

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM 10-Q**

**QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2025

or

**TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from \_\_\_\_\_ to \_\_\_\_\_.

Commission File Number: 001-37923

**CRISPR THERAPEUTICS AG**

(Exact name of registrant as specified in its charter)

**Switzerland**  
(State or other jurisdiction of  
incorporation or organization)

**Baarerstrasse 14**  
**6300 Zug, Switzerland**  
(Address of principal executive offices)

**Not Applicable**  
(I.R.S. Employer  
Identification No.)

**Not Applicable**  
(Zip Code)

**+41 (0)41 561 32 77**

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
<b>Common Shares, nominal value CHF 0.03</b>	<b>CRSP</b>	<b>The Nasdaq Global Market</b>

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES  NO

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). YES  NO

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or emerging growth company. See definition of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large Accelerated filer	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes  No

As of November 6, 2025, there were 95,300,233 shares of registrant's common shares outstanding.

Throughout this Quarterly Report on Form 10-Q, the “Company,” “CRISPR,” “CRISPR Therapeutics,” “we,” “us,” and “our,” except where the context requires otherwise, refer to CRISPR Therapeutics AG and its consolidated subsidiaries; “our board of directors” refers to the board of directors of CRISPR Therapeutics AG; and we generally refer to CASGEVY® (exagamglogene autotemcel [exa-cel]), as “CASGEVY”.

“CRISPR Therapeutics®” standard character mark and design logo, “CRISPRX™,” “CRISPR TX™,” “CTX112™,” “CTX131™,” “CTX211™,” “CTX213™,” “CTX310™,” “CTX320™,” “CTX340™,” “CTX450™,” “CTX460™” and SyNTase™ are trademarks and registered trademarks of CRISPR Therapeutics AG. CASGEVY® and the CASGEVY logo are registered trademarks of Vertex Pharmaceuticals Incorporated, and Vertex Pharmaceuticals Incorporated is the manufacturer and exclusive license holder of CASGEVY. All other trademarks and registered trademarks contained in this Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, trademarks, service marks and trade names referred to in this Quarterly Report on Form 10-Q may appear without the ® or ™ symbols and any such omission is not intended to indicate waiver of any such rights.

## FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q are forward-looking statements. These statements are often identified by the use of words such as “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “potential,” “will,” “would” or the negative or plural of these words or similar expressions or variations, although not all forward-looking statements contain these identifying words. Forward-looking statements in this Quarterly Report on Form 10-Q include, but are not limited to, statements about:

- our strategic plans to develop and, if approved, subsequently commercialize any product candidates we may develop, including plans and expectations for the commercialization of, and anticipated benefits of, CASGEVY, as well as plans for patient access to CASGEVY;
- the safety, efficacy and clinical progress of various clinical programs, including those for CASGEVY, CTX112, CTX131, CTX211, CTX310, CTX320 and SRSD107;
- the status of clinical trials, including development timelines and discussions with regulatory authorities related to product candidates under development by us and us and our collaborators;
- the results of preclinical studies and clinical trials, including ongoing clinical trials and any planned clinical trials, and research and development programs;
- the actual or potential benefits of regulatory designations, such as orphan drug, fast track and regenerative medicine advanced therapy, or RMAT, in the United States or such European equivalents;
- our ability to advance product candidates into, and successfully complete, clinical trials;
- the size and growth potential of the markets for our product candidates and our ability to serve those markets, including our estimates regarding the addressable patient population and potential market opportunity for our current and future product candidates;
- the rate and degree of market acceptance of our product candidates and the success of competing therapies that are or become available;
- our internal manufacturing capabilities and operation of our cell therapy manufacturing facility;
- our intellectual property coverage and positions, including those of our licensors and third parties as well as the status and potential outcome of proceedings involving any such intellectual property;
- the expected benefits of our collaborations;
- our strategy, goals, and anticipated financial performance;
- our anticipated expenses, ability to obtain funding for our operations and the sufficiency of our cash resources;
- the therapeutic value, development, and commercial potential of gene editing technologies and therapies, including CRISPR/Cas9 and SyNTase, as well as other technologies we develop and use; and
- the volatility of capital markets and unfavorable macroeconomic conditions resulting from factors including rising inflation, international tariffs, interest rate and currency rate fluctuations, economic sanctions and economic slowdown or recession, banking instability, monetary policy changes, geopolitical tensions or the outbreak of hostilities or war.

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and assumptions that could cause our actual results and the timing of certain events to differ materially from future results expressed or implied by the forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified herein, and those discussed in the section titled “Risk Factors,” set forth in Part II, Item 1A of this Quarterly Report on Form 10-Q, if any, our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on February 11, 2025, and in other SEC filings. You should not rely upon forward-looking statements as predictions of future events. Such forward-looking statements speak only as of the date of this report. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make or enter into.

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This Quarterly Report on Form 10-Q 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.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results, performance or achievements may be materially different from what we expect. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

Investors and others should note that we announce material information to our investors using our investor relations website (<https://crisprtx.gcs-web.com/>), SEC filings, press releases, public conference calls and webcasts. We use these channels as well as social media to communicate with the public about our Company, our business, our product candidates and other matters. It is possible that the information we post on social media could be deemed to be material information. Therefore, we encourage investors, the media, and others interested in our Company to review the information we post on the social media channels listed on our investor relations website.

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**PART I—FINANCIAL INFORMATION****Item 1. Financial Statements**

**CRISPR Therapeutics AG**  
**Condensed Consolidated Balance Sheets**  
(unaudited, in thousands, except share and per share data)

	As of	
	September 30, 2025	December 31, 2024
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 286,497	\$ 298,257
Marketable securities	1,629,213	1,605,569
Accounts receivable	—	25,000
Prepaid expenses and other current assets	13,382	8,306
Total current assets	1,929,092	1,937,132
Property and equipment, net	120,831	134,093
Marketable securities, non-current	28,412	—
Restricted cash	8,006	11,519
Operating lease assets	134,659	143,461
Other non-current assets	24,308	15,829
Total assets	<u>\$ 2,245,308</u>	<u>\$ 2,242,034</u>
<b>Liabilities and shareholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 11,798	\$ 14,709
Accrued expenses	84,182	41,072
Deferred revenue, current	1,295	3,845
Accrued tax liabilities	3,426	451
Operating lease liabilities	18,256	17,288
Other current liabilities	—	10,417
Total current liabilities	118,957	87,782
Deferred revenue, non-current	12,323	12,323
Operating lease liabilities, net of current portion	192,768	206,405
Other non-current liabilities	5,278	3,444
Total liabilities	329,326	309,954
Commitments and contingencies, see Note 7		
Shareholders' equity:		
Common shares, CHF 0.03 nominal value, 132,477,166 shares authorized at September 30, 2025 and at December 31, 2024, 93,998,110 and 85,912,297 shares issued at September 30, 2025 and December 31, 2024, respectively, 93,872,794 and 85,741,981 shares outstanding at September 30, 2025 and December 31, 2024, respectively	3,013	2,698
Treasury shares, at cost, 125,316 and 170,316 shares at September 30, 2025 and December 31, 2024, respectively	(60)	(62)
Additional paid-in capital	3,724,981	3,293,556
Accumulated deficit	(1,816,938)	(1,365,952)
Accumulated other comprehensive income	4,986	1,840
Total shareholders' equity	1,915,982	1,932,080
Total liabilities and shareholders' equity	<u>\$ 2,245,308</u>	<u>\$ 2,242,034</u>

*The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.*

**CRISPR Therapeutics AG**  
**Condensed Consolidated Statements of Operations and Comprehensive Loss**  
(unaudited, in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
<b>Revenue:</b>				
Collaboration revenue	\$ —	\$ —	\$ —	\$ —
Grant revenue	889	602	2,646	1,623
Total revenue	889	602	2,646	1,623
<b>Operating expenses:</b>				
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)
<b>Other income:</b>				
Other income, net	26,237	25,064	61,841	75,924
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)
<b>Net loss per common share — basic</b>				
	\$ (1.17)	\$ (1.01)	\$ (5.12)	\$ (3.92)
<b>Basic weighted-average common shares outstanding</b>				
	91,305,337	85,234,926	88,124,241	83,988,063
<b>Net loss per common share — diluted</b>				
	\$ (1.17)	\$ (1.01)	\$ (5.12)	\$ (3.92)
<b>Diluted weighted-average common shares outstanding</b>				
	91,305,337	85,234,926	88,124,241	83,988,063

*The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.*

**CRISPR Therapeutics AG**  
**Condensed Consolidated Statements of Shareholders' Equity**  
(unaudited, in thousands, except share and per share data)

	Common Shares		Treasury Shares		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income/(Loss)	Total Shareholders' Equity
	Shares	CHF 0.03 Nominal Value	Shares	Amount, at cost				
<b>Balance at December 31, 2023</b>	80,044,378	\$ 2,497	170,316	\$ (62)	\$ 2,878,155	\$ (999,700)	\$ 1,913	\$ 1,882,803
Issuance of common stock, net of issuance costs of \$3.8 million	3,929,610	132	—	—	277,015	—	—	277,147
Vesting of restricted shares	214,913	7	—	—	—	—	—	7
Exercise of vested options, net of issuance costs of \$0.6 million	632,683	22	—	—	23,844	—	—	23,866
Purchase of common stock under ESPP	16,026	—	—	—	764	—	—	764
Stock-based compensation expense	—	—	—	—	19,405	—	—	19,405
Other comprehensive loss	—	—	—	—	—	—	(3,465)	(3,465)
Net loss	—	—	—	—	—	(116,591)	—	(116,591)
<b>Balance at March 31, 2024</b>	<u>84,837,610</u>	<u>\$ 2,658</u>	<u>170,316</u>	<u>\$ (62)</u>	<u>\$ 3,199,183</u>	<u>\$ (1,116,291)</u>	<u>\$ (1,552)</u>	<u>\$ 2,083,936</u>
Vesting of restricted shares	163,013	6	—	—	—	—	—	6
Exercise of vested options, net of issuance costs of \$0.2 million	44,878	1	—	—	1,069	—	—	1,070
Stock-based compensation expense	—	—	—	—	23,672	—	—	23,672
Other comprehensive loss	—	—	—	—	—	—	(1,327)	(1,327)
Net loss	—	—	—	—	—	(126,408)	—	(126,408)
<b>Balance at June 30, 2024</b>	<u>85,045,501</u>	<u>\$ 2,665</u>	<u>170,316</u>	<u>\$ (62)</u>	<u>\$ 3,223,924</u>	<u>\$ (1,242,699)</u>	<u>\$ (2,879)</u>	<u>\$ 1,980,949</u>
<b>Issuance of common stock</b>	97,040	\$ 4	—	\$ —	\$ 5,939	\$ —	\$ —	\$ 5,943
Vesting of restricted shares	10,500	1	—	—	—	—	—	1
Exercise of vested options	161,516	14	—	—	2,732	—	—	2,746
Purchase of common stock under ESPP	21,219	—	—	—	974	—	—	974
Stock-based compensation expense	—	—	—	—	21,543	—	—	21,543
Other comprehensive income	—	—	—	—	—	—	13,444	13,444
Net loss	—	—	—	—	—	(85,942)	—	(85,942)
<b>Balance at September 30, 2024</b>	<u>85,335,776</u>	<u>\$ 2,684</u>	<u>170,316</u>	<u>\$ (62)</u>	<u>\$ 3,255,112</u>	<u>\$ (1,328,641)</u>	<u>\$ 10,565</u>	<u>\$ 1,939,658</u>

	Common Shares		Treasury Shares			Accumulated Other Comprehensive Income/(Loss)	Total Shareholders' Equity	
	Shares	CHF 0.03 Nominal Value	Shares	Amount, at cost	Additional Paid-in Capital			
<b>Balance at December 31, 2024</b>	85,741,981	\$ 2,698	170,316	\$ (62)	\$ 3,293,556	\$ (1,365,952)	\$ 1,840	\$ 1,932,080
Issuance of common stock, net of issuance costs of \$0.1 million	162,482	5	—	—	8,632	—	—	8,637
Vesting of restricted shares	396,943	15	—	—	—	—	—	15
Exercise of vested options, net of issuance costs of \$0.2 million	39,996	3	—	—	1,261	—	—	1,264
Purchase of common stock under ESPP	19,522	—	—	—	653	—	—	653
Stock-based compensation expense	—	—	—	—	20,212	—	—	20,212
Other comprehensive income	—	—	—	—	—	—	2,295	2,295
Net loss	—	—	—	—	—	(135,996)	—	(135,996)
<b>Balance at March 31, 2025</b>	<u>86,360,924</u>	<u>\$ 2,721</u>	<u>170,316</u>	<u>\$ (62)</u>	<u>\$ 3,324,314</u>	<u>\$ (1,501,948)</u>	<u>\$ 4,135</u>	<u>\$ 1,829,160</u>
Issuance of common stock, net of issuance costs of \$0.7 million	1,842,105	65	—	—	70,540	—	—	70,605
Vesting of restricted shares	104,941	5	—	—	—	—	—	5
Exercise of vested options, net of issuance costs of \$0.1 million	68,469	3	—	—	2,388	—	—	2,391
Stock-based compensation expense	—	—	—	—	17,607	—	—	17,607
Other comprehensive loss	—	—	—	—	—	—	(94)	(94)
Net loss	—	—	—	—	—	(208,549)	—	(208,549)
<b>Balance at June 30, 2025</b>	<u>88,376,439</u>	<u>\$ 2,794</u>	<u>170,316</u>	<u>\$ (62)</u>	<u>\$ 3,414,849</u>	<u>\$ (1,710,497)</u>	<u>\$ 4,041</u>	<u>\$ 1,711,125</u>
Issuance of common stock, net of issuance costs of \$6.5 million	4,899,409	198	—	—	275,120	—	—	275,318
Vesting of restricted shares	88,703	3	—	—	—	—	—	3
Exercise of vested options, net of issuance costs of \$0.3 million	483,637	18	(45,000)	2	17,585	—	—	17,605
Purchase of common stock under ESPP	24,606	—	—	—	823	—	—	823
Stock-based compensation expense	—	—	—	—	16,604	—	—	16,604
Other comprehensive income	—	—	—	—	—	—	945	945
Net loss	—	—	—	—	—	(106,441)	—	(106,441)
<b>Balance at September 30, 2025</b>	<u>93,872,794</u>	<u>\$ 3,013</u>	<u>125,316</u>	<u>\$ (60)</u>	<u>\$ 3,724,981</u>	<u>\$ (1,816,938)</u>	<u>\$ 4,986</u>	<u>\$ 1,915,982</u>

