



## Intellia Therapeutics Reports Financial Results for Third Quarter 2016

November 1, 2016

- First company to report high *in vivo* editing data using CRISPR/Cas9 in animal models
- Further validation of lipid nanoparticle delivery platform
- Ended quarter with \$290.6 million in cash and cash equivalents

CAMBRIDGE, Mass., Nov. 01, 2016 (GLOBE NEWSWIRE) -- Intellia Therapeutics, Inc. (NASDAQ:NTLA), a leading genome editing company focused on the development of potentially curative therapeutics using CRISPR/Cas9 technology, today reported results for the quarter ended September 30, 2016 and provided an update on recent highlights and upcoming events.

"We have demonstrated substantial progress in our research, including being the first company to present data showing high levels of *in vivo* editing in animal models using systemic lipid nanoparticles to deliver CRISPR/Cas9 components," said Nesson Bermingham, Ph.D., Chief Executive Officer and Founder, Intellia Therapeutics. "We continue to make further enhancements and remain focused on advancing the development of CRISPR/Cas9-based therapeutics for patients with severe unmet medical needs."

### Recent Highlights

- Intellia presented preclinical data demonstrating *in vivo* gene editing using lipid nanoparticles (LNPs) to deliver CRISPR/Cas9. These data were presented at the Genome Engineering: The CRISPR/Cas Revolution meeting in Cold Spring Harbor, New York. In several preclinical studies, the data showed:
  - Progress in achieving *in vivo* editing, reporting an efficiency of approximately 60 percent in mouse liver at the transthyretin (TTR) target site after a single intravenous administration, which was consistent across different lobes of the liver. This resulted in an associated decrease in serum TTR protein levels of up to approximately 80 percent;
  - Dose-dependent editing by LNP delivery;
  - Undetectable Cas9 mRNA and guide RNA (gRNA) in the liver at 72 hours post administration; and
  - Repair patterns in mouse liver cells *in vivo* being best predicted *in vitro* by primary mouse liver cells rather than cell lines.
- Intellia presented four posters at the recent European Society for Gene and Cell Therapy Congress (ESGCT) in Florence, Italy. The data presentations included an update on the Company's *in vivo* delivery and DNA repair data and new methods for analyzing off-target activity. In its presentation on off-target analysis, Intellia described improved computational methods for readily identifying guide RNAs with zero to few off-target events, an essential step in developing CRISPR/Cas9-based therapeutics.

### Third Quarter 2016 Financial Results

As of September 30, 2016, Intellia had \$290.6 million in cash and cash equivalents. Net loss for the third quarter 2016 was \$7.5 million, compared to \$3.0 million in the same period in 2015.

Collaboration revenue was \$4.9 million in the third quarter 2016, compared to \$1.7 million in the same period of 2015. For the Novartis collaboration, Intellia recognized \$2.0 million and \$1.7 million in the third quarters of 2016 and 2015, respectively. The Regeneron collaboration, announced in April 2016, for which the Company recognized \$2.9 million in the third quarter of 2016, was the primary driver of the increase in collaboration revenue.

Research and development expenses in the third quarter 2016 were \$7.9 million, compared to \$3.5 million in the same period in 2015. This increase in expenses is primarily attributable to accelerating the development of our CRISPR/Cas9 platform and advancing our sentinel indications. These expenses include compensation and benefits for employees, including equity-based compensation, and expansion of Intellia's facilities and laboratories.

General and administrative expenses were \$4.7 million in the third quarter of 2016, compared to \$1.5 million for the same period in 2015. The increase in general and administrative expenses is primarily driven by expenses to support the Company's overall growth and costs associated with being a publicly traded company.

### Upcoming Events

Intellia will present at the *Fortune* Brainstorm Health 2016 Conference in San Diego on November 2, 2016, the Credit Suisse Healthcare Conference in Arizona on November 7, 2016, and the Jefferies 2016 Healthcare Conference in London on November 16, 2016.

