CRISPR Therapeutics Provides Business Update and Reports Third Quarter 2026 Financial Results

– CASGEVY® revenue of $99 million in the third quarter of 2026, up 30% quarter-over-quarter and nearly six times the third quarter of 2025 –

– CASGEVY approved in the U.K. for children 5 years of age and older with severe SCD or TDT, and in the United Arab Emirates and Bahrain for children 2 years of age and older –

– FDA granted Regenerative Medicine Advanced Therapy (RMAT) designations to CTX310® for severe hypertriglyceridemia and elevated LDL-C –

– Phase 1b data for CTX310 in severe hypertriglyceridemia to be presented in a late-breaking oral presentation at AHA Scientific Sessions 2026; Phase 1 data for zugo-cel in systemic sclerosis to be presented at ACR Convergence 2026 –

– Completed enrollment in the Phase 2 clinical trial of CTX611 in patients undergoing total knee arthroplasty –

– Appointed Siva Sakhamuri, Ph.D., as Chief Technical Officer and A.J. Silver as Chief Financial Officer –

ZUG, Switzerland and BOSTON, Nov. 02, 2026 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP) today reported financial results for the third quarter ended September 30, 2026.

"The third quarter marked meaningful progress for CRISPR Therapeutics," said Samarth Kulkarni, Ph.D., Chairman and Chief Executive Officer of CRISPR Therapeutics. "CASGEVY is reaching more patients, including younger children following new approvals in the U.K. and the Middle East, and the FDA's RMAT designations for CTX310 underscore its potential to address significant unmet needs in cardiovascular disease. With new clinical data from CTX310 and zugo-cel in the coming weeks and enrollment complete in our Phase 2 trial of CTX611, we are entering a pivotal period for our pipeline."

Recent Highlights and Outlook

Hemoglobinopathies and CASGEVY® (exagamglogene autotemcel)

- CASGEVY, the world's first approved CRISPR-based therapy, has been shown to reduce or eliminate vaso-occlusive crises (VOCs) in eligible patients with sickle cell disease (SCD) and transfusion requirements in eligible patients with transfusion-dependent beta thalassemia (TDT). CASGEVY is approved in 39 countries across North America, Europe, and the Middle East for eligible patients 12 years of age and older, with approvals for younger children in five of those countries.
- CASGEVY generated third quarter 2026 revenue of $99 million, representing 30% growth quarter-over-quarter and nearly six times the revenue reported in the third quarter of 2025. Twice as many in the first nine months of 2026 as in all of 2025, and more than 750 patients have initiated treatment worldwide.
- CASGEVY's availability for younger patients continues to expand. In July, the FDA approved CASGEVY for children 2 years of age and older with SCD or TDT, making it the first genetic therapy for SCD and TDT approved for this age group, and multiple children have since initiated treatment in the U.S. The Kingdom of Saudi Arabia, the United Arab Emirates, and the Kingdom of Bahrain have also approved CASGEVY for children 2 years of age and older, and the U.K. Medicines and Healthcare products Regulatory Agency (MHRA) approved CASGEVY for children 5 years of age and older with severe SCD or TDT.
- Building on CASGEVY, CRISPR Therapeutics is advancing an *in vivo* hematopoietic stem cell editing approach that could extend gene editing to many more patients with SCD and TDT. Using lipid nanoparticle (LNP)-mediated delivery, the approach is designed to edit stem cells directly in the body, potentially simplifying treatment and broadening global access.

***In Vivo* Liver Editing**

CRISPR Therapeutics continues to advance a diversified portfolio of *in vivo* gene editing programs leveraging its proprietary liver-

directed LNP delivery platform.

- The Phase 1 clinical trial of CTX310®, targeting angiopoietin-related protein 3 (ANGPTL3) is ongoing.
- CRISPR Therapeutics today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designations to CTX310 for the treatment of severe hypertriglyceridemia (sHTG) and for the treatment of patients with elevated LDL-C. RMAT designation provides the benefits of Fast Track and Breakthrough Therapy designations, including early interactions with the FDA and potential eligibility for priority review and accelerated approval.
- In August, CRISPR Therapeutics presented Phase 1a durability data for CTX310 in a late-breaking session at the European Society of Cardiology (ESC) Congress 2026 and simultaneously published in *The New England Journal of Medicine*. A single course treatment of CTX310 produced deep and durable reductions in circulating ANGPTL3, triglycerides (TG), and low-density lipoprotein (LDL) that were sustained through one year of follow-up. At the highest dose, the mean reductions from baseline were 79% in ANGPTL3, 48% in triglycerides, and 53% in LDL-C in a mixed patient cohort with high triglycerides, high LDL-C, or both. CTX310 was well tolerated with no treatment-related serious adverse events.
- Phase 1b data in patients with sHTG will be presented in a late-breaking oral presentation at the American Heart Association (AHA) Scientific Sessions 2026 on November 9, 2026, at 8:00 a.m. CT.
- The Phase 1 clinical trial of CTX340™, targeting angiotensinogen (AGT), in patients with refractory hypertension is ongoing.
- The Phase 1 clinical trial of CTX460™, targeting SERPINA1 for alpha-1 antitrypsin deficiency (AATD), is ongoing. CTX460 is the first investigational candidate from the Company's SyNTase™ editing platform.
- CTX321™, the Company's next-generation LPA program, is progressing through IND/CTA-enabling studies.

