CRISPR Therapeutics Highlights Strategic Priorities and Anticipated 2026 Milestones

-2026 is poised to be a data- and milestone-rich year across the portfolio-

-CASGEVY® launch acceleration continues, supporting multi-billion-dollar potential-

-Broad pipeline supported by leading gene-editing and siRNA platforms across cardiovascular, autoimmune, oncology and rare diseases-

-Starting 2026 with a strong balance sheet with approximately $2 billion in cash, cash equivalents, and marketable securities-

ZUG, Switzerland and BOSTON, January 12, 2026 - CRISPR Therapeutics (Nasdaq: CRSP), a biopharmaceutical company focused on creating transformative gene-based medicines for serious diseases, today highlighted its strategic priorities and anticipated 2026 milestones.

"Entering 2026, CRISPR Therapeutics is well positioned, with CASGEVY® gaining momentum, and multiple programs with encouraging data advancing rapidly through clinical trials across a diverse set of therapeutic areas," said Samarth Kulkarni, Ph.D., Chairman and Chief Executive Officer of CRISPR Therapeutics. "Across the portfolio, clinical data demonstrated the transformative potential of CTX310™ for patients suffering from severe cardiovascular disease, as well as zugo-cel in oncology and autoimmune diseases. We also added a siRNA pillar through our partnership with Sirius and are advancing our lead siRNA asset, CTX611™ targeting Factor XI through the clinic. Beyond these clinical stage programs, we have multiple additional assets in preclinical development that leverage our best-in-class advanced editing platform. Together, our broad portfolio, strong balance sheet and effective operating model reinforce our confidence as we move into the next phase."

Anticipated Key Milestones in 2026

CRISPR Therapeutics anticipates several key milestones across its portfolio:

- Continued global commercialization and adoption of CASGEVY, with ongoing quarterly updates.
- Global regulatory submissions for CASGEVY in patients ages 5 – 11 are expected to be initiated by Vertex in the first half of 2026.
- Updates from CTX310® are expected in the second half of 2026.
- Updates from the Lp(a) program are expected in 2026.
- Top-line Phase 2 clinical data from CTX611 in patients undergoing total knee arthroplasty (TKA) are expected in the second half of 2026.
- Updates across autoimmune disease and immuno-oncology for zugocabtagene geleucel (zugo-cel; formerly CTX112™) are expected in the second half of 2026.

- The Company expects to initiate a clinical trial for CTX460 in alpha-1 antitrypsin deficiency (AATD) in mid-2026.
- The Company expects to initiate a clinical trial for CTX340 in refractory hypertension in the first half of 2026.

Portfolio Highlights and Outlook

Hemoglobinopathies and CASGEVY (exagamglogene autotemcel [exa-cel])

- CASGEVY now approved in the U.S., the United Kingdom, the EU, the Kingdom of Saudi Arabia, the Kingdom of Bahrain, Qatar, Canada, Switzerland, the United Arab Emirates, and Kuwait for patients 12 years and older with sickle cell disease (SCD) or transfusion-dependent beta thalassemia (TDT).
- CASGEVY exceeded $100M in revenue in 2025, reflecting more than 60 patients receiving CASGEVY infusions.
- Achieved a nearly three-fold increase in patient initiations and first cell collections in 2025 compared to 2024.
- Regulatory submissions for CASGEVY in patients aged 5-11 years with SCD and TDT are expected in the first half of 2026.
- CASGEVY awarded a Commissioner's National Priority Voucher for pediatric submissions in SCD and TDT, enabling accelerated regulatory review once submissions are complete.
- Continued development of next-generation conditioning approaches with the potential to expand addressable patient populations for SCD and TDT with CASGEVY.
- Continued development of a lipid nanoparticle (LNP) based *in vivo* approach for editing hematopoietic stem cells (HSCs), with the potential to address a broader patient population in SCD and TDT.

***In Vivo* Liver Editing**

CRISPR Therapeutics continues to advance a diversified portfolio of *in vivo* gene editing programs leveraging its proprietary LNP delivery platform.

- In 2025, CRISPR Therapeutics presented data for CTX310, demonstrating deep and durable reductions of triglycerides (TG) and low-density lipoprotein (LDL) following a single-course intravenous infusion, with a well-tolerated safety profile. Based on the positive Phase 1 results, the Company has advanced CTX310, targeting angiopoietin-related protein 3 (*ANGPTL3*), into Phase 1b clinical trials, prioritizing development in severe hypertriglyceridemia (sHTG) and refractory hypercholesterolemia. The Company expects to provide an update in the second half of 2026.

