CRISPR Therapeutics Presents Phase 1b Data for CTX310® Demonstrating Reductions in ANGPTL3 and Triglycerides in Patients with Severe Hypertriglyceridemia at the American Heart Association (AHA) Scientific Sessions 2026

-Data presented in a late-breaking oral presentation at AHA-

-A single-course treatment with CTX310 produced a mean reduction from baseline of 65% in circulating ANGPTL3 and 63% in triglycerides (TG) at Day 30 in patients with severe hypertriglyceridemia (sHTG), who had a median baseline TG level above 1,000 mg/dL-

-CTX310 was well tolerated, with no treatment-related serious adverse events and no clinically relevant elevations in liver transaminases-

ZUG, Switzerland and BOSTON, Nov. 09, 2026 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP) today presented clinical data from its Phase 1b clinical trial evaluating CTX310®, an investigational, *in vivo* CRISPR/Cas9 gene editing therapy targeting angiopoietin-like protein 3 (ANGPTL3), in patients with severe hypertriglyceridemia (sHTG). A single-course treatment with CTX310 produced mean reductions from baseline at Day 30 of 65% in circulating ANGPTL3 and 63% in triglycerides (TG) in participants with a median baseline TG level above 1,000 mg/dL. These results show that a single-course treatment with CTX310 can deeply reduce ANGPTL3 and triglycerides in patients with severe hypertriglyceridemia, reinforcing its potential as a one-time gene editing treatment for this high-risk population.

These data were presented today during a late-breaking session at the AHA Scientific Sessions 2026 and build on the Phase 1a results, including one-year durability data presented at the European Society of Cardiology (ESC) Congress 2026 and published in *The New England Journal of Medicine*. The U.S. Food and Drug Administration (FDA) recently granted Regenerative Medicine Advanced Therapy (RMAT) designations to CTX310 for the treatment of sHTG and for the treatment of patients with elevated low-density lipoprotein cholesterol (LDL-C). CRISPR Therapeutics continues to advance CTX310 in the Phase 1b clinical trial, with U.S. and ex-U.S. trials ongoing.

“Severe hypertriglyceridemia is a serious condition associated with an elevated risk of acute pancreatitis and cardiovascular disease, and many patients continue to have elevated triglycerides despite currently available therapies,” said Naimish Patel, M.D., Chief Medical Officer, CRISPR Therapeutics. “These are the first data for CTX310 in a dedicated cohort of patients with severe hypertriglyceridemia, and they show substantial triglyceride lowering after a single-course treatment with CTX310. We are encouraged by these results in a population with significant unmet need.”

“In participants with severe hypertriglyceridemia, a single-course treatment with CTX310 was well tolerated and substantially lowered triglyceride levels,” said Ashish Sarraju, M.D., preventive cardiologist, Cleveland Clinic, who presented the data. “These results are promising and represent a new pathway for lipid management, with the potential of a one-time treatment to address multiple lipid disorders that remain difficult to control with current therapies.”

Phase 1b Clinical Trial Design

The Phase 1b portion of the study is a multicenter, open-label trial. In the sHTG cohort, 10 participants with fasting serum TG above 500 mg/dL received a single intravenous (IV) infusion of CTX310 at a fixed flat dose of 40 mg (body weight below 85 kg) or 50 mg (body weight of 85 kg or above). The weight-based flat dosing approach was designed to approximate the 0.8 mg/kg dose evaluated in the Phase 1a trial. The cohort includes one participant from Phase 1a who received a dose of 56.5 mg. The trial is evaluating safety and tolerability, including dose-limiting toxicities, as well as percent changes over time in ANGPTL3, TG and other lipid parameters, including non-high-density lipoprotein cholesterol (non-HDL-C). The trial is enrolling at sites in New Zealand, Australia, the United Kingdom and the United States.

At baseline, participants had a median TG level above 1,000 mg/dL. All participants had diabetes, three (30%) had a history of pancreatitis, and two (20%) had clinical atherosclerotic cardiovascular disease.

