CRISPR Therapeutics Provides Business Update and Reports Third Quarter 2025 Financial Results

-Positive Phase 1 data for CTX310® presented in a late-breaking presentation at the American Heart Association (AHA) Scientific Sessions and simultaneously published in The New England Journal of Medicine*-*

-CASGEVY® momentum accelerating; nearly 300 patients have been referred to Authorized Treatment Centers (ATCs), approximately 165 patients have completed their first cell collection and 39 have received infusions across all regions; Vertex expects clear line of sight to over $100 million in total CASGEVY revenue this year and significant growth expected in 2026-

-Pediatric development of exa-cel is advancing in SCD and TDT, with enrollment in two global Phase 3 studies completed; initial data will be presented at the upcoming American Society of Hematology (ASH) annual meeting-

-Clinical trials ongoing for CTX112™, targeting CD19, across multiple indications; broad updates for CTX112 in autoimmune disease and oncology expected by year-end-

-Preclinical data for CTX460™, presented at the European Society of Gene and Cell Therapy (ESGCT) annual congress, demonstrated in vivo *gene correction of alpha-1 antitrypsin deficiency (AATD) with a potential best-in-class profile; clinical trial initiation planned for mid-2026-*

-Phase 2 clinical trial ongoing for SRSD107, targeting Factor XI for the prevention of thromboembolic disorders; first patient dosed and trial expanded to additional sites in Europe-

-Strong balance sheet with approximately $1.9 billion in cash, cash equivalents, and marketable securities as of September 30, 2025-

ZUG, Switzerland and BOSTON, Nov. 10, 2025 (GLOBE NEWSWIRE) -- CRISPR Therapeutics (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today reported financial results for the third quarter ended September 30, 2025.

"This has been another strong quarter of execution and progress across our portfolio," said Samarth Kulkarni, Ph.D., Chairman and Chief Executive Officer of CRISPR Therapeutics. "CASGEVY® momentum continues to build globally, reflecting growing patient engagement and clinical advancement. Enrollment has been completed in two global Phase 3 pediatric studies, and dosing is on track to complete this quarter. Additionally, positive Phase 1 data for CTX310® presented in a late-breaking presentation at the American Heart Association Scientific Sessions and published in *The New England Journal of Medicine*, highlight the breadth and potential of our platform to address serious cardiovascular disease. We continue to advance our broader pipeline, including dosing the first patient in the Phase 2 clinical trial of SRSD107 and unveiling our novel SyNTase™ editing platform with CTX460™, highlighting continued innovation and expansion of our therapeutic toolkit. With strong execution and growing momentum across our programs, CRISPR Therapeutics is well positioned to lead the next wave of gene editing innovation and deliver potentially transformative therapies to patients."

Recent Highlights and Outlook

Hemoglobinopathies and 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), designed to eliminate both vaso-occlusive crises (VOCs) and transfusion requirements. CASGEVY is approved in the U.S., Great Britain, the EU, the Kingdom of Saudi Arabia (KSA), the Kingdom of Bahrain (Bahrain), Qatar, Canada, Switzerland and the United Arab Emirates (UAE) for the treatment of both SCD and TDT. Across these markets, there are more than 60,000 eligible patients in these countries, including approximately 37,000 in North America and Europe and more than 23,000 in the Middle East. With a well-established treatment pathway and a growing number of patients progressing through each stage, CASGEVY has strong momentum heading into late 2025 and beyond.
    - In September, a reimbursement agreement was reached in Italy for patients with SCD and TDT. Italy represents the

largest population of individuals with TDT in Europe, with approximately 5,000 people aged 12 years and older with TDT and approximately 2,300 with SCD.

- Globally, since launch through September 30th, 2025, approximately 165 patients with SCD or TDT have completed their first cell collection, including 50 people in the third quarter. 39 patients have received infusions of CASGEVY, including 10 infused in the third quarter of 2025. Nearly 300 patients have been referred by their physicians to an authorized treatment center (ATC) to begin the treatment process. This includes 110 cell collections in the first nine months of 2025, double the total for all of 2024.

- Enrollment of children 5 to 11 years of age with SCD or TDT in two global Phase 3 studies of CASGEVY has been completed, and dosing is expected to be completed this quarter. Initial data from these studies will be presented at the American Society of Hematology (ASH) annual meeting on December 6th, 2025.

- The number of ATCs initiating patients continues to increase in the U.S., Europe, and the Middle East. Through the end of September, 25 ATCs had initiated more than 5 patients, with at least one ATC in each region initiating 20 or more patients.

- Momentum continues to build through the final months of 2025. With continued uptake and reimbursement progress across major regions, Vertex expects a clear line of sight to over $100 million in total CASGEVY revenue this year with significant growth expected in 2026.