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

**CRISPR Therapeutics AG**  
**Condensed Consolidated Statements of Cash Flows**  
**(unaudited, in thousands)**

	<b>Nine Months Ended September 30,</b>	
	<b>2025</b>	<b>2024</b>
<b>Operating activities:</b>		
Net loss	\$ (450,986)	\$ (328,941)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation and amortization	13,818	14,417
Equity-based compensation	54,423	64,620
Other non-cash items, net	(15,214)	(23,463)
Acquired in-process research and development	96,253	—
Changes in:		
Accounts receivable	25,000	200,000
Prepaid expenses and other assets	(515)	5,109
Accounts payable and accrued expenses	40,473	(19,433)
Deferred revenue	(2,550)	(1,311)
Operating lease assets and liabilities	(3,867)	(3,380)
Other liabilities, net	(9,296)	(361)
Net cash used in operating activities	<u>(252,461)</u>	<u>(92,743)</u>
<b>Investing activities:</b>		
Purchase of property, plant and equipment	(397)	(1,647)
Purchase of in-process research and development	(25,000)	—
Investment in equity securities	(9,700)	(20,385)
Purchases of marketable securities	(745,197)	(1,199,072)
Maturities of marketable securities	711,290	834,795
Net cash used in investing activities	<u>(69,004)</u>	<u>(386,309)</u>
<b>Financing activities:</b>		
Proceeds from issuance of common shares, net of issuance costs	286,599	285,736
Proceeds from exercise of options and ESPP contributions, net of issuance costs	23,013	29,372
Net cash provided by financing activities	<u>309,612</u>	<u>315,108</u>
Effect of exchange rate changes on cash	94	66
Decrease in cash	<u>(11,759)</u>	<u>(163,878)</u>
Cash, cash equivalents and restricted cash, beginning of period	<u>309,776</u>	<u>401,068</u>
Cash, cash equivalents and restricted cash, end of period	<u>\$ 298,017</u>	<u>\$ 237,190</u>
<b>Supplemental disclosure of non-cash investing and financing activities</b>		
Equity issuance costs in accounts payable, accrued expenses, and other long-term liabilities	\$ 6,680	\$ 3,005
Acquired in-process research and development expense related to issuance of common shares	\$ 71,253	\$ —
<b>Reconciliation to amounts within the condensed consolidated balance sheets</b>		
	<b>As of September 30,</b>	
	<b>2025</b>	<b>2024</b>
Cash and cash equivalents	\$ 286,497	\$ 225,670
Prepaid expenses and other current assets	3,514	—
Restricted cash	8,006	11,520
<b>Cash, cash equivalents and restricted cash at end of period</b>	<u>\$ 298,017</u>	<u>\$ 237,190</u>

*The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.*

**CRISPR Therapeutics AG**  
**Notes to Condensed Consolidated Financial Statements**  
**(unaudited)**

## **1. Basis of Presentation and Significant Accounting Policies**

### ***Basis of Presentation***

The accompanying condensed consolidated financial statements are unaudited and have been prepared by the Company in accordance with accounting principles generally accepted in the United States of America, or U.S. GAAP.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. The Company views its operations and manages its business in one operating segment, which is the business of discovering, developing and commercializing therapies derived from or incorporating genome-editing technology. Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of operations for the three and nine-month interim periods ended September 30, 2025 and 2024.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These interim financial statements should be read in conjunction with the audited financial statements for the year ended December 31, 2024, which are contained in the 2024 Annual Report on Form 10-K filed with the Securities and Exchange Commission, or the SEC, on February 11, 2025.

### ***Use of Estimates***

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, revenue recognition, equity-based compensation expense and reported amounts of expenses during the period. The Company bases its estimates on historical experience and other market-specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions. Changes in estimates are reflected in reported results in the period in which they become known.

### ***Significant Accounting Policies***

The significant accounting policies used in preparation of these condensed consolidated financial statements for the three and nine months ended September 30, 2025 are consistent with those discussed in Note 2 to the consolidated financial statements in the Company's 2024 Annual Report on Form 10-K filed with the SEC on February 11, 2025 and discussed in Note 1 in the condensed consolidated financial statements in the Company's Quarterly Report on Form 10-Q filed with the SEC on August 4, 2025 with respect to acquired in-process research and development expenses and changes to the Company's policy around collaboration arrangements.

#### ***Acquired In-process Research and Development Expenses***

In-process research and development that is associated to a product that has not yet achieved regulatory approval and is acquired in a transaction that does not qualify as a business combination under U.S. GAAP is recorded as "Acquired in-process research and development" in the Company's condensed consolidated statements of operations and comprehensive loss in accordance with Accounting Standards Codification, or ASC, 730, *Research and development expense*, or ASC 730, as the asset acquired does not have an alternative future use. The Company classifies asset acquisitions of acquired in-process research and development as investing activities on its condensed consolidated statements of cash flows.

#### ***Collaboration Arrangements***

The Company records the elements of its collaboration agreements that represent joint operating activities in accordance with ASC 808, *Collaborative Agreements*, or ASC 808. Accordingly, the elements of the collaboration agreements that represent activities in which both parties are active participants and to which both parties are exposed to the significant risks and rewards that are dependent on the commercial success of the activities, are recorded as collaborative arrangements.

The Company evaluates the proper presentation of the commercial activities and the profit and loss sharing associated with the collaboration agreements. ASC 808 states that when payments between parties in a collaborative arrangement are not within the scope of other authoritative accounting literature, the income statement classification should be based on the nature of the arrangement, the nature of its business operations and the contractual terms of the arrangement. To the extent that these payments are not within the scope of other authoritative accounting literature, the income statement classification for the payments shall be based on an analogy to authoritative accounting literature or if there is no appropriate analogy, a reasonable, rational and consistently applied accounting policy election.

Collaboration costs specific to the Vertex Hemoglobinopathy Agreements (as defined in Note 6) accounted for under ASC 808

are presented within “collaboration expense, net” in the condensed consolidated statements of operations and comprehensive loss. Refer to Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for further discussion on the Vertex Hemoglobinopathy Agreements.

### Recent Accounting Pronouncements

In December 2023, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, 2023-09, *Improvements to Income Tax Disclosures*, which requires entities, on an annual basis, to disclose disaggregated information about their effective tax rate reconciliation and income taxes paid. The disclosure requirements will be applied on a prospective basis, with the option to apply them retrospectively. The standard is effective for fiscal years beginning after December 15, 2024, with early adoption permitted. The Company has adopted ASU 2023-09 and is currently evaluating the guidance, which is expected to only impact annual disclosures with no impact to the Company’s results of operations.

In November 2024, the FASB issued ASU 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures*, and in January 2025, the FASB issued ASU 2025-01, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures: Clarifying the Effective Date*. ASU 2024-03 requires disclosure of additional information about specific expense categories in the notes to the financial statements on an interim and annual basis. ASU 2024-03, as clarified by ASU 2025-01, is effective for fiscal years beginning after December 15, 2026, and for interim periods beginning after December 15, 2027, with early adoption permitted. The Company is currently evaluating the disclosure requirements related to this new standard.

## 2. Marketable Securities

The following table summarizes cash equivalents and marketable securities held at September 30, 2025 and December 31, 2024 (in thousands), which are recorded at fair value. The table below excludes \$201.9 million and \$193.9 million of cash at September 30, 2025 and December 31, 2024, respectively.

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
<b>September 30, 2025</b>				
Cash equivalents:				
Money market funds	\$ 82,068	\$ —	\$ —	\$ 82,068
Commercial paper	2,499	—	—	2,499
Total cash equivalents	84,567	—	—	84,567
Marketable securities:				
Corporate debt securities	1,097,526	4,250	(93)	1,101,683
Certificates and term deposits	182,469	—	—	182,469
Government-sponsored enterprise securities	276,625	714	(4)	277,335
Commercial paper	82,517	26	(9)	82,534
Total marketable debt securities	1,639,137	4,990	(106)	1,644,021
Corporate equity securities	10,386	3,218	—	13,604
Total marketable securities	1,649,523	8,208	(106)	1,657,625
Total cash equivalents and marketable securities	\$ 1,734,090	\$ 8,208	\$ (106)	\$ 1,742,192
<b>December 31, 2024</b>				
Cash equivalents:				
Money market funds	\$ 74,155	\$ —	\$ —	\$ 74,155
Corporate debt securities	882	—	—	882
Commercial paper	29,271	—	(9)	29,262
Total cash equivalents	104,308	—	(9)	104,299
Marketable securities:				
U.S. Treasury securities	5,936	2	—	5,938
Corporate debt securities	1,136,255	3,442	(1,592)	1,138,105
Certificates of deposit	52,372	—	—	52,372
Government-sponsored enterprise securities	266,877	482	(497)	266,862
Commercial paper	127,805	34	(39)	127,800
Total marketable debt securities	1,589,245	3,960	(2,128)	1,591,077
Corporate equity securities	10,387	4,600	(495)	14,492
Total marketable securities	1,599,632	8,560	(2,623)	1,605,569
Total cash equivalents and marketable securities	\$ 1,703,940	\$ 8,560	\$ (2,632)	\$ 1,709,868

The following table summarizes the net unrealized gain (loss) recorded on marketable debt and equity securities during the three and nine months ended September 30, 2025 and 2024 (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Unrealized gain recorded on marketable debt securities	\$ 1.0	\$ 13.4	\$ 3.1	\$ 8.6
Unrealized gain (loss) recorded on marketable equity securities	5.5	—	(0.9)	—

Unrealized gains and losses on the Company's marketable debt securities are included in comprehensive loss in the condensed consolidated statements of operations and comprehensive loss. Unrealized gains and losses due to the change in fair value of the Company's marketable equity securities are included in other income (expense), net, in the condensed consolidated statements of operations and comprehensive loss.

The following table summarizes the net unrealized gain (loss) position of the Company's marketable debt and equity securities as of September 30, 2025 and December 31, 2024 (in millions):

	September 30, 2025	December 31, 2024
Unrealized gain position of marketable debt securities	\$ 4.9	\$ 1.8
Unrealized gain position of marketable equity securities	3.2	4.1

The following table summarizes the aggregate fair value of marketable debt securities that were in an unrealized loss position as of September 30, 2025 and December 31, 2024 by the length of time the security has been in a loss position (in millions):

	September 30, 2025	December 31, 2024
Debt securities in an unrealized loss position for 12 months or less	\$ 125.9	\$ 451.9
Debt securities in an unrealized loss position for more than 12 months	40.4	31.8
Total debt securities in an unrealized loss position	\$ 166.3	\$ 483.7

As of September 30, 2025, there were \$28.4 million in marketable debt securities in an unrealized loss position for more than twelve months with maturities beyond one year. As of December 31, 2024, no marketable debt securities in an unrealized loss position for more than twelve months had maturities beyond one year.

The Company determined that there is no material credit risk associated with the above investments as of September 30, 2025. The Company has the intent and ability to hold such securities until recovery. As a result, the Company did not record any charges for credit-related impairments for its marketable securities for the three and nine months ended September 30, 2025 and 2024. No available-for-sale debt securities held as of September 30, 2025 had remaining maturities greater than thirty months.

#### *Equity Investments Without Readily Determinable Fair Value*

The Company holds investments in privately-held companies in the form of equity securities without readily determinable fair values and in which the Company does not have a controlling interest or significant influence. These investments had a net carrying value of \$22.6 million and \$12.9 million as of September 30, 2025 and December 31, 2024, respectively, and are classified within other non-current assets on the condensed consolidated balance sheets. There were no upward or downward adjustments for observable price changes or impairment charges recorded for the three and nine months ended September 30, 2025 and 2024 related to these equity securities.

### 3. Fair Value Measurements

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the fair value hierarchy classification of such fair values as of September 30, 2025 and December 31, 2024 (in thousands):

	Total	Fair Value Measurements at September 30, 2025		
		Level 1	Level 2	Level 3
Cash and cash equivalents:				
Cash	\$ 201,930	\$ 201,930	\$ —	\$ —
Money market funds	82,068	82,068	—	—
Commercial paper	2,499	—	2,499	—
Marketable securities:				
Corporate debt securities	1,101,683	—	1,101,683	—
Certificates and term deposits	182,469	—	182,469	—
Government-sponsored enterprise securities	277,335	—	277,335	—
Commercial paper	82,534	—	82,534	—
Corporate equity securities	13,604	577	13,027	—
<b>Total</b>	<b>\$ 1,944,122</b>	<b>\$ 284,575</b>	<b>\$ 1,659,547</b>	<b>\$ —</b>

	Total	Fair Value Measurements at December 31, 2024		
		Level 1	Level 2	Level 3
Cash and cash equivalents:				
Cash	\$ 193,958	\$ 193,958	\$ —	\$ —
Money market funds	74,155	74,155	—	—
Corporate debt securities	882	—	882	—
Commercial paper	29,262	—	29,262	—
Marketable securities:				
U.S. Treasury securities	5,938	—	5,938	—
Corporate debt securities	1,138,105	—	1,138,105	—
Certificates of deposit	52,372	—	52,372	—
Government-sponsored enterprise securities	266,862	—	266,862	—
Commercial paper	127,800	—	127,800	—
Corporate equity securities	14,492	2,391	12,101	—
<b>Total</b>	<b>\$ 1,903,826</b>	<b>\$ 270,504</b>	<b>\$ 1,633,322</b>	<b>\$ —</b>

Marketable securities classified as Level 1 within the valuation hierarchy consist of corporate equity securities with quoted prices in active markets. Marketable securities classified as Level 2 within the valuation hierarchy generally consist of U.S. treasury securities and government agency securities, certificates of deposit, corporate bonds, commercial paper and warrants to purchase common shares of publicly traded companies. The Company estimates the fair value of these marketable securities by taking into consideration valuations obtained from third-party pricing sources.

