



# CRISPR Therapeutics and Capsida Biotherapeutics Announce Strategic Collaboration to Develop Gene-Edited Therapies for Amyotrophic Lateral Sclerosis and Friedreich's Ataxia

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*- Collaboration leverages expertise and capabilities of both companies towards developing life-changing treatments for severe neurological diseases -*

ZUG, Switzerland and CAMBRIDGE, Mass. and THOUSAND OAKS, Calif., June 15, 2021 (GLOBE NEWSWIRE) -- [CRISPR Therapeutics](#) (Nasdaq: CRSP), a biopharmaceutical company focused on developing transformative gene-based medicines for serious diseases, and [Capsida Biotherapeutics Inc.](#), a biotechnology company dedicated to developing breakthrough gene therapies using fully integrated adeno-associated virus (AAV) engineering, cargo development and manufacturing, today announced a strategic partnership to research, develop, manufacture and commercialize *in vivo* gene editing therapies delivered with engineered AAV vectors for the treatment of familial amyotrophic lateral sclerosis (ALS) and Friedreich's ataxia.

Under the agreement, CRISPR Therapeutics will lead research and development of the Friedreich's ataxia program and perform gene-editing activities for both programs, and Capsida will lead research and development of the ALS program and conduct capsid engineering for both programs. Capsida's high-throughput AAV engineering platform generates capsids optimized to target specific tissue types and limits transduction of tissues and cell types that are not relevant to the target disease, potentially allowing for improved efficacy and safety. CRISPR Therapeutics and Capsida



equally share all research, development and commercialization costs and profits worldwide related to the collaboration product. As part of the collaboration, Capsida will also be responsible for process development and clinical manufacture of both programs and have the option to manufacture commercial products generated under the agreement.

“We are excited to enter this collaboration with Capsida. The combination of Capsida’s AAV engineering platform and CRISPR Therapeutics’ gene-editing platform has the potential to enable transformative gene-edited therapies for patients with neurological diseases,” said Samarth Kulkarni, Ph.D., Chief Executive Officer of CRISPR Therapeutics. “This new partnership is one more step in our overall strategy of bringing together innovative and complementary technologies to unlock the full potential of our core platform.”

“Bringing together Capsida’s fully integrated, tissue targeting gene therapy platform with CRISPR Therapeutics’ leading gene-editing capabilities gives us the potential to develop first-in-class gene therapies for patients with severe neurological disorders and expand the reach of Capsida’s broadly enabling capabilities,” said Robert Cuddihy, M.D., Chief Executive Officer of Capsida Biotherapeutics.

### **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 platform. CRISPR/Cas9 is a revolutionary gene editing technology that allows for precise, directed changes to genomic DNA. CRISPR Therapeutics has established a portfolio of therapeutic programs across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine and rare diseases. To accelerate and expand its efforts, CRISPR Therapeutics has established strategic collaborations with leading companies including Bayer, Vertex Pharmaceuticals and ViaCyte, Inc. CRISPR Therapeutics AG is headquartered in Zug, Switzerland, with its wholly-owned U.S. subsidiary, CRISPR Therapeutics, Inc., and R&D operations based in Cambridge, Massachusetts, and business offices in San Francisco, California and London, United Kingdom. For more information, please visit [www.crisprtx.com](http://www.crisprtx.com).



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### **CRISPR Therapeutics Forward-Looking Statement**

*This press release may contain a number of “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including statements made by Dr. Kulkarni and Dr. Cuddihy in this press release, as well as statements regarding CRISPR Therapeutics’ expectations about any or all of the following: (i) the future activities of the parties pursuant to the collaboration and the expected benefits of CRISPR Therapeutics’ collaboration with Capsida; and (ii) the therapeutic value, development, and commercial potential of CRISPR/Cas9 gene editing technologies and therapies. Without limiting the foregoing, the words “believes,” “anticipates,” “plans,” “expects” and similar expressions are intended to identify forward-looking statements. You are cautioned that forward-looking statements are inherently uncertain. Although CRISPR Therapeutics believes that such statements are based on reasonable assumptions within the bounds of its knowledge of its business and operations, 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: CRISPR Therapeutics may not realize the potential benefits of the collaboration; uncertainties inherent in the initiation and completion of preclinical studies, including availability and timing of results from preclinical studies; whether results from a preclinical study will be favorable and predictive of future results of future studies or clinical trials; uncertainties about regulatory approvals and that future competitive or other market factors may adversely affect the commercial potential for product candidates; potential impacts due to the coronavirus pandemic, such as the timing and progress of preclinical studies; uncertainties regarding the intellectual property protection for CRISPR Therapeutics’ technology and intellectual property belonging to third parties, and the outcome of proceedings (such as an interference, an opposition or a similar proceeding) involving all or any portion of such intellectual property; and those risks and uncertainties described under the heading “Risk Factors” in CRISPR Therapeutics’ most recent annual report on Form 10-K, quarterly report on Form 10-Q, and in any other subsequent filings made by CRISPR Therapeutics with the U.S. Securities and Exchange Commission, which are available on the SEC’s website at [www.sec.gov](http://www.sec.gov). Existing and prospective*



*Therapeutics disclaims any obligation or undertaking to update or revise any forward-looking statements contained in this press release, other than to the extent required by law.*

### **About Capsida Biotherapeutics**

Capsida Biotherapeutics Inc. is developing tissue-targeted gene therapies using its biologically driven, high-throughput adeno-associated virus (AAV) engineering and proprietary cargo development platform. As a fully integrated gene therapy company, Capsida is combining its differentiated AAV engineering and screening capabilities with cargo development and state-of-the-art manufacturing to establish a proprietary pipeline of groundbreaking gene therapies across a range of therapeutic areas for indications that are unreachable with current technologies. The company's leadership is backed by decades of successful biologics manufacturing experience and deep AAV biology expertise. Visit us at [www.capsida.com](http://www.capsida.com) to learn more.

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May 25, 2021 08:00 ET

Source: [CRISPR Therapeutics AG](#)

May 12, 2021 11:00 ET

Source: [CRISPR Therapeutics AG](#)



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CAMBRIDGE, Mass. and ZUG, Switzerland and BOSTON, May 12, 2021 (GLOBE NEWSWIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and CRISPR Therapeutics (Nasdaq: CRSP) today announced two...

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