siRNA-based Programs

CRISPR Therapeutics' small interfering RNA (siRNA)-based portfolio, developed in collaboration with Sirius Therapeutics, includes programs targeting cardiovascular and thromboembolic diseases.

- CRISPR Therapeutics has completed enrollment in the Phase 2 clinical trial of CTX611 (SRSD107), a long-acting siRNA therapeutic targeting Factor XI (FXI), in patients undergoing total knee arthroplasty (TKA), with an update expected in the second half of 2026. CTX611 has potential across a broad range of thromboembolic indications. CRISPR Therapeutics will lead global Phase 3 development, with Sirius Therapeutics overseeing development in greater China.
- CRISPR Therapeutics has the option to nominate up to two additional siRNA targets, with an update expected in 2027.

Autoimmune Disease and Immuno-Oncology

Zugocabtagene geleucel (zugo-cel; formerly CTX112™) continues to advance across both autoimmune disease and hematologic malignancies.

- In autoimmune disease, zugo-cel is being evaluated in three ongoing Phase 1 trials: a rheumatology basket including systemic lupus erythematosus (SLE), systemic sclerosis (SSc), and inflammatory myositis (IM); a hematology basket in immune thrombocytopenic purpura (ITP) and warm autoimmune hemolytic anemia (wAIHA); and a trial in autoimmune neurologic diseases, including progressive multiple sclerosis (PMS), neuromyelitis optica spectrum disorder (NMOSD), myelin oligodendrocyte glycoprotein antibody-associated disease (MOGAD), N-methyl-D-aspartate receptor (NMDAR) and leucine-rich glioma-inactivated protein 1 (LGI1) autoimmune encephalitis (AIE), and stiff person syndrome (SPS). Enrollment is ongoing globally.
- Phase 1 clinical data in patients with refractory SSc will be presented in a poster presentation at the American College of Rheumatology (ACR) Convergence 2026 on November 8 at 10:30 a.m. ET.
- In immuno-oncology, the Phase 1/2 clinical trial of zugo-cel in B-cell malignancies is ongoing, with updates anticipated in the second half of 2026. A combination study evaluating zugo-cel with pirtobrutinib in aggressive B-cell lymphomas, under the Company's existing collaboration with Lilly, is also ongoing.

CRISPR Therapeutics is also advancing a proprietary *in vivo* CAR-T platform with potential applications across autoimmune disease and oncology.

The Company is pursuing two modalities, supported by an antibody-conjugated LNP delivery system targeting immune cells: a transient, re-dosable mRNA-based CAR-T and a non-viral, integrating CAR-T, for which the Company is evaluating multiple integration approaches. Both programs are in the IND/CTA-enabling phase, focused on a best-in-class profile.

Regenerative Medicine

- The Company is developing CTX213™, a deviceless beta cell replacement candidate for Type 1 diabetes, consisting of unencapsulated precursor islet cells derived from edited induced pluripotent stem cells (iPSCs). CTX213 has demonstrated compelling preclinical efficacy through direct administration and is progressing toward the clinic.

Other Corporate Matters

- Earlier today, CRISPR Therapeutics announced the appointment of Sivakesava Sakhamuri, Ph.D., as Chief Technical Officer, effective immediately. Dr. Sakhamuri brings more than 30 years of technical operations and quality leadership across biologics, vaccines, oligonucleotides, RNA therapeutics, and LNP delivery, most recently as Chief Technical Officer at Orna Therapeutics.
- In October, CRISPR Therapeutics announced the appointment of A.J. Silver, the Company's Senior Vice President of Finance, as Chief Financial Officer, effective October 19, 2026, succeeding Raju Prasad, Ph.D., who stepped down to pursue external opportunities.
- The Company's *in vivo* and cell therapy programs are supported by a wholly-owned GMP manufacturing facility in Framingham, Massachusetts. The facility provides end-to-end production capabilities, supports both clinical and future commercial supply, and enables an industry-leading cost of goods.

