



Regeneron and Intellia Announce Expanded Research Collaboration to Develop CRISPR-Based Therapies for the Treatment of Neurological and Muscular Diseases

October 3, 2023

- *Collaboration combines Intellia's leading genome editing platform, including its proprietary Nme2Cas9 technology, with Regeneron's proprietary antibody-targeted viral vector delivery technologies to jointly advance in vivo programs outside of the liver for neurological and muscular diseases*

TARRYTOWN, N.Y. and CAMBRIDGE, Mass., Oct. 03, 2023 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ:REGN) and Intellia Therapeutics, Inc. (NASDAQ:NTLA) today announced an expanded research collaboration to develop additional *in vivo* CRISPR-based gene editing therapies focused on neurological and muscular diseases. This builds on the success of the companies' [existing collaboration](#) and continues to combine both companies' deep biology and technology expertise. The collaboration will leverage Regeneron's proprietary antibody-targeted adeno-associated virus (AAV) vectors and delivery systems and Intellia's proprietary Nme2 CRISPR/Cas9 (Nme2Cas9) systems adapted for viral vector delivery and designed to precisely modify a target gene.

"To date, the widespread use of genetic medicines has generally been limited by the inability to deliver a genetic payload to cells of interest in the body beyond the liver. This expansion of our longstanding and productive collaboration with Intellia is taking advantage of new technology and innovations to unlock these opportunities," said Aris Baras, M.D., Senior Vice President and Co-Head of Regeneron Genetic Medicines.

"Regeneron has invented and preclinically validated a proprietary antibody-directed AAV approach that builds on our decades of experience in antibodies and newly developed AAV capsid engineering technologies to deliver innovative payloads across many targeted tissue types and disease settings. We're excited to put this approach to the test in combination with Intellia's industry-leading gene editing systems, in hopes of generating important new medicines for people with serious neurological and muscular diseases," said Christos Kyratsous, Ph.D., Senior Vice President, Research, and Co-Head of Regeneron Genetic Medicines.

"We are excited to expand our successful collaboration with Regeneron to now accelerate the development of CRISPR-based therapies outside of the liver for the treatment of neurological and muscular diseases with significant unmet need," said Intellia President and Chief Executive Officer John Leonard, M.D. "At Intellia, we are continuously innovating our editing and delivery solutions to realize the full potential of CRISPR gene editing as a new therapeutic modality. This collaboration is representative of our long-standing belief that the most groundbreaking solutions will come from selecting the best tools for each individual application, all of which are enabled by our industry-leading genome editing toolbox."

Under the terms of the expanded agreement, the companies will initially research two *in vivo* non-liver targets. Intellia will lead the design of the editing methodology and Regeneron will lead the design of the targeted viral vector delivery approach. Each company will have the opportunity to lead potential development and commercialization of product candidates for one target, and the company that is not leading development and commercialization will have the option to enter into a co-development and co-commercialization agreement for the target.

About Regeneron

Regeneron is a leading biotechnology company that invents, develops, and commercializes life-transforming medicines for people with serious diseases. Founded and led for 35 years by physician-scientists, Regeneron's unique ability to repeatedly and consistently translate science into medicine has led to numerous FDA-approved treatments and product candidates in development, almost all of which were homegrown in Regeneron's laboratories. Regeneron's medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, hematologic conditions, infectious diseases, and rare diseases.

Regeneron is accelerating and improving the traditional drug development process through our proprietary *VelociSuite*[®] technologies, such as *VelocImmune*[®], which uses unique genetically humanized mice to produce optimized fully human antibodies and bispecific antibodies, and through ambitious research initiatives such as the Regeneron Genetics Center, which is conducting one of the largest genetics sequencing efforts in the world.

For additional information about Regeneron, please visit www.regeneron.com or follow Regeneron on [LinkedIn](#).

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 intelliactx.com. Follow us on X (formerly known as Twitter) [@intelliactx](#).

