Intellia Therapeutics Announces Second Quarter 2020 Financial Results

August 6, 2020

- *Submitted first regulatory application to the U.K.'s MHRA to initiate a Phase 1 study of NTLA-2001 for the treatment of transthyretin amyloidosis; on track to dose first patient by year-end*
- *Expanded Regeneron collaboration, receiving $100 million through upfront cash and equity investment and agreeing to co-develop treatments for hemophilia A and B using CRISPR-mediated targeted transgene insertion technology*
- *On track to submit an IND or IND-equivalent in 2021 for each of two wholly owned programs: NTLA-5001 for the treatment of acute myeloid leukemia and NTLA-2002 for the treatment of hereditary angioedema*
- *Ended quarter with strong cash position of $436.8 million*

CAMBRIDGE, Mass., Aug. 06, 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 second quarter ended June 30, 2020.

"We are very pleased to announce our first regulatory submission to initiate a Phase 1 study of NTLA-2001, which is an important milestone as we deliver on our mission to develop curative, CRISPR/Cas9-based treatments for severe diseases. Our CTA keeps us on track to dose our first ATTR patient by year-end and is a critical step in executing our global development strategy for the first systemically delivered CRISPR-based therapy to enter clinical trial," said Intellia President and Chief Executive Officer, John Leonard, M.D. "With our recently expanded collaboration with Regeneron and subsequent financing, we are well-capitalized to advance our initial wave of development programs and continue our work developing innovative modular platform capabilities."

Second Quarter 2020 and Recent Operational Highlights

- **ATTR Program:** Intellia announced today it has submitted its first Clinical Trial Application (CTA) to the United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA) to initiate a Phase 1 study of NTLA-2001 for the treatment of transthyretin amyloidosis (ATTR). Based on the Company's *in vivo* liver knockout approach, NTLA-2001 is a potential single-course therapy for patients living with ATTR. Pending CTA authorization and subject to the impact of COVID-19, the Company is on track to dose its first patient by the end of 2020 with the first systemically delivered CRISPR/Cas9-based therapy to enter the clinic. The Company is submitting additional regulatory applications to enable enrollment in other countries as part of its global development strategy. 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 acute myeloid leukemia (AML). The Company's approach aims to develop a broadly applicable treatment for AML patients, regardless of the mutational subtypes of the cancer. At the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting, held from May 12-15, 2020, Intellia presented data on its proprietary T cell engineering process in support of NTLA-5001. The data showed that the Company's proprietary process enables multiple, highly efficient, sequential edits in T cells, whether knocking out or inserting genes. This technology yields engineered cells with high anti-tumor activity and favorable attributes, including a desired memory phenotype, which is associated with longer-lasting treatment effects. Importantly, chromosomal translocations (i.e., undesired chromosomal rearrangements) were similar to background levels in untreated cells. 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. Additional efforts are underway to evaluate potential use of the WT1-targeted TCR construct to treat solid tumors.
- **HAE Program:** NTLA-2002 is a wholly owned, *in vivo* development candidate for the treatment of hereditary angioedema (HAE). Building on Intellia's modular lipid nanoparticle (LNP) delivery system, NTLA-2002 is designed to knock out the *prekallikrein B1 (KLKB1)* gene in the liver after a single course of treatment, which is expected to prevent improperly regulated bradykinin production and therefore, reduce HAE attacks. In a non-human primate (NHP) study of its lead LNP formulation for NTLA-2002, the knockout of *KLKB1* resulted in a therapeutically relevant reduction of serum kallikrein levels and activity following a single dose. Consistent with the durability achieved in earlier NHP studies for its lead *in vivo* program, the Company has now demonstrated sustained kallikrein activity reduction for 10 months in an ongoing study. Based on these results, Intellia believes NTLA-2002 could be efficacious and durable in preventing HAE attacks following a single course of treatment. Intellia continues to progress IND-enabling activities and is on track to submit an IND or IND-equivalent for NTLA-2002 in 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. Additionally, to supplement its *ex vivo* capabilities, the Company established research collaborations and license agreements with TeneoBio and GEMoaB GmbH to develop engineered cell therapies for immuno-oncology and autoimmune diseases. Intellia's broad platform innovations and expanded capabilities will drive

the next wave of *in vivo* and *ex vivo* clinical candidates.

- **Regeneron Collaboration:** In June 2020, Intellia and Regeneron announced an expansion of their existing collaboration to co-develop potential products for the treatment of hemophilia A and B. The collaboration expansion builds upon the jointly-developed targeted transgene insertion capabilities designed to durably restore a missing protein and to overcome the limitations of traditional gene therapy. The collaboration also provides Regeneron with rights to develop products for additional *in vivo* CRISPR/Cas9-based therapeutic liver targets. Under the expansion, the term of the collaboration has been extended until April 2024, at which point Regeneron has an option to renew for an additional two years. In addition, the expansion grants Regeneron a royalty-bearing, non-exclusive license to certain Intellia intellectual property to develop and commercialize up to 10 *ex vivo* CRISPR/Cas9 products in defined cell types. Intellia received $100 million through an upfront payment of $70 million and an additional $30 million equity investment from Regeneron at $32.42 per share.
- **Financing:** In June 2020, Intellia closed an underwritten public offering of 6,301,370 shares of common stock, including the exercise in full of the underwriters' option to purchase additional shares, at the public offering price of $18.25 per share. Intellia received aggregate gross proceeds of approximately $115 million, before underwriting discounts and commissions and offering expenses.

