



## Regeneron and Intellia Therapeutics Expand Collaboration to Develop CRISPR/Cas9-Based Treatments

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TARRYTOWN, N.Y. and CAMBRIDGE, Mass., June 1, 2020 /PRNewswire/ --

- *Regeneron and Intellia to co-develop potential hemophilia A and B treatments using their jointly-owned targeted transgene insertion capabilities*
- *Regeneron gains rights to develop products for additional *in vivo* targets and new rights for *ex vivo* product development*
- *Intellia receives \$100 million through upfront cash and equity investment*

Regeneron Pharmaceuticals, Inc. (NASDAQ: **REGN**) and Intellia Therapeutics, Inc. (NASDAQ: **NTLA**) announced an expansion of their [existing collaboration](#) to provide Regeneron with rights to develop products for additional *in vivo* CRISPR/Cas9-based therapeutic targets and for the companies to jointly develop potential products for the treatment of hemophilia A and B. Regeneron also receives non-exclusive rights to independently develop and commercialize *ex vivo* gene edited products. Intellia will receive an upfront payment of \$70 million, and Regeneron will make an additional equity investment in Intellia of \$30 million at \$32.42 per share.

Regeneron and Intellia have worked together to make significant advances with Intellia's CRISPR/Cas9 platform to enable the targeted insertion of therapeutic proteins and antibodies. This collaboration expansion allows the companies to leverage more fully their jointly-developed targeted transgene insertion capabilities and potentially accelerate efforts to discover and develop new therapeutics, including products for hemophilia A and B. In preclinical studies, the companies demonstrated the first CRISPR/Cas9-mediated targeted transgene insertion in the liver of non-human primates, which generated normal or higher levels of circulating human Factor IX. Factor IX is a blood-clotting protein that is missing or defective in hemophilia B patients. These results suggest that transgene insertion may provide a functional *Factor 9* gene, which encodes for this important protein.

"The Regeneron team works hard to push the boundaries of science and technology, and we believe the precise *in vivo* gene insertion capabilities jointly developed with Intellia could be a promising therapeutic platform with significant potential in many diseases, including those that have been historically difficult to treat," said George D. Yancopoulos, M.D., Ph.D., Co-Founder, President and Chief Scientific Officer, Regeneron. "We're pleased to expand our work with Intellia, a like-minded group of scientists focused on maximizing the potential of CRISPR/Cas9 in order to help as many patients as possible."

"We're excited to work with Regeneron on what could potentially be a cure for hemophilia A and B in this expansion of our successful collaboration that builds on our leading insertion capabilities," said Intellia's Chief Executive Officer and President, John M. Leonard, M.D. "We believe that our CRISPR/Cas9-based technology addresses the limitations of current replacement and gene therapy approaches, and importantly, may provide a durable, potentially life-long solution to these genetic diseases."

Under the amended agreement, the term of the companies' existing collaboration is extended until April 2024, with Regeneron having an option to renew for an additional two years. Regeneron will have rights to discover and develop CRISPR/Cas9-based therapeutic products for an additional five *in vivo* liver targets, for a total of up to 15 targets. As currently set forth in the existing collaboration, the parties will jointly research these *in vivo* targets, and thereafter certain targets may be developed by Regeneron or Intellia, or co-developed under certain conditions. Per the terms of the original agreement, Regeneron will pay potential royalties and milestone payments for the *in vivo* products it independently develops. In addition, Regeneron will receive a royalty-bearing, non-exclusive license to certain Intellia intellectual property to develop and commercialize up to 10 *ex vivo* CRISPR/Cas9 products in defined cell types.

### About Regeneron Pharmaceuticals, Inc.

Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents life-transforming medicines for people with serious diseases. Founded and led for 30 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to seven FDA-approved treatments and numerous product candidates in development, all of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, infectious diseases, pain and rare diseases.

Regeneron is accelerating and improving the traditional drug development process through our proprietary *VelociSuite*<sup>®</sup> technologies, including *VelocImmune*<sup>®</sup> which uses a unique genetically-humanized mouse 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 the company, please visit [www.regeneron.com](http://www.regeneron.com) or follow @Regeneron on Twitter.

