



## Intellia Therapeutics Announces Second Quarter 2021 Financial Results and Highlights Recent Company Progress

August 5, 2021

- *Established proof of concept for the Company's modular in vivo delivery platform with first-ever clinical data from systemically administered CRISPR genome editing candidate in humans*
- *Interim Phase 1 data from first two cohorts show NTLA-2001 was well-tolerated and achieved deep TTR reduction, with a maximum 96% serum TTR reduction by day 28, demonstrating potential as single-dose treatment for transthyretin (ATTR) amyloidosis; plan to report additional data from ongoing study this year*
- *Plans to initiate first-in-human studies for both NTLA-2002 for hereditary angioedema (HAE) and NTLA-5001 for acute myeloid leukemia (AML) this year*
- *Launched CAR T-cell company with Blackstone Life Sciences and Cellex Cell Professionals; concurrent agreement with Cellex gives Intellia access to donor cells and dedicated manufacturing capacity to support wholly-owned ex vivo portfolio*
- *Ended the quarter with strong cash position of \$551.3 million, and raised an additional \$648.1 million of net proceeds from follow-on offering in July*

CAMBRIDGE, Mass., Aug. 05, 2021 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading clinical-stage genome editing company focused on developing curative therapeutics using CRISPR/Cas9 technology both *in vivo* and *ex vivo*, today reported financial results for the second quarter ended June 30, 2021, and recent operational highlights.

"This quarter marked an exciting new chapter for Intellia. We presented the first clinical data in history supporting precision editing of a disease-causing gene within the body following a single, systemic dose of CRISPR/Cas9. These data open a new era of medicine – one that holds the potential of curing genetic disease. The interim data from our ongoing Phase 1 trial of NTLA-2001 support its potential to halt and even reverse the relentless progression of ATTR amyloidosis with one dose. More broadly, they offer proof of concept for our LNP-based platform for systemic *in vivo* delivery. With these results, we believe we are unlocking the treatment of diseases that originate in the liver and introducing a rapid and reproducible development path that accelerates our *in vivo* portfolio," said Intellia President and Chief Executive Officer John Leonard, M.D.

"In addition, together with Blackstone and Cellex, we launched a new company combining our allogeneic cell engineering platform with a clinically validated switchable, universal CAR-T construct. This new venture confers numerous benefits to Intellia, extending the reach of our technology beyond our core internal focus and giving us a meaningful stake in the company's success, even as we retain our rights across a diverse *ex vivo* landscape. This transaction also fortified our leading cell engineering capabilities by securing access to high-quality donor cells and dedicated manufacturing capacity to support the development of our wholly-owned *ex vivo* programs."

Dr. Leonard continued, "We are well-positioned to build on this quarter's momentum as we close in on a number of upcoming milestones. We look forward to initiating Phase 1 trials this year for our next two candidates, NTLA-2002 for HAE and NTLA-5001 for AML. We will also share additional interim data from NTLA-2001 later this year. With a strong financial position and a well-validated platform, we intend to expand and accelerate development across our full-spectrum pipeline."

### Second Quarter 2021 and Recent Operational Highlights

- **NTLA-2001 for ATTR Amyloidosis:** NTLA-2001 is the first systemically delivered CRISPR-based therapy dosed in a patient with the potential to be a curative treatment for 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, 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, and Regeneron Pharmaceuticals, Inc. (Regeneron).
  - In June, Intellia and Regeneron announced positive interim clinical data from the first two cohorts in the ongoing dose-escalation portion of a Phase 1 trial evaluating NTLA-2001 in adults with hereditary ATTR amyloidosis with polyneuropathy (ATTRv-PN). A single dose of either 0.1 mg/kg or 0.3 mg/kg of NTLA-2001 was administered systemically. Among the three patients in the 0.1 mg/kg dose group, treatment with NTLA-2001 led to mean serum TTR reductions of 52% by day 28. In the second cohort of three patients, a single 0.3 mg/kg dose of NTLA-2001 exceeded that of existing ATTRv-PN therapies with an 87% mean reduction in serum TTR levels by day 28, including one patient with a 96% reduction. NTLA-2001 demonstrated a dose-dependent response and an encouraging safety profile, with no serious adverse events in the first six patients by day 28. These results, the first-ever clinical data supporting safety and efficacy of *in vivo* CRISPR genome editing in humans, were [presented](#) at the 2021 Peripheral Nerve Society Annual Meeting and [published](#) in *The New England Journal of Medicine*.
  - Intellia is continuing to enroll the study to determine if higher doses could result in a deeper reduction in serum TTR protein levels with potential to translate into more meaningful clinical benefit. Once the recommended dose has been identified, Part 2 of the study, a single-dose expansion cohort, will be initiated later this year. After completion of the Phase 1 trial, Intellia intends to move to pivotal studies for both polyneuropathy and cardiomyopathy

manifestations of ATTR amyloidosis.

