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

-CASGEVY® momentum building; >75 authorized treatment centers (ATCs) activated globally, achieving the target goal and ~115 patients have had cells collected across all regions; positioning the program for strong future growth-

-Clinical trial ongoing for CTX310™, targeting ANGPTL3, with preliminary data showing dose-dependent reductions of up to 82% in triglycerides (TG) and 86% in low-density lipoprotein (LDL), and a well-tolerated safety profile; data presentation anticipated at a medical meeting in the second half of 2025-

-Clinical trial ongoing for CTX320 ™, targeting the LPA *gene; update expected in the first half of 2026-*

-Clinical trials ongoing for CTX112™ and CTX131™, targeting CD19 and CD70 across multiple indications; broad updates fo CTX112 in oncology and autoimmune diseases expected in the second half of 2025 with CTX131 updates also expected in 2025-

-Received European Medicines Agency (EMA) Authorization to Initiate a Phase 2 Clinical Trial of SRSD107 for Thromboembolic Disorders-

-Strong balance sheet with approximately $1.7 billion in cash, cash equivalents, and marketable securities as of June 30, 2025-

ZUG, Switzerland and BOSTON, Aug. 04, 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 second quarter ended June 30, 2025.

“We are entering the second half of the year with momentum across both our commercial and clinical programs,” said Samarth Kulkarni, Ph.D., Chairman and Chief Executive Officer of CRISPR Therapeutics. “The activation of 75 authorized treatment centers for CASGEVY has been achieved, marking a meaningful step in expanding patient access, while clinical trials across multiple other programs continue to advance. Looking ahead, we expect several key milestones including the presentation of complete Phase 1 data for CTX310, as well as updates across our oncology and autoimmune portfolios. Our focus remains on delivering transformative therapies for patients with critical unmet needs.”

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. Building on the foundational launch in 2024, significant progress is being made to bring this transformative therapy to patients worldwide.
    - The target of activating 75 authorized treatment centers (ATCs) globally has been achieved, marking an important milestone in the commercial rollout of CASGEVY. Since launch through June 30, approximately 115 patients have completed their first cell collection, and 29 patients have received infusions of CASGEVY, including 16 infused in the second quarter. The launch of CASGEVY is building strong momentum, positioning the program for significant growth and broader impact.
    - Through reimbursement agreements, Vertex has secured access for eligible SCD and TDT patients in 10 countries. Recent agreements include Northern Ireland, Scotland and Denmark. Efforts are ongoing with government and reimbursement authorities globally to secure access for eligible patients.
    - CRISPR Therapeutics continues to advance its next-generation approaches designed to significantly broaden the addressable patient population for SCD and TDT. The Company’s internally developed targeted conditioning program, an anti-CD117 (c-Kit) antibody-drug conjugate (ADC), remains on track in preclinical development. In parallel, the Company is making continued progress in its *in vivo* editing platform aimed at enabling direct editing of hematopoietic stem cells (HSC) without the need for conditioning. By potentially eliminating the need for conditioning, this approach could unlock access to transformative therapies for a significantly larger patient

population.

- **Immuno-Oncology and Autoimmune Disease Programs**

    - Clinical trials are ongoing for the Company's next-generation allogeneic CAR T product candidates, CTX112™ and CTX131™, targeting CD19 and CD70, respectively, across multiple indications. Both candidates incorporate novel potency edits designed to significantly enhance CAR T cell expansion and cytotoxicity, positioning them as potential best-in-class therapies.
    - CTX112, targeting CD19, is in development for hematologic malignancies and autoimmune diseases. Preliminary clinical data support a differentiated profile with strong clinical benefit combined with the convenience of an "off-the-shelf" therapy.
        - In relapsed or refractory B-cell malignancies, encouraging results from the ongoing Phase 1/2 clinical trial led to the FDA granting Regenerative Medicine Advanced Therapy (RMAT) designation for CTX112 in relapsed or refractory follicular lymphoma and marginal zone lymphoma.
        - A Phase 1 trial of CTX112 is ongoing in autoimmune indications, including systemic lupus erythematosus (SLE), systemic sclerosis and inflammatory myositis. Preliminary safety, pharmacokinetic, and pharmacodynamic data from oncology trials support its potential in autoimmune indications.
        - The Company plans to provide a broad update for CTX112 in oncology and autoimmune disease in the second half of 2025.

    - CTX131™, targeting CD70, is in development for both solid tumors and hematologic malignancies. Clinical trials for CTX131 are ongoing, with an update expected in 2025.
    - CRISPR Therapeutics' immuno-oncology and autoimmune disease efforts are supported by a wholly-owned, U.S. manufacturing facility located in Framingham, MA. This investment enables the production of clinical and commercial-stage good manufacturing practice (GMP) materials across the Company's allogeneic cell therapy programs.

