



## Intellia Therapeutics Announces Fourth Quarter and Full-Year 2021 Financial Results and Highlights Recent Company Progress

February 24, 2022

- *On track to present additional interim data from dose-escalation portion of the ongoing Phase 1 study in patients with transthyretin (ATTR) amyloidosis with polyneuropathy on February 28, at 4:30 p.m. ET*
- *Plans to present interim data from ongoing first-in-human study of NTLA-2002 for hereditary angioedema (HAE) in 2H 2022*
- *Nominated two new development candidates – NTLA-6001, its first ex vivo allogeneic CAR-T candidate for CD30+ expressing hematologic cancers, and NTLA-2003, an in vivo knockout candidate for liver associated alpha1-antitrypsin deficiency (AATD)*
- *Expanded gene editing technology platform through acquisition of Rewrite Therapeutics*
- *Broadened future product pipeline with ONK Therapeutics, Kyverna Therapeutics and SparingVision collaboration agreements*
- *Entered into a lease agreement to build a GMP manufacturing facility to support production of its CRISPR-based therapies*

CAMBRIDGE, Mass., Feb. 24, 2022 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on developing potentially curative therapies leveraging CRISPR-based technologies, today reported operational highlights and financial results for the fourth quarter and year ended December 31, 2021.

"We began 2022 with strong momentum, executing against our strategic priorities for the advancement of our CRISPR-based pipeline and platform. We continued to make steady progress across our multiple clinical programs. In addition, we nominated two new development candidates – NTLA-2003 and NTLA-6001. Notably, we are looking forward to sharing additional data from the landmark study of NTLA-2001 next week," said Intellia President and Chief Executive Officer John Leonard, M.D. "In parallel, we continue to propel our own scientific innovation, as well as leverage external capabilities from across the industry to generate the next wave of clinical candidates. As part of this strategy, we completed several important transactions to further bolster our industry-leading genome editing toolbox and pipeline. Intellia is well-positioned to extend its leadership position as we aim to harness the full potential of genomic medicines."

### Fourth Quarter 2021 and Recent Operational Highlights

#### *In Vivo* Program Updates

- **NTLA-2001 for ATTR amyloidosis:** NTLA-2001 is the first investigational CRISPR-based therapy to be systemically delivered to edit genes inside the human body and has the potential to be the first single-dose treatment for transthyretin (ATTR) amyloidosis. Delivered with the Company's *in vivo* lipid nanoparticle (LNP) technology, NTLA-2001 offers the possibility of halting and reversing the disease by driving a deep, potentially lifelong reduction in transthyretin (TTR) protein after a single dose. NTLA-2001 is part of a co-development/co-promotion agreement between Intellia, the lead party for this program, and Regeneron Pharmaceuticals, Inc. (Regeneron).
  - Intellia will be hosting a virtual investor event on February 28, 2022, at 4:30 p.m. ET to present additional interim clinical data from the ongoing Phase 1 study of NTLA-2001 in patients with hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN). Data to be presented will come from all four ATTRv-PN dose cohorts in Part 1, the single-ascending dose portion, and include safety and serum TTR reduction for Cohorts 3 and 4, as well as an early look at durability across all cohorts. The Company remains on track to initiate Part 2, a single-cohort expansion, in the polyneuropathy arm in the first quarter of 2022.
  - Intellia continues to dose patients in the cardiomyopathy arm of the Phase 1 study with NTLA-2001. In December 2021, Intellia initiated dosing in new dose-escalation cohorts in patients with ATTR amyloidosis with cardiomyopathy (ATTR-CM) as part of the expanded Phase 1 study. Enrollment across both ATTRv-PN and ATTR-CM patient populations is expected to complete in 2022.
- **NTLA-2002 for HAE:** NTLA-2002 leverages Intellia's proprietary *in vivo* LNP delivery technology to knock out the *KLKB1* gene in the liver with the potential to permanently reduce total plasma kallikrein protein and activity, a key mediator of hereditary angioedema (HAE). This investigational approach aims to prevent attacks for people living with HAE by providing continuous suppression of plasma kallikrein activity following a single dose and to eliminate the significant treatment burden associated with currently available HAE therapies.