### 4. Property and Equipment, net

Property and equipment, net, consists of the following (in thousands):

	As of	
	September 30, 2025	December 31, 2024
Computer equipment	\$ 4,344	\$ 3,833
Furniture, fixtures and other	8,554	8,554
Laboratory equipment	41,897	42,008
Leasehold improvements	145,852	145,852
Construction work in process	5,797	6,118
Total property and equipment, gross	206,444	206,365
Accumulated depreciation	(85,613)	(72,272)
<b>Total property and equipment, net</b>	<b>\$ 120,831</b>	<b>\$ 134,093</b>

Depreciation expense for the three and nine months ended September 30, 2025 was \$4.4 million and \$13.8 million, respectively. Depreciation expense for the three and nine months ended September 30, 2024 was \$4.8 million and \$14.4 million, respectively.

## 5. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	As of	
	September 30, 2025	December 31, 2024
Payroll and employee-related costs	\$ 10,154	\$ 18,443
Research and development costs	10,535	15,549
Collaboration costs	57,968	—
Licensing fees	640	1,850
Professional fees	3,009	3,086
Intellectual property costs	1,313	1,513
Other	563	631
Total	<u>\$ 84,182</u>	<u>\$ 41,072</u>

## 6. Significant Contracts

### *Agreements with Vertex*

For purposes of this Note 6 and Note 7, CASGEVY (exagamglogene autotemcel [exa-cel]) is referred to as “CASGEVY”.

#### *2015 collaboration*

In 2015, the Company entered into a strategic collaboration, option and license agreement, or the 2015 Collaboration Agreement, with Vertex Pharmaceuticals Incorporated, or Vertex. The 2015 Collaboration Agreement is focused on the use of the Company’s CRISPR/Cas9 gene editing technology to discover and develop potential new treatments aimed at the underlying genetic causes of human disease. The Company and Vertex amended the 2015 Collaboration Agreement in 2017 and 2019 with Amendment No. 1 and Amendment No. 2, respectively, namely to clarify Vertex’s option rights under the 2015 Collaboration Agreement and to modify certain definitions and provisions of the 2015 Collaboration Agreement to make them consistent with the JDA (as defined below) and a strategic collaboration and license agreement from 2019 for the development and commercialization of products for the treatment of Duchenne muscular dystrophy and myotonic dystrophy Type 1. In 2017, Vertex exercised an option granted to it under the 2015 Collaboration Agreement to obtain a co-exclusive license to develop and commercialize hemoglobinopathy and beta-globin targets, and in 2019, Vertex exercised the remaining options granted to it under the 2015 Collaboration Agreement to exclusively license certain collaboration targets developed under the 2015 Collaboration Agreement.

#### *Hemoglobinopathies collaboration*

In 2017, following Vertex’s exercise of its option to obtain a co-exclusive license to develop and commercialize hemoglobinopathy and beta-globin targets, the Company and Vertex entered into a joint development and commercialization agreement, or the JDA, and agreed for potential hemoglobinopathy treatments, including CASGEVY, the Company and Vertex would share equally all research and development costs and worldwide revenues. In 2021, the Company and Vertex amended and restated the JDA, or the A&R Vertex JDCA (as amended and in effect, from time to time), pursuant to which the parties agreed to, among other things, (a) adjust the governance structure for the collaboration and adjust the responsibilities of each party thereunder, whereby Vertex leads and has all decision making (i.e., control) in relation to the CASGEVY program prospectively; (b) adjust the allocation of net profits and net losses between the parties with respect to CASGEVY only, which will be allocated 40% to the Company and 60% to Vertex, prospectively; and (c) exclusively license (subject to the Company’s reserved rights to conduct certain activities) certain intellectual property rights to Vertex relating to the specified product candidates and products (including CASGEVY) that may be researched, developed, manufactured and commercialized on a worldwide basis under the A&R Vertex JDCA. Additionally, for 2022, 2023, and 2024, the Company had an option to defer a portion of its share of costs under the A&R Vertex JDCA if spending on the CASGEVY program exceeded specified amounts. Any deferred amounts under the A&R Vertex JDCA, as amended, are only payable to Vertex as an offset against future profitability of the CASGEVY program and the amounts payable are capped at a specified maximum amount per year.

In December 2023, the Company entered into an amendment to the A&R Vertex JDCA, or Amendment No. 1 to the A&R Vertex JDCA, with Vertex related to the global development, manufacturing, and commercialization of CASGEVY. Pursuant to Amendment No. 1 to the A&R Vertex JDCA, among other things, the Company and Vertex agreed to (a) allocate certain costs arising from a license agreement with a third party, resulting in a current payment due to Vertex by the Company of \$20.0 million upon an event specified in Amendment No. 1 to the A&R Vertex JDCA, and (b) adjust, under certain specified circumstances, the timing of and portion of the Company’s share of costs it is permitted to defer under the agreement.

### *Letter Agreement*

In May 2024, Vertex and the Company entered into a letter agreement, or the Letter Agreement, with respect to the priority review voucher issued by the U.S. Food and Drug Administration to Vertex as the sponsor of the rare pediatric disease product application for CASGEVY. Vertex and the Company agreed that if Vertex utilizes or transfers the priority review voucher prior to the first calendar year in which the CASGEVY program generates a net profit, Vertex will pay the Company \$43.0 million or an amount equal to 42% of the net proceeds from such transfer, as applicable. If the CASGEVY program begins generating calendar-year net profits prior to such utilization or transfer, Vertex will instead pay the Company up to \$43.0 million set-off by deductions Vertex would otherwise be eligible to take against the CASGEVY program's net profits due to the Company related to amounts deferred previously by the Company.

### *Collaboration in the field of diabetes*

In 2021, the Company and ViaCyte, Inc., or ViaCyte, entered into a joint development and commercialization agreement, or the ViaCyte JDCA, to jointly develop and commercialize product candidates and shared products for the diagnosis, treatment or prevention of diabetes type 1, diabetes type 2 or insulin dependent / requiring diabetes throughout the world. In the third quarter of 2022, Vertex acquired ViaCyte, and ViaCyte became a wholly-owned subsidiary of Vertex. In March 2023, (1) the Company and ViaCyte entered into an amendment to the ViaCyte JDCA, or the ViaCyte JDCA Amendment, and adjusted certain rights and obligations of the Company and ViaCyte under the ViaCyte JDCA, and (2) the Company and Vertex entered into a non-exclusive license agreement, or the Non-Ex License Agreement, pursuant to which the Company agreed to license to Vertex, on a non-exclusive basis, certain of its gene editing intellectual property to exploit certain products for the diagnosis, treatment or prevention of diabetes type 1, diabetes type 2 or insulin dependent / requiring diabetes throughout the world. Subsequently, ViaCyte elected to opt-out of the ViaCyte JDCA. Per the opt-out terms, the ongoing collaboration assets are now wholly owned by the Company, subject to a royalty on future sales owed to ViaCyte. The opt-out became effective in early February 2024.

In 2024, the Company received a \$10.0 million research milestone achieved in the fourth quarter of 2024 and recorded a receivable of \$25.0 million as of December 31, 2024 related to an additional research milestone achieved under the Non-Ex License Agreement in the fourth quarter of 2024. The Company is eligible to receive additional milestone payments under the Non-Ex License Agreement of up to \$125.0 million in aggregate, which are dependent on the achievement of pre-determined research, development and commercial milestones for certain products utilizing the licensed intellectual property. Additionally, the Company is eligible to receive tiered royalties on the sales of certain products in the low to mid-single digits.

### **Accounting Analysis**

For purposes of this Note 6, the 2015 Collaboration Agreement, Amendment No. 1, Amendment No. 2, A&R Vertex JDCA, and Amendment No. 1 to the A&R Vertex JDCA are collectively referred to as the "Vertex Hemoglobinopathy Agreements" and the Non-Ex License Agreement and ViaCyte JDCA Amendment are collectively referred to as the "March 2023 Diabetes Agreements."

The Vertex Hemoglobinopathy Agreements and the March 2023 Diabetes Agreements include components of a customer-vendor relationship as defined under ASC 606, *Revenue from Contracts with Customers*, or ASC 606, collaborative arrangements as defined under ASC 808 and research and development costs as defined under ASC 730, *Research and Development*, or ASC 730. Specifically, with regards to the March 2023 Diabetes Agreements, the Company concluded that the non-exclusive license is a performance obligation under ASC 606 and the ongoing research and development services under the ViaCyte JDCA Amendment are a unit of account under ASC 808.

The Company has determined that recognition criteria for the Letter Agreement has not been met and will not be met until the priority review voucher is (i) utilized or (ii) there is sufficient profitability such that Vertex is obligated to pay the Company under the Letter Agreement.

### **Accounting Analysis Under ASC 606**

#### *Accounting for the March 2023 Diabetes Agreements*

##### *Recognition of Revenue*

No revenue was recognized under the March 2023 Diabetes Agreements for the three and nine months ended September 30, 2025 and 2024.

##### *Milestones under the Non-Ex License Agreement*

As of September 30, 2025, the Company is eligible to receive potential future milestone payments from Vertex of up to \$125.0 million in the aggregate under the Non-Ex License Agreement depending on the achievement of pre-determined research, development and commercial milestones for certain products utilizing the licensed intellectual property. Additionally, the Company is eligible to receive tiered royalties on the sales of certain products in the low to mid-single digits.

Each of the remaining milestones under the Non-Ex License Agreement are fully constrained as of September 30, 2025. There is uncertainty as to whether the events to obtain the research and developmental milestones will be achieved given the nature of clinical development and the stage of the CRISPR/Cas9 technology. The remaining research, development and regulatory milestones will be

constrained until it is probable that a significant revenue reversal will not occur. Commercial milestones and royalties relate predominantly to a license of intellectual property and are determined by sales or usage-based thresholds. The commercial milestones and royalties are accounted for under the royalty recognition constraint and will be accounted for as constrained variable consideration. The Company applies the royalty recognition constraint for each commercial milestone and will not recognize revenue for each until the subsequent sale of a licensed product (achievement of each) occurs.

#### *Accounting for the Vertex Hemoglobinopathy Agreements*

##### *Recognition of Revenue*

No revenue was recognized under the Vertex Hemoglobinopathy Agreements for the three and nine months ended September 30, 2025 and 2024.

##### *Milestones under the Vertex Hemoglobinopathy Agreements*

The Company has evaluated the milestones that may be received in connection with the Vertex Hemoglobinopathy Agreements.

Under the 2015 Collaboration Agreement and subsequent amendments, the Company is eligible to receive up to \$410.0 million in additional development, regulatory and commercial milestones and royalties on net product sales for each of the three collaboration targets that Vertex licensed in 2019. Each milestone is payable only once per collaboration target, regardless of the number of products directed to such collaboration target that achieve the relevant milestone event.

The Company has the option to conduct research at its own cost in certain defined areas. If such research is beneficial to a program under the Vertex Hemoglobinopathy Agreements and the applicable product ultimately achieves regulatory approval in such areas, the Company could be entitled to receive from Vertex certain milestone payments aggregating to high eight digits.

Each of the remaining milestones described above are fully constrained as of September 30, 2025. There is uncertainty that the events to obtain the research and developmental milestones will be achieved given the nature of clinical development and the stage of the CRISPR/Cas9 technology. The remaining research, development and regulatory milestones will be constrained until it is probable that a significant revenue reversal will not occur. Commercial milestones and royalties relate predominantly to a license of intellectual property and are determined by sales or usage-based thresholds. The commercial milestones and royalties are accounted for under the royalty recognition constraint and will be accounted for as constrained variable consideration. The Company applies the royalty recognition constraint for each commercial milestone and will not recognize revenue for each until the subsequent sale of a licensed product (achievement of each) occurs.

#### *Accounting Analysis under ASC 808*

##### *Vertex Hemoglobinopathy Agreements*

In connection with the Vertex Hemoglobinopathy Agreements, the Company identified the following collaborative elements, which are accounted for under ASC 808: (i) development and commercialization services for shared products, including any transition services related to CASGEVY under the A&R Vertex JDCA; (ii) R&D Services for follow-on products; and (iii) committee participation. The related impact of the cost sharing under the Vertex Hemoglobinopathy Agreements is included within collaboration expense, net, in the condensed consolidated statements of operations and comprehensive loss.

During the three and nine months ended September 30, 2025, the Company recognized \$57.1 million and \$159.8 million of collaboration expense, net, respectively. Collaboration expense, net, during the three and nine months ended September 30, 2025 was net of \$0.4 million and \$1.1 million of reimbursements from Vertex, respectively.

In the third quarter of 2024, the Company exercised its option to defer specified costs on the CASGEVY program in excess of \$110.3 million under the A&R Vertex JDCA, as amended. During the three and nine months ended September 30, 2024, the Company recognized \$11.2 million and \$110.3 million of collaboration expense, net, respectively. Collaboration expense, net, during the three and nine months ended September 30, 2024 was net of \$0.5 million and \$1.8 million of reimbursements from Vertex, respectively.

#### *Additional Accounting Considerations*

The Company is eligible to receive potential future payments of up to \$775.0 million upon the successful achievement of specified development, regulatory and commercial milestones under a strategic collaboration and license agreement with Vertex from 2019 for the development and commercialization of products for the treatment of Duchenne muscular dystrophy, or DMD, and myotonic dystrophy Type 1, or DM1. The Company is also eligible to receive tiered royalties on future net sales on any products that may result from this collaboration; however, the Company has the option to forego the DM1 milestones and royalties to co-develop and co-commercialize all DM1 products globally.

Each of the remaining milestones are fully constrained as of September 30, 2025. There is uncertainty that the events to obtain the research and developmental milestones will be achieved given the nature of clinical development and the stage of the CRISPR/Cas9 technology. The remaining research, development and regulatory milestones will be constrained until it is probable that a significant revenue reversal will not occur. Commercial milestones and royalties relate predominantly to a license of intellectual property and are determined by sales or usage-based thresholds. The commercial milestones and royalties are accounted for under the royalty recognition constraint and will be accounted for as constrained variable consideration. The Company applies the royalty

recognition constraint for each commercial milestone and will not recognize revenue for each until the subsequent sale of a licensed product (achievement of each) occurs.

