Intellia Therapeutics Announces Fourth Quarter and Full-Year 2020 Financial Results

February 25, 2021

- *Expects to report initial data from Phase 1 study of NTLA-2001, a potentially curative single-course therapy for transthyretin amyloidosis (ATTR), in 2021*
- *On track to submit an IND or IND-equivalent in mid-2021 for NTLA-5001 for the treatment of acute myeloid leukemia (AML)*
- *Intends to submit an IND or IND-equivalent in 2H 2021 for NTLA-2002 for the treatment of hereditary angioedema (HAE)*
- *Plans to nominate at least one new development candidate in 2021 from broad research efforts and continued platform innovation*
- *Ended 2020 with strong cash position of $597.4 million*

CAMBRIDGE, Mass., Feb. 25, 2021 (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 fourth quarter and year ended December 31, 2020.

“Intellia’s achievements in 2020 reflect important progress on both our full-spectrum strategy and our mission to deliver curative genome editing treatments for people with severe diseases. Dosing our first patient with NTLA-2001, the first-ever systemically delivered CRISPR-based therapy, was a major milestone for our team, as we completed our transition to a clinical-stage company in the fourth quarter,” said Intellia President and Chief Executive Officer, John Leonard, M.D. “Looking ahead, we are focused on our three core priorities for 2021: clinical validation of our approach with NTLA-2001, advancement and expansion of our *in vivo* and *ex vivo* pipeline, and continued platform innovation. We have made steady progress in our global Phase 1 study of NTLA-2001 and look forward to sharing our first clinical data this year. Additionally, we are on track to submit first-in-human regulatory applications to begin clinical studies of NTLA-5001 for AML and NTLA-2002 for HAE, and we plan to nominate at least one new development candidate from our research portfolio.”

Fourth Quarter 2020 and Recent Operational Highlights

- **NTLA-2001 for ATTR:** NTLA-2001 is the first systemically delivered CRISPR-based therapy dosed in a patient and could potentially be the first curative treatment for ATTR. By applying the Company’s *in vivo* lipid nanoparticle (LNP) delivery technology, NTLA-2001 offers the possibility of halting and reversing the disease with potent, lifelong transthyretin (TTR) protein reduction after a single course of treatment. NTLA-2001 is part of a co-development/co-promotion agreement between Intellia, the lead party, and Regeneron Pharmaceuticals, Inc. (Regeneron).
    - The Company anticipates reporting interim clinical data from the Phase 1 study this year. These results are expected to characterize the emerging safety and activity profile of NTLA-2001 at the initial dose levels.
    - In November, Intellia dosed the first patient in its global Phase 1 study evaluating NTLA-2001 in adults with hereditary ATTR with polyneuropathy (hATTR-PN). The Company continues to enroll patients in the study and is submitting additional regulatory applications in other countries as part of its ongoing, global development strategy.
    - Intellia intends to evaluate NTLA-2001 in a broader ATTR population of both polyneuropathy and cardiomyopathy patients following its Phase 1 safety assessment and dose optimization.
- **NTLA-5001 for AML:** NTLA-5001 is a potential best-in-class engineered T cell therapy designed to treat all genetic subtypes of AML. This investigational candidate is an autologous T cell receptor (TCR)-T cell therapy targeting the Wilms’ Tumor 1 (WT1) antigen utilizing Intellia’s proprietary cell engineering process.
    - Intellia plans to submit an Investigational New Drug (IND) application or equivalent regulatory application for NTLA-5001 in mid-2021. This first-in-human trial is expected to evaluate the safety and activity of NTLA-5001 in patients with persistent or recurrent AML who have previously received first-line therapies.
    - NTLA-5001 shows high anti-tumor activity in proof-of-concept mouse models of acute leukemias. The preclinical

data presented at the American Society of Hematology (ASH) Annual Meeting in December highlighted faster expansion and superior function of T cells manufactured by Intellia's proprietary approach, compared to standard T cell engineering approaches currently in use.

- The Company is also evaluating the potential use of NTLA-5001 to treat WT1-positive solid tumors in preclinical studies.

- **NTLA-2002 for HAE:** NTLA-2002 aims to prevent attacks for people living with HAE after a single course of treatment. Intellia is applying its modular LNP delivery system to develop NTLA-2002 to knock out the *KLKB1* gene in the liver to permanently reduce plasma kallikrein activity. This approach is expected to provide continuous suppression of kallikrein activity and eliminate the significant treatment burden associated with currently available therapies for HAE patients.