Regeneron Forward-Looking Statements

This press release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. ("Regeneron" or the "Company"), and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of products marketed or otherwise commercialized by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Products") and product

candidates being developed by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Product Candidates") and research and clinical programs now underway or planned, such as the research programs with Intellia Therapeutics, Inc. to develop in vivo CRISPR-based gene editing therapies focused on neurological and muscular diseases discussed in this press release; the potential for any license, collaboration, or supply agreement, including Regeneron's agreements with Sanofi and Bayer (or their respective affiliated companies, as applicable), as well as Regeneron's collaboration with Intellia discussed in this press release, to be cancelled or terminated; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators or licensees (including those conducted as part of the collaboration with Intellia discussed in this press release) may be replicated in other studies and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; the potential of utilizing for therapeutic purposes Regeneron's novel adeno-associated virus (AAV) vectors and delivery systems and Intellia's proprietary Nme2 CRISPR/Cas9 (Nme2Cas9) systems adapted for viral vector delivery as discussed in this press release; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's Product Candidates and new indications for Regeneron's Products; uncertainty of the utilization, market acceptance, and commercial success of Regeneron's Products and Regeneron's Product Candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary, including those discussed or referenced in this press release) on any of the foregoing or any potential regulatory approval of Regeneron's Products and Regeneron's Product Candidates; the ability of Regeneron's collaborators, licensees, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's Products and Regeneron's Product Candidates; the ability of Regeneron to manage supply chains for multiple products and product candidates; safety issues resulting from the administration of Regeneron's Products and Regeneron's Product Candidates in patients, including serious complications or side effects in connection with the use of Regeneron's Products and Regeneron's Product Candidates in clinical trials; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron's ability to continue to develop or commercialize Regeneron's Products and Regeneron's Product Candidates; ongoing regulatory obligations and oversight impacting Regeneron's Products, research and clinical programs, and business, including those relating to patient privacy; the availability and extent of reimbursement of Regeneron's Products from third-party payers, including private payer healthcare and insurance programs, health maintenance organizations, pharmacy benefit management companies, and government programs such as Medicare and Medicaid; coverage and reimbursement determinations by such payers and new policies and procedures adopted by such payers; competing drugs and product candidates that may be superior to, or more cost effective than, Regeneron's Products and Regeneron's Product Candidates; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its financial projections or guidance and changes to the assumptions underlying those projections or guidance; the impact of public health outbreaks, epidemics, or pandemics (such as the COVID-19 pandemic) on Regeneron's business; and risks associated with intellectual property of other parties and pending or future litigation relating thereto (including without limitation the patent litigation and other related proceedings relating to EYLEA® (afibercept) Injection and REGEN-COV® (casirivimab and imdevimab)), other litigation and other proceedings and government investigations relating to the Company and/or its operations, the ultimate outcome of any such proceedings and investigations, and the impact any of the foregoing may have on Regeneron's business, prospects, operating results, and financial condition. A more complete description of these and other material risks can be found in Regeneron's filings with the U.S. Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2022 and its Form 10-Q for the quarterly period ended June 30, 2023. Any forward-looking statements are made based on management's current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update (publicly or otherwise) any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.

Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron's media and investor relations website (<https://investor.regeneron.com>) and its LinkedIn page (<https://www.linkedin.com/company/regeneron-pharmaceuticals>).

Intellia 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: research and development of a proprietary Nme2Cas9 system adapted for viral vector delivery and the combination of that system with Regeneron Pharmaceuticals, Inc.'s ("Regeneron") proprietary antibody-targeted adeno-associated virus vectors and delivery systems to develop product candidates for neurological and muscular diseases, including development and design of editing methodologies for such product candidates; ability to accelerate the development of CRISPR-based therapies outside of the liver for the treatment of neurological and muscular diseases with significant unmet need; advancement and expansion of its CRISPR/Cas9 technology to develop human therapeutic products, including its ability to continuously innovate its editing and delivery solutions to realize the full potential of CRISPR gene editing as a new therapeutic modality and to enable groundbreaking solutions with its industry-leading genome editing toolbox; ability to lead the potential development and commercialization of product candidates for in vivo non-liver targets; ability to maintain and expand its intellectual property portfolio related to CRISPR/Cas9 technology; and ability to optimize the impact of its collaborations on its development programs, including but not limited to its collaboration with Regeneron.

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 Regeneron and its other licensors and licensees; risks related to the ability of its licensors to protect and maintain their intellectual property position; uncertainties related to the research, development and commercialization of product candidates for neurological and muscular diseases, including product candidates that combine Intellia's proprietary Nme2Cas9 system with Regeneron's proprietary antibody-targeted adeno-associated virus vectors and delivery systems; the risk that any one or more of Intellia's product candidates, or product candidates it develops with Regeneron, 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 collaboration with Regeneron 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 or quarterly report of 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. 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.

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Source: Intellia Therapeutics, Inc.