



CRISPR Therapeutics and Casebia Therapeutics Announce Exclusive Development and Option Agreement with StrideBio

April 17, 2017

CRISPR Therapeutics and Casebia will partner with StrideBio to develop novel AAV vectors for in vivo CRISPR/Cas9-based therapies

BASEL, Switzerland and CAMBRIDGE, Mass. and DURHAM, N.C., April 17, 2017 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (NASDAQ:CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, and Casebia Therapeutics, a joint-venture established by CRISPR Therapeutics and Bayer AG for developing CRISPR-based therapeutics in select disease areas, today announced they have signed a collaboration agreement with StrideBio, LLC, a US-based company developing novel AAV vectors for *in vivo* gene delivery applications.

Under the terms of the agreement, StrideBio will use its proprietary platform to develop AAV vectors with improved properties such as tissue specificity and reduced susceptibility to immune responses. CRISPR Therapeutics and Casebia will have an option to exclusively license AAV vectors with desired properties for use in their *in vivo* gene-editing programs. StrideBio will receive development funding, milestones and royalties on licensed vectors, and retain certain rights to use the novel AAV vectors for gene therapy applications.

"Beyond our *ex vivo* lead programs, we see tremendous potential to apply our CRISPR/Cas9 platform to diseases requiring *in vivo* delivery. StrideBio has a unique platform that can identify next-generation AAV vectors which are much more effective than those currently in use. This partnership positions us at the forefront of viral delivery technology for our *in vivo* programs," said **Samarth Kulkarni, Chief Business Officer of CRISPR Therapeutics**.

StrideBio's AAV vector development platform makes use of structure-guided evolution to discover novel capsids with improved properties for *in vivo* gene delivery applications. The underlying technology is based on the work of Dr. Aravind Asokan at the University of North Carolina at Chapel Hill and Dr. Mavis Agbandje-McKenna at the University of Florida.

"This collaboration is another example of how CRISPR Therapeutics and Casebia can partner to improve our shared gene editing platform. Casebia has a focus on *in vivo* gene editing in the liver, eye, and heart where AAV vectors are a promising delivery modality, and where StrideBio's improved vectors can advance our ability to develop effective therapeutics," said **Jim Burns, President and CEO of Casebia**.

"We founded StrideBio to meet the challenge of pre-existing immunity in patients. Building on our deep understanding of how neutralizing antibodies interact with different AAV serotypes, we have established a unique technology platform that will allow us to evolve advanced AAV vectors that are both resistant to pre-existing immunity and have improved tissue specificity. This collaboration with CRISPR Therapeutics and Casebia will allow us to greatly accelerate our progress in that area," said **Aravind Asokan, co-founder of StrideBio, LLC**.

About CRISPR Therapeutics

CRISPR Therapeutics is a leading gene-editing company focused on developing transformative gene-based medicines for serious diseases using its proprietary CRISPR/Cas9 gene-editing platform. CRISPR / Cas9 is a revolutionary technology that allows for precise, directed changes to genomic DNA. The company's multi-disciplinary team of world-class researchers and drug developers is working to translate this technology into breakthrough human therapeutics in a number of serious diseases. Additionally, CRISPR Therapeutics has established strategic collaborations with Bayer AG and Vertex Pharmaceuticals to develop CRISPR-based therapeutics in diseases with high unmet need. The foundational CRISPR/Cas9 patent estate for human therapeutic use was licensed from the company's scientific founder Emmanuelle Charpentier, Ph.D. CRISPR Therapeutics is headquartered in Basel, Switzerland with its R&D operations based in Cambridge, Massachusetts. For more information, please visit www.crisprtx.com.

About Casebia Therapeutics

Casebia Therapeutics is a joint venture between CRISPR Therapeutics and Bayer AG, focused on discovering, developing and commercializing new CRISPR/Cas9-based breakthrough therapeutics to treat blood disorders, blindness, and heart disease. Formed in the first quarter of 2016, the company began operations in the U.S. in August of 2016. Casebia has access to gene-editing technology from CRISPR Therapeutics in specific disease areas, as well as access to protein engineering expertise and relevant disease know-how through Bayer. Casebia is a free-standing entity, equally owned by CRISPR Therapeutics and Bayer, with its own scientific leadership and management team. Casebia is headquartered in Cambridge, MA with research operations in Cambridge, MA, and San Francisco, CA. For more information, please visit www.casebia.com.

About StrideBio

StrideBio, LLC is a gene therapy company focused on creating and developing novel adeno-associated viral (AAV) vector technologies and therapeutics for rare diseases. Our **STR**ucture Inspired **DE**sign approach holds the potential to generate unique AAV capsids capable of overcoming the challenges of pre-existing neutralizing antibodies and improving gene transfer efficiency in patients. This powerful new approach has broad application, enabling gene addition, gene silencing and gene editing modalities for a wide range of diseases, including rare genetic diseases. StrideBio is headquartered in Durham, NC. For more information, please visit www.stridebio.com.

CRISPR Forward-Looking Statement

Certain statements set forth in this press release constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, but not limited to, statements concerning: the therapeutic value, development, and commercial potential of CRISPR/Cas-9 gene editing technologies and therapies and the intellectual property protection of our technology and therapies. You are cautioned

that forward-looking statements are inherently uncertain. Although the company believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, the forward-looking statements are neither promises nor guarantees and they are necessarily subject to a high degree of uncertainty and risk. Actual performance and results may differ materially from those projected or suggested in the forward-looking statements due to various risks and uncertainties. These risks and uncertainties include, among others: uncertainties inherent in the initiation and completion of preclinical and clinical studies for the Company's product candidates; uncertainties regarding the intellectual property protection for our technology and intellectual property belonging to third parties; availability and timing of results from preclinical and clinical studies; whether results from a preclinical trial will be predictive of future results of the future trials; expectations for regulatory approvals to conduct trials or to market products; and those risks and uncertainties described in Item 1A under the heading "Risk Factors" in the company's most recent annual report on Form 10-K, and in any other subsequent filings made by the company with the U.S. Securities and Exchange Commission (SEC), which are available on the SEC's website at www.sec.gov. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date they are made.

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