CRISPR Therapeutics to Present Preclinical Data on Alpha-1 Antitrypsin Deficiency (AATD) Utilizing Novel SyNTase Gene Editing Technology at the European Society of Gene and Cell Therapy (ESGCT) 2025 Annual Congress

ZUG, Switzerland and BOSTON, Oct. 01, 2025 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today announced the acceptance of an abstract for oral presentation at the European Society of Gene and Cell Therapy (ESGCT) 2025 Annual Congress, taking place October 7-10, 2025. The presentation will introduce the Company's novel SyNTase gene editing technology and highlight its application in single-dose *in vivo* gene correction to treat Alpha-1 Antitrypsin Deficiency (AATD), a rare genetic disorder.

CRISPR Therapeutics has developed SyNTase editing, a proprietary, next-generation, site-specific gene correction platform. SyNTase editors represent a significant advance over currently described prime editing systems by combining compact Cas9 proteins with a novel class of engineered polymerases. Together, these components enable gene editing with greater efficiency and precision, while also supporting scalable manufacturing.

Using AI-guided structural modeling and large-scale screening, the polymerase was optimized to support gene correction activity based on synthetic nucleotide templates. When integrated with Cas9, SyNTase editors can utilize engineered templates with improved serum stability, enabling higher target correction efficiency.

The abstract describes that SyNTase editing produces high levels of editing (up to 95%) in SERPINA1-E342K human hepatocyte cell models without any detectable (<0.5%) off-target effects. In a humanized mouse model, SyNTase editing components encapsulated in a lipid nanoparticle (LNP) enabled highly efficient, specific, and potentially curative gene correction with a single intravenous (IV) dose (≤0.5 mg/kg) with a well-tolerated safety profile. In a custom humanized rat model of AATD, SyNTase editing achieved potent gene correction of the E342 mutation with >70% mRNA correction and >3-fold total serum AAT upregulation, exceeding the established clinically protective threshold. Together, these data provide proof-of-concept for a potentially best-in-class therapeutic modality to address the underlying cause of AATD and support SyNTase editing as a promising platform for the treatment of many monogenic disorders. Additional results beyond those included in the abstract will be presented at the conference.

Presentation Details
Title: Single-dose *in vivo* gene correction of AATD via LNP-delivered SyNTase editors
Abstract Number: OR096
Session Type: Oral Presentation
Session Title: SESSION 12c: Gene Editing III: Technology & applications
Session Date and Time: Friday, October 10, 2025, 11:00 a.m. – 1:00 p.m. CEST

The accepted abstract is available online on the ESGCT website for congress registrants. Any updated data, new graphics, and follow-up information to be presented during the oral presentation sessions is embargoed until 8:00 a.m. CEST on the day of the presentation. A copy of the presentation will be available at www.crisprtx.com once the presentation concludes.

About CRISPR Therapeutics
Since its inception over a decade ago, CRISPR Therapeutics has evolved from a research-stage company advancing gene editing programs into a leader that celebrated the historic approval of the first-ever CRISPR-based therapy. The Company has a diverse portfolio of product candidates across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine, cardiovascular, autoimmune, and rare diseases. In 2018, CRISPR Therapeutics advanced the first-ever CRISPR/Cas9 gene-edited therapy into the clinic to investigate the treatment of sickle cell disease and transfusion-dependent beta thalassemia. Beginning in late 2023, CASGEVY® (exagamglogene autotemcel [exa-cel]) was approved in several countries to treat eligible patients with either of these conditions. The Nobel Prize-winning CRISPR technology 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 formed strategic partnerships with leading companies including 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. To learn more, visit www.crisprtx.com.

CRISPR Therapeutics Forward-Looking Statement

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, statements regarding any or all of the following: (i) CRISPR Therapeutics preclinical studies, clinical trials and pipeline products and programs, including, without limitation, manufacturing capabilities, status of such studies and trials and expectations regarding data, safety and efficacy generally; (ii) data included in the above-described oral presentation and above-described abstract and any associated poster; and (iii) the therapeutic value, development, and commercial potential of gene editing technologies and therapies, including CRISPR/Cas9, as well as other technologies. Risks that contribute to the uncertain nature of the forward-looking statements include, without limitation, the risks and uncertainties discussed 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. 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. We disclaim 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.

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