As of September 30, 2025, there was \$12.3 million of non-current deferred revenue related to the strategic collaboration and license agreement with Vertex from 2019 for the development and commercialization of products for the treatment of DMD and DM1, which is unchanged from December 31, 2024. The transaction price allocated to the remaining performance obligations was \$12.3 million.

#### ***Agreement with Sirius Therapeutics***

On May 19, 2025, the Company entered into a collaboration, option and license agreement, or the Sirius Agreement, with Sirius Therapeutics and certain of its affiliates, or Sirius, pursuant to which, among other things, (1) Sirius and the Company will collaborate on the research, development, manufacture, commercialization and use of certain collaboration products utilizing Sirius' siRNA technology for targeting Factor XI, including SRSD107, collectively, the Sirius Collaboration Products; and (2) Sirius granted to the Company options to exclusively license Sirius siRNA technology to target up to two licensed targets from a list of seven reserved targets for the research, development, manufacture and commercialization of licensed products, collectively the Sirius Licensed Products, in exchange for the potential to receive certain option fees, milestone payments and royalties.

In connection with entering into the Sirius Agreement, the Company made an upfront cash payment to Sirius of \$25.0 million and also entered into a share issuance agreement with Sirius, pursuant to which the Company registered and issued to Sirius 1,842,105 common shares equal to approximately \$70.0 million based on a price per common share equal to \$38.00, nominal value CHF 0.03 per share, or the Sirius Shares.

With respect to Sirius Collaboration Products, the Company and Sirius will share equally all development and commercialization costs. For the first collaboration product candidate successfully developed, the Company will be the lead party responsible for commercialization efforts in the United States and Sirius will be the lead party responsible for commercialization efforts in Greater China. The parties will determine the lead party responsible for commercialization in the rest of the world at a future date. The Company and Sirius will share equally net profits and net losses incurred under the Sirius Agreement with respect to all Sirius Collaboration Products, except in the event that a party opts out of the joint development and commercialization. The Company will pay Sirius certain specified future development and regulatory milestones of up to an aggregate of \$87.5 million for the first Sirius Collaboration Products to achieve the applicable milestone events. At the Company's sole election, such milestone payments may be paid in cash, common shares of the Company, or a combination thereof.

With respect to the Sirius Licensed Products, if the Company elects to exercise its option to a licensed target to research, develop, manufacture and commercialize Sirius Licensed Products, the Company will make a one-time \$10.0 million payment per option exercise, each, a Sirius Option Payment, to Sirius. The Sirius Option Payment is payable up to two times. In addition, the Company will pay Sirius certain specified future development, regulatory and sales milestones of up to an aggregate of \$300.0 million for the first Sirius Licensed Product relating to each licensed target to achieve the applicable milestone events, as well as tiered royalty payments in the mid-single digits to low double digits range on future sales of a commercialized Sirius Licensed Product. The royalty payments are subject to reduction under certain specified conditions set forth in the Sirius Agreement. In addition, at the Company's sole election, such milestones may be paid in cash, common shares of the Company, or a combination thereof. The Company is solely responsible for all research, development, manufacturing and global commercialization activities and associated costs for the Sirius Licensed Products, as well as all associated costs related to Sirius activities set forth in any applicable research plan relating thereto.

#### ***Accounting for the Sirius Agreement***

The Company determined that substantially all the fair value of the upfront payment under the Sirius Agreement was attributable to acquired in-process research and development for which there was no alternative future use and that no substantive processes were acquired that would constitute a business. As a result, the Company recorded \$96.3 million to acquired in-process research and development in the condensed consolidated statement of operations and comprehensive loss. The \$96.3 million represented the \$25.0 million upfront cash payment to Sirius, as well as \$71.3 million in expense related to the issuance of the Sirius Shares at a fair value of \$38.68 per share, which was the fair value of the Company's common shares on the effective date of the Sirius Agreement.

The Sirius Agreement includes components of collaborative arrangements as defined under ASC 808 and research and development costs as defined under ASC 730.

Specifically, development and commercialization costs for the Sirius Collaboration Products contain collaborative elements accounted for under ASC 808, and the related impact of cost sharing for the Sirius Collaboration Products under the Sirius Agreement is included within research and development expenses in the condensed consolidated statement of operations and comprehensive loss. Costs related to Sirius Licensed Products under the Sirius Agreement are included within research and development expenses in the consolidated statement of operations and comprehensive loss.

Research and development costs under the Sirius Agreement were not material for the three and nine months ended September 30, 2025.

## 7. Commitments and Contingencies

### *Leases*

Refer to Note 7 to the consolidated financial statements in the Company's 2024 Annual Report on Form 10-K filed with the SEC on February 11, 2025 for discussion of the Company's lease arrangements.

### *Litigation*

In the ordinary course of business, the Company is or has been from time to time involved in lawsuits, investigations, proceedings and threats of litigation related to, among other things, the Company's intellectual property estate (including certain in-licensed intellectual property), commercial arrangements and other matters. Such proceedings may include quasi-litigation, *inter partes* administrative proceedings in the U.S. Patent and Trademark Office and the European Patent Office or patent offices in other countries, involving the Company's intellectual property estate including certain in-licensed intellectual property. The outcome of any of the foregoing, regardless of the merits, is inherently uncertain. In addition, litigation and related matters are costly and may divert the attention of Company's management and other resources that would otherwise be engaged in other activities. If the Company is unable to prevail in any such proceedings, the Company's business, results of operations, liquidity and financial condition could be adversely affected.

### *Letters of Credit*

As of September 30, 2025, the Company had restricted cash of \$11.5 million, representing letters of credit securing the Company's obligations under certain leased facilities. The letters of credit are secured by cash held in a restricted depository account, with \$3.5 million included in "Prepaid expenses and other current assets" and \$8.0 million included in "Restricted cash" on the Company's condensed consolidated balance sheet as of September 30, 2025.

### *Research, Manufacturing, License and Intellectual Property Agreements*

The Company has engaged several research institutions and companies to identify new delivery strategies and applications of the Company's gene editing technology. The Company is also a party to a number of license agreements which require significant upfront payments and may be required to make future royalty payments and potential milestone payments from time to time. In addition, the Company is also a party to intellectual property agreements, which require maintenance and milestone payments from time to time. Further, the Company is a party to a number of manufacturing agreements that require upfront payments for the future performance of services.

In connection with these agreements, on a product-by-product basis, the counterparties are eligible to receive up to low eight-digit potential payments upon specified research, development and regulatory milestones. In addition, on a product-by-product basis, the counterparties are eligible to receive potential commercial milestone payments based on specified annual sales thresholds. The potential payments are low-single digit percentages of the specified annual sales thresholds. The counterparties are also eligible to receive low single-digit royalties on future net sales.

Contractual amounts due under license and intellectual property agreements are accrued and, if a contingency exists related to the interpretation of the amounts due under the license agreement, the Company recognizes a liability for the amount that is probable and estimable. When no amount within the range of potential payments is a better estimate than any other amount, the minimum amount in the range is accrued. In the second quarter of 2025, a third-party licensor formally engaged with the Company regarding certain matters under their intellectual property contracts with the Company that may lead to further actions that could result in additional amounts being owed by the Company to such third party. As of September 30, 2025, the Company is unable to provide an estimate of a reasonably possible loss or range of loss. The ultimate resolution of this matter could result in an outcome that is materially different from the amounts accrued as of September 30, 2025.

Under certain circumstances and if certain contingent future events occur, Vertex is eligible to receive up to \$395.0 million in potential specified research, development, regulatory and commercial milestones and tiered single-digit percentage royalties on future net sales related to a specified target under an amendment to the 2015 Collaboration Agreement (as such term is defined in Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q above). In addition, Vertex has the option to conduct research at its own cost in certain defined areas that, if beneficial to a program under the Vertex Hemoglobinopathy Agreements and the applicable product ultimately achieves regulatory approval, could result in the Company owing Vertex certain milestone payments aggregating to high eight digits, subject to certain limitations on the profitability of the program.

Under the A&R Vertex JDCA, as amended, for 2022, 2023 and 2024, the Company had an option to defer a portion of its share of costs if spending on the CASGEVY program exceeded specified amounts, which the Company exercised in each such year, resulting in deferred costs of \$221.8 million, in the aggregate. Any deferred amounts under the A&R Vertex JDCA, as amended, are only payable to Vertex as an offset against future profitability of the CASGEVY program and the amounts payable are capped at a specified maximum amount per year. These deferred costs on the CASGEVY program will be recognized by the Company when recoverability of such deferred amounts by Vertex is probable and the amount can be reasonably estimated. As of September 30, 2025, no contingent payments have been accrued to date.

The Company may be required to make future potential payments to Sirius under the Sirius Agreement defined and described in Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q. Potential payments to Sirius include (i) up to \$20.0 million in Sirius Option Payments, (ii) up to \$300.0 million in certain specified future development, regulatory and sales milestones for the first Sirius Licensed Product relating to each licensed target to achieve the applicable milestones, as well as tiered royalty payments in the mid-single digits to low double digits range on future sales of a commercialized Sirius Licensed Product, and (iii) up to \$87.5 million in certain specified future development and regulatory milestones related to the Sirius Collaboration Products.

## 8. Share Capital

All of the Company's common shares are authorized under Swiss corporate law with a nominal value of 0.03 CHF per share. Though the nominal value of common shares is stated in Swiss francs, the Company continues to use U.S. dollars as its reporting currency for preparing the condensed consolidated financial statements.

As of September 30, 2025, the Company's share capital consists of 95,047,997 registered common shares with a nominal value of CHF 0.03 per share, 8,202,832 registered common shares reserved for potential issuance of bonds or similar instruments and 19,537,850 registered common shares reserved for the Company's employee equity incentive plans. In addition, the Board of Directors is authorized to conduct one or more increases of the share capital at any time until June 8, 2028, or the expiration of the capital band if earlier, up to an upper limit of CHF 3,142,094.52 by issuing a corresponding number of registered shares with a nominal value of CHF 0.03 each to be fully paid in. As of September 30, 2025, the number of shares that may be issued under the capital band is 9,688,487 registered common shares.

### *Common Share Issuances*

#### *At-the-Market Offerings*

The Company has entered into an Open Market Sale Agreement<sup>SM</sup>, or the Sales Agreement, with Jefferies LLC under which the Company, at its sole discretion, is able to offer and sell, from time to time at prevailing market prices, its common shares. The following are in connection with the Sales Agreement.

#### *2021 ATM*

In January 2021, the Company filed a prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to \$600.0 million, or, together with the subsequent prospectus supplements filed in July 2021 and August 2024 relating to the common shares remaining under the original prospectus supplement, the 2021 ATM. For the nine months ended September 30, 2025, the Company issued and sold an aggregate of 5.1 million common shares under the 2021 ATM at an average price of \$57.39 per share for aggregate proceeds of \$286.8 million, which were net of equity issuance costs of \$3.7 million, excluding stamp taxes of \$2.9 million. Activity under the 2021 ATM was not material for the nine months ended September 30, 2024. As of September 30, 2025, the Company has issued and sold an aggregate of 7.0 million common shares under the 2021 ATM at an average price of \$75.40 per share for aggregate proceeds of \$520.0 million, which were net of equity issuance costs of \$6.8 million, excluding stamp taxes of \$5.2 million. As of September 30, 2025, common shares having aggregate gross proceeds up to \$73.2 million remain available under the 2021 ATM.

From September 30, 2025 through the date of issuance of this Quarterly Report on Form 10-Q, the Company has issued and sold an aggregate of 1.0 million common shares under the 2021 ATM at an average price of \$70.53 per share for aggregate proceeds of \$72.2 million, which were net of equity issuance costs of \$1.0 million, excluding stamp taxes of \$0.7 million.

#### *2025 ATM*

In October 2025, the Company filed a new prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to \$600.0 million, or the 2025 ATM. Through the date of issuance of this Quarterly Report on Form 10-Q, the Company has issued and sold an aggregate of 0.2 million common shares under the 2025 ATM at an average price of \$73.53 per share for aggregate proceeds of \$11.7 million, which were net of equity issuance costs of \$0.1 million, excluding stamp taxes of \$0.1 million. Common shares having aggregate gross proceeds up to \$588.3 million remain available under the 2025 ATM.

#### *Share Issuance Agreement with Sirius Therapeutics*

As described in Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q, the Company and Sirius entered into a share issuance agreement and the Company registered and issued 1,842,105 of the Company's common shares to Sirius, nominal value CHF 0.03 per share, at an issue price of \$38.00 per share as partial consideration for entering into the Sirius Agreement.

#### *Additional Financings*

In February 2024, the Company entered into an investment agreement for the sale of approximately \$280.0 million of its common shares to a group of institutional investors in a registered direct offering, at a price per share of \$71.50. The Company received net proceeds of \$279.0 million, excluding stamp taxes due of \$2.8 million.

## 9. Stock-based Compensation

During the three and nine months ended September 30, 2025 and 2024, the Company recognized the following stock-based compensation expense (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development	\$ 7,578	\$ 11,949	\$ 25,115	\$ 36,542
General and administrative	9,026	9,594	29,308	28,078
<b>Total</b>	<b>\$ 16,604</b>	<b>\$ 21,543</b>	<b>\$ 54,423</b>	<b>\$ 64,620</b>

### Stock option activity

The following table summarizes stock option activity for the nine months ended September 30, 2025:

	Shares	Weighted-average exercise price per share
Outstanding at December 31, 2024	7,288,883	\$ 58.07
Granted	1,072,255	41.91
Exercised	(592,102)	36.85
Cancelled or forfeited	(1,038,119)	65.18
Outstanding at September 30, 2025	6,730,917	\$ 56.27
Exercisable at September 30, 2025	4,639,031	\$ 58.98
Vested and expected to vest at September 30, 2025	6,730,917	\$ 56.27

As of September 30, 2025, total unrecognized compensation expense related to stock options was \$60.1 million, which the Company expects to recognize over a remaining weighted-average period of 2.4 years.