- In December 2021, Intellia announced the first patient was dosed with NTLA-2002. The first-in-human study is expected to evaluate the safety, tolerability and activity of NTLA-2002 in adults with Type I or Type II HAE.
- The Company anticipates presenting interim data from the Phase 1/2 study in the second half of 2022. The data are expected to characterize the emerging safety and activity profile of NTLA-2002, and to potentially demonstrate preliminary proof of concept.
- **NTLA-3001 for AATD-associated lung disease:** NTLA-3001 is a wholly owned, first-in-class, CRISPR-mediated *in vivo* targeted gene insertion development candidate for the treatment of alpha-1 antitrypsin deficiency (AATD)-associated lung disease. It is designed with the aim to precisely insert a healthy copy of the *SERPINA1* gene, which encodes the alpha-1 antitrypsin (A1AT) protein, with the potential to restore permanent expression of functional A1AT protein to therapeutic levels after a single dose. This approach seeks to eliminate the need for sub-optimal weekly IV infusions of A1AT augmentation therapy or lung transplant in severe cases.
  - Intellia continues to conduct Investigational New Drug (IND)-enabling activities for NTLA-3001, with plans to file an IND or IND-equivalent in 2023.
- **NTLA-2003 for AATD-associated liver disease:** NTLA-2003 is a wholly owned *in vivo* knockout development candidate for the treatment of AATD-associated liver disease. It is designed to inactivate the *SERPINA1* gene responsible for the production of abnormal A1AT protein in the liver. This approach aims to halt the progression of liver disease and eliminate the need for liver transplant in severe cases.
  - Today, Intellia announced the nomination of a new development candidate, NTLA-2003, for treatment of AATD-associated liver disease. The Company is advancing towards IND-enabling activities for this program.

#### Ex Vivo Program Updates

- **NTLA-5001 for AML:** NTLA-5001 is an autologous T cell receptor (TCR)-T cell therapy engineered to target the Wilms' Tumor 1 (WT1) antigen for the treatment of all genetic subtypes of acute myeloid leukemia (AML).
  - In the fourth quarter of 2021, Intellia initiated screening of patients in the Phase 1/2a study of NTLA-5001 for patients with AML. The Company has begun enrolling patients and expects to dose its first patient in the coming weeks. Later this year, the Company plans to provide guidance around timing of the first expected data readout, with the goal of demonstrating clinical proof of concept for its TCR-based platform.
- **NTLA-6001 for CD30+ Lymphomas:** NTLA-6001 is Intellia's wholly owned allogeneic CAR-T development candidate targeting CD30 for the treatment of CD30-expressing hematologic cancers, including relapsed or refractory classical Hodgkin's Lymphoma (cHL).
  - Today, Intellia announced the nomination of its first *ex vivo* allogeneic development candidate, NTLA-6001, for CD30-expressing hematologic cancers, including cHL. NTLA-6001 is developed using Intellia's proprietary allogeneic cell engineering platform, which leverages a novel combination of sequential gene edits. Preclinical data presented on its differentiated allogeneic engineering platform showed allogeneic T cells were shielded from immune rejection, both host T and natural killer (NK) cell attack.
  - Intellia is advancing towards IND-enabling activities and plans to present preclinical data leading to the development of NTLA-6001 at an upcoming scientific conference in 2022.

#### Research and Corporate Updates

- **Modular Platform and Pipeline Expansion:** Intellia is expanding its industry-leading genome editing platform and scientific leadership through editing, delivery and cell engineering innovations that may enable broader *in vivo* and *ex vivo* applications.
  - Following the nomination of NTLA-2003, Intellia plans to advance at least one new *in vivo* development candidate by the end of 2022.
  - The Company plans to highlight additional advances to its proprietary technology capabilities, including both genome editing and delivery tools, at upcoming scientific conferences in 2022.
- **Collaboration Updates**
  - In February, the Company [executed](#) a licensing and collaboration agreement with ONK Therapeutics Ltd. (ONK) for the development of allogeneic CRISPR-edited NK cell therapies for the treatment of cancer. The agreement grants ONK a non-exclusive license to Intellia's proprietary *ex vivo* CRISPR/Cas9-based genome editing platform and its LNP-based delivery technologies and exclusive rights to certain guide RNAs for development of up to five NK cell therapies. ONK will be responsible for preclinical and clinical development for the engineered NK cell therapies

covered under the agreement. In addition, the agreement grants Intellia options to co-develop and co-commercialize up to two products worldwide with rights to lead commercialization in the U.S.

