UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2026

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: 001-43482

ADARx Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**84-4145188**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
5871 Oberlin Drive, Suite 200 San Diego, California	**92121**
(Address of principal executive offices)	**(Zip Code)**

(877) 232-7974
(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
Common Stock, $0.0001 par value per share	ADRX	The Nasdaq Global Select Market

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, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☒		

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 the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 6, 2026, the registrant had 111,193,446 shares of common stock, $0.0001 par value per share, outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), which statements are subject to substantial risks and uncertainties and are based on estimates and assumptions. All statements other than statements of historical facts, including statements concerning our plans, objectives, goals, strategies, future events, future revenues or performance, financing needs, plans or intentions relating to therapeutic candidates and markets, and business trends and other information contained in this Quarterly Report on Form 10-Q are forward-looking statements, including statements related to:

- the success, cost, timing and potential indications of our product development activities and clinical trials, including our Phase 3 STOP-HAE clinical trial of onvuzosiran, our Phase 2 clinical trials of agazisiran, and our Phase 1 and Phase 2 clinical trials of ADX-626;
- the timing of our planned investigational new drug application ("IND") submissions to the U.S. Food and Drug Administration (the "FDA") or similar regulatory submissions to comparable foreign regulatory authorities for our therapeutic candidates, including ADX-077 and ADX-199;
- the timing of the initiation, enrollment and completion of, and the availability of data from, our planned and ongoing clinical trials;
- our ability to obtain and maintain regulatory approval of our therapeutic candidates in any of the indications for which we plan to develop them, and any related restrictions, limitations and/or warnings in the label of an approved therapeutic candidate;
- our ability to obtain funding for our operations, including funding necessary to complete the clinical trials of any of our therapeutic candidates, and our expectations regarding the period over which our existing cash, cash equivalents and short-term investments will fund our operations;
- our plans to research and develop our therapeutic candidates;
- our ability to advance our clinical programs in parallel, advance our preclinical therapeutic candidates into clinical development and submit additional INDs;
- the size of the markets for our therapeutic candidates, and our ability to serve those markets;
- our ability to successfully commercialize our therapeutic candidates;
- the rate and degree of market acceptance of our therapeutic candidates;
- our ability to develop and maintain sales and marketing capabilities, whether alone or with potential future collaborators;
- the performance of our third-party suppliers and manufacturers;
- our ability to leverage programs within our initial target indications and to progress additional programs to further develop our pipeline;
- the potential of our preclinical studies and clinical trials to demonstrate safety and efficacy of our therapeutic candidates and other positive results;
- our collaboration with AbbVie, including the timing and amount of any option exercise, milestone, royalty or other payments we may receive and our ability to realize the potential benefits of the collaboration;
- our ability to identify and enter into additional license agreements and collaborations and realize the potential benefits of such agreements and collaborations;
- our reliance on third-party manufacturers to successfully manufacture therapeutic candidates for preclinical use, clinical trials and on a larger scale for commercial use, if approved;
- the success of competing therapies that are or become available;
- existing regulations and regulatory developments in the United States and other jurisdictions;
- the implementation of our business model and strategic plans for our business and operations;
- our ability to attract and retain key scientific or management personnel;
- our anticipated use of the net proceeds from our initial public offering ("IPO") and concurrent private placement, estimates of our expenses, capital requirements, and needs for additional financing;

- our expectations regarding the impact of macroeconomic conditions or geopolitical events, such as global health pandemics, geopolitical conflicts, economic uncertainty, interest rates, tariffs and inflation on our business and operations;
- our expectations regarding the period during which we will qualify as an emerging growth company under the Jumpstart Our Business Startups Act (the "JOBS Act") and a smaller reporting company; and
- our expectations regarding our ability to obtain and maintain intellectual property protection for our technology and therapeutic candidates, and our ability to operate our business without infringing on the intellectual property rights of others.