### About Intellia Therapeutics

Intellia Therapeutics is a leading 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 permanently editing disease-associated genes in the human body with a single treatment course. Our combination of deep scientific, technical and clinical development experience, along with our leading intellectual property portfolio, puts us 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](http://intelliatx.com); Follow us on Twitter @intelliatweets.

#### Forward-Looking Statements

*This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. These forward looking statements include, but are not limited to, statements regarding our ability to advance CRISPR/Cas9 into therapeutic products for severe and life-threatening diseases; the potential timing and advancement of our clinical trials; the impact of our collaborations with Novartis and Regeneron on our development programs; the potential indications we may pursue, including our sentinel indications; the potential timing of regulatory filings regarding our development programs; and potential commercialization opportunities for product candidates. Any forward-looking statements in this press release are based on management's current expectations 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, the risk that any one or more of our product candidates will not be successfully developed and commercialized, the risk of cessation or delay of any of the ongoing or planned clinical trials and/or our development of our product candidates, the risk that the results of previously conducted studies involving similar product candidates will not be repeated or observed in ongoing or future studies involving current product candidates, the risk that our collaboration with Novartis or Regeneron will not continue or will not be successful, and risks related to our ability to protect and maintain our intellectual property position. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in our most recent quarterly report on Form 10-Q filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Intellia Therapeutics undertakes no duty to update this information unless required by law.*

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

|  | Three Months Ended<br>September 30, |            | Nine Months Ended September<br>30, |            |
|--|-------------------------------------|------------|------------------------------------|------------|
|  | 2016                                | 2015       | 2016                               | 2015       |
| Collaboration revenue  | \$ 4,869                            | \$ 1,688   | \$ 10,852                          | \$ 4,351   |
| Operating expenses:  |                                     |            |                                    |            |
| Research and development   | 7,861                               | 3,458      | 20,509                             | 6,795      |
| General and administrative   | 4,705                               | 1,531      | 11,680                             | 5,474      |
| Total operating expenses   | 12,566                              | 4,989      | 32,189                             | 12,269     |
| Operating loss   | (7,697)                             | (3,301)    | (21,337)                           | (7,918)    |
| Interest income  | 215                                 | -          | 266                                | -          |
| Loss before income taxes   | (7,482)                             | (3,301)    | (21,071)                           | (7,918)    |
| Income tax benefit   | -                                   | 282        | -                                  | 766        |
| Net loss   | \$ (7,482)                          | \$ (3,019) | \$ (21,071)                        | \$ (7,152) |
| Net loss per share attributable to common stockholders,<br>basic and diluted | \$ (0.22)                           | \$ (10.27) | \$ (1.16)                          | \$ (48.65) |
| Weighted average shares outstanding, basic and diluted                       | 34,316                              | 294        | 18,098                             | 147        |

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

|                                      | September 30,<br>2016 | December 31,<br>2015 |
|--------------------------------------|-----------------------|----------------------|
| Cash and cash equivalents            | \$ 290,618            | \$ 75,816            |
| Total assets                         | 301,469               | 82,139               |
| Total liabilities                    | 83,345                | 14,783               |
| Convertible preferred stock          | -                     | 88,557               |
| Total stockholders' equity (deficit) | 218,124               | (21,201)             |

Media Contact:

Jennifer Mound Smoter  
Chief External Affairs & Communications Officer  
+1 857-706-1071  
[jenn.smoter@intelliatx.com](mailto:jenn.smoter@intelliatx.com)

Investor Contacts:

John Graziano  
Trout Group  
+ 1 646-378-2942  
[jgraziano@troutgroup.com](mailto:jgraziano@troutgroup.com)

Chad Rubin  
Trout Group  
+ 1 646-378-2947  
[crubin@troutgroup.com](mailto:crubin@troutgroup.com)



Intellia Therapeutics, Inc.