### Restricted stock activity

The following table summarizes restricted stock activity for the nine months ended September 30, 2025:

	Shares	Weighted-Average Grant Date Fair Value
Unvested balance at December 31, 2024	2,397,643	\$ 59.21
Granted	795,371	43.60
Vested	(590,587)	63.08
Cancelled or forfeited	(561,698)	59.51
Unvested balance at September 30, 2025	2,040,729	\$ 51.36

As of September 30, 2025, total unrecognized compensation expense related to unvested restricted common shares was \$78.4 million, which the Company expects to recognize over a remaining weighted-average vesting period of 2.7 years.

## 10. Net Loss Per Share Attributable to Common Shareholders

Basic net loss per share is calculated by dividing net loss attributable to common shareholders by the weighted-average number of common shares outstanding during the period. Diluted net loss per share is calculated by adjusting weighted-average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period using the treasury stock method. For purposes of the diluted net loss per share calculation, stock options, unvested restricted common shares and ESPP shares are considered to be common stock equivalents but are excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive; therefore, basic and diluted net loss per share were the same for all periods presented as a result of the Company's net loss. The Company's net loss is net loss attributable to common shareholders for all periods presented.

The Company did not include the securities in the following table in the computation of the net loss per share calculations for the three and nine months ended September 30, 2025 and 2024 because the effect would have been anti-dilutive during each period:

	September 30,	
	2025	2024
Outstanding options	6,730,917	7,053,202
Unvested restricted common shares	2,040,729	1,927,984
ESPP	5,829	8,313
Total	8,777,475	8,989,499

## 11. Income Taxes

During the three and nine months ended September 30, 2025, the Company recorded an income tax provision of \$0.6 million and \$3.0 million, respectively, representing an effective tax rate of (0.6%) and (0.7%), respectively. During the three and nine months ended September 30, 2024, the Company recorded an income tax provision of \$0.9 million and \$2.9 million, respectively, representing an effective tax rate of (1.0%) and (0.9%), respectively. The income tax provision for the three and nine months ended September 30, 2025 is primarily attributable to the income generated by the Company's U.S. subsidiaries. The difference in the statutory tax rate and effective tax rate is primarily a result of the jurisdictional mix of earnings, research credits generated, and the valuation allowance recorded against certain deferred tax assets. The Company maintains a valuation allowance against certain deferred tax assets that are not more-likely-than-not realizable.

On July 4, 2025, the "One Big Beautiful Bill Act" was enacted, introducing significant changes to the U.S. federal income tax code. Key provisions include modifications to the treatment of research and development, or R&D, expenditures, adjustments to interest deductibility, and changes to international tax rules that may affect the Company's global structure. As a biotechnology company with substantial investments in early-stage R&D and clinical development, the Company is currently assessing the implications of the legislation, particularly the restoration of immediate expensing for qualified R&D. The provisions of the bill are expected to reduce the Company's taxable income in 2025, resulting in a decrease in current tax expense. Conversely, the remeasurement of deferred tax assets and liabilities under ASC 740, *Income Taxes*, to reflect the new law has resulted in an increase in deferred tax expense recorded as a discrete item within income tax expense during the three months ended September 30, 2025. The Company will continue to monitor developments and assess the impact of the legislation on its deferred tax assets, effective tax rate and long-term tax planning strategies.

## 12. Segment Information

The Company operates as one operating segment, which is the business of discovering, developing and commercializing therapies derived from or incorporating genome-editing technology. The determination of a single business segment is consistent with the consolidated financial information regularly provided to the Company's chief operating decision maker, or CODM. The Company's chief executive officer, as the CODM, uses consolidated, single-segment financial information for purposes of evaluating performance, making operating decisions, allocating resources and planning and forecasting for future periods.

The CODM assesses performance and decides how to allocate resources based on consolidated net loss. The measure is used to monitor budget verses actual results to evaluate the performance of the segment.

The measure of segment assets is reported on the condensed consolidated balance sheets as total consolidated assets. All material long-lived assets are located in the United States. Long-lived assets consist of property and equipment, net, and operating lease right-of-use assets.

The following table illustrates information about segment revenue, significant segment expenses and segment operating loss for the periods presented (in thousands):

	Three Months Ended September		Nine Months Ended September	
	2025	2024	2025	2024
Revenue:	\$ 889	\$ 602	\$ 2,646	\$ 1,623
Less <sup>1</sup> :				
Research and development expense <sup>2</sup>	49,065	67,693	169,130	194,173
Acquired in-process research and development <sup>3</sup>	—	—	96,253	—
General and administrative expense <sup>4</sup>	5,749	5,622	19,106	20,200
Collaboration expense, net	57,115	11,153	159,777	110,250
Stock-based compensation expense	16,604	21,543	54,423	64,620
Depreciation expense	4,415	4,721	13,764	14,358
Other segment items <sup>5</sup>	(25,618)	(24,188)	(58,821)	(73,037)
Segment net loss	(106,441)	(85,942)	(450,986)	(328,941)
Reconciliation of profit or loss:				
Adjustments or reconciling items	—	—	—	—
Consolidated net loss	(106,441)	(85,942)	(450,986)	(328,941)

(1) The significant expense categories and amounts align with the segment-level information that is regularly provided to the CODM.

(2) Research and development expense for the three and nine months ended September 30, 2025 excludes \$7.6 million and \$25.1 million of stock-based compensation expense, respectively, and \$2.3 million and \$7.0 million of depreciation expense, respectively. Research and development expense for the three and nine months ended September 30, 2024 excludes \$11.9 million and \$36.5 million of stock-based compensation expense, respectively, and \$2.5 million and \$7.8 million of depreciation expense, respectively.

(3) Acquired in-process research and development expense for the nine months ended September 30, 2025 relates to expense of \$25.0 million related to the upfront cash payment to Sirius in the second quarter of 2025, as well as expense of \$71.3 million related to the issuance of the Company's common shares issued to Sirius as part of the Sirius Agreement in the second quarter of 2025, as described further in Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

(4) General and administrative expense for the three and nine months ended September 30, 2025 excludes \$9.0 million and \$29.3 million of stock-based compensation expense, respectively, and \$2.2 million and \$6.7 million of depreciation expense, respectively. General and administrative expense for the three and nine months ended September 30, 2024 excludes \$9.6 million and \$28.1 million of stock-based compensation expense, respectively, and \$2.2 million and \$6.6 million of depreciation expense, respectively.

(5) Other segment items include interest income, net, the change in fair value of corporate equity securities and income tax expense.

The Company operates in the United States and Switzerland. Collaboration revenue is attributed to the CRISPR Therapeutics AG entity, which is domiciled in Switzerland.

## Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with (i) our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and (ii) our audited consolidated financial statements and related notes and management’s discussion and analysis of financial condition and results of operations included in our Annual Report on Form 10-K for the year ended December 31, 2024 filed with the Securities and Exchange Commission, or the SEC, on February 11, 2025. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and impact and potential impacts on our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including, without limitation, those factors set forth in the “Risk Factors” section of our Annual Report on Form 10-K for the year ended December 31, 2024 and the “Risk Factors” section of subsequent Quarterly Reports on Form 10-Q, our actual results or timing of certain events could differ materially from the results or timing described in, or implied by, these forward-looking statements.

### Overview

Our mission is to create transformative gene-based medicines for serious human diseases. We are a leading gene editing company focused on the development of CRISPR-based therapeutics, including by using CRISPR/Cas9 technology. CRISPR/Cas9 is a revolutionary technology for gene editing, the process of precisely altering specific sequences of genomic DNA. We aim to apply this technology to disrupt, delete, correct and insert genes to treat genetic diseases and to engineer advanced cellular therapies. We have advanced this technology from discovery to an approved medicine with unparalleled speed, culminating in the landmark first approval of a CRISPR-based therapy, CASGEVY (exagamglogene autotemcel [exa-cel]), in 2023 with our collaborators at Vertex Pharmaceuticals Incorporated, or Vertex. We have established a portfolio of therapeutic programs spanning four core franchises: hemoglobinopathies, CAR T, *in vivo* approaches and type 1 diabetes, or T1D. Depending on the program, we take either an *ex vivo* approach, in which we edit cells outside of the human body before administering them to the patient, or an *in vivo* editing approach, where we deliver the CRISPR-based therapeutic directly to target cells within the human body.

We continue to innovate on our platform to develop next-generation technologies that can enable new therapies. Through our efforts, we aim to unlock the full potential of CRISPR-based therapeutics to create medicines that can transform people’s lives. We believe that our innovative research, translational expertise, and clinical development experience, position us as a leader in the development of CRISPR-based therapeutics and may enable us to create an entirely new class of highly effective and potentially curative therapies for patients with both rare and common diseases for whom current biopharmaceutical approaches have had limited success.

#### **Hemoglobinopathies**

##### **CASGEVY**

CASGEVY is a non-viral, *ex vivo* CRISPR/Cas9 gene-edited cell therapy, in which a patient’s own hematopoietic stem and progenitor cells are edited at the erythroid specific enhancer region of the *BCL11A* gene through a precise double-strand break. This edit results in the production of high levels of fetal hemoglobin in red blood cells, which can compensate for the defective adult hemoglobin in patients with sickle cell disease, or SCD, or transfusion-dependent beta thalassemia, or TDT. CASGEVY is the first therapy to emerge from our strategic partnership with Vertex and is being advanced under a joint development and commercialization agreement between us and Vertex and certain of its affiliates.

In 2023, CASGEVY became the first-ever approved CRISPR-based gene-editing therapy in the world. To date, CASGEVY has been approved in the United States, European Union, Great Britain, Kingdom of Saudi Arabia, Kingdom of Bahrain, Qatar, Canada, Switzerland, and the United Arab Emirates for the treatment of eligible patients 12 years and older with SCD or TDT. We and Vertex continue to investigate CASGEVY, including in (1) three clinical trials designed to assess the safety and efficacy of a single dose of CASGEVY in patients 12 to 35 years of age with severe SCD and TDT, respectively, (2) two clinical trials in patients 5 to 11 years of age, one in severe SCD and a second in TDT, and (3) long-term follow-up clinical trials designed to follow participants for up to 15 years after CASGEVY infusion. CASGEVY safety data presented to date is generally consistent with an autologous stem cell transplant and myeloablative conditioning. Efficacy data presented to date support the profile of this therapy as a potential one-time functional cure for people with severe SCD and TDT.

##### **Additional candidates**

We continue to advance our internally developed targeted conditioning program, as well as *in vivo* hematopoietic stem cell editing approaches utilizing lipid nanoparticle-mediated delivery through preclinical studies. Both initiatives could significantly expand the addressable patient populations for SCD and TDT.

#### **CAR T**

We believe CRISPR/Cas9 has the potential to create the next generation of CAR T cell therapies that may have a superior product profile and allow broader patient access compared to current autologous therapies. We are advancing several cell therapy

programs for autoimmune and/or oncology indications. These product candidates incorporate edits designed to enhance CAR T potency, reduce CAR T exhaustion and evade the immune system. In addition, these next-generation candidates exhibit increased manufacturing robustness, with a higher and more consistent number of CAR T cells produced per batch. Our autoimmune disease and immuno-oncology programs are supported by our wholly-owned, GMP manufacturing facility located in Framingham, Massachusetts.

#### *CD19 candidates*

CTX112, our next-generation allogeneic CAR T program targeting Cluster of Differentiation 19, or CD19, is being developed for both hematologic malignancies and autoimmune indications. It 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, as well as an ongoing clinical trial in adult patients with relapsed or refractory B-cell malignancies who have received at least two prior lines of therapy. CTX112 has been granted RMAT designation by the U.S. Food and Drug Administration for the treatment of relapsed or refractory follicular lymphoma and marginal zone lymphoma.

#### *CD70 candidates*

CTX131, our next-generation allogeneic CAR T program targeting Cluster of Differentiation 70, was previously in development for both solid tumors and hematologic malignancies. While the Phase 1 data are encouraging, we have strategically redirected resources away from this program to advance other programs with the greatest potential for long-term value creation.

#### *Additional candidates*

Our CRISPR/Cas9 platform enables us to innovate continuously by incorporating incremental edits into next-generation products. We are advancing several additional investigational CAR T programs.

#### ***In Vivo***

Our *in vivo* gene editing strategy focuses on gene disruption and whole gene correction – the two technologies required to address the vast majority of the most prevalent severe monogenic diseases as well as many common diseases. We have established a leading platform for *in vivo* gene editing and are rapidly advancing a pipeline of *in vivo* gene editing candidates targeting major unmet needs in cardiovascular and metabolic diseases using our proprietary lipid nanoparticle, or LNP, delivery platform. Our first *in vivo* programs target the liver, taking advantage of validated LNP delivery technologies, and aim to treat diseases where we can produce a strong therapeutic effect by safely disrupting a gene with well-understood genetic association.

#### *Cardiovascular and metabolic diseases*

Our first two *in vivo* programs utilizing our proprietary LNP platform, CTX310 and CTX320, aim to address cardiovascular disease by disrupting the validated targets angiotensin-like protein 3, or ANGPTL3, and lipoprotein (a), or Lp(a), respectively.

CTX310 is being investigated in an ongoing Phase 1 first-in-human clinical trial targeting ANGPTL3, a gene that encodes for key protein involved in the regulation of low-density lipoprotein and triglyceride levels, in four patient groups: homozygous familial hypercholesterolemia, severe hypertriglyceridemia, heterozygous familial hypercholesterolemia, or mixed dyslipidemias. Both low-density lipoprotein and triglyceride are validated surrogate endpoints accepted by regulatory agencies.

CTX320 is being investigated in an ongoing Phase 1 clinical trial targeting *LPA*, the gene encoding apo(a), a critical component of Lp(a), in patients with elevated Lp(a), a genetically determined risk factor associated with increased incidence of major adverse cardiovascular events.

