



Intellia Therapeutics Announces First Quarter 2021 Financial Results

May 6, 2021

- Plan to report initial safety and activity data from Phase 1 study of NTLA-2001, a potentially curative single-dose therapy for transthyretin amyloidosis (ATTR), in mid-2021
- On track to submit an IND or IND-equivalent in mid-2021 to advance NTLA-5001 for acute myeloid leukemia (AML)
- Expect to submit an IND or IND-equivalent in 2H 2021 for NTLA-2002 for hereditary angioedema (HAE)
- Ended the quarter with a strong cash position of \$600.8 million

CAMBRIDGE, Mass., May 06, 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 first quarter ended March 31, 2021.

"Intellia continues to build momentum as we progress our full-spectrum pipeline towards key upcoming milestones this year. In mid-2021, we anticipate reporting initial clinical data for NTLA-2001 for ATTR, the first-ever systemically delivered CRISPR-based therapy. The company also plans on submitting first-in-human regulatory filings for NTLA-5001 for AML and NTLA-2002 for HAE, and remains on track to nominate at least one additional development candidate from our research efforts," said Intellia President and Chief Executive Officer John Leonard, M.D. "During the first quarter, we shared important advancements that expanded the breadth of our modular platform, such as introducing our proprietary base editor and establishing preclinical proof-of-concept for systemic *in vivo* editing in bone marrow. We believe our platform innovations will drive future pipeline growth and expand our ability to develop potentially curative treatments for patients with a range of severe diseases."

First Quarter 2021 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 a 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 administration. 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 ongoing single ascending dose portion of the Phase 1 study evaluating NTLA-2001 in adults with hereditary ATTR with polyneuropathy (hATTR-PN) at a scientific or medical meeting in mid-2021. The data are expected to characterize the emerging safety and activity profile of NTLA-2001 at the initial dose levels.
 - In March, Intellia [announced](#) that the European Commission (EC) granted NTLA-2001 orphan drug designation. Orphan drug designation is granted to therapies that are intended for the treatment, prevention, or diagnosis of life threatening or chronically debilitating rare diseases where there are either no treatments or no satisfactory therapeutic options in the European Union.
 - 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 expects 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.
 - The Company is evaluating the potential 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 treatment consisting of a single administration. 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.

- The Company expects to submit an IND or equivalent regulatory application for NTLA-2002 in the second half of 2021. Intellia is leveraging insights gained from the development of 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.
- In March, Intellia presented preclinical results confirming greater reductions in serum kallikrein protein levels and activity versus the current standard of care for HAE, sustained over 17 months following a single dose in an ongoing non-human primate study of its cyno-specific LNP formulation for NTLA-2002. Additionally, the Company presented data from a humanized KLKB1 mouse model of bradykinin-mediated vascular permeability, establishing that a single administration of NTLA-2002 prevented captopril-induced vascular leakage. These results, which affirm NTLA-2002's therapeutic hypothesis of preventing HAE attacks, were presented at the American Academy of Allergy, Asthma & Immunology (AAAAI) 2021 Annual Meeting.
- **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 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 autoimmune diseases.
 - Intellia remains on track to nominate at least one additional development candidate in 2021.
 - The Company plans to [present](#) preclinical data at the American Society of Gene and Cell Therapy (ASGCT) 24th Annual Meeting, taking place virtually May 11 – 14, 2021, highlighting research advancements and platform innovations.
 - In March, the Company [presented](#) preclinical data introducing Intellia's novel, proprietary cytosine deaminase base editing technology. The data demonstrated the technology's potential for enhanced cell engineering, with multiple simultaneous gene knockouts achieving >90% T cell editing efficiency and no detectable increase in translocation above background levels. These results, which expand Intellia's modular *ex vivo* capabilities, were presented at the seventh Cold Spring Harbor Laboratory (CSHL) virtual scientific meeting on Nucleic Acid Therapies.
 - In March, the Company [presented](#) preclinical data establishing proof-of-concept for systemic, *in vivo* genome editing in a tissue outside the liver. Intellia's non-viral delivery platform achieved dose-dependent, therapeutically meaningful editing of bone marrow and hematopoietic stem cells (HSCs), lasting one year following a single dose in mice. These results, which extend Intellia's modular *in vivo* capabilities to treat inherited blood disorders such as sickle cell disease, were presented at the Keystone eSymposium: Precision Engineering of the Genome, Epigenome and Transcriptome.
- **Board of Directors:**
 - In April, Intellia appointed Georgia Keresty, Ph.D., M.P.H., to the Company's Board of Directors. Dr. Keresty has held key global roles in pharmaceutical research and development, operations, manufacturing and distribution, quality, compliance and regulatory affairs. Most recently, Dr. Keresty served as chief operating officer and global head, medical sciences and development operations for Takeda Research and Development, a division of Takeda Pharmaceuticals USA, Inc.