  - Intellia commenced clinical manufacturing activities to support the Company's plans to submit an IND or equivalent regulatory application in the second half of 2021.

  - The Company is applying insights gained from NTLA-2001 to expedite clinical development of NTLA-2002. The first-in-human trial is expected to evaluate safety, tolerability and activity in patients with HAE.

  - The Company plans to present additional preclinical results in support of NTLA-2002 at the upcoming American Academy of Allergy, Asthma & Immunology (AAAAI) 2021 Annual Meeting, taking place virtually from February 26 – March 1, 2021.

- **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 developing capabilities for innovative CRISPR/Cas9-mediated *in vivo* editing in multiple tissue types, targeted transgene insertion and an allogeneic approach for the development of "off-the-shelf" T cell therapies. These efforts will support new therapeutic candidates for genetic diseases requiring removal and/or restoration of a protein, and next-generation engineered cell therapies for cancers and auto-immune diseases.

  - Intellia plans to nominate at least one additional development candidate in 2021 and expects to present preclinical data at upcoming scientific conferences highlighting research advancements and platform innovations.

  - Intellia demonstrated the modularity of its targeted insertion approach for a second target, in non-human primates, showing insertion of the *SERPINA1* gene produced normal levels of human alpha-1 antitrypsin (AAT) after a single administration. In December, these results were presented at the Alpha-1 Foundation's 20th Gordon L. Snider Critical Issues Workshop: The Promise of Gene-Based Interventions of Alpha-1 Antitrypsin Deficiency. Intellia is advancing multiple genome editing strategies that may treat both lung and liver manifestations of AAT deficiency (AATD), which occur due to mutations in the *SERPINA1* gene.

  - Intellia is advancing preclinical validation of *in vivo* hematopoietic stem cell (HSC) genome editing using the Company's proprietary non-viral delivery systems and CRISPR/Cas9 technology to potentially cure sickle cell disease. This research is supported by a grant from the Bill & Melinda Gates Foundation.

- **Financing:** In December, Intellia closed an underwritten public offering of 5,513,699 shares of common stock, including the exercise in full of the underwriters' option to purchase additional shares, at the public offering price of $36.50 per share. Intellia received aggregate gross proceeds of approximately $201 million, before underwriting discounts and commissions and offering expenses.

Upcoming Events

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

- AAAAI Annual Meeting, February 26–March 1, Virtual
- Keystone Symposium: Precision Engineering of the Genome, Epigenome and Transcriptome, March 8-10, Virtual
- Barclays Capital Global Healthcare Conference, March 8, Virtual
- Oppenheimer's 31st Annual Healthcare Conference, March 16, Virtual
- Cold Spring Harbor Laboratories meeting on Nucleic Acid Therapies, March 24 - 26, Virtual

Upcoming Milestones

The Company has set forth the following for pipeline progression:

- ATTR: Report initial clinical data from Phase 1 study of NTLA-2001 in 2021
- AML: Submit an IND or IND-equivalent for NTLA-5001 in mid-2021
- HAE: Submit an IND or IND-equivalent for NTLA-2002 in 2H 2021
- Nominate at least one new development candidate in 2021

Fourth Quarter and Full-Year 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $597.4 million as of December 31, 2020, compared to $284.5 million as of December 31, 2019. The increase was driven by net proceeds of $296.6 million from our follow-on public offerings, $100.0 million upfront payment from the Regeneron collaboration expansion, which included a $30.0 million equity investment, $49.5 million of net equity proceeds raised from the Company's "At the Market" (ATM) agreement, $18.2 million from the Regeneron and Novartis Institutes for BioMedical Research, Inc. (Novartis) collaborations and $13.2 million in proceeds from employee-based stock plans. These increases were offset in part by cash used to fund operations of approximately $164.6 million.
- **Collaboration Revenue:** Collaboration revenue decreased by $4.3 million to $6.6 million during the fourth quarter of 2020, compared to $10.9 million during the fourth quarter of 2019. The decrease was primarily driven by a decrease in Novartis revenue as the research portion of the collaboration ended in December 2019.
- **R&D Expenses:** Research and development expenses increased by $6.5 million to $38.2 million during the fourth quarter of 2020, compared to $31.7 million during the fourth quarter of 2019. 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 $1.8 million to $10.8 million during the fourth quarter of 2020, compared to $9.0 million during the fourth quarter of 2019. This increase was primarily related to employee related expenses, including stock-based compensation of $1.3 million.
- **Net Loss:** The Company's net loss was $42.2 million for the fourth quarter of 2020, compared to $28.3 million during the fourth quarter of 2019.

Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of December 31, 2020 will enable the Company to fund its robust R&D plans, anticipated operating expenses and capital expenditure requirements at least through 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 Fourth Quarter and Full-Year 2020 Earnings

The Company will discuss these results on a conference call today, February 25, 2021, 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.

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 February 25, 2021 at 12 p.m. ET.

About Intellia Therapeutics
Intellia Therapeutics is a leading clinical-stage 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 and CRISPR/Cas9 at intelliatx.com. 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 our: being able to enroll and dose the necessary subjects in our clinical studies for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR"), provide timing on the first expected data readout, and successfully submit additional regulatory applications in other countries; ability to evaluate NTLA-2001 in a broader ATTR population; the ability to demonstrate NTLA-5001 as a potential

best-in-class engineered T cell therapy designed to treat all genetic subtypes of acute myeloid leukemia ("AML"); plans to submit an investigational new drug ("IND") application or equivalent regulatory application for NTLA-5001 in mid-2021; expectations of evaluating the safety and activity of NTLA-5001 in patients with persistent or recurrent AML who have previously received first-line therapies; plans to evaluate the potential use of NTLA-5001 to treat WT1-positive solid tumors in preclinical studies; plans to submit an IND or equivalent regulatory application for NTLA-2002 in the second half of 2021; expectations of evaluating safety, tolerability and measures of activities of NTLA-2002 in patients with hereditary angioedema ("HAE"); plans to nominate at least one additional development candidate in 2021; plans to advance and complete preclinical studies for our programs; development of our modular platform to advance our complex genome editing capabilities; further development of our proprietary genome editing tools for research and therapeutic development sequential editing; presentation of additional data at upcoming scientific conferences, and other preclinical data in 2021; advancement and expansion of our CRISPR/Cas9 technology to develop human therapeutic products, as well as our ability to maintain and expand our related intellectual property portfolio; ability to demonstrate our platform's modularity and replicate or apply results achieved in preclinical studies, including those in our ATTR, AML, and HAE programs, in any future studies, including human clinical trials; ability to develop other *in vivo* or *ex vivo* cell therapeutics of all types, and those targeting Wilms' Tumor 1 ("WT1") in AML in particular, using CRISPR/Cas9 technology; ability to optimize the impact of our collaborations on our development programs, including but not limited to our collaboration with Regeneron Pharmaceuticals, Inc.; statements regarding the timing of regulatory filings and clinical trial execution, including dosing of patients, regarding our development programs; potential commercial opportunities, including value and market, for our product candidates; our expectations regarding our use of capital and other financial results during 2021; 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 our ability to protect and maintain our intellectual property position; risks related to our relationship with third parties, including our licensors and licensees; risks related to the ability of our licensors to protect and maintain their intellectual property position; uncertainties related to regulatory agencies' evaluation of regulatory filings and other information related to our product candidates; uncertainties related to the authorization, initiation and conduct of studies and other development requirements for our product candidates; the risk that any one or more of our 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 our collaborations with Regeneron or our 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 December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
Collaboration revenue	$ 6,595	$ 10,936	$ 57,994	$ 43,103
Operating expenses:				
Research and development	38,231	31,731	150,408	108,413
General and administrative	10,763	8,976	44,169	41,058
Total operating expenses	48,994	40,707	194,577	149,471
Operating loss	(42,399)	(29,771)	(136,583)	(106,368)
Interest income	207	1,495	2,352	6,835
Net loss	$ (42,192)	$ (28,276)	$ (134,231)	$ (99,533)
Net loss per share, basic and diluted	$ (0.69)	$ (0.57)	$ (2.40)	$ (2.11)
Weighted average shares outstanding, basic and diluted	61,306	49,350	55,987	47,247

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

	December 31, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 597,371	$ 284,472
Total assets	676,322	334,280
Total liabilities	149,250	64,399
Total stockholders' equity	527,072	269,881

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