***In Vivo* Liver Editing**

- CRISPR Therapeutics is advancing a pipeline of *in vivo* gene editing candidates addressing major unmet needs in cardiovascular, metabolic and rare diseases using its proprietary, de-risked lipid nanoparticle (LNP) delivery platform.

- CTX310®, targeting ANGPTL3, is in an ongoing Phase 1 clinical trial in patients with homozygous familial hypercholesterolemia (HoFH), severe hypertriglyceridemia (SHTG), heterozygous familial hypercholesterolemia (HeFH), or mixed dyslipidemias. Phase 1 data were presented in a late-breaking session at the American Heart Association (AHA) Scientific Sessions and published simultaneously in *The New England Journal of Medicine (NEJM)*. Results from the Phase 1 clinical trial highlight the potential of CTX310 to safely and durably lower both triglycerides (TG) and low-density lipoprotein (LDL) following a single-course IV administration. CRISPR Therapeutics is advancing CTX310 into Phase 1b clinical trials, prioritizing development in sHTG and mixed dyslipidemia.

- CTX320™ is in an ongoing Phase 1 clinical trial targeting the *LPA* gene in patients with elevated lipoprotein(a) [Lp(a)], a genetically determined risk factor associated with an increased incidence of major adverse cardiovascular events (MACE). Elevated Lp(a) levels affect up to 20% of the global population and remain unaddressed by current therapies. The Company plans to provide an update in the first half of 2026.

- CRISPR Therapeutics continues to advance its preclinical *in vivo* programs: CTX460™, targeting *SERPINA1* for the treatment of alpha-1 antitrypsin deficiency (AATD); CTX340™, targeting *AGT* for the treatment of refractory hypertension; and CTX450™, targeting *ALAS1* for the treatment of acute hepatic porphyria (AHP).

  - CTX460 is the first investigational candidate using the Company's novel SyNTase editing platform, unveiled in October. SyNTase is designed to enable precise, *in vivo* gene correction, and represents an important expansion of CRISPR Therapeutics' toolkit. Preclinical data presented at the European Society of Gene and Cell Therapy (ESGCT) Annual Congress demonstrated >90% mRNA correction, a 5-fold increase in total AAT levels, and >99% serum M-AAT:Z-AAT ratio in AATD disease models. These findings provide preclinical proof-of-concept for precise, single-dose *in vivo* gene correction using the SyNTase editing platform and support the potential best-in-class profile of CTX460. CRISPR Therapeutics expects to initiate a clinical trial of CTX460 in mid-2026.

  - Preclinical data from CTX340 were presented in a late-breaking poster presentation at the American Heart Association (AHA) Scientific Sessions. In a spontaneous hypertensive rat model, CTX340 showed >70% liver editing and mean arterial pressure reduction of 53 mmHg compared to vehicle that was durable throughout the

study (~8.5 months). Furthermore, in non-human primates, CTX340 showed greater than 90% AGT reduction with a two-dose regimen showing the additive effects of repeat dosing and enabling dose titration. CTX340 was well tolerated with no hypotension or hypokalemia observed. CTX340 is currently in IND/CTA-enabling studies.

Autoimmune Disease and Immuno-Oncology

- CTX112™, targeting CD19, is being developed for autoimmune disease and hematologic malignancies and has received Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA) for the treatment of relapsed or refractory follicular lymphoma and marginal zone lymphoma. A Phase 1 clinical trial in autoimmune disease is underway in systemic lupus erythematosus (SLE), systemic sclerosis and inflammatory myositis. In parallel, a Phase 1 clinical trial of CTX112 in relapsed or refractory B-cell malignancies is ongoing. The Company plans to provide a broad update on CTX112 by year-end.

- CTX131™, targeting CD70, was previously in development for both solid tumors and hematologic malignancies. While the Phase 1 data are encouraging, the Company has strategically redirected resources away from this program to advance other programs with the greatest potential for long-term value creation.

- CRISPR Therapeutics is leveraging its expertise and proprietary lipid nanoparticle (LNP) delivery platform, mRNA, and conjugation capabilities to advance an *in vivo* CAR T platform with the ability to address autoimmune disease and oncology.

- CRISPR Therapeutics' autoimmune disease and immuno-oncology programs are supported by a wholly-owned, GMP manufacturing facility located in Framingham, Massachusetts, which provides end-to-end production capabilities for its cell therapy portfolio and supports both clinical and future commercial supply.

siRNA

- In September, CRISPR Therapeutics and its partner Sirius Therapeutics announced that the first patient was dosed in Europe in the Phase 2 clinical trial of SRSD107, a long-acting Factor XI (FXI) small interfering RNA (siRNA) for thromboembolic disorders. The trial is evaluating the safety and efficacy of SRSD107 in preventing venous thromboembolism (VTE) following total knee arthroplasty (TKA) and will inform dose selection for future pivotal trials. SRSD107 has the potential to be a best-in-class FXI inhibitor, showing deep reductions in FXI via semi-annual subcutaneous injection. SRSD107 is being co-developed by CRISPR Therapeutics and Sirius Therapeutics as part of a broader strategic collaboration to advance RNA-based medicines.