Third Quarter 2026 Financial Results

- **Cash Position**: Cash, cash equivalents, and marketable securities were $2,276.6 million as of September 30, 2026, compared to $1,975.8 million as of December 31, 2025. The increase in cash was primarily driven by net proceeds of $585.4 million from the issuance of convertible senior notes in March 2026, offset by operating expenses.
- **R&D Expenses**: R&D expenses were $71.9 million for the third quarter of 2026, compared to $58.9 million for the third quarter of 2025. The increase in R&D expense was primarily attributable to an increase in external research and development expenses and sublicense fees, offset by a decrease in employee-related costs.
- **Acquired In-Process R&D Expenses**: Acquired in-process R&D expenses were $11.7 million for the third quarter of 2026. Acquired in-process R&D expenses for the third quarter of 2026 primarily consisted of a development milestone under the Sirius Agreement that was recognized in the third quarter of 2026. No acquired in-process R&D expenses were recognized during the third quarter of 2025.
- **G&A Expenses**: General and administrative expenses were $17.9 million for the third quarter of 2026, compared to $16.9 million for the third quarter of 2025.
- **Collaboration Expense**: Collaboration expense, net, was $41.3 million for the third quarter of 2026, compared to $57.1 million for the third quarter of 2025. The decrease was primarily attributable to an increase in the Company's share of CASGEVY revenue.
- **Net Loss**: Net loss was $111.4 million for the third quarter of 2026, compared to a net loss of $106.4 million for the third quarter of 2025.

About CASGEVY® (exagamglogene autotemcel [exa-cel])

CASGEVY® is a non-viral, *ex vivo* CRISPR/Cas9 gene-edited cell therapy for eligible patients with sickle cell disease (SCD) or transfusion-dependent beta thalassemia (TDT), in which a patient's own hematopoietic stem and progenitor cells are edited at the erythroid specific enhancer region of the BCL11A gene. This edit results in the production of high levels of fetal hemoglobin (HbF; hemoglobin F) in red blood cells. HbF is the form of the oxygen-carrying hemoglobin that is naturally present during fetal development, which then switches to the adult form of hemoglobin after birth. CASGEVY has been shown to reduce or eliminate VOCs for patients with SCD and transfusion requirements for patients with TDT. CASGEVY is approved for eligible SCD and TDT

patients 12 years and older by multiple regulatory bodies around the world, and for younger children in select countries.

About the CRISPR Therapeutics – Vertex Collaboration for CASGEVY

CRISPR Therapeutics and Vertex established a strategic research collaboration in 2015 to discover and develop therapies leveraging CRISPR/Cas9 technology to address the underlying genetic causes of human disease. CASGEVY is the first approved therapy to emerge from this collaboration. Under an amended agreement, Vertex leads global development, manufacturing, and commercialization of CASGEVY, and Vertex and CRISPR Therapeutics share profits and program costs on a 60/40 basis. Vertex is the manufacturer and exclusive license holder of CASGEVY.

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-related 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 CTX611 (SRSD107)

CTX611 is a novel double-stranded, long-acting siRNA, designed to target the human coagulation factor XI, or FXI, messenger RNA and inhibit FXI protein expression. Through modulation of the intrinsic coagulation pathway, CTX611 is intended to provide anticoagulant and antithrombotic effects with a decreased risk of bleeding compared to other anti-thrombotics. Supported by clinical experience conducted by Sirius Therapeutics in two Phase 1 clinical trials, the Company and Sirius Therapeutics are developing CTX611 as a long-acting FXI inhibitor with the potential to support infrequent, including semi-annual, subcutaneous administration.

About the CRISPR Therapeutics – Sirius Therapeutics Collaboration for siRNA

CRISPR Therapeutics and Sirius Therapeutics entered into a strategic collaboration in 2025 to develop and commercialize siRNA therapies for thromboembolic disorders and other serious diseases. The lead program, CTX611, is a long-acting siRNA targeting FXI, which the companies will co-develop on an equal cost-and-profit-sharing basis. CRISPR Therapeutics will lead commercialization in the U.S., while Sirius will lead commercialization in greater China. The collaboration also provides CRISPR Therapeutics with the option to license up to two additional siRNA programs.