- The Company intends to present additional interim data from the dose-escalation portion of the Phase 1 study at a scientific or medical meeting this year.
- **NTLA-2002 for HAE:** NTLA-2002 leverages Intellia's modular *in vivo* LNP delivery technology to knock out the *KLKB1* gene in the liver with the potential to permanently reduce plasma kallikrein protein and activity, a key mediator of HAE. This 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 June, Intellia announced that it had submitted its first Clinical Trial Application (CTA) to the New Zealand Medicines and Medical Devices Safety Authority for NTLA-2002 to initiate a first-in-human study.
  - The Company expects to enroll the first patient by year-end and is also submitting additional regulatory applications to enable enrollment in other countries. The first-in-human trial is expected to evaluate safety, tolerability and activity in patients with HAE, and will continue to leverage insights gained from the development of NTLA-2001.
- **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 AML.
  - Intellia announced today it has submitted its first CTA to the United Kingdom Medicines and Healthcare products Regulatory Agency for NTLA-5001 to initiate a Phase 1 study.
  - The Company is submitting additional regulatory applications and expects to initiate patient screening in a Phase 1 study by year-end. 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.
- **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, as well as next-generation engineered cell therapies for cancers and autoimmune diseases.
  - In June, Intellia [announced](#) the launch of a new universal CAR-T cell therapy company in collaboration with Blackstone Life Sciences and Cellex Cell Professionals GmbH ("Cellex"), which closed on July 30. The new company will combine clinical-stage universal CAR-T platforms with Intellia's differentiated allogeneic cell engineering platform to develop therapies for immuno-oncology and autoimmune diseases. The agreement allows Intellia to advance its *ex vivo* pipeline with a key stake in the new company, with options to co-develop two allogeneic universal CAR-T candidates.
  - Through a concurrent agreement with Cellex, Intellia also established a preferred relationship including access to donor cells and dedicated manufacturing capacity to support the development of Intellia's wholly-owned *ex vivo* programs.
  - Intellia plans to nominate at least one additional development candidate from across its pipeline in 2021. In addition, the Company plans to nominate its first allogeneic development candidate by the first half of 2022.
  - The Company plans to present at upcoming scientific conferences, with *in vivo* and *ex vivo* updates, including an allogeneic solution that enables the next generation of engineered cell therapies.
- **Corporate:**
  - In July, the Company closed an underwritten public offering of 4,758,620 shares of common stock, including the exercise in full of the underwriters' option to purchase an additional 620,689 shares of common stock, at the public offering price of \$145.00 per share. Intellia raised aggregate net proceeds of \$648.1 million, which were net of estimated equity issuance costs of \$41.9 million.
  - In June, Intellia appointed James Basta, J.D., as Executive Vice President, General Counsel and Corporate Secretary. Mr. Basta has over two decades of combined in-house corporate and law firm experience and joins Intellia from Kura Oncology, where he served as Chief Legal Officer. Earlier in his career, Mr. Basta held various leadership roles in the legal department at Biogen and was a Partner at Baker McKenzie.
  - In July, Intellia appointed Ian Karp as Senior Vice President, Investor Relations and Corporate Communications. Mr. Karp brings over two decades of pharmaceutical and biotech industry experience across investor relations, corporate communications, corporate development and product commercialization. Mr. Karp joins Intellia from

Karyopharm Therapeutics, where he served as Senior Vice President, Investor and Public Relations, and was formerly the Head of Global Investor Relations at Shire plc.

### Upcoming Events

The Company plans to participate in the following events during the third quarter of 2021:

- Wells Fargo Securities Healthcare Conference, September 9-10, Virtual
- H.C. Wainwright 23rd Annual Global Investment Conference, September 15, Virtual

### Upcoming Milestones

The Company has set forth the following guidance for pipeline progression:

- ATTR:
  - Report additional interim clinical data from Phase 1 study of NTLA-2001 later this year
  - Initiate Part 2 of the NTLA-2001 Phase 1 study, a single-dose expansion cohort, later this year
- HAE: Initiate enrollment in the first-in-human study of NTLA-2002 by year-end
- AML: Initiate patient screening in the Phase 1 study of NTLA-5001 by year-end
- Pipeline Expansion:
  - Nominate at least one new development candidate in 2021, and
  - Nominate the Company's first allogeneic development candidate by 1H 2022