Safety and Tolerability

A single-course treatment with CTX310 was well tolerated. As of the data cutoff, seven participants had been followed for at least 90 days. There were no serious adverse events related to CTX310 and no clinically relevant elevations in aspartate

aminotransferase (AST) or alanine aminotransferase (ALT). One participant experienced a Grade 2 infusion-related reaction, which resolved, and the participant was able to complete the infusion. There were no Grade 3 or higher adverse events related to CTX310.

Efficacy Highlights

These results include clinical data from 10 participants with sHTG who received a single-course treatment with CTX310. All participants had at least 30 days of follow-up as of the data cutoff.

Following a single-course treatment with CTX310, the following reductions were observed at Day 30:

- A mean reduction in circulating ANGPTL3 protein of 65%.
- A mean TG reduction of 63%.
- Mean reductions of 51% in non-HDL-C and 23% in apolipoprotein B (ApoB), reflecting reductions in atherogenic lipoproteins, and a mean LDL-C reduction of 16% from a low mean baseline of 60 mg/dL.
- Reductions in ANGPTL3 and TG were maintained in participants followed for at least 90 days.
- No events of acute pancreatitis were reported on study, in a population in which 30% of participants had a history of pancreatitis.
- Together, these data support the potential of a single-course treatment with CTX310 to deliver substantial reductions in triglycerides and atherogenic lipoproteins with a favorable tolerability profile in patients with sHTG.

CRISPR Therapeutics continues to advance CTX310 across multiple lipid disorders, with additional Phase 1b cohorts ongoing.

About *In Vivo* Liver Editing Programs

CRISPR Therapeutics has established a proprietary lipid nanoparticle (LNP) delivery platform to enable gene editing in the liver using both CRISPR/Cas9 and its novel, proprietary SyNTase™ editing technology. The Company's *in vivo* portfolio includes three cardiovascular programs: CTX310, targeting angiopoietin-like protein 3 (ANGPTL3), in development for heterozygous and homozygous familial hypercholesterolemia, mixed dyslipidemias, and severe hypertriglyceridemia; CTX340™, targeting angiotensinogen (AGT), in development for refractory hypertension; and CTX321™, targeting LPA, in development for patients with elevated lipoprotein(a) [Lp(a)]. In addition, the Company's disclosed development candidates also include CTX460™, targeting SERPINA1 using SyNTase editing, for the treatment of alpha-1 antitrypsin deficiency (AATD).

About CRISPR Therapeutics

CRISPR Therapeutics is a leading biopharmaceutical company focused on developing transformative gene-based medicines for serious human diseases. Founded over a decade ago as an early pioneer in CRISPR/Cas9 gene editing, the Company has evolved from a pioneering research-stage organization into an industry leader, marking a historic milestone with the approval of CASGEVY® (exagamglogene autotemcel [exa-cel]), the world's first CRISPR-based therapy, for eligible patients with sickle cell disease and transfusion-dependent beta thalassemia. Today, CRISPR Therapeutics is advancing a broad, diversified pipeline spanning hemoglobinopathies, cardiovascular disease, autoimmune disease, oncology, regenerative medicine and rare diseases. The Company is also expanding its gene editing toolkit through SyNTase™ editing, its novel, proprietary platform designed to enable precise, efficient, and scalable gene correction. To accelerate its impact, CRISPR Therapeutics has established strategic collaborations with leading biopharmaceutical partners, 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.

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References

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2. Laffin L, et al. Durability of CRISPR-Cas9 Gene Editing Targeting ANGPTL3 with CTX310. The New England Journal of Medicine. 2026.

CRISPR Special Note Regarding Forward-Looking Statements

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, potential expansion into new indications and expectations regarding data, safety and efficacy generally; (ii) data included in the above-described oral presentation and any associated abstracts or presentations, as well as the ability to use data from ongoing and planned clinical trials for the design and initiation of further clinical trials; and (iii) the therapeutic value, development, and commercial potential of gene editing technologies and therapies, including CRISPR/Cas9 and SyNTase, 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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