#### *Additional candidates*

We continue to advance our earlier stage *in vivo* programs through preclinical studies, including: CTX340, targeting angiotensinogen for the treatment of refractory hypertension, as well as CTX450, targeting 5'-aminolevulinic acid synthase 1 for the treatment of acute hepatic porphyria; and CTX460, targeting *SERPINA1* for the treatment of alpha-1 antitrypsin deficiency. CTX340 and CTX450 are currently progressing through IND/CTA-enabling studies. CTX460 is the first investigational candidate we are advancing using our novel SyNTase editing platform. SyNTase enables precise, *in vivo* gene correction, and represents an important expansion of our toolkit. We are also pursuing additional delivery technologies, including LNPs, for delivery to tissues beyond the liver, including hematopoietic stem cells.

#### ***SRSD107***

In May 2025, we entered into a strategic collaboration with Sirius Therapeutics and certain of its affiliates, or Sirius, pursuant to which, among other things, we and Sirius will jointly develop and commercialize small interfering RNA, or siRNA therapies, beginning with SRSD107. SRSD107 is a next generation, long-acting siRNA designed to selectively inhibit human coagulation factor XI, or FXI, a key driver of pathological thrombosis with minimal impact on normal hemostasis. By targeting FXI, SRSD107 aims to reduce thrombotic events while minimizing the risk of bleeding, representing a differentiated approach compared to Factor Xa inhibitors. In addition, SRSD107 may offer the potential for reversibility not observed with other anti-Factor XI modalities. The addressable population for SRSD107 includes patients with atrial fibrillation, venous thromboembolism, or VTE, cancer-associated thrombosis, chronic Coronary Artery Disease, chronic Peripheral Vascular Disease, end-stage renal disease requiring hemodialysis,

and patients undergoing major orthopedic surgery, where bleeding risk limits existing therapies.

Two Phase 1 clinical trials of SRSD107 have been completed by Sirius, and single doses of SRSD107 have been well tolerated. SRSD107 demonstrated robust pharmacodynamic effects, including reductions of over 93% in FXI levels and FXI activity, along with more than a twofold increase in activated partial thromboplastin time relative to baseline. These effects were sustained, with responses maintained for up to six months post-dosing. SRSD107 is being investigated in an ongoing Phase 2 clinical trial evaluating the safety and efficacy of the candidate in preventing VTE following total knee arthroplasty.

### ***Type 1 Diabetes***

We are developing gene-edited stem cell-derived therapies for the treatment of T1D. We believe our gene editing capabilities have the potential to enable a beta-cell replacement product candidate that may deliver durable benefit to patients without the need for long-term immunosuppression. We have three parallel efforts to achieve this goal: (1) CTX211, an allogeneic, gene-edited, hypimmune, stem cell derived product candidate in a device that is implanted into patients and intended to produce insulin in a glucose-dependent manner, and which is in an ongoing clinical trial; (2) CTX213, a research stage deviceless beta cell replacement product candidate consisting of unencapsulated precursor islet cells derived from edited stem cells; and (3) we have granted a non-exclusive license to certain of our CRISPR/Cas9 intellectual property to Vertex to accelerate Vertex's development of hypimmune cell therapies for T1D in exchange for certain milestones and royalties.

### ***CRISPR-X***

While we have made significant progress with our current portfolio of programs, we recognize that we need to continue to innovate to unlock the full power of gene editing and bring potentially transformative therapies to even more patients. We have a dedicated early-stage research team called CRISPR-X that focuses on innovating next-generation editing modalities. CRISPR-X is developing technologies to enable whole gene correction and insertion via non-viral DNA delivery and all-RNA systems, without requiring homology-directed repair or viral delivery of DNA.

### ***Partnerships***

Given the numerous potential therapeutic applications for CRISPR/Cas9, we have partnered strategically to broaden the indications we can pursue and accelerate development of programs by accessing specific technologies and/or disease-area expertise. We maintain broad partnerships to develop gene editing-based therapeutics in specific disease areas.

#### ***Hemoglobinopathies***

In 2015, we partnered with Vertex and entered into a strategic collaboration, option and license agreement, which focused on the discovery and development of gene-based treatments for hemoglobinopathies and cystic fibrosis using CRISPR/Cas9 gene-editing technology. In 2017, Vertex exercised its option to co-develop and co-commercialize the hemoglobinopathies program and we entered into a joint development and commercialization agreement with Vertex, which we amended and restated in 2021, pursuant to which, among other things, we are co-developing and co-commercializing CASGEVY for TDT and SCD.

#### ***Diabetes***

Beginning in 2018, we partnered with ViaCyte, Inc., or ViaCyte (now a wholly-owned subsidiary of Vertex), to pursue the discovery, development and commercialization of gene-edited allogeneic stem cell therapies for the treatment of diabetes. In 2023, ViaCyte elected to opt-out of the collaboration with us for the co-development and co-commercialization of gene-edited stem cell therapies for the treatment of diabetes. Per the opt-out terms, the ongoing collaboration assets will be wholly owned by us, subject to a royalty on future sales owed to ViaCyte. Our product candidate, CTX211, being developed for the potential treatment of T1D, resulted from this collaboration, and which we are continuing to advance in a Phase 1 clinical trial. Additionally, in 2023, we entered into a non-exclusive license agreement with Vertex for Vertex to utilize certain of our gene-editing intellectual property to exploit certain products for the diagnosis, treatment or prevention of T1D, diabetes type 2 or insulin dependent/requiring diabetes throughout the world. To date, we have recognized revenue of \$205.0 million in upfront and milestone payments and remain eligible to receive additional research and development milestones and royalties on future products under the license.

#### ***Sirius Therapeutics***

In May 2025, we entered into a collaboration, option and license agreement, or the Sirius Agreement, with Sirius, pursuant to which, among other things, we and Sirius will collaborate on the research, development, manufacture, commercialization and use of the Sirius Collaboration Products; and (2) Sirius granted us options to exclusively license Sirius siRNA technology to target up to two licensed targets from a list of seven reserved targets for the research, develop, manufacture and commercialization of Sirius Licensed Products, in exchange for the potential to receive certain option fees, milestone payments and royalties. In connection with entering into the Sirius Agreement, we made an upfront cash payment to Sirius of \$25.0 million and also entered into a share issuance agreement with Sirius, pursuant to which we agreed to issue to Sirius the Sirius Shares.

With respect to Sirius Collaboration Products, we and Sirius will share equally all development and commercialization costs. For the first collaboration product candidate successfully developed, we will be the lead party responsible for commercialization efforts in the United States and Sirius will be the lead party responsible for commercialization efforts in Greater China. The parties

will share equally the net profits and net losses incurred under the Sirius Agreement with respect to all Sirius Collaboration Products, except in the event that a party opts out of the joint development and commercialization. We will also pay Sirius future one-time development and regulatory milestones with respect to Sirius Collaboration Products that achieve the applicable milestone events.

#### *Other Partnerships*

We have entered into a number of additional collaborations and license agreements in other therapeutic areas, including an additional agreement with Vertex for the treatment of Duchenne muscular dystrophy and myotonic dystrophy type 1, and others to support and complement our hematopoietic stem cell, CAR T, *in vivo* and T1D programs and platform.

### **Financial Overview**

Since our inception in October 2013, we have devoted substantially all of our resources to our research and development efforts, identifying potential product candidates, undertaking drug discovery and preclinical development activities, building and protecting our intellectual property estate, establishing internal manufacturing capabilities, organizing and staffing our company, business development activities, business planning, raising capital and providing general and administrative support for these operations. To date, we have primarily financed our operations through private placements of our preferred shares, common share issuances, convertible loans and payments related to certain of our license and collaboration agreements with strategic partners.

We have a history of recurring losses and expect to continue to incur losses for the foreseeable future; however, we have been in a net income position in certain previous years due to certain payments associated with our collaboration and license agreements with Vertex. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase as we continue our current research programs and development activities; seek to identify additional research programs and additional product candidates; conduct initial drug application supporting preclinical studies and initiate clinical trials for our product candidates; pursue business development activities; initiate preclinical testing and clinical trials for any other product candidates we identify and develop; seek regulatory approval for our product candidates; maintain, defend, protect and expand our intellectual property estate; further develop our gene editing platform; hire additional research, clinical and scientific personnel; incur facilities costs associated with such personnel growth; continue to develop internal manufacturing capabilities and infrastructure; and incur additional costs associated with operating as a public company.

#### *Revenue Recognition*

We have not generated any revenue to date from sales of any wholly-owned product and do not expect to do so in the near future. Revenue recognized for the three and nine months ended September 30, 2025 and 2024 was not material. For additional information about our revenue recognition policy, see Note 2, “Summary of Significant Accounting Policies,” in our Annual Report on Form 10-K for the year ended December 31, 2024 filed with the SEC on February 11, 2025, as well as Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

#### *Research and Development Expenses*

Research and development expenses consist primarily of costs incurred for our research activities, including our product discovery efforts and the development of our product candidates, which include:

- employee-related expenses, including salaries, benefits and equity-based compensation expense;
- costs of services performed by third parties that conduct research and development and preclinical and clinical activities on our behalf;
- costs of purchasing lab supplies and non-capital equipment used in our preclinical activities and in manufacturing preclinical study materials, as well as supplies and materials used to manufacture clinical drug material;
- consultant fees;
- facility costs, including rent, depreciation and maintenance expenses; and
- fees and other payments related to acquiring and maintaining licenses under certain of our third-party licensing agreements.

Our external research and development expenses support our various preclinical and clinical programs, and, as such, we do not break down external research and development expenses further. Our internal research and development expenses consist of payroll and benefits expenses, facilities expense, and other indirect research and development expenses incurred in support of overall research and development activities and, as such, are not allocated to a specific development stage or therapeutic area. Research and development costs are expensed as incurred. Nonrefundable advance payments for research and development goods or services to be received in the future are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed. At this time, we cannot reasonably estimate or know the nature, timing or estimated costs of the efforts that will be necessary to complete the development of any product candidates we may identify and develop. This is due to the numerous risks and uncertainties associated with developing such product candidates, including the uncertainty of:

- successful completion of preclinical studies and IND-enabling studies;

- successful enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of the product, if and when approved, whether alone or in collaboration with others;
- acceptance of the product, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

A change in the outcome of any of these variables with respect to the development of any product candidates or the subsequent commercialization of any product candidates we may successfully develop could significantly change the costs, timing and viability associated with the development of that product candidate.

Research and development activities are central to our business model. We expect to continue to incur research and development costs consistent with research and development at companies of our size and stage of development, which may increase in the foreseeable future as our current development programs progress, new programs are added and we continue to prepare regulatory filings. These increases will likely include the costs related to the implementation and expansion of clinical trial sites and related patient enrollment, monitoring, program management and manufacturing expenses for current and future clinical trials.

#### ***Acquired In-Process Research and Development Expenses***

Asset acquisition costs related to acquired technology are expensed as acquired in-process research and development at the point that they have no established alternative future use. We classify asset acquisitions of acquired in-process research and development as investing activities on our condensed consolidated statements of cash flows.

#### ***General and Administrative Expenses***

General and administrative expenses consist primarily of employee-related expenses, including salaries, benefits and equity-based compensation, for personnel in executive, finance, accounting, business development, human resources and other general and administrative functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services.

We expect to continue to incur general and administrative expenses consistent with general and administrative functions at research and development companies of our size and stage of development, which may increase in the future to support continued research and development activities, and potential commercialization of our product candidates. In addition, we anticipate ongoing expenses related to the reimbursements of third-party patent related expenses in connection with certain of our in-licensed intellectual property.

#### ***Collaboration Expense, Net***

Collaboration expense, net, consists of operating expense under our collaboration with Vertex for the hemoglobinopathies program.

#### ***Other Income (Expense), Net***

Other income (expense), net consists primarily of interest income earned on investments, as well as the change in fair value of corporate equity securities.

## Results of Operations

Comparison of three months ended September 30, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		Period to Period Change
	2025	2024	
<b>Revenue:</b>			
Collaboration revenue	\$ —	\$ —	\$ —
Grant revenue	889	602	287
Total revenue	889	602	287
<b>Operating expenses:</b>			
Research and development	58,902	82,160	(23,258)
Acquired in-process research and development	—	—	—
General and administrative	16,931	17,419	(488)
Collaboration expense, net	57,115	11,153	45,962
Total operating expenses	132,948	110,732	22,216
Loss from operations	(132,059)	(110,130)	(21,929)
Other income, net	26,237	25,064	1,173
Loss before income taxes	(105,822)	(85,066)	(20,756)
Provision for income taxes	(619)	(876)	257
Net loss	<u>\$ (106,441)</u>	<u>\$ (85,942)</u>	<u>\$ (20,499)</u>

### Collaboration Revenue

There was no collaboration revenue for the three months ended September 30, 2025 and 2024. Please refer to Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for further information.

### Research and Development Expenses

Research and development expenses were \$58.9 million for the three months ended September 30, 2025, compared to \$82.2 million for the three months ended September 30, 2024. The following table summarizes our research and development expenses for the three months ended September 30, 2025 and 2024, together with the changes in those items in dollars (in thousands):

	Three Months Ended September 30,		Period to Period Change
	2025	2024	
External research and development expenses	\$ 14,908	\$ 25,375	\$ (10,467)
Employee-related expenses	13,871	18,443	(4,572)
Facility expenses	21,629	25,651	(4,022)
Stock-based compensation expenses	7,577	11,949	(4,372)
Other expenses	68	607	(539)
Sublicense and license fees	849	135	714
Total research and development expenses	<u>\$ 58,902</u>	<u>\$ 82,160</u>	<u>\$ (23,258)</u>

The decrease of approximately \$23.3 million was primarily attributable to the following:

- \$10.5 million of decreased external research and development costs, primarily associated with a decrease in variable external research and manufacturing costs;
- \$8.9 million of decreased employee-related expenses, including stock-based compensation expenses, primarily driven by decreased headcount; and
- \$4.0 million of decreased facility expenses primarily driven by lower laboratory-related costs.

### General and Administrative Expenses

General and administrative expenses were \$16.9 million for the three months ended September 30, 2025, compared to general and administrative expenses of \$17.4 million for the three months ended September 30, 2024. The decrease of approximately \$0.5 million was primarily associated with a decrease in employee-related costs.