- CRISPR Therapeutics and Sirius Therapeutics have expanded the Phase 2 clinical trial with additional centers in Europe. SRSD107 is being developed for a range of thromboembolic and clotting-related indications, including arterial fibrillation (AF), cancer-associated thrombosis (CAT), chronic kidney disease (CKD), peripheral vascular disease (PVD), chronic coronary artery disease (CAD), ischemic stroke and VTE.

Regenerative Medicine

- CRISPR Therapeutics continues to advance its regenerative medicine efforts for Type 1 diabetes (T1D). Beyond CTX211™, the Company is developing next-generation programs that leverage induced pluripotent stem cell (iPSC) derived, allogeneic, gene-edited, beta islet cell precursors. These approaches aim to achieve insulin independence in T1D patients without requiring chronic immunosuppression. The Company expects to provide an update this year.

Upcoming Events

- The Company will participate in the following events in November:

    - Guggenheim 2nd Annual Healthcare Innovation Conference
      Date: Wednesday, November 12, 2025
      Time: 11:30 a.m. ET

- Jefferies Global Healthcare Conference
  Date: Wednesday, November 19, 2025
  Time: 1:00 p.m. GMT

Third Quarter 2025 Financial Results

- **Cash Position:** Cash, cash equivalents, and marketable securities were $1,944.1 million as of September 30, 2025, compared to $1,903.8 million as of December 31, 2024. The increase in cash was primarily driven by proceeds from the issuance of common shares, option exercise activity and interest income, offset by operating expenses, as well as the $25.0 million upfront cash payment made as part of the Sirius Agreement.

- **R&D Expenses:** R&D expenses were $58.9 million for the third quarter of 2025, compared to $82.2 million for the third quarter of 2024. The decrease in R&D expense was primarily driven by a decrease in variable external research and manufacturing costs, as well as a decrease in employee-related expenses, including stock-based compensation expenses.

- **G&A Expenses:** General and administrative expenses were $16.9 million for the third quarter of 2025, compared to $17.4 million for the third quarter of 2024.

- **Collaboration Expense:** Collaboration expense, net, was $57.1 million for the third quarter of 2025, compared to $11.2 million for the third quarter of 2024. In the third quarter of 2024, we exercised our option to defer specified costs under the CASGEVY program in excess of the deferral limit of $110.3 million under the A&R Vertex JDCA, as amended. The increase in collaboration expense, net, was primarily attributable to the timing of when we reached the deferral limit in 2024, as no such limit was applicable in 2025.

- **Net Loss:** Net loss was $106.4 million for the third quarter of 2025, compared to a net loss of $85.9 million for the third quarter of 2024.

About CASGEVY® (exagamglogene autotemcel [exa-cel])
CASGEVY® is a non-viral, *ex vivo* CRISPR/Cas9 gene-edited cell therapy for eligible patients with SCD or 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.

About the CRISPR Therapeutics - Vertex Collaboration for CASGEVY
CRISPR Therapeutics and Vertex established a strategic research collaboration in 2015 to discover and develop therapies using 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 collaboration agreement, Vertex leads global development, manufacturing, and commercialization of CASGEVY and shares program costs and profits worldwide 60/40 with CRISPR Therapeutics. Vertex is the manufacturer and exclusive license holder of CASGEVY.

About CTX112
CTX112 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. CTX112 is being investigated in ongoing clinical trials in adult patients with systemic lupus erythematosus, systemic sclerosis, and inflammatory myositis and in adult patients with relapsed or refractory B-cell malignancies.

About *In Vivo* 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 technologies. The Company's *in vivo* portfolio includes its lead investigational programs, CTX310 (directed towards angiopoietin-related protein 3 (*ANGPTL3*)) and CTX320 (directed towards *LPA*, the gene encoding apolipoprotein(a) (apo(a)), a major component of lipoprotein(a) [Lp(a)]). Both are validated therapeutic targets for cardiovascular disease. CTX310 and CTX320 are in ongoing clinical trials in patients with heterozygous familial hypercholesterolemia, homozygous familial hypercholesterolemia, mixed dyslipidemias, or severe hypertriglyceridemia, and

in patients with elevated lipoprotein(a), respectively. In addition, the Company's research and preclinical development candidates include: CTX460™, targeting SERPINA1 for the treatment of alpha-1 antitrypsin deficiency (AATD); CTX340™, targeting AGT fo the treatment of refractory hypertension; and CTX450™, targeting ALAS1 for the treatment of acute hepatic porphyria (AHP).