In some cases, you can identify forward-looking statements by terms such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target" or "will" or the negative of these terms or other similar expressions intended to identify statements about the future. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report on Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

You should read the section titled "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. As a result of these factors, we cannot assure you that the forward-looking statements in this Quarterly Report on Form 10-Q will prove to be accurate. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

You should read this Quarterly Report on Form 10-Q and the documents that we reference in this Quarterly Report on Form 10-Q and have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements in this Quarterly Report on Form 10-Q by these cautionary statements.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

ADARx Pharmaceuticals, Inc.
Condensed consolidated balance sheets
(in thousands, except share and per share data)
(unaudited)

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 570,610	$ 66,517
Short-term investments	330,397	413,479
Prepaid expenses and other current assets	17,507	13,379
Total current assets	918,514	493,375
Property and equipment, net	4,397	4,734
Operating lease right-of-use assets	6,053	7,336
Other non-current assets	100	2,210
Total assets	$ 929,064	$ 507,655
Liabilities, convertible preferred stock, and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 9,346	$ 5,844
Accrued expenses and other current liabilities	11,594	6,371
Deferred revenue, current portion	20,924	16,557
Lease liabilities, current portion	1,710	1,713
Total current liabilities	43,574	30,485
Lease liabilities, less current portion	4,749	6,028
Deferred revenue, less current portion	305,524	314,994
Other long-term liabilities	815	781
Total liabilities	354,662	352,288
Commitments and contingencies (Note 8)		
Convertible preferred stock:		
Series A convertible preferred stock, $0.0001 par value; zero and 31,315,236 shares authorized as of September 30, 2026 and December 31, 2025; zero and 26,726,324 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	—	37,553
Series B convertible preferred stock, $0.0001 par value; zero and 14,653,302 shares authorized as of September 30, 2026 and December 31, 2025; zero and 12,506,015 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	—	74,787
Series B-1 convertible preferred stock, $0.0001 par value; zero and 6,913,353 shares authorized as of September 30, 2026 and December 31, 2025; zero and 5,900,272 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	—	45,710
Series C convertible preferred stock, $0.0001 par value; zero and 24,038,463 shares authorized as of September 30, 2026 and December 31, 2025; zero and 20,515,872 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	—	199,572
Stockholders' equity (deficit):		
Preferred stock, $0.0001 par value; 10,000,000 and zero shares authorized, zero shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	—	—
Common stock, $0.0001 par value; 999,999,999 and 102,000,000 shares authorized as of September 30, 2026 and December 31, 2025; 107,255,963 and 9,659,572 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	11	—
Additional paid-in capital	873,785	16,489
Accumulated other comprehensive (loss) income	(2,152)	1,955
Accumulated deficit	(297,242)	(220,699)
Total stockholders' equity (deficit)	574,402	(202,255)
Total liabilities, convertible preferred stock, and stockholders' equity (deficit)	$ 929,064	$ 507,655

See accompanying notes to condensed consolidated financial statements.

ADARx Pharmaceuticals, Inc.
Condensed consolidated statements of operations and comprehensive loss
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Collaboration revenue	$ 2,217	$ 1,555	$ 5,103	$ 1,763
Operating expenses:				
Research and development	26,713	19,416	75,032	49,381
General and administrative	7,681	5,146	19,195	14,399
Total operating expenses	34,394	24,562	94,227	63,780
Loss from operations	(32,177)	(23,007)	(89,124)	(62,017)
Other income, net:				
Interest income	3,479	4,622	11,234	9,363
Other income	581	502	1,347	1,127
Total other income, net	4,060	5,124	12,581	10,490
Net loss	$ (28,117)	$ (17,883)	$ (76,543)	$ (51,527)
Other comprehensive income (loss):				
Foreign currency translation adjustment	3	(6)	32	(28)
Unrealized (losses) gains on available-for-sale securities	(1,372)	435	(4,139)	1,236
Total comprehensive loss	$ (29,486)	$ (17,454)	$ (80,650)	$ (50,319)
Net loss per share, basic and diluted	$ (1.74)	$ (1.85)	$ (6.46)	$ (5.35)
Weighted-average shares outstanding, basic and diluted	16,127,078	9,654,689	11,840,561	9,632,648

See accompanying notes to condensed consolidated financial statements.