### Collaboration Expense, Net

Collaboration expense, net, was \$57.1 million for the three months ended September 30, 2025, compared to \$11.2 million for the three months ended September 30, 2024. In the third quarter of 2024, we exercised our option to defer specified costs under the CASGEVY program in excess of the \$110.3 million deferral limit under the A&R Vertex JDCA, as amended. The increase of

approximately \$46.0 million 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. Absent the deferral limit, the increase was primarily attributable to an increase in our share of CASGEVY operating expenses related to increased commercial and manufacturing costs for CASGEVY when compared to the prior period, which was offset by an increase in our share of CASGEVY revenue.

### **Other Income, Net**

Other income was \$26.2 million for the three months ended September 30, 2025, compared to \$25.1 million of income for the three months ended September 30, 2024. The increase of approximately \$1.2 million was primarily due to the change in fair value of corporate equity securities during the three months ended September 30, 2025, offset by a decrease in interest income earned on cash, cash equivalents and marketable securities for the three months ended September 30, 2025.

### **Comparison of nine months ended September 30, 2025 and 2024 (in thousands):**

	<b>Nine Months Ended September 30,</b>		<b>Period to Period Change</b>
	<b>2025</b>	<b>2024</b>	
<b>Revenue:</b>			
Collaboration revenue	\$ —	\$ —	\$ —
Grant revenue	2,646	1,623	1,023
Total revenue	2,646	1,623	1,023
<b>Operating expenses:</b>			
Research and development	201,280	238,498	(37,218)
Acquired in-process research and development	96,253	—	96,253
General and administrative	55,143	54,853	290
Collaboration expense, net	159,777	110,250	49,527
Total operating expenses	512,453	403,601	108,852
Loss from operations	(509,807)	(401,978)	(107,829)
Other income, net	61,841	75,924	(14,083)
Loss before income taxes	(447,966)	(326,054)	(121,912)
Provision for income taxes	(3,020)	(2,887)	(133)
Net loss	\$ (450,986)	\$ (328,941)	\$ (122,045)

### **Collaboration Revenue**

There was no collaboration revenue for the nine months ended September 30, 2025 and 2024. Please refer to Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for further information.

### **Research and Development Expenses**

Research and development expenses were \$201.3 million for the nine months ended September 30, 2025, compared to \$238.5 million for the nine months ended September 30, 2024. The following table summarizes our research and development expenses for the nine months ended September 30, 2025 and 2024, together with the changes in those items in dollars (in thousands):

	<b>Nine Months Ended September 30,</b>		<b>Period to Period Change</b>
	<b>2025</b>	<b>2024</b>	
External research and development expenses	\$ 53,593	\$ 63,083	\$ (9,490)
Employee related expenses	50,571	58,015	(7,444)
Facility expenses	68,853	73,486	(4,633)
Stock-based compensation expenses	25,115	36,542	(11,427)
Other expenses	772	1,467	(695)
Sublicense and license fees	2,376	5,905	(3,529)
Total research and development expenses	\$ 201,280	\$ 238,498	\$ (37,218)

The decrease of approximately \$37.2 million was primarily attributable to the following:

- \$18.9 million of decreased employee-related expenses, including stock-based compensation expenses, primarily driven by decreased headcount;
- \$9.5 million of decreased external research and development costs, primarily associated with a decrease in variable external research and manufacturing costs;
- \$4.6 million of decreased facility-related expenses, primarily driven by lower lab consumable costs; and
- \$3.5 million of decreased sublicense and license fees, primarily attributable to a decrease in research and development

licenses and milestones payable under certain strategic partnerships and other research and development agreements.

#### ***Acquired In-Process Research and Development Expenses***

Acquired in-process research and development expenses were \$96.3 million for the nine months ended September 30, 2025. There was no acquired in-process research and development expenses for the nine months ended September 30, 2024. The \$96.3 million acquired in-process research and development expense is attributable to the costs incurred upon entering the Sirius Agreement during the second quarter of 2025, as described in Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

#### ***General and Administrative Expenses***

General and administrative expenses were \$55.1 million for the nine months ended September 30, 2025, compared to general and administrative expenses of \$54.9 million for the nine months ended September 30, 2024. The increase of approximately \$0.3 million was primarily associated with an increase in stock-based compensation expense, offset by a decrease in intellectual property costs.

#### ***Collaboration Expense, Net***

Collaboration expense, net, was \$159.8 million for the nine months ended September 30, 2025, compared to \$110.3 million for the nine months ended September 30, 2024. In the third quarter of 2024, we exercised our option to defer specified costs under the CASGEVY program in excess of the \$110.3 million deferral limit under the A&R Vertex JDCA, as amended. The increase of approximately \$49.5 million 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. Absent the deferral limit, the increase was primarily attributable to an increase in our share of CASGEVY operating expenses related to increased commercial and manufacturing costs for CASGEVY when compared to the prior period, which was offset by an increase in our share of CASGEVY revenue.

#### ***Other Income, Net***

Other income was \$61.8 million for the nine months ended September 30, 2025, compared to \$75.9 million of income for the nine months ended September 30, 2024. The decrease of approximately \$14.1 million was primarily due to a decrease in interest income earned on cash, cash equivalents and marketable securities for the nine months ended September 30, 2025.

### **Liquidity and Capital Resources**

We have predominantly incurred losses and cumulative negative cash flows from operations since our inception. As of September 30, 2025, we had \$1,944.1 million in cash, cash equivalents and marketable securities, of which approximately \$184.7 million was held outside of the United States, and an accumulated deficit of \$1,816.9 million. We anticipate that we will continue to incur losses for at least the next several years. We expect to continue to incur research and development costs and general and administrative expenses consistent with costs associated with research and development at companies of our size and stage of development, and, as a result, we will need additional capital to fund our operations, which we may raise through public or private equity or debt financings, strategic collaborations, or other sources.

#### ***At-the-Market Offerings***

We have entered into an Open Market Sale Agreement<sup>SM</sup>, or the Sales Agreement, with Jefferies LLC under which we, at our sole discretion, are able to offer and sell, from time to time at prevailing market prices, our common shares. The following are in connection with the Sales Agreement.

##### ***2021 ATM***

In January 2021, we filed a prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to \$600.0 million, or, together with the subsequent prospectus supplements filed in July 2021 and August 2024 relating to our common shares remaining under our original prospectus supplement, the 2021 ATM. For the nine months ended September 30, 2025, we issued and sold an aggregate of 5.1 million common shares under the 2021 ATM at an average price of \$57.39 per share for aggregate proceeds of \$286.8 million with equity issuance costs of \$3.7 million, excluding stamp taxes of \$2.9 million. As of September 30, 2025, we have issued and sold an aggregate of 7.0 million common shares under the 2021 ATM at an average price of \$75.40 per share for aggregate proceeds of \$520.0 million, which were net of equity issuance costs of \$6.8 million, excluding stamp taxes of \$5.2 million. As of September 30, 2025, common shares having aggregate gross proceeds up to \$73.2 million remain available under the 2021 ATM.

From September 30, 2025 through the date of issuance of this Quarterly Report on Form 10-Q, we have issued and sold an aggregate of 1.0 million common shares under the 2021 ATM at an average price of \$70.53 per share for aggregate proceeds of \$72.2 million, which were net of equity issuance costs of \$1.0 million, excluding stamp taxes of \$0.7 million.

### *2025 ATM*

In October 2025, we filed a new prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to \$600.0 million, or the 2025 ATM. Through the date of issuance of this Quarterly Report on Form 10-Q, we have issued and sold an aggregate of 0.2 million common shares under the 2025 ATM at an average price of \$73.53 per share for aggregate proceeds of \$11.7 million, which were net of equity issuance costs of \$0.1 million, excluding stamp taxes of \$0.1 million. Common shares having aggregate gross proceeds up to \$588.3 million remain available under the 2025 ATM.

### *Share Issuance Agreement with Sirius Therapeutics*

As described in Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q, we and Sirius entered into a share issuance agreement and we registered and issued 1,842,105 common shares to Sirius, nominal value CHF 0.03 per share, at an issue price of \$38.00 per share as partial consideration for entering into the Sirius Agreement.

### *Additional Financings*

In February 2024, we entered into an investment agreement for the sale of approximately \$280.0 million of our common shares to a group of institutional investors in a registered direct offering, at a price per share of \$71.50. We received net proceeds of \$279.0 million, excluding stamp taxes due of \$2.8 million.

### *Funding Requirements*

Our primary uses of capital are, and we expect will continue to be, research and development activities, manufacturing activities, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses, patent prosecution, filing, defense and intellectual property maintenance costs, business development activities, and general overhead costs, including costs associated with operating as a public company. We expect to continue to incur operating expenses consistent with costs associated with research and development companies of our size and stage of development, which may increase in the future to support continued research and development activities and potential commercialization of our product candidates.

Although we and our partner, Vertex, have received marketing approvals for CASGEVY in certain jurisdictions, we cannot guarantee we and Vertex will receive additional marketing approvals for CASGEVY or we will receive marketing approvals for our other product candidates in the future. Most of our programs are still in early stages of research and development and the outcome of our efforts is uncertain, and we cannot estimate the actual amounts necessary to successfully complete the development, manufacture and commercialization of any current or future product candidates, if approved, or whether, or when, we may achieve profitability. Until such time as we can generate substantial product revenues, if ever, we expect to finance our cash needs through a combination of equity financings, debt financings and payments received in connection with our collaboration and license agreements. We intend to consider opportunities to raise additional funds through the sale of equity or debt securities when market conditions are favorable to us to do so. However, the trading prices for our common shares and other biopharmaceutical companies have been highly volatile. As a result, we may face difficulties raising capital through sales of our common shares or such sales may be on unfavorable terms. In addition, a recession, depression or other sustained adverse market event, including resulting from the uncertain geopolitical environment, global trade policy and global economic conditions, could materially and adversely affect our business and the value of our common shares. To the extent that we raise additional capital through the future sale of equity or debt securities, the ownership interests of our shareholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing shareholders. If we raise additional funds through license or collaboration arrangements in the future, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

### *Outlook*

Based on our research and development plans and our timing expectations related to the progress of our programs, we expect our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditures for at least the next 24 months without giving effect to any additional proceeds we may receive under our license agreements and collaborations, including with Vertex, and any other capital raising transactions we may complete. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we expect. Given our need for additional financing to support the long-term clinical development and future commercialization of our programs, as applicable, we intend to consider additional financing opportunities when market terms are favorable to us.

Our ability to generate revenue and achieve profitability depends significantly on our success in many areas, including: developing our delivery technologies and our gene editing technology platform; selecting appropriate product candidates to develop; completing research and preclinical and clinical development of selected product candidates; obtaining regulatory approvals and marketing authorizations for product candidates for which we complete clinical trials; developing a sustainable and scalable manufacturing process for product candidates; launching and commercializing product candidates for which we obtain regulatory approvals and marketing authorizations, either directly or with a collaborator or distributor; obtaining market acceptance of our

product candidates, either directly or with a collaborator or distributor, if approved, including for CASGEVY; addressing any competing technological and market developments; negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter; maintaining good relationships with our collaborators and licensors; maintaining, defending, protecting and expanding our estate of intellectual property rights, including patents, trade secrets and know-how; and attracting, hiring and retaining qualified personnel.

## Cash Flows

The following table provides information regarding our cash flows for each of the periods below (in thousands):

	Nine Months Ended September 30,		Period to Period Change
	2025	2024	
Net cash used in operating activities	\$ (252,461)	\$ (92,743)	\$ (159,718)
Net cash used in investing activities	(69,004)	(386,309)	317,305
Net cash provided by financing activities	309,612	315,108	(5,496)
Effect of exchange rate changes on cash	94	66	28
Net decrease in cash	<u>\$ (11,759)</u>	<u>\$ (163,878)</u>	<u>\$ 152,119</u>

### Operating Activities

Net cash used in operating activities was \$252.5 million for the nine months ended September 30, 2025, compared to \$92.7 million for the nine months ended September 30, 2024. The \$159.7 million increase in net cash used in operating activities was primarily driven by the timing of receipt of milestone payments from Vertex which were recorded in accounts receivable as of the respective year-end periods and paid in the subsequent year (\$200.0 million paid in the first quarter of 2024 compared to \$25.0 million paid in the first quarter of 2025).

### Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2025 was \$69.0 million, compared to net cash used in investing activities of \$386.3 million for the nine months ended September 30, 2024. The decrease in net cash used in investing activities was primarily driven by a net decrease in purchases of our marketable securities.

### Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2025 was \$309.6 million, compared with \$315.1 million for the nine months ended September 30, 2024. Net cash provided by financing activities for the nine months ended September 30, 2025 consisted primarily of \$286.6 million in net proceeds from the sale of common shares issued in connection with our 2021 ATM, as well as option exercise proceeds of \$23.0 million, net of issuance costs. Net cash provided by financing activities for the nine months ended September 30, 2024 consisted of net proceeds from the sale of approximately \$280.0 million of our common shares to a group of institutional investors in a registered direct offering, at a price per share of \$71.50, as well as option exercise proceeds of \$29.4 million, net of issuance costs.

## Critical Accounting Policies and Estimates

There have been no material changes in our critical accounting policies and estimates in the preparation of our condensed consolidated financial statements during the three and nine months ended September 30, 2025 compared to those discussed in our Annual Report on Form 10-K for the year ended December 31, 2024 filed with the SEC on February 11, 2025 and discussed in Note 1 in the condensed consolidated financial statements in the Company's Quarterly Report on Form 10-Q filed with the SEC on August 4, 2025 with respect to acquired in-process research and development expenses and changes to the Company's policy around collaboration arrangements.

### Acquired In-process Research and Development Expenses

In-process research and development that is acquired in a transaction that does not qualify as a business combination under U.S. GAAP and that does not have an alternative future use is recorded as "Acquired in-process research and development" in our condensed consolidated statements of operations and comprehensive loss.

