Intellia Therapeutics Announces $15 Million in Funding to Develop Therapeutic Products Utilizing CRISPR-Cas9 Gene Editing Technology

November 18, 2014

Scientific Pioneers, Industry Veterans Advance Company toward IND Filing

CAMBRIDGE, Mass.—November 18, 2014—Intellia Therapeutics, a new company formed to develop therapeutic products using CRISPR-Cas9 technology for gene editing and repair, announced today that it has closed a Series A investment round with $15 million in financing led by Atlas Venture and Novartis. Created by Atlas Venture and Caribou Biosciences, together with leading scientists who have pioneered and shaped the field of CRISPR biology, Intellia leverages exclusive access to one of the most comprehensive intellectual property portfolios covering the therapeutic application of this transformative technology.

"Discovery of the CRISPR-Cas9 system has been a significant advance toward the long-elusive therapeutic goal of targeting and repairing specific genetic defects," said Nessan Bermingham, Ph.D., Chief Executive Officer and co-founder of Intellia. "We have assembled an experienced team with a track record of success in all phases of development, from discovery through translation, clinical testing and commercialization. Together with our key advisors, we are focused squarely on clinical drug development as we progress toward our first IND filing. Our goal is to ensure that patients suffering from genetic-based diseases gain access to these potentially life-altering therapeutics as swiftly as possible."

Since the completion of the Human Genome Project in 2000, physicians have sought an approach capable of translating emerging genomic insights into meaningful medicines. The CRISPR-Cas9 system, whose mechanism was revealed in 2012, has emerged as the leading technology for therapeutic gene modification. CRISPR-Cas9 harnesses the body's natural DNA repair machinery to enable the repair, knockout or replacement of specific genes in the human genome.

"We believe the impact of this technology will be far-reaching, leading to new therapies for diseases that have been underserved with current therapeutic approaches," said Jean-François Formela, M.D., Partner, Atlas Venture. "Intellia was created to play a leading role in CRISPR-Cas9 therapeutic development, which will be greatly accelerated by decades of innovation across cell and gene therapy, RNA modification and stabilization and oligonucleotide delivery."

"Translating the potential of the CRISPR-Cas9 technology to focused pre-clinical and clinical development programs at Intellia is an exciting step forward in the evolution of our cell engineering platform," said Rachel Haurwitz, Ph.D., Chief Executive Officer and co-founder of Caribou Biosciences. "The management team, advisors and investors assembled at Intellia have an outstanding track record of developing therapeutics that will transform the lives of patients."

Development Focus

Intellia's initial therapeutic focus is *ex vivo* applications, wherein cells are removed from the body (collected from blood or bone marrow), modified to correct disease-causing genes and returned to the patient for therapeutic benefit. Near-term *ex vivo* applications include blood disorders, therapeutic protein production and cancer, focused on such approaches as CAR-T and checkpoint inhibitor regulation.

Intellia has also initiated longer-term development of *in vivo* applications, administered either systemically or locally to correct genes residing within specific cells of the body. *In vivo* applications include ophthalmic, central nervous system (CNS), muscle, liver, anti-infective and other disease states.

Management Team and Directors

To accelerate these efforts, Intellia has assembled a fully integrated management team with deep commercial, clinical, scientific and technical experience. In addition to founding Chief Executive Officer, Nessan Bermingham, Intellia is led by Chief Scientific Officer Thomas Barnes, Ph.D., former Vice President of Discovery at Eleven Biotherapeutics; Chief Medical Officer John Leonard, M.D., former Chief Scientific Officer at AbbVie; Chief Technology Officer David Morrissey, Ph.D., former Head of siRNA at Novartis; and General Counsel José Rivera, former Divisional Vice President and Associate General Counsel, Intellectual Property Group at Abbott.

Teams are aligned around three priority areas – platform development, delivery technology and prioritization of therapeutic applications – with specialized scientific advisory boards dedicated to each.

The company's founders and Board of Directors include Nessan Bermingham, Rachel Haurwitz, Ph.D., Chief Executive Officer and co-founder of Caribou Biosciences; Andy May, Ph.D., Chief Scientific Officer of Caribou Biosciences; Rodolphe Barrangou, Ph.D., Associate Professor at North Carolina State University; Erik Sontheimer, Ph.D., Professor at University of Massachusetts Medical School; Luciano Marraffini, Ph.D., Assistant Professor at Rockefeller University; and Derrick Rossi, Ph.D., Assistant Professor at Harvard Medical School. Additional board members include

Jean-François Formela, M.D., Partner at Atlas Venture, and John Leonard, Chief Medical Officer at Intellia.

About Intellia Therapeutics

Intellia Therapeutics was formed in 2014 to lead the industry in one of the most promising new areas of therapeutic development: gene editing and repair using CRISPR-Cas9 technology. Intellia holds exclusive access to one of the most comprehensive intellectual property platforms available for the therapeutic use of CRISPR-Cas9. The company is advancing a broad pipeline toward clinical development, including *ex vivo* and *in vivo* approaches. Intellia closed a Series A round in 2014 led by Atlas Venture and Novartis.

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