Upcoming Events

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

- ASGCT 24th Annual Meeting, May 11–14, Virtual
- Jefferies Healthcare Conference, June 2, 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 mid-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

First Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$600.8 million as of March 31, 2021, compared to \$597.4 million as of December 31, 2020. The increase was driven by \$45.3 million of net equity proceeds raised from the Company's "At the Market" (ATM) agreement, \$13.3 million in proceeds from employee-based stock plans and \$2.4 million from the Regeneron collaboration. These increases were offset in part by cash used to fund operations of approximately \$57.6 million.
- **Collaboration Revenue:** Collaboration revenue decreased by \$6.5 million to \$6.4 million during the first quarter of 2021, compared to \$12.9 million during the first quarter of 2020. The decrease was primarily driven by the \$5.0 million milestone payment earned from Novartis Institutes for BioMedical Research, Inc. (Novartis) for the IND submission of OTQ923 in 2020.
- **R&D Expenses:** Research and development expenses increased by \$4.6 million to \$39.3 million during the first quarter of 2021, compared to \$34.7 million during the first 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 \$2.3 million to \$13.6 million during the first quarter of 2021, compared to \$11.3 million during the first quarter of 2020. This increase was primarily related to employee related expenses, including stock-based compensation of \$0.9 million.
- **Net Loss:** The Company's net loss was \$46.2 million for the first quarter of 2021, compared to \$31.8 million during the first quarter of 2020.

Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of March 31, 2021 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 First Quarter Earnings

The Company will discuss these results on a conference call today, May 6, 2021, at 8 a.m. E.T.

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.intelliatrix.com beginning on May 6, 2021 at 12 p.m. E.T.

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 administration, 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 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 amyloidosis ("ATTR") pursuant to its clinical trial applications ("CTA"), including submitting additional regulatory applications in other countries and evaluating NTLA-2001 in a broader ATTR population; clinical data from the ongoing single ascending dose portion of the Phase 1 study evaluating NTLA-2001; expectation to submit an Investigational New Drug ("IND") application or equivalent regulatory submission for NTLA-5001 for the treatment of acute myeloid leukemia ("AML") in mid-2021; expectation to submit an IND application or equivalent regulatory submission for NTLA-2002 for the treatment of hereditary angioedema ("HAE"); identification and nomination of a new development candidate in 2021; potential advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, including *in vivo* therapies in multiple tissue types and allogeneic T cell therapies; development of our modular platform to advance our complex genome editing capabilities, including insertion and base editing technologies; ability to maintain and expand its related intellectual property portfolio; ability to optimize the impact of its collaborations on its development programs, including but not limited to its collaborations with Regeneron Pharmaceuticals, Inc., including its co-development programs for ATTR; statements regarding the timing of regulatory filings regarding its development programs; and expectations regarding use of capital and its 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 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 Regeneron 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 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 March 31,	
	2021	2020
Collaboration revenue	\$ 6,445	\$ 12,916
Operating expenses:		
Research and development	39,276	34,650
General and administrative	13,594	11,314
Total operating expenses	52,870	45,964
Operating loss	(46,425)	(33,048)
Interest income	220	1,242
	\$ (46,205)	\$ (31,806)
Net loss per share, basic and diluted	\$ (0.69)	\$ (0.63)
Weighted average shares outstanding, basic and diluted	67,183	50,491

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

	March 31, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	\$ 600,798	\$ 597,371
Total assets	716,891	676,322
Total liabilities	171,018	149,250
Total stockholders' equity	545,873	527,072

Intellia Contacts:

Investors:

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

Media:

Julie Ferguson
Interim Head of External Affairs & Communications
+1-312-385-0098
julie.ferguson@intelliatx.com



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