- CTX320™, targeting *LPA,* has demonstrated reductions of up to 73% in the dose escalation phase of the clinical trial. In parallel, the Company is advancing a next-generation *LPA* program, CTX321™, incorporating an updated guide RNA that demonstrates approximately two-fold greater potency in preclinical testing, while utilizing the same LNP delivery system. CTX321 is currently in IND/CTA-enabling studies, with an Lp(a) program update expected in 2026.
- In addition to its clinical-stage programs, CRISPR Therapeutics continues to advance several preclinical *in vivo* gene editing candidates, including:
    - CTX460™, targeting SERPINA1 for the treatment of alpha-1 antitrypsin deficiency (AATD), is the first investigational candidate to emerge from the Company's SyNTase™ editing platform. The Company expects to initiate a clinical trial for CTX460 in mid-2026.
    - CTX340™, targeting angiotensinogen (*AGT*) for refractory hypertension, is currently in IND/CTA-enabling studies. The Company expects to initiate a clinical trial for CTX340 in the first half of 2026.

siRNA-based Programs

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

- CTX611 (SRSD107), a long-acting siRNA targeting Factor XI (FXI), is in an ongoing Phase 2 clinical trial in patients undergoing total knee arthroplasty (TKA). The Company expects to provide an update in the second half of 2026.

- CRISPR Therapeutics will lead global Phase 3 development of CTX611, excluding Greater China. The program targets a range of thromboembolic and clotting-related indications and represents a multi-billion-dollar market opportunity, including atrial fibrillation (AF), venous thromboembolism (VTE), ischemic stroke, cancer-associated thrombosis (CAT), chronic kidney disease (CKD), peripheral vascular disease (PVD), chronic coronary artery disease (CAD).

Autoimmune Disease and Immuno-Oncology

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

- In autoimmune disease, Phase 1 clinical trials are ongoing across multiple indications, including systemic lupus erythematosus (SLE), systemic sclerosis (SSc), and inflammatory myositis and a second Phase 1 trial in immune thrombocytopenia purpura (ITP) and warm autoimmune hemolytic anemia (wAIHA). The first patient with SLE, refractory to 9 prior therapies with a baseline Systemic Lupus Erythematosus Disease Activity Index 2000 (SLEDAI-2K) score of 8, has maintained drug-free DORIS clinical remission through month 9 following CAR-T therapy. The second SLE patient with a baseline SLEDAI-2K score of 8, has sustained B cell depletion with

SLEDAI-2K score of 0 through month 2 following CAR-T therapy. The Company expects to provide updates in the second half of 2026.

- The Phase 1/2 clinical trial of zugo-cel in B-cell malignancies is ongoing. The Company expects to provide updates in the second half of 2026. CRISPR Therapeutics has also established a collaboration and clinical supply agreement with Lilly to evaluate zugo-cel together with pirtobrutinib in aggressive B-cell lymphomas, further expanding the program's development in oncology.

Regenerative Medicine

CRISPR Therapeutics continues to advance its regenerative medicine portfolio, including its efforts in diabetes. Clinical data generated from CTX211™ were promising, demonstrating detectable C-peptide levels 12 months after implantation. These data have informed the Company's approach to hypoimmune cell engineering, supporting a transition to a next-generation candidate, CTX213™. CTX213 has demonstrated compelling preclinical efficacy and is progressing towards the clinic. The Company expects to provide additional updates as development progresses.

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 *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 being developed for 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 for the treatment of refractory hypertension and CTX321, targeting LPA for the treatment of patients with elevated lipoprotein(a).

About CTX611 (SRSD107)

CTX611 is a novel double-stranded siRNA, designed to target the human coagulation FXI messenger RNA and inhibit FXI protein expression. Through modulation of the intrinsic coagulation pathway, CTX611 is intended to provide anticoagulant and antithrombotic effects. Supported by clinical experience to date, CTX611 is being developed as a long-acting FXI inhibitor with the potential to support infrequent, including semi-annual, subcutaneous administration.

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. Zugo-cel is an off-the-shelf allogeneic CAR-T that utilizes CRISPR Cas9 for targeted gene knockout and CAR insertion for immune evasion and enhanced T effector cell potency. Zugo-cel is given following a standard lymphodepletion regimen without the need for HLA matching. Zugo-cel 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 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, CTX611, is a long-acting siRNA targeting FXI. Under the agreement, the companies will co-develop CTX611 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.

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 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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