About Zugocabtagene Geleucel (zugo-cel; formerly CTX112)

Zugocabtagene geleucel (zugo-cel) is a wholly-owned, allogeneic chimeric antigen receptor (CAR) T cell therapy product candidate targeting Cluster of Differentiation 19 (CD19), in development for both autoimmune and immuno-oncology indications. The off-the-shelf therapy leverages CRISPR/Cas9 for targeted gene knockout and CAR insertion, enabling immune evasion and enhanced T effector cell potency, and is administered following a standard lymphodepletion regimen without the need for human leukocyte antigen (HLA) matching. In autoimmune disease, zugo-cel is being investigated in three ongoing basket trials. The rheumatology basket trial is enrolling adult patients with systemic lupus erythematosus (SLE), systemic sclerosis (SSc), and inflammatory myositis (IM). The hematology basket trial is enrolling patients with immune thrombocytopenic purpura (ITP) and warm autoimmune hemolytic anemia (wAIHA). The neuroimmune basket trial is enrolling patients with progressive multiple sclerosis (PMS), neuromyelitis optica spectrum disorder (NMOSD), myelin oligodendrocyte glycoprotein antibody-associated Disease (MOGAD), N-methyl-D-aspartate receptor (NMDAR) and leucine-rich glioma-inactivated Protein 1 (LGI1) autoimmune encephalitis (AIE), and stiff person syndrome (SPS). In immuno-oncology, zugo-cel is being investigated in a trial in adult patients with relapsed or refractory B-cell malignancies.

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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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 made by Dr. Kulkarni in this press release, as well as 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 this press release, as well as the ability to use data from ongoing and planned studies and clinical trials for the design and initiation of further studies and clinical trials; (iii) CRISPR Therapeutics strategy, goals, anticipated financial performance and the sufficiency of its cash resources; (iv) plans and expectations for the commercialization of and anticipated benefits of CASGEVY, including anticipated patient access to CASGEVY; (v) regulatory submissions and authorizations, including timelines for and expectations regarding regulatory agency decisions; (vi) the expected benefits of its collaborations; and (vii) 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 its 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.

This press release also contains information regarding our industry, our business and the markets for certain of our product candidates, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Unless otherwise expressly stated, we obtained this industry, business, market and other data from market research firms and other third parties, including medical publications, government data and similar sources. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. This press release discusses investigational therapies and is not intended to convey conclusions about efficacy or safety as to those investigational therapies or uses of such investigational therapies. There is no guarantee that any investigational therapy will successfully complete clinical development or gain approval from applicable regulatory authorities.

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CRISPR Therapeutics AG
Condensed Consolidated Statements of Operations
(Unaudited, In thousands except share data and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Collaboration revenue	$ 12,323	$ —	$ 23,323	$ —
Grant revenue	346	889	985	2,646

Total revenue	12,669	889	24,308	2,646
Operating expenses:				
Research and development	71,888	58,902	207,614	201,280
Acquired in-process research and development	11,675	—	14,148	96,253
General and administrative	17,867	16,931	52,603	55,143
Collaboration expense, net	41,284	57,115	127,505	159,777
Total operating expenses	142,714	132,948	401,870	512,453
Loss from operations	(130,045)	(132,059)	(377,562)	(509,807)
Total other income, net	18,682	26,237	53,913	61,841
Net loss before income taxes	(111,363)	(105,822)	(323,649)	(447,966)
Provision for income taxes	(74)	(619)	(1,873)	(3,020)
Net loss	(111,437)	(106,441)	(325,522)	(450,986)
Foreign currency translation adjustment	2	(28)	(20)	94
Unrealized (loss) gain on marketable securities	(9,456)	973	(18,884)	3,052
Comprehensive loss	$ (120,891)	$ (105,496)	$ (344,426)	$ (447,840)
Net loss per common share — basic	$ (1.15)	$ (1.17)	$ (3.38)	$ (5.12)
Basic weighted-average common shares outstanding	96,741,070	91,305,337	96,437,995	88,124,241
Net loss per common share — diluted	$ (1.15)	$ (1.17)	$ (3.38)	$ (5.12)
Diluted weighted-average common shares outstanding	96,741,070	91,305,337	96,437,995	88,124,241

CRISPR Therapeutics AG
Condensed Consolidated Balance Sheets Data
(Unaudited, in thousands)

	As of	
	September 30, 2026	December 31, 2025
Cash and cash equivalents	$ 192,027	$ 347,559
Marketable securities	2,080,521	1,628,269
Marketable securities - non-current	4,021	—
Working capital	2,144,117	1,836,551
Total assets	2,556,412	2,265,243
Total shareholders' equity	1,648,068	1,921,813

Source: CRISPR Therapeutics AG