ADARx Pharmaceuticals, Inc.
Condensed consolidated statements of convertible preferred stock and stockholders' equity (deficit)
(in thousands, except share data)
(unaudited)

	Series A convertible preferred stock		Series B convertible preferred stock		Series B-1 convertible preferred stock		Series C convertible preferred stock		Common stock		Additional paid-in	Accumulated other comprehensive	Accumulated	Total stockholders' equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	capital	income/(loss)	deficit	(deficit)
Balance as of December 31, 2025	26,726,324	$ 37,553	12,506,015	$ 74,787	5,900,272	$ 45,710	20,515,872	$ 199,572	9,659,572	$ —	$ 16,489	$ 1,955	$ (220,699)	$ (202,255)
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	853	—	2	—	—	2
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	837	—	—	837
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(1,649)	—	(1,649)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(22,667)	(22,667)
Balance as of March 31, 2026	26,726,324	$ 37,553	12,506,015	$ 74,787	5,900,272	$ 45,710	20,515,872	$ 199,572	9,660,425	$ —	$ 17,328	$ 306	$ (243,366)	$ (225,732)
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	9,153	—	22	—	—	22
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	1,506	—	—	1,506
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(1,089)	—	(1,089)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(25,759)	(25,759)
Balance as of June 30, 2026	26,726,324	$ 37,553	12,506,015	$ 74,787	5,900,272	$ 45,710	20,515,872	$ 199,572	9,669,578	$ —	$ 18,856	$ (783)	$ (269,125)	$ (251,052)
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	432,360	—	450	—	—	450
Issuance of common stock in initial public offering, net of underwriting discounts and commissions and offering costs	—	—	—	—	—	—	—	—	26,250,000	3	407,379	—	—	407,382
Issuance of common stock in concurrent private placement, net of placement agent fee	—	—	—	—	—	—	—	—	5,255,542	1	87,557	—	—	87,558
Conversion of convertible preferred stock into common stock on initial public offering	(26,726,324)	$ (37,553)	(12,506,015)	$ (74,787)	(5,900,272)	$ (45,710)	(20,515,872)	$ (199,572)	65,648,483	7	357,615	—	—	357,622
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	1,928	—	—	1,928
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(1,369)	—	(1,369)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(28,117)	(28,117)
Balance as of September 30, 2026	—	$ —	—	$ —	—	$ —	—	$ —	107,255,963	$ 11	$ 873,785	$ (2,152)	$ (297,242)	$ 574,402

	Series A convertible preferred stock		Series B convertible preferred stock		Series B-1 convertible preferred stock		Series C convertible preferred stock		Common stock		Additional paid-in	Accumulated other comprehensive	Accumulated	Total stockholders'
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	capital	income	deficit	deficit
Balance as of December 31, 2024	26,726,324	$ 37,553	12,506,015	$ 74,787	5,900,272	$ 45,710	20,515,872	$ 199,572	9,632,904	$ —	$ 10,131	$ 335	$ (147,611)	$ (137,145)
Vesting of early exercised stock options	—	—	—	—	—	—	—	—	—	—	134	—	—	134
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	1,730	—	—	1,730
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(84)	—	(84)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	$ (15,551)	$ (15,551)
Balance as of March 31, 2025	26,726,324	$ 37,553	12,506,015	$ 74,787	5,900,272	$ 45,710	20,515,872	$ 199,572	9,632,904	$ —	$ 11,995	$ 251	$ (163,162)	$ (150,916)
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	17,069	—	83	—	—	83
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	1,146	—	—	1,146
Other comprehensive income	—	—	—	—	—	—	—	—	—	—	—	863	—	863
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	$ (18,093)	$ (18,093)
Balance as of June 30, 2025	26,726,324	$ 37,553	12,506,015	$ 74,787	5,900,272	$ 45,710	20,515,872	$ 199,572	9,649,973	$ —	$ 13,224	$ 1,114	$ (181,255)	$ (166,917)
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	8,534	—	8	—	—	8
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	1,441	—	—	1,441
Other comprehensive income	—	—	—	—	—	—	—	—	—	—	—	429	—	429
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	$ (17,883)	$ (17,883)
Balance as of September 30, 2025	26,726,324	$ 37,553	12,506,015	$ 74,787	5,900,272	$ 45,710	20,515,872	$ 199,572	9,658,507	$ —	$ 14,673	$ 1,543	$ (199,138)	$ (182,922)