- In December 2021, the Company [executed](#) a licensing and collaboration agreement with Kyverna Therapeutics (Kyverna) for the development of KYV-201, an allogeneic CD19 CAR-T cell investigational candidate for the treatment of select autoimmune diseases. This is a novel approach aimed at targeting CD19 for inflammatory diseases as compared to traditional oncology indications. Kyverna will lead and fund preclinical and clinical development for KYV-201 and, as part of the agreement, Intellia granted Kyverna rights to use its proprietary *ex vivo* CRISPR/Cas9-based allogeneic platform in exchange for an equity stake in Kyverna. Intellia will be eligible to receive certain development and commercial milestone payments, as well as low- to mid-single-digit royalties on potential future sales and may choose to exercise an option to lead U.S. commercialization for KYV-201 under a co-development and co-commercialization agreement.
- In October 2021, the Company [executed](#) a strategic collaboration with SparingVision to develop novel genomic medicines utilizing Intellia's proprietary CRISPR/Cas9 technology for the treatment of ocular diseases. In addition, Intellia received an equity stake in SparingVision. As part of the collaboration, Intellia will receive an option for exclusive U.S. commercialization rights for product candidates arising from two of three collaboration targets, eligibility for development and commercial milestone payments, as well as royalties on potential future sales of products arising from the collaboration. The companies will additionally research and develop novel self-inactivating AAV vectors and LNP-based approaches to address delivery of CRISPR/Cas9 genome reagents to the retina.

#### • Corporate Updates

- In February, Intellia [completed](#) the acquisition of Rewrite Therapeutics, Inc. (Rewrite), a private biotechnology company focused on advancing novel DNA writing technologies. Rewrite's DNA writing technology may enable a range of precise editing strategies. These include targeted corrections, insertions, deletions and the full range of single-nucleotide changes, which could provide new ways to edit disease-causing genes and broaden the therapeutic potential for genomic medicines.
- In February, the Company [announced](#) a lease agreement to develop a 140,000-square-foot manufacturing facility in Waltham, Massachusetts, to support the manufacturing of key components for its CRISPR-based investigational therapies. The new manufacturing facility will be Good Manufacturing Practice (GMP) compliant and support both the preclinical through commercial supply for key components of Intellia's CRISPR-based therapies. Additionally, this facility, in combination with existing capabilities and partnerships, will provide capacity and capabilities in support of Intellia's expanding pipeline and commercial readiness.

#### Upcoming Events

The Company will participate in the following events during the first quarter of 2022:

- AAAAI Annual Meeting, February 25-28, Phoenix
- Cowen Healthcare Conference, March 7, Virtual
- Barclays Capital Global Healthcare Conference, March 15, Miami
- Guggenheim Healthcare Talks Genomic Medicines and Rare Disease Day, March 31, Virtual

#### Upcoming Milestones

The Company has set forth the following for pipeline progression:

- NTLA-2001 for ATTR amyloidosis:
  - Report additional interim data from Phase 1 study on February 28
  - Initiate Part 2, a single-dose expansion cohort, of the Phase 1 study of NTLA-2001 in Q1 2022
  - Complete enrollment of Phase 1 study for both ATTRv-PN and ATTR-CM subjects in 2022
- NTLA-2002 for HAE: Present interim data from Phase 1/2 study in 2H 2022
- NTLA-3001 for AATD: Plan to file an IND or IND-equivalent in 2023
- NTLA-5001 for AML: Continue to enroll patients in Phase 1/2a study in 2022

- NTLA-6001 for CD30+ Lymphomas: Plan to present preclinical data at an upcoming scientific conference in 2022
- Pipeline Expansion:
  - Advance at least one new *in vivo* development candidate by the end of 2022
  - Advance additional novel platform capabilities in 2022

#### Fourth Quarter and Full-Year 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$1,086.0 million as of December 31, 2021, compared to \$597.4 million as of December 31, 2020. The increase was driven by net proceeds of \$648.3 million from a follow-on offering in the third quarter of 2021, \$45.3 million of net proceeds from the Company's "At the Market" (ATM) agreement, \$43.1 million in proceeds from employee-based stock plans, and \$6.3 million of funding for cost-sharing agreements received from Regeneron. These increases were offset in part by cash used to fund operations of approximately \$254.7 million.
- **Collaboration Revenue:** Collaboration revenue increased by \$6.3 million to \$12.9 million during the fourth quarter of 2021, compared to \$6.6 million during the fourth quarter of 2020. This increase was primarily driven by \$5.8 million in revenue recorded in 2021 from our joint venture with AvenCell.
- **R&D Expenses:** Research and development expenses increased by \$32.9 million to \$71.2 million during the fourth quarter of 2021, compared to \$38.2 million during the fourth 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 \$11.3 million to \$22.1 million during the fourth quarter of 2021, compared to \$10.8 million during the fourth quarter of 2020. This increase was primarily related to employee related expenses, including stock-based compensation of \$3.8 million.
- **Net Loss:** The Company's net loss was \$81.2 million for the fourth quarter of 2021, compared to \$42.2 million during the fourth quarter of 2020.

#### Conference Call to Discuss Fourth Quarter and Full-Year 2021 Results

The Company will discuss these results on a conference call today, Thursday, February 24, at 8:00 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.
- Please visit this [link](#) for a simultaneous live webcast of the 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 [intelliatx.com](https://intelliatx.com), beginning on February 24, at 12:00 p.m. ET.