About SRSD107

SRSD107 is a novel double-stranded small interfering ribonucleic acid (siRNA). SRSD107 specifically targets the human coagulation factor XI (FXI) mRNA and inhibits FXI protein expression, thereby blocking the intrinsic coagulation pathway and promoting anticoagulant/anti-thrombotic effects. SRSD107 is being co-developed by CRISPR Therapeutics and Sirius Therapeutics as part of a strategic collaboration to advance innovative treatments for cardiovascular and clotting-related diseases.

About the CRISPR Therapeutics and Sirius Therapeutics Collaboration

CRISPR Therapeutics and Sirius Therapeutics entered into a strategic collaboration in 2025 to develop and commercialize novel small interfering RNA (siRNA) therapies for thromboembolic disorders and other serious diseases. The lead program, SRSD107, is a long-acting siRNA targeting Factor XI (FXI) with the potential to offer best-in-class efficacy and safety. Under the agreement, the companies will co-develop SRSD107 and share costs and profits equally. CRISPR Therapeutics will lead commercialization in the U.S., while Sirius will lead in Greater China. The collaboration also provides CRISPR Therapeutics with the option to license up to two additional siRNA programs. This partnership expands CRISPR Therapeutics' therapeutic portfolio into RNA-based medicines, complementing its ongoing efforts in gene editing and broadening its impact across serious and chronic diseases. For Sirius, the collaboration marks a major milestone in its mission to deliver innovative RNA-based therapies globally, leveraging deep expertise in siRNA design and delivery.

About CTX211

CTX211 is an allogeneic, gene-edited, stem cell-derived investigational therapy for the treatment of type 1 diabetes (T1D), which incorporates gene edits that aim to make cells hypoimmune and enhance cell fitness. This immune-evasive cell replacement therapy is designed to enable patients to produce their own insulin in response to glucose.

About CRISPR Therapeutics

Founded over a decade ago, CRISPR Therapeutics is a leading gene editing company focused on developing transformative medicines for serious diseases. 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, approved for eligible patients with sickle cell disease and transfusion-dependent beta thalassemia. CRISPR Therapeutics is advancing a broad and diversified pipeline across hemoglobinopathies, oncology, regenerative medicine, cardiovascular and autoimmune, and rare diseases. The Company continues to expand its leadership in gene editing through the development of SyNTase™ editing, a novel and proprietary gene-editing platform designed to enable precise, efficient, and scalable gene correction. To accelerate and expand 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.

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 clinical trials for the design and initiation of further 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 additional 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,	
	2025	2024	2025	2024
Revenue:				
Collaboration revenue	$ —	$ —	$ —	$ —
Grant revenue	889	602	2,646	1,623
Total revenue	$ 889	602	$ 2,646	$ 1,623
Operating expenses:				
Research and development	58,902	82,160	201,280	238,498
Acquired in-process research and development	—	—	96,253	—
General and administrative	16,931	17,419	55,143	54,853
Collaboration expense, net	57,115	11,153	159,777	110,250
Total operating expenses	132,948	110,732	512,453	403,601
Loss from operations	(132,059)	(110,130)	(509,807)	(401,978)
Total other income, net	26,237	25,064	61,841	75,924
Net loss before income taxes	(105,822)	(85,066)	(447,966)	(326,054)
Provision for income taxes	(619)	(876)	(3,020)	(2,887)
Net loss	(106,441)	(85,942)	(450,986)	(328,941)
Foreign currency translation adjustment	(28)	76	94	66
Unrealized gain on marketable securities	973	13,368	3,052	8,586
Comprehensive loss	$ (105,496)	$ (72,498)	$ (447,840)	$ (320,289)
Net loss per common share — basic	$ (1.17)	$ (1.01)	$ (5.12)	$ (3.92)
Basic weighted-average common shares outstanding	91,305,337	85,234,926	88,124,241	83,988,063
Net loss per common share — diluted	$ (1.17)	$ (1.01)	$ (5.12)	$ (3.92)
Diluted weighted-average common shares outstanding	91,305,337	85,234,926	88,124,241	83,988,063

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

	As of	
	September 30, 2025	December 31, 2024
Cash and cash equivalents	$ 286,497	$ 298,257
Marketable securities	1,629,213	1,605,569
Marketable securities, non-current	28,412	—
Working capital	1,810,135	1,849,350
Total assets	2,245,308	2,242,034
Total shareholders' equity	1,915,982	1,932,080

Source: CRISPR Therapeutics AG