Upcoming Events

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

- Wedbush PacGrow Healthcare Conference, August 11, Virtual
- Baird Healthcare Conference, September 9, Virtual

Upcoming Milestones

The Company has set forth the following for pipeline progression:

- ATTR: Plan to dose first patient by end of 2020
- 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

Second Quarter 2020 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were $436.8 million as of June 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, $14.4 million of funding received under existing collaborations with Regeneron and Novartis and $2.1 million in proceeds from employee-based stock plans. These increases were offset in part by cash used to fund operations of approximately $86.5 million.
- **Collaboration Revenue:** Collaboration revenue increased by $5.1 million to $16.3 million during the second quarter of 2020, compared to $11.1 million during the second quarter of 2019. The increase in collaboration revenue in 2020 was mainly driven by an $8.4 million one-time cumulative catch-up adjustment related to the extension of the Regeneron collaboration.
- **R&D Expenses:** Research and development expenses increased by $12.3 million to $37.8 million during the second quarter of 2020, compared to $25.5 million during the second quarter of 2019. This increase was primarily driven by IND-enabling activities related to our lead programs, research personnel growth to support these programs, and the expansion of the development organization.
- **G&A Expenses:** General and administrative expenses decreased by $1.6 million to $11.5 million during the second quarter of 2020, compared to $13.1 million during the second quarter of 2019. This decrease was primarily driven by a decrease in legal expenses, which were principally related to a decrease in certain activities related to intellectual property matters.
- **Net Loss:** The Company's net loss was $32.4 million for the second quarter of 2020, compared to $25.7 million during the second quarter of 2019.

Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of June 30, 2020 will enable the Company to fund its 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 Second Quarter 2020 Earnings

The Company will discuss these results on a conference call today, August 6, 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 August 6, 2020 at 12 p.m. ET.

About Intellia Therapeutics
Intellia Therapeutics is a leading genome editing company focused on developing proprietary, curative therapeutics using the CRISPR/Cas9 system. Intellia believes the CRISPR/Cas9 technology has the potential to transform medicine by permanently editing disease-associated genes in the human body with a single treatment course, and through improved cell therapies that can treat cancer and immunological diseases, or can replace patients' diseased cells. The combination of deep scientific, technical and clinical development experience, along with its leading intellectual property portfolio, puts Intellia in a unique position to unlock broad therapeutic applications of the CRISPR/Cas9 technology and create a new class 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" 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: receiving authorization to initiate clinical studies for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR") pursuant to its clinical trial application ("CTA") or similar regulatory applications, and the planned dosing of first patients by the end of 2020; plans to complete manufacturing activities and submit an investigational new drug application ("IND") or equivalent regulatory filing 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 complete manufacturing activities and submit an IND or equivalent regulatory filing for NTLA 2002, a development candidate for its hereditary angioedema ("HAE") program in the second half of 2021; plans to advance and complete preclinical studies, including any necessary non-human primate studies, for its ATTR program, AML program, HAE program and other in vivo and ex vivo programs; 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; 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; 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; 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 optimize the impact of its collaborations on its development programs, including but not limited to its collaborations with Novartis or Regeneron, including its co-development programs for hemophilia A and hemophilia B; statements regarding the timing of regulatory filings regarding its development programs; use of capital, expenses, future accumulated deficit and other 2020 financial results or in the future; and ability to fund operations at least through 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; 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. 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,	
	2020	2019	2020	2019
Collaboration revenue	$ 16,263	$ 11,118	$ 29,179	$ 21,551
Operating expenses:				
Research and development	37,771	25,460	72,421	49,169
General and administrative	11,526	13,118	22,840	23,651
Total operating expenses	49,297	38,578	95,261	72,820
Operating loss	(33,034)	(27,460)	(66,082)	(51,269)
Interest income	641	1,777	1,883	3,646
Net loss	$ (32,393)	$ (25,683)	$ (64,199)	$ (47,623)
Net loss per share, basic and diluted	$ (0.61)	$ (0.56)	$ (1.24)	$ (1.05)
Weighted average shares outstanding, basic and diluted	53,369	45,814	51,938	45,526

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

	June 30, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 436,803	$ 284,472
Total assets	490,830	334,280
Total liabilities	139,243	64,399
Total stockholders' equity	351,587	269,881

Intellia Contacts:

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

Media:
Jennifer Mound Smoter
Senior Vice President
External Affairs & Communications
+1 857-706-1071
jenn.smoter@intelliatx.com

Source: Intellia Therapeutics, Inc.