### Collaboration Arrangements

We record the elements of our collaboration agreements that represent joint operating activities in accordance with ASC 808. Accordingly, the elements of the collaboration agreements that represent activities in which both parties are active participants and to which both parties are exposed to the significant risks and rewards that are dependent on the commercial success of the activities, are recorded as collaborative arrangements.

We evaluate the proper presentation of the commercial activities and the profit and loss sharing associated with the collaboration agreements. ASC 808 states that when payments between parties in a collaborative arrangement are not within the scope of other

authoritative accounting literature, the income statement classification should be based on the nature of the arrangement, the nature of its business operations and the contractual terms of the arrangement. To the extent that these payments are not within the scope of other authoritative accounting literature, the income statement classification for the payments shall be based on an analogy to authoritative accounting literature or if there is no appropriate analogy, a reasonable, rational and consistently applied accounting policy election.

Collaboration costs specific to the Vertex Hemoglobinopathy Agreements (as defined in Note 6) accounted for under ASC 808 are presented within “collaboration expense, net” in the condensed consolidated statements of operations and comprehensive loss. Refer to Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for further discussion on the Vertex Hemoglobinopathy Agreements.

### **Recent Accounting Pronouncements**

Refer to Note 1 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for a discussion of recent accounting pronouncements.

### **Item 3. Qualitative and Quantitative Disclosures about Market Risk**

#### *Interest Rate Sensitivity*

We are exposed to market risk related to changes in interest rates. As of September 30, 2025, we had cash, cash equivalents and marketable securities of \$1,944.1 million, primarily invested in U.S. treasury securities and government agency securities, corporate bonds, commercial paper and money market accounts invested in U.S. government agency securities. Due to the conservative nature of these instruments, we do not believe that we have a material exposure to interest rate risk. If interest rates were to increase or decrease by 1%, the fair value of our investment portfolio would increase or decrease by an immaterial amount.

#### *Foreign Currency Exchange Rate Risk*

As a result of our foreign operations, we face exposure to movements in foreign currency exchange rates, primarily the Swiss Franc and British Pound, against the U.S. dollar. The current exposures arise primarily from cash, accounts payable and intercompany receivables and payables. Changes in foreign exchange rates affect our consolidated statement of operations and distort comparisons between periods. To date, foreign currency transaction gains and losses have not been material to our financial statements, and we have not engaged in any foreign currency hedging transactions.

#### *Inflation*

Inflation generally affects us by increasing our cost of labor, clinical trial and manufacturing costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the three and nine months ended September 30, 2025 and 2024.

### **Item 4. Controls and Procedures.**

#### *Management’s Evaluation of our Disclosure Controls and Procedures*

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities and Exchange Act of 1934 is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC’s rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of September 30, 2025, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities and Exchange Act of 1934). Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2025, our disclosure controls and procedures were effective at the reasonable assurance level.

#### *Changes in Internal Control over Financial Reporting*

During the quarter ended September 30, 2025, there were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II—OTHER INFORMATION

### Item 1. Legal Proceedings.

In the ordinary course of business, we are from time to time involved in lawsuits, investigations, proceedings and threats of litigation related to, among other things, our intellectual property estate (including certain in-licensed intellectual property), commercial arrangements and other matters. Such proceedings may include quasi-litigation, *inter partes* administrative proceedings in the U.S. Patent and Trademark Office and the European Patent Office involving our intellectual property estate including certain in-licensed intellectual property. There are currently no claims or actions pending against us that, in the opinion of our management, are likely to have a material adverse effect on our business.

There have been no material developments with respect to the legal proceedings previously disclosed in “Item 3. Legal Proceedings” of our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 filed with the SEC on February 11, 2025.

### Item 1A. Risk Factors.

We are updating and supplementing our risk factor previously disclosed in Item 1A of our Quarterly Report on Form 10-Q for the quarter ended June 30, 2025 filed with the SEC on August 4, 2025 to revise the following risk factor:

*Disruptions At The FDA, The SEC and Other Government Agencies Could Hinder Their Ability To Hire And Retain Key Leadership And Other Personnel, Prevent New Products And Services From Being Developed Or Commercialized In A Timely Manner, Or Otherwise Prevent Those Agencies From Performing Normal Business Functions, Which Could Negatively Impact Our Business And Our Timelines.*

Recently, beginning on October 1, 2025, the U.S. government shut down and remains shut down as of the date of this filing, during which time certain regulatory agencies, such as the U.S. Food and Drug Administration, or FDA, and the SEC, have furloughed critical employees and stopped critical activities. Additionally, on October 10, 2025, the U.S. government implemented substantial layoffs and workforce reductions in connection with the ongoing federal government shutdown, which has resulted in the suspension or delay of various government-funded programs. While we continue to monitor developments, there is no assurance that affected government employees or contractors will be reinstated and that government-funded programs will resume. Without appropriation of additional funding to federal agencies, our business operations related to our product development for the U.S. market could be impacted. Inadequate funding for the FDA, the SEC and other government agencies, including from government shut downs, or other disruptions to these agencies’ operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business. Additionally, any reduction in personnel may result in longer review times by the FDA, the SEC and other agencies.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, shifting policy priorities as a result of changes in the Presidential administration and political appointees tasked to oversee the agency, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. Moreover, disruptions and personnel turnover, as a result of leadership changes, staff reductions or otherwise, at the FDA and other agencies may slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which could adversely affect our business. For example, changes and cuts in FDA staffing also could result in delays in the FDA’s responsiveness or in its ability to review IND submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all.

Over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, or if staffing changes prevent the FDA, the SEC or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, including formal and informal interactions with product developers, it could significantly impact the ability of the FDA, the SEC or other regulatory authority to timely review and process our regulatory submissions, which could have a material adverse effect on our business and our timelines. In addition, government funding of the SEC, and other government agencies on which our operations may rely is subject to the impacts of political events, which are inherently fluid and unpredictable.

There have been no material changes to the risk factors previously disclosed in Part I, Item 1A (Risk Factors) of our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 filed with the SEC on February 11, 2025 and Part II, Item 1A (Risk Factors) of our Quarterly Report on Form 10-Q for the quarter ended March 31, 2025 filed with the SEC on May 6, 2025 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2025 filed with the SEC on August 4, 2025. In addition to the risks described in our Annual Report on Form 10-K and any quarterly report on Form 10-Q, you should carefully consider the other information set forth in this Form 10-Q and the information in our other filings with the SEC, as they could materially affect our business, financial condition or future results of operations.

**Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.**

None.

**Item 3. Defaults Upon Senior Securities.**

Not applicable.

**Item 4. Mine Safety Disclosures.**

Not applicable.

**Item 5. Other Information.**

(a) On November 8, 2025, we announced positive Phase 1 data from our ongoing clinical trial evaluating CTX310, an investigational, *in vivo* CRISPR/Cas9 gene-editing therapy targeting ANGPTL3. A single-course treatment with CTX310 produced dose-dependent, durable reductions in circulating ANGPTL3 with a mean reduction from baseline of -73% (maximum -89%), a mean reduction in triglycerides, or TG, of -55% (maximum -84%) and a mean reduction of low-density lipoprotein, or LDL, of -49% (maximum -87%) at the highest dose. These data were presented during a late-breaking session at the American Heart Association Scientific Sessions and published simultaneously in *The New England Journal of Medicine* in a peer-reviewed article entitled “Phase 1 Trial of CRISPR-Cas9 Gene Editing Targeting ANGPTL3.”

The Phase 1, open label, dose-escalation trial evaluated single-course intravenous doses of CTX310 ranging from 0.1 to 0.8 mg/kg (lean body weight) targeting *ANGPTL3* in four patient groups: homozygous familial hypercholesterolemia, severe hypertriglyceridemia, or sHTG, heterozygous familial hypercholesterolemia, or mixed dyslipidemias (elevated TG and LDL). Eligible participants had uncontrolled TG levels >150 mg/dL and/or LDL cholesterol >100 mg/dL (or >70 mg/dL for those with established atherosclerotic cardiovascular disease) despite background standard of care per local guidelines. The majority of participants were receiving statins and/or ezetimibe, while 40% were taking PCSK9 inhibitors. The trial was designed to evaluate safety and tolerability as primary endpoints, with changes in circulating ANGPTL3 protein, TG, and LDL as secondary endpoints.

Single-course ascending doses of CTX310 were administered to 15 participants across sequential cohorts, and all participants completed at least 28 days of follow-up as of the data cutoff. CTX310 was generally well tolerated, and no dose-limiting toxicities or serious adverse events related to treatment.

Adverse events were generally mild to moderate. One participant experienced an allergic reaction that resolved the following day with supportive care. Infusion-related reactions occurred in three participants (two at 0.6 mg/kg and one at 0.8 mg/kg dose), all Grade 2. All events resolved, and all participants completed their infusions. One participant with elevated transaminases level at baseline had a Grade 2 elevation of transaminases that peaked by Day 4 and resolved completely by Day 14 without any rise in bilirubin.

Overall, CTX310 demonstrated a well-tolerated safety and tolerability profile that supports continued advancement of the program.

These new results build upon previously disclosed top-line data from 12 participants across the first four sequential cohorts, corresponding to lean body weight-based doses of dose level, or DL, 1 [0.1 mg/kg], DL2 [0.3 mg/kg], DL3 [0.6 mg/kg] and DL4 [0.8 mg/kg]. All participants had at least 30 days of follow-up.

- Dose-dependent reductions in circulating ANGPTL3 protein: Mean (range) among participants treated with 0.1, 0.3, 0.6, 0.7, and 0.8 mg/kg doses were 10% (-22 to 71), 9% (-25 to 64), -33% (-51 to -19), -80% (-87 to -73), and -73% (-89 to -67), respectively, at Day 30 following CTX310 infusion.
- Among participants treated at 0.8 mg/kg, TG reductions of up to 84% were observed, with a mean reduction of 55% at Day 60 following CTX310 infusion.
- In participants with elevated TG (>150 mg/dL) at baseline, mean reductions of 60% were observed at the therapeutic dose levels at Day 60 following CTX310 infusion.
- Among participants treated at 0.8 mg/kg, LDL reductions of up to 87% were observed, with a mean reduction of 49% at Day 60 following CTX310 infusion.
- Two participants on background PCSK9 inhibitors achieved >80% reduction in LDL from baseline.

We are advancing CTX310 into Phase 1b clinical trials, prioritizing development in sHTG and mixed dyslipidemia.

(b) Not applicable.

(c) From time to time, our officers (as defined in Rule 16a-1(f)) and directors may enter into Rule 10b5-1 or non-Rule 10b5-1 trading arrangements (as each such term is defined in Item 408 of Regulation S-K). During the three months ended September 30, 2025, our officers and directors took the following actions with respect to 10b5-1 trading arrangements:

Name (Title)	Action Taken (Date of Action)	Type of Trading Arrangement	Nature of Trading Arrangement	Duration of Trading Arrangement	Aggregate Number of Securities
James R. Kasinger (General Counsel)	Adoption August 6, 2025	Trading plan intended to satisfy the affirmative defense conditions of Securities Exchange Act Rule 10b5-1(c).	Sale of the Company's common stock pursuant to the terms of the plan.	Active through June 30, 2026	93,632
Raju Prasad, Ph.D. (Chief Financial Officer)	Adoption August 18, 2025	Trading plan intended to satisfy the affirmative defense conditions of Securities Exchange Act Rule 10b5-1(c).	Sale of the Company's common stock pursuant to the terms of the plan.	Active through June 22, 2026	74,672
Samarth Kulkarni, Ph.D. (Chief Executive Officer)	Adoption August 27, 2025	Trading plan intended to satisfy the affirmative defense conditions of Securities Exchange Act Rule 10b5-1(c).	Sale of the Company's common stock pursuant to the terms of the plan.	Active through June 30, 2026	90,000

In addition, on August 27, 2025, Samarth Kulkarni, Ph.D., our Chief Executive Officer, terminated a Rule 10b5-1 trading arrangement he had previously adopted with respect to the sale of up to an aggregate of 75,000 common shares of the Company pursuant to the terms of such trading plan. Dr. Kulkarni's Rule 10b5-1 trading arrangement was adopted on June 10, 2025 and was active through March 31, 2026. As of the date of termination of such rule 10b5-1 trading arrangement, Dr. Kulkarni had not sold any common shares under the terms of such trading plan.

**Item 6. Exhibits**

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth in the Exhibit Index below.

Exhibit Number	Description of Document
31.1*	<a href="#">Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</a>
31.2*	<a href="#">Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</a>
32.1*+	<a href="#">Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</a>
101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the inline XBRL document.
101.SCH*	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104*	Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101)

\* Filed herewith.

+ The certification attached as Exhibit 32.1 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of CRISPR Therapeutics AG under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

**CRISPR Therapeutics AG**

Dated: November 10, 2025

By: /s/ Samarth Kulkarni  
Samarth Kulkarni, Ph.D.  
Chief Executive Officer  
(Principal Executive Officer)

Dated: November 10, 2025

By: /s/ Raju Prasad  
Raju Prasad, Ph.D.  
Chief Financial Officer  
(Principal Financial Officer)

## CERTIFICATIONS

I, Samarth Kulkarni, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CRISPR Therapeutics AG;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 10, 2025

By: /s/ Samarth Kulkarni  
Samarth Kulkarni, Ph.D.  
Chief Executive Officer  
(Principal Executive Officer)

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## CERTIFICATIONS

I, Raju Prasad, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CRISPR Therapeutics AG;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 10, 2025

By: /s/ Raju Prasad  
Raju Prasad, Ph.D.  
Chief Financial Officer  
(Principal Financial and Accounting Officer)

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CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of CRISPR Therapeutics AG (the “Company”) for the period ended September 30, 2025 as filed with the Securities and Exchange Commission on or about the date hereof (the “Report”), the undersigned officers of the Company hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to his or her knowledge:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company as of, and for, the periods presented in the Report.

/s/ Samarth Kulkarni  
Samarth Kulkarni, Ph.D.  
Chief Executive Officer  
(Principal Executive Officer)

November 10, 2025

/s/ Raju Prasad  
Raju Prasad, Ph.D.  
Chief Financial Officer  
(Principal Financial and Accounting Officer)

November 10, 2025

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