See accompanying notes to condensed consolidated financial statements.

ADARx Pharmaceuticals, Inc.
Condensed consolidated statements of cash flows
(in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2026	2025
Cash flows from operating activities:		
Net loss	$ (76,543)	$ (51,527)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities:		
Stock-based compensation	4,271	4,317
Depreciation	920	707
Amortization of right-of-use assets	1,283	1,111
Accretion on investments	(396)	(2,501)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(4,090)	(9,431)
Other non-current assets	2,109	(1,471)
Accounts payable	2,017	1,806
Accrued expenses and other current liabilities	66	(240)
Operating lease liabilities	(1,282)	(890)
Deferred revenue	(5,103)	333,237
Net cash (used in) provided by operating activities	(76,748)	275,118
Cash flows from investing activities:		
Purchase of investments	(51,751)	(369,872)
Proceeds from maturities of investments	131,000	142,000
Purchase of property and equipment	(641)	(2,205)
Net cash provided by (used in) investing activities	78,608	(230,077)
Cash flows from financing activities:		
Proceeds from exercise of stock options	474	91
Proceeds from initial public offering, net of underwriting discounts and commissions	415,013	—
Proceeds from concurrent private placement	89,344	—
Payments for deferred initial public offering costs	(2,733)	—
Net cash provided by financing activities	502,098	91
Effect of exchange rate changes on cash and cash equivalents	135	11
Net increase in cash and cash equivalents	504,093	45,143
Cash and cash equivalents, beginning of the period	66,517	14,036
Cash and cash equivalents, end of the period	$ 570,610	$ 59,179
Supplemental disclosure of non-cash activities:		
Purchases of property and equipment in accounts payable and accrued expenses and other current liabilities	$ 26	$ 86
Operating lease right-of-use assets obtained in exchange for operating lease liabilities	$ —	$ 4,506
Conversion of convertible preferred stock upon initial public offering	$ 357,622	$ —
Deferred offering costs included in accounts payable and accrued expenses	$ 6,684	$ —
Vesting of early exercised stock options	$ —	$ 134

See accompanying notes to condensed consolidated financial statements.

ADARx Pharmaceuticals, Inc.

Notes to unaudited condensed consolidated financial statements

1. Nature of business

Description of business

ADARx Pharmaceuticals, Inc. (collectively with its wholly owned subsidiary, "ADARx" or the "Company") is headquartered in San Diego, California, and was incorporated in the State of Delaware in December 2019. The Company is a late-clinical stage biotechnology company focused on developing next-generation RNA therapeutics designed to treat a broad spectrum of diseases. The Company is focused on advancing and expanding its pipeline of highly potent, selective and durable small interfering RNA ("siRNA") therapeutic candidates, including three clinical-stage programs and two advanced preclinical programs. Its three hepatic-targeted programs are agazisiran, onvuzosiran and ADX-626, and its two extrahepatic-targeted programs are ADX-077 and ADX-199. Beyond its named programs, the Company plans to continue to leverage its proprietary technologies to strategically expand its pipeline of next-generation siRNA therapeutics. The Company's goal is to control the expression of specific disease drivers with highly selective RNA targeted therapies, delivering life-changing treatments for patients with urgent unmet medical needs.

Initial public offering and concurrent private placement

On September 