- ***In Vivo* Liver Editing Programs**

    - CRISPR Therapeutics is advancing a pipeline of *in vivo* gene editing candidates targeting major unmet needs in cardiovascular and metabolic diseases using its proprietary lipid nanoparticle (LNP) delivery platform.
    - CTX310™ is in an ongoing Phase 1 clinical trial targeting ANGPTL3 in patients with homozygous familial hypercholesterolemia (HoFH), severe hypertriglyceridemia (SHTG), heterozygous familial hypercholesterolemia (HeFH), or mixed dyslipidemias. ANGPTL3 loss-of-function mutations are linked to reduced in low-density lipoprotein cholesterol (LDL-C), triglycerides (TG), and a lower risk of atherosclerotic cardiovascular disease (ASCVD), without adverse effects on overall health. In the U.S., more than 40 million patients have elevated LDL, severely elevated TGs or both. CTX310 is initially focused on a high-risk subset of this group with the greatest unmet medical need and limited effective treatment options.
        - In June, the Company reported data for CTX310, demonstrating dose-dependent reductions in ANGPTL3, TG, and LDL following a single administration. As dose-range finding continues, data to date demonstrate peak reductions of up to 82% in TG and LDL reductions of up to 86% at DL4 without any clinically significant changes in liver enzymes and a safety and tolerability profile consistent with previous findings.
        - These initial results represent a significant milestone in the advancement of CRISPR Therapeutics' proprietary LNP delivery technologies for gene editing in the liver. The Company anticipates presenting the complete Phase 1 data for CTX310 at a medical meeting in the second half of 2025.
    - 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 two preclinical programs: CTX340™, targeting angiotensinogen (AGT) for the treatment of refractory hypertension, and CTX450™, targeting 5' aminolevulinic acid synthase 1 (ALAS1) for the treatment of acute hepatic porphyrias (AHP). Both candidates are currently in IND/CTA-enabling studies.

- **SRSD107**

    - In May, the Company entered a strategic collaboration with Sirius Therapeutics to jointly develop and commercialize small interfering RNA (siRNA) therapies, beginning with SRSD107, a long-acting Factor XI (FXI) siRNA. Under the partnership, development and commercialization will be shared, with CRISPR Therapeutics leading efforts in the U.S. and Sirius in Greater China. The agreement also grants CRISPR Therapeutics the option to nominate two additional siRNA targets for future development. This collaboration expands CRISPR Therapeutics' capabilities, enabling the development of a broader range of transformative gene-based medicines beyond its current gene-editing programs in the clinic.

- In July, the European Medicines Agency (EMA) authorized the initiation of a Phase 2 clinical trial of SRSD107 for thromboembolic disorders. The study is designed to evaluate the safety and efficacy of SRSD107 in preventing post-operative venous thromboembolism in patients undergoing total knee arthroplasty and aims to confirm its anticoagulant potential.

- **Regenerative Medicine Programs**

  - CRISPR Therapeutics continues to advance its regenerative medicine efforts in Type 1 diabetes (T1D). In addition to CTX211™, the Company is developing next-generation programs focusing on induced pluripotent stem cell (iPSC) derived, allogeneic, gene-edited, beta islet cell precursors. These approaches aim to enable insulin independence in T1D patients without the need for chronic immunosuppression. The Company expects to provide an update in 2025.

- **Second Quarter 2025 Financial Results**

  - **Cash Position:** Cash, cash equivalents, and marketable securities were $1,721.2 million as of June 30, 2025, compared to $1,903.8 million as of December 31, 2024. The decrease in cash was primarily driven by operating expenses, as well as the $25.0 million upfront cash payment made as part of the Sirius Agreement, offset by proceeds from interest income and proceeds from the issuance of common shares and option exercise activity.
  - **R&D Expenses:** R&D expenses were $69.9 million for the second quarter of 2025, compared to $80.2 million for the second quarter of 2024. The decrease in R&D expense was primarily driven by a decrease in employee-related expenses, including stock-based compensation expenses.
  - **Acquired In-Process R&D Expenses**: Acquired in-process R&D expenses were $96.3 million for the second quarter of 2025 related to costs incurred upon entering the Sirius Agreement during the second quarter of 2025.
  - **G&A Expenses:** General and administrative expenses were $18.9 million for the second quarter of 2025, compared to $19.5 million for the second quarter of 2024.
  - **Collaboration Expense:** Collaboration expense, net, was $45.2 million for the second quarter of 2025, compared to $52.1 million for the second quarter of 2024. The decrease in collaboration expense, net, was primarily attributable to an increase in CASGEVY revenue, as well as a decrease in operating expenses for the program.
  - **Net Loss:** Net loss was $208.5 million for the second quarter of 2025, compared to a net loss of $126.4 million for the second 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 recurrent vaso-occlusive crises (VOCs) for patients with SCD and transfusion requirements for patients with TDT. CASGEVY is approved for certain indications in multiple jurisdictions for eligible patients.