### Second Quarter 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$551.3 million as of June 30, 2021, compared to \$597.4 million as of December 31, 2020. The decrease was driven by cash used to fund operations of approximately \$115.1 million, which was offset in part by \$45.3 million of net equity proceeds from the Company's "At the Market" (ATM) agreement, \$20.5 million in proceeds from employee-based stock plans, and \$3.2 million of funding for cost-sharing agreements received from Regeneron. The \$551.3 million cash position as of June 30, 2021 does not include the proceeds from the July 2021 follow-on offering of common stock.
- **Collaboration Revenue:** Collaboration revenue decreased by \$9.7 million to \$6.6 million during the second quarter of 2021, compared to \$16.3 million during the second quarter of 2020. The decrease was primarily driven by an \$8.4 million one-time cumulative catch-up adjustment related to the modification of the 2016 Regeneron agreement recorded during Q2 2020.
- **R&D Expenses:** Research and development expenses increased by \$21.1 million to \$58.9 million during the second quarter of 2021, compared to \$37.8 million during the second quarter of 2020. This increase was primarily driven by a \$10.0 million one-time payment related to the third amendment to the 2014 Novartis Agreement as well as employee-related expenses due to the continued expansion of the development organization.
- **G&A Expenses:** General and administrative expenses increased by \$5.2 million to \$16.7 million during the second quarter of 2021, compared to \$11.5 million during the second quarter of 2020. This increase was primarily related to employee related expenses, including stock-based compensation, of \$2.1 million.
- **Net Loss:** The Company's net loss was \$68.8 million for the second quarter of 2021, compared to \$32.4 million during the second quarter of 2020.

### Financial Guidance

Intellia expects that its cash, cash equivalents and marketable securities as of June 30, 2021, along with the proceeds from the July 2021 public offering of common stock, will enable the Company to fund its robust R&D plans, anticipated operating expenses and capital expenditure requirements beyond 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 Earnings

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 [www.intelliatrix.com](http://www.intelliatrix.com), beginning on August 5, 2021 at 12 p.m. ET.

### About Intellia Therapeutics

Intellia Therapeutics, a leading clinical-stage genome editing company, is developing novel, potentially curative therapeutics using CRISPR/Cas9 technology. To fully realize the transformative potential of CRISPR/Cas9, 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 CRISPR/Cas9 to create new classes of genetic medicine. Learn more at [intelliatx.com](http://intelliatx.com). Follow us on Twitter [@intelliatweets](https://twitter.com/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 complete clinical studies for NTLA-2001 for the treatment of transthyretin ("ATTR") amyloidosis pursuant to its clinical trial applications ("CTA"), including submitting additional regulatory applications in other countries; clinical data from the ongoing single-ascending dose portion of the Phase 1 study evaluating NTLA-2001; expectations of approvals of its CTAs to regulatory authorities in New Zealand for NTLA-2002 for the treatment of hereditary angioedema ("HAE") and the United Kingdom for NTLA-5001 for the treatment of acute myeloid leukemia ("AML"); identification and nomination of new development candidates; expectations for the new universal CAR-T cell company launched in collaboration with Blackstone Life Sciences Advisors L.L.C. and Cellex Cell Professionals GmbH; 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; statements regarding the timing of regulatory filings regarding its development programs; plans to attend and present data at scientific conferences later this year; and our expectations regarding our use of capital and our ability to fund operations beyond 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; risks related to the COVID-19 pandemic including the effects of the Delta variant; 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 June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Collaboration revenue	\$ 6,550	\$ 16,263	\$ 12,995	\$ 29,179
Operating expenses:				
Research and development	58,884	37,771	98,160	72,421
General and administrative	16,683	11,526	30,277	22,840
Total operating expenses	75,567	49,297	128,437	95,261
Operating loss	(69,017)	(33,034)	(115,442)	(66,082)
Interest income	211	641	431	1,883
Net loss	\$ (68,806)	\$ (32,393)	\$ (115,011)	\$ (64,199)
Net loss per share, basic and diluted	\$ (1.01)	\$ (0.61)	\$ (1.70)	\$ (1.24)
Weighted average shares outstanding, basic and diluted	68,164	53,369	67,675	51,938

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

	June 30, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	\$ 551,281	\$ 597,371
Total assets	672,243	676,322
Total liabilities	177,430	149,250
Total stockholders' equity	494,813	527,072

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Source: Intellia Therapeutics, Inc.