# Intellia Therapeutics Announces Second Quarter 2019 Financial Results and Company Update

August 1, 2019

- *NTLA-2001: Intends to submit an investigational new drug application in mid-2020 for lead* in vivo *program for the treatment of transthyretin amyloidosis*
- *On track to nominate first engineered cell therapy development candidate for the treatment of acute myeloid leukemia by end of 2019*
- *Ends quarter with strong cash position of $276 million to provide funding through at least two years*

CAMBRIDGE, Mass., Aug. 01, 2019 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), reported operational highlights and financial results for the second quarter ended June 30, 2019.

"We continue to build our *in vivo* and engineered cell therapy pipeline in support of our mission to develop curative genome editing therapies for patients," said Intellia President and Chief Executive Officer John Leonard, M.D. "With IND-enabling toxicology studies underway for NTLA-2001, we are well-positioned to submit an IND in mid-2020 for the first systemically delivered CRISPR/Cas9-based therapy for transthyretin amyloidosis. Additionally, by the end of this year, we plan to select our first engineered cell therapy development candidate to treat acute myeloid leukemia."

**Second Quarter 2019 and More Recent Operational Highlights**

- **ATTR Program:** Intellia's first *in vivo* development candidate, NTLA-2001, is for the treatment of transthyretin amyloidosis (ATTR). As part of an ongoing durability study of the Company's lead lipid nanoparticle (LNP) formulation in support of NTLA-2001, we have demonstrated six months of durable liver editing with sustained reduction of circulating transthyretin (TTR) protein (average reduction >95%) following a single dose in non-human primates (NHPs). The Company announced today it has conducted its pre-investigational new drug (IND) meeting with the U.S. Food and Drug Administration (FDA), has initiated IND-enabling toxicology studies and expects to submit an IND application for NTLA-2001 in mid-2020. Additionally, the Company's supply chain operations are in place to support manufacturing of Phase 1 materials. NTLA-2001 is being co-developed with Regeneron Pharmaceuticals, Inc. (Regeneron), with Intellia as the lead development and commercialization party.
- **AML Program:** Intellia's lead T cell receptor (TCR)-based therapy uses engineered T cells to target Wilms' Tumor 1 (WT1) for the treatment of acute myeloid leukemia (AML). During the second quarter of 2019, the Company and its research collaborators at IRCCS Ospedale San Raffaele initiated functional testing of multiple lead TCRs in patient-derived xenograft models and remain on track to nominate a development candidate by the end of 2019. In parallel, Intellia has begun establishing manufacturing capabilities to support clinical evaluation.

- ***In Vivo* Insertion in NHPs:** As previously disclosed, Intellia demonstrated the first CRISPR/Cas9-mediated, targeted transgene insertion in the liver of NHPs, using *Factor 9 (F9)* as a model gene. *F9* is a gene that encodes for the Factor IX (FIX) protein, a blood-clotting protein that is missing or defective in hemophilia B patients. The study is a collaborative effort between Intellia and Regeneron, using Intellia's LNP delivery system of CRISPR/Cas9 with an adeno-associated virus (AAV) containing a proprietary bi-directional insertion template. Following a single dose of the hybrid LNP-AAV delivery system containing an *F9* DNA template, the Company demonstrated that the circulating human FIX protein levels achieved at day 14 were durable through the two months of completed observation and were within the normal human range (source: Amiral et al, Clin. Chem., 1984). Additionally, the NHP data expands on the durability of clinically relevant human FIX protein levels achieved in mice for over 12 months. Intellia is currently working closely with Regeneron on moving the program forward and is also independently evaluating the hybrid LNP-AAV delivery system for targeted insertion across several other transgenes of interest in an *in vivo* setting.

- **Expanded Management Team**: In May, Intellia announced the appointment of Laura Sepp-Lorenzino, Ph.D., as executive vice president and chief scientific officer. Dr. Sepp-Lorenzino brings decades of leadership and research and development experience and leads Intellia's drug research organization.

**Upcoming Milestones**

The Company has set forth the following for pipeline progression:

- ATTR:
  - Commence manufacturing of NTLA-2001 Phase 1 materials by the end of 2019
  - Submit IND application for NTLA-2001 in mid-2020
- AML:
  - Nominate first engineered cell therapy development candidate by the end of 2019

- Platform:
  - Present additional *in vivo* and engineered cell therapy data at upcoming scientific conferences by the end of 2019