About the CRISPR Therapeutics - Vertex Collaboration for CASGEVY
CRISPR Therapeutics and Vertex entered into a strategic research collaboration in 2015 focused on the use of CRISPR/Cas9 to discover and develop potential new treatments aimed at the underlying genetic causes of human disease. CASGEVY represents the first potential treatment to emerge from the joint research program. Under an amended collaboration agreement, Vertex now leads global development, manufacturing, and commercialization of CASGEVY and splits program costs and profits worldwide 60/40 with CRISPR Therapeutics. Vertex is the manufacturer and exclusive license holder of CASGEVY.

About CTX112
CTX112 is being developed for both oncology and autoimmune indications. CTX112 is a next-generation, wholly-owned, allogeneic CAR T product candidate targeting Cluster of Differentiation 19, or CD19, which incorporates edits designed to evade the immune system, enhance CAR T potency, and reduce CAR T exhaustion. CTX112 is being investigated in an ongoing clinical trial designed to assess safety and efficacy of the product candidate in adult patients with relapsed or refractory B-cell malignancies who have received at least two prior lines of therapy. In addition, CTX112 is being investigated in an ongoing clinical trial designed to assess the safety and efficacy of the product candidate in adult patients with systemic lupus erythematosus, systemic sclerosis, and inflammatory myositis.

About CTX131
CTX131 is being developed for both solid tumors and hematologic malignancies, including T cell lymphomas (TCL). CTX131 is a next-generation, wholly-owned, allogeneic CAR T product candidate targeting Cluster of Differentiation 70, or CD70, an antigen expressed on various solid tumors and hematologic malignancies. CTX131 incorporates edits designed to evade the immune

system, prevent fratricide, enhance CAR T potency, and reduce CAR T exhaustion. CTX131 is being investigated in ongoing clinical trials designed to assess the safety and efficacy of the product candidate in adult patients with relapsed or refractory solid tumors and hematologic malignancies, including TCL.

About *In Vivo* Programs

CRISPR Therapeutics has established a proprietary lipid nanoparticle (LNP) platform for the delivery of CRISPR/Cas9 to the liver. 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 CTX340 and CTX450, targeting angiotensinogen (*AGT*) for refractory hypertension and 5'-aminolevulinate synthase 1 (*ALAS1*) for acute hepatic porphyria (AHP), respectively.

About SRSD107

SRSD107 is a novel double-stranded small interfering ribonucleic acid (siRNA). Developed initially by Sirius Therapeutics, 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.

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. A Phase 1 clinical trial for CTX211 for the treatment of T1D is ongoing.

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 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, 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 June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenue:				
Collaboration revenue	$ —	$ —	$ —	$ —
Grant revenue	892	517	1,757	1,021
Total revenue	$ 892	517	$ 1,757	$ 1,021
Operating expenses:				
Research and development	69,894	80,165	142,378	156,338
Acquired in-process research and development	96,253	—	96,253	—
General and administrative	18,916	19,481	38,212	37,434
Collaboration expense, net	45,153	52,131	102,662	99,097
Total operating expenses	230,216	151,777	379,505	292,869
Loss from operations	(229,324)	(151,260)	(377,748)	(291,848)
Total other income, net	22,067	26,139	35,604	50,860
Net loss before income taxes	(207,257)	(125,121)	(342,144)	(240,988)
Provision for income taxes	(1,292)	(1,287)	(2,401)	(2,011)
Net loss	(208,549)	(126,408)	(344,545)	(242,999)
Foreign currency translation adjustment	80	2	121	(9)
Unrealized (loss) gain on marketable securities	(174)	(1,329)	2,080	(4,783)

Comprehensive loss	$ (208,643)	$ (127,735)	$ (342,344)	$ (247,791)
Net loss per common share — basic	$ (2.40)	$ (1.49)	$ (3.98)	$ (2.92)
Basic weighted-average common shares outstanding	87,069,690	84,920,929	86,507,330	83,357,780
Net loss per common share — diluted	$ (2.40)	$ (1.49)	$ (3.98)	$ (2.92)
Diluted weighted-average common shares outstanding	87,069,690	84,920,929	86,507,330	83,357,780

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

	As of	
	June 30, 2025	December 31, 2024
Cash and cash equivalents	$ 193,618	$ 298,257
Marketable securities	1,527,619	1,605,569
Working capital	1,629,304	1,849,350
Total assets	2,029,711	2,242,034
Total shareholders' equity	1,711,125	1,932,080

Source: CRISPR Therapeutics AG