



## **CRISPR Therapeutics to Present Oral Presentation at the American Society of Gene and Cell Therapy (ASGCT) 2024 Annual Meeting**

ZUG, Switzerland and BOSTON, April 22, 2024 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today announced an oral presentation highlighting the Company's lipid nanoparticle (LNP) approach for ocular editing will be presented at the American Society of Gene & Cell Therapy (ASGCT) 2024 Annual Meeting, taking place May 7 – 11, 2024, in Baltimore, MD and virtually.

The abstract describes our proprietary capabilities to deliver to and edit genes in the eye, opening a potential new focus area. Multiple LNPs as well as modified gRNAs and mRNAs were screened to achieve maximal editing *in vivo*. These optimized components have been applied to target myocilin (MYOC). Mutations of MYOC in trabecular meshwork cells have been linked to severe glaucomatous conditions. In human primary trabecular meshwork cells, up to 95% MYOC editing and 85% protein knockdown were seen. This novel approach aims to facilitate glaucoma treatment using transient expression of editing machinery targeting MYOC.

**Title:** Development of an *In Vivo* Non-Viral Ocular Editing Platform and Application to Potential Treatments for Glaucoma

**Session Type:** In-Person Oral Presentation

**Session Title:** Ophthalmic and Auditory: Delivery Innovations

**Abstract Number:** 87

**Location:** Room 318 – 323

**Session Date and Time:** Wednesday, May 8, 2024, 1:30 p.m. – 3:15 p.m. ET

The accepted abstract is available online on the [ASGCT website](https://www.asgct.org/). The data are embargoed until 6:00 a.m. ET on the presentation day, Wednesday May 8, 2024. A copy of the presentation will be available at [www.crisprtx.com](https://www.crisprtx.com) once the presentation concludes.

### **About CRISPR Therapeutics**

Since its inception over a decade ago, CRISPR Therapeutics has transformed from a research-stage company advancing programs in the field of gene editing, to a company that recently celebrated the historic approval of the first-ever CRISPR-based therapy and has a diverse portfolio of product candidates across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine, cardiovascular, autoimmune, and rare diseases. CRISPR Therapeutics advanced the first-ever CRISPR/Cas9 gene-edited therapy into the clinic in 2018 to investigate the treatment of sickle cell disease or transfusion-dependent beta thalassemia, and beginning in late 2023, CASGEVY™ (exagamglogene autotemcel) was approved in some countries to treat eligible patients with either of those conditions. The Nobel Prize-winning CRISPR science has revolutionized biomedical research and represents a powerful, clinically validated approach with the potential to create a new class of potentially transformative medicines. To accelerate and expand its efforts, CRISPR Therapeutics has established strategic partnerships with leading companies including Bayer and Vertex Pharmaceuticals. CRISPR Therapeutics AG is headquartered in Zug, Switzerland, with its wholly-owned U.S. subsidiary, CRISPR Therapeutics, Inc., and R&D operations based in Boston, Massachusetts and San Francisco, California, and business offices in London, United Kingdom. To learn more, visit [www.crisprtx.com](https://www.crisprtx.com).

### **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 regarding CRISPR Therapeutics’ expectations about any or all of the following: (i) its ongoing and/or planned preclinical studies, clinical trials and pipeline products and programs, including, without limitation, the status of such studies and trials, potential expansion into new indications and expectations regarding data generally (including expected timing of data releases) as well as the data in the above-described abstract and any associated poster and the data that is being presented as described above; (ii) the safety, efficacy and clinical progress of its various clinical and preclinical programs including the program described in the oral presentation and poster; (iii) the data that will be generated by ongoing and planned preclinical studies and/or clinical trials, and the ability to use that data for the design and initiation of further preclinical studies and/or clinical trials; and (iv) 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: the efficacy and safety results from ongoing pre-clinical studies and/or clinical trials will not continue or be repeated in ongoing or planned pre-clinical studies and/or clinical trials or may not support regulatory submissions; pre-clinical study and/or clinical trial results may not be favorable or support further development; one or more of its product candidate programs will not proceed as planned for technical, scientific or commercial reasons; future competitive or other market factors may adversely affect the commercial potential for its product candidates; uncertainties inherent in the initiation and completion of preclinical studies for its product candidates and whether results from such studies will be predictive of future results of future studies or clinical trials; uncertainties about regulatory approvals to conduct trials or to market products; uncertainties regarding the intellectual property protection for its 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 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). CRISPR 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.

**Investor Contact:**

Susan Kim

+1-617-307-7503

[susan.kim@crisprtx.com](mailto:susan.kim@crisprtx.com)

**Media Contact:**

Rachel Eides

+1-617-315-4493

[rachel.eides@crisprtx.com](mailto:rachel.eides@crisprtx.com)



Source: CRISPR Therapeutics AG