### About Intellia Therapeutics

Intellia Therapeutics is a leading genome editing company focused on developing proprietary, 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, and through improved cell therapies that can treat cancer and immunological diseases, or can replace patients' diseased cells. The combination of deep scientific, technical and clinical development experience, along with its leading intellectual property portfolio, puts Intellia 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 [intelliatax.com](http://intelliatax.com) and follow us on Twitter @intelliatweets.

### Regeneron Forward-Looking Statements and Use of Digital Media

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 impact of SARS-CoV-2 (the virus that has caused the COVID-19 pandemic) on Regeneron's business and its employees, collaborators, suppliers, and other third parties on which Regeneron relies, Regeneron's and its collaborators' ability to continue to conduct research and clinical programs, Regeneron's ability to manage its supply chain, net product sales of products marketed by Regeneron and/or its collaborators (collectively, "Regeneron's Products"), and the global economy; the nature, timing, and possible success and therapeutic applications of Regeneron's Products and Regeneron's product candidates and research and clinical programs now underway or planned, such as the programs discussed in this press release to develop products for in vivo CRISPR/Cas9-based therapeutic targets with Intellia Therapeutics, Inc. (including for the treatment of hemophilia A and B); the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators (including based on the collaboration discussed in this press release) may lead to advancement of product candidates to clinical trials or therapeutic applications; the potential for any license or collaboration agreement, including Regeneron's agreements with Sanofi, Bayer, and Teva Pharmaceutical Industries Ltd. (or their respective affiliated companies, as applicable), as well as Regeneron's collaboration with Intellia Therapeutics, Inc. discussed in this press release, to be cancelled or terminated without any further product success; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's product candidates and new indications for Regeneron's Products; unforeseen safety issues resulting from the administration of Regeneron's Products and product candidates in patients, including serious complications or side effects in connection with the use of Regeneron's Products and 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 product candidates; ongoing regulatory obligations and oversight impacting Regeneron's Products, research and clinical programs, and business, including those relating to patient privacy; uncertainty of market acceptance and commercial success of Regeneron's Products and product candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary) on the commercial success of Regeneron's Products and product candidates; 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 Regeneron's Products and product candidates; the ability of Regeneron to manufacture and manage supply chains for multiple products and product candidates; the ability of Regeneron's collaborators, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's Products and 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; 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 Dupixent<sup>®</sup> (dupilumab) and Praluent<sup>®</sup> (alirocumab)), 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, 2019 and its Form 10-Q for the quarterly period ended March 31, 2020. 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 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 (<http://newsroom.regeneron.com>) and its Twitter feed (<http://twitter.com/regeneron>).

### **Intellia's 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: planned submission of an investigational new drug ("IND") application or similar clinical trial application for NTLA-2001 for the treatment of transthyretin amyloidosis ("ATTR") in mid-2020 and its planned dosing of first patients in the second half of 2020; plans to submit an IND application or similar clinical trial application for NTLA-5001, its first T cell receptor ("TCR")-directed engineered cell therapy development candidate for its acute myeloid leukemia ("AML") program in the first half of 2021; plans to submit an IND or similar clinical trial application for its hereditary angioedema ("HAE") program in the second half of 2021; plans to advance and complete preclinical studies, including non-human primate studies for its ATTR program and HAE programs, and other animal studies supporting other in vivo and ex vivo programs, including its AML program; development of a proprietary LNP/AAV hybrid delivery system, as well as its modular platform to advance its complex genome editing capabilities, such as gene insertion; further development of its proprietary cell engineering process for multiple sequential editing; presentation of additional data at upcoming scientific conferences, and other preclinical data in 2020; 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; ability to demonstrate its platform's modularity and replicate or apply results achieved in preclinical studies, including those in its ATTR, AML, and HAE programs, in any future studies, including human clinical trials; ability to develop other in vivo or ex vivo cell therapeutics of all types, and those targeting WT1 in AML in particular, using CRISPR/Cas9 technology; ability to optimize the impact of its collaborations on its development programs, including but not limited to its collaborations with Novartis or Regeneron Pharmaceuticals, Inc., Regeneron's ability to enter into a co-development and co-promotion agreement for the HAE program, and the potential timing and receipt of future milestones and royalties based on Intellia's collaboration with Regeneron; and statements regarding the timing of regulatory filings and clinical trial execution, including dosing of patients in its development programs.

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 regulatory agencies' evaluation of regulatory filings and other information related to its product candidates; uncertainties related to the 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 Novartis or Regeneron or its other ex vivo 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. 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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