**Second Quarter 2019 Financial Results**

- **Cash Position:** Cash, cash equivalents and marketable securities were $275.8 million as of June 30, 2019, compared to $314.1 million as of December 31, 2018. The decrease was driven by cash used to fund operations of $59.8 million, which was offset in part by $7.9 million of net equity proceeds raised from the Company's At The Market ("ATM") offerings, $7.0 million of funding received under the Novartis collaboration, $4.1 million of ATTR cost reimbursements made by Regeneron, and $2.6 million in proceeds from employee-based stock plans.
- **ATM Proceeds Due:** The Company has $27.1 million in current assets on the balance sheet as of June 30, 2019 related to proceeds due from the ATM offerings that were received in the first week of July 2019.
- **Collaboration Revenue:** Collaboration revenue increased by $3.4 million to $11.1 million during the second quarter of 2019, compared to $7.7 million during the second quarter of 2018. The increase in collaboration revenue in 2019 was primarily driven by amounts recognized from the expansion of the existing collaboration with Novartis, as well as by amounts recognized under the Company's ATTR Co/Co agreement with Regeneron. As previously disclosed, Regeneron is obligated to fund approximately 50% of the development costs for the ATTR program.
- **R&D Expenses:** Research and development expenses increased by $2.0 million to $25.5 million during the second quarter of 2019, compared to $23.5 million during the second quarter of 2018. This increase was primarily driven by the advancement of Intellia's pipeline and the expansion of the research and development organization.
- **G&A Expenses:** General and administrative expenses increased by $5.3 million to $13.1 million during the second quarter of 2019, compared to $7.8 million during the second quarter of 2018. This increase was primarily driven by personnel-related costs to support Intellia's larger research and development organization, and an increase in legal and intellectual property (IP) costs.
- **Net Loss:** The Company's net loss was $25.7 million for the second quarter of 2019, compared to $22.2 million during the second quarter of 2018.

**Financial Guidance**

Intellia expects that its cash, cash equivalents, marketable securities and proceeds due from the ATM offerings as of June 30, 2019, as well as technology access and funding from Novartis and Regeneron, will enable Intellia to fund its anticipated operating expenses and capital expenditure requirements into the second half of 2021. This expectation excludes any potential milestone payments or extension fees that could be earned and distributed under the collaboration agreements with Novartis and Regeneron or any strategic use of capital not currently in the Company's base-case planning assumptions.

**Conference Call to Discuss Second Quarter 2019 Earnings**

The Company will discuss these results on a conference call today, August 1, 2019, at 8 a.m. ET.

To join the call:

- U.S. callers should dial 866-575-6539 and use conference ID# 6223506, approximately five minutes before the call.
- International callers should dial +1 856-344-9299 and use conference ID# 6223506, approximately five minutes before the call.

A replay of the call will be available through the Events and Presentations page of the Investor Relations section of the Company's website at www.intelliatx.com, beginning on August 1, 2019 at 12 p.m. ET.

**About Intellia Therapeutics**

Intellia Therapeutics is a leading genome editing company focused on developing 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 ability to conduct successful investigational new drug application ("IND")-enabling toxicology studies of NTLA-2001; its plans to commence related manufacturing efforts of Phase 1 materials for NTLA-2001; its planned submission of an IND application for NTLA-2001 in mid-2020; its plans to generate preclinical and other data necessary to nominate a first engineered cell therapy development candidate for its AML program by the end of 2019; its plans to advance and complete preclinical studies, including non-human primate studies for its ATTR program, AML program and other *in vivo* and *ex vivo* programs; develop our proprietary LNP/AAV hybrid delivery system to advance our complex genome editing capabilities, such as gene insertion; its presentation of additional data at upcoming scientific conferences regarding CRISPR-mediated, targeted transgene insertion in the liver of NHPs, using F9 as a model gene, via the Company's proprietary LNP-AAV delivery technology, and other preclinical data by the end of 2019; the advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, as well as maintain and expand its related intellectual property portfolio; the ability to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies, including those in its ATTR and AML programs, in any future studies, including human clinical trials; its 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; the ability to continue its growth and realize the anticipated contribution of the members of its board of directors and executives to its operations and progress; the impact of its collaborations on its development programs, including but not limited to its collaboration with Regeneron Pharmaceuticals, Inc.; statements regarding the timing of regulatory filings regarding its development programs; its use of capital, including ATM receivables, expenses, future accumulated deficit and other financial results during the second quarter of 2019; and the ability to fund operations into the second half of 2021.

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 our intellectual property position, including through our arbitration proceedings against Caribou; risks related to Intellia's relationship with third parties, including our licensors; risks related to the ability of our licensors to protect and maintain their intellectual property position; uncertainties related to the initiation and conduct of studies and other development requirements for our 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 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, | |
|---|---|---|---|---|
| | **2019** | **2018** | **2019** | **2018** |
| Collaboration revenue | $ 11,118 | $ 7,677 | $ 21,551 | $ 15,146 |
| Operating expenses: | | | | |
| Research and development | 25,460 | 23,467 | 49,169 | 45,960 |
| General and administrative | 13,118 | 7,805 | 23,651 | 15,211 |
| Total operating expenses | 38,578 | 31,272 | 72,820 | 61,171 |
| Operating loss | (27,460) | (23,595) | (51,269) | (46,025) |
| Interest income | 1,777 | 1,376 | 3,646 | 2,450 |
| Net loss | $ (25,683) | $ (22,219) | $ (47,623) | $ (43,575) |
| Net loss per share, basic and diluted | $ (0.56) | $ (0.52) | $ (1.05) | $ (1.03) |
| Weighted average shares outstanding, basic and diluted | 45,814 | 42,836 | 45,526 | 42,441 |

**INTELLIA THERAPEUTICS, INC.**
**CONSOLIDATED STATEMENTS OF OPERATIONS (UNAUDITED)**
**(Amounts in thousands)**

| | June 30, 2019 | December 31, 2018 |
|---|---|---|
| Cash, cash equivalents and marketable securities | $ 275,838 | $ 314,059 |
| Total assets | 352,441 | 347,315 |
| Total liabilities | 75,575 | 69,395 |

| | | |
|---|---|---|
| Total stockholders' equity | 276,866 | 277,920 |

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