#### About Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics leveraging CRISPR-based technologies. To fully realize the transformative potential of CRISPR-based technologies, Intellia is pursuing two primary approaches. The company's *in vivo* programs use intravenously administered CRISPR as the therapy, in which proprietary delivery technology enables highly precise editing of disease-causing genes directly within specific target tissues. Intellia's *ex vivo* programs use CRISPR to create the therapy by using engineered human cells to treat cancer and autoimmune diseases. Intellia's deep scientific, technical and clinical development experience, along with its robust intellectual property portfolio, have enabled the company to take a leadership role in harnessing the full potential of genome editing to create new classes of genetic medicine. Learn more at [intelliatx.com](https://intelliatx.com). Follow us on Twitter [@intelliatx](https://twitter.com/intelliatx).

#### 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 extend its leadership successfully and harness the full potential of genomic medicines to bolster its genome editing capabilities and pipeline; its ability to deploy its financial resources, including cash and cash equivalents and revenue from operations, successfully; the safety, efficacy, success and advancement of its clinical programs for NTLA-2001 for the treatment of transthyretin amyloidosis, NTLA-2002 for the treatment of hereditary angioedema, and NTLA-5001 for the treatment of acute myeloid leukemia pursuant to its clinical trial applications ("CTA") and IND submissions, including the expected timing of data releases, regulatory filings, and the initiation, enrollment, and completion of clinical trials; the advancement of development candidates, including NTLA-3001 for the treatment of alpha-1 antitrypsin deficiency (AATD)-associated lung disease, NTLA-2003 for AATD-associated liver disease, and NTLA-6001 for CD30+ lymphomas; the ability to generate data to initiate clinical trials and the timing of CTA and IND submissions, including for NTLA-3001 for the AATD-associated lung disease, NTLA-2003 for

AATD-associated liver disease, and NTLA-6001 for CD30+ lymphomas; the expansion of its CRISPR/Cas9 technology and related technologies to advance additional development candidates and timing expectations of advancing such development candidates; its ability to maintain and expand its related intellectual property portfolio; expectations of the potential impact of the coronavirus disease 2019 pandemic, including the impact of the Delta and Omicron variants on strategy, future operations and timing of its clinical trials; its expectations of Rewrite's DNA writing technology in enabling a range of precise editing strategies and the resulting therapeutic potential of such technology; the ability to optimize the impact of its collaborations on its development programs, including, but not limited to, its collaborations with Regeneron, including its co-development program with Regeneron for ATTR amyloidosis, with Kyverna for the development of KYV-201, with ONK for the development of engineered NK cell therapies, and with SparingVision for the development of ocular therapies; and the ability to complete and utilize its planned manufacturing facility, including to manufacture GMP-compliant clinical and commercial supply.

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, including uncertainties related to regulatory approvals to conduct clinical trials; risks related to the development and/or commercialization of any of Intellia's or its collaborators' product candidates, including that they may not be successfully developed and commercialized; risks related to the results of preclinical studies or clinical studies, including that they may not be predictive of future results in connection with future studies; risks related to clinical study results, including that they may not be positive; risks related to the Rewrite acquisition, including that it may not result in the development of a writing technology or otherwise result in enabling precise editing strategies; risks related to Intellia's reliance on collaborations, including that its collaborations with Regeneron, ONK or Kyverna or its other collaborations will not continue or will not be successful; and risks related to completing and maintaining a GMP-compliant manufacturing facility. 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 and quarterly report on Form 10-Q, 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,	
	2021	2020	2021	2020
Collaboration revenue	\$ 12,854	\$ 6,595	\$ 33,053	\$ 57,994
Operating expenses:				
Research and development	71,161	38,231	229,807	150,408
General and administrative	22,108	10,763	71,096	44,169
Total operating expenses	93,269	48,994	300,903	194,577
Operating loss	(80,415)	(42,399)	(267,850)	(136,583)
Other (expense) income, net:				
Loss from equity method investment	(1,325)	-	(1,325)	-
Interest income	503	207	1,283	2,352
Total other (expense) income, net	(822)	207	(42)	2,352
Net loss	\$ (81,237)	\$ (42,192)	\$ (267,892)	\$ (134,231)
Net loss per share, basic and diluted	\$ (1.09)	\$ (0.69)	\$ (3.78)	\$ (2.40)
Weighted average shares outstanding, basic and diluted	74,427	61,306	70,894	55,987

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

	December 31, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	\$ 1,086,049	\$ 597,371
Total assets	1,294,464	676,322
Total liabilities	254,220	149,250
Total stockholders' equity	1,040,244	527,072

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Source: Intellia Therapeutics, Inc.