diseases. Intellia's combination of deep scientific, technical and clinical development experience, along with its leading intellectual property portfolio, puts it in a unique position to unlock broad therapeutic applications of the CRISPR/Cas9 technology and create new classes of therapeutic products. Learn more about Intellia Therapeutics and CRISPR/Cas9 at intelliatx.com and follow us on Twitter @intelliatweets.

Forward-Looking Statements
This press release contains "forward-looking statements" of Intellia Therapeutics, Inc. ("Intellia", "we" or "our") 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: being able to initiate clinical studies for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR") pursuant to its clinical trial applications ("CTA"), including dosing of a first patient by the end of 2020, and submitting similar regulatory applications in other countries; plans to submit an investigational new drug ("IND") application or similar clinical trial application for NTLA-5001, its first T cell receptor ("TCR")-directed engineered cell therapy development candidate for its acute myeloid leukemia ("AML") program in the first half of 2021; plans to submit an IND or similar clinical trial application for its hereditary angioedema ("HAE") program in the second half of 2021; plans to advance and complete preclinical studies, including non-human primate studies for its HAE and other programs, and other animal studies supporting other in vivo and ex vivo programs, including its AML program; development of a proprietary LNP/AAV hybrid delivery system, as well as its modular platform to advance its complex genome editing capabilities, such as gene insertion; further development of its proprietary cell engineering process for multiple sequential editing; presentation of additional data at upcoming scientific conferences, and other preclinical data in 2020; 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; ability to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies, including those in its ATTR, AML, and HAE programs, in any future studies, including human clinical trials; expectations of the potential impact of the coronavirus disease 2019 pandemic on strategy, future operations and timing of its clinical trials or IND submissions; ability to develop other in vivo or ex vivo cell therapeutics of all types, and those targeting WT1 in AML in particular, using CRISPR/Cas9 technology; ability to optimize the impact of its collaborations on its development programs, including but not limited to its collaborations with Novartis Institutes for BioMedical Research, Inc. ("Novartis") or Regeneron Pharmaceuticals, Inc. ("Regeneron"), including its co-development programs for hemophilia A and hemophilia B; statements regarding the timing of regulatory filings and clinical trial execution, including dosing of patients, regarding its development programs; the potential commercial opportunities, including value and market, for our product candidates; our expectations regarding our use of capital and other financial results during 2020; and our ability to fund operations for at least 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 regulatory agencies' evaluation of regulatory filings and other information related to its product candidates; 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, including those that are co-developed, 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; and the risk that Intellia's collaborations with Novartis or Regeneron or its other ex vivo 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 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 (“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,	
	2020	2019	2020	2019
Collaboration revenue	$ 22,220	$ 10,616	$ 51,399	$ 32,167
Operating expenses:				
Research and development	39,756	27,513	112,177	76,682
General and administrative	10,566	8,431	33,406	32,082
Total operating expenses	50,322	35,944	145,583	108,764
Operating loss	(28,102)	(25,328)	(94,184)	(76,597)
Interest income	262	1,694	2,145	5,340
Net loss	$ (27,840)	$ (23,634)	$ (92,039)	$ (71,257)
Net loss per share, basic and diluted	$ (0.47)	$ (0.49)	$ (1.70)	$ (1.53)
Weighted average shares outstanding, basic and diluted	58,754	48,554	54,218	46,547

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

	September 30, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 407,947	$ 284,472
Total assets	458,555	334,280
Total liabilities	128,935	64,399
Total stockholders' equity	329,620	269,881

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Source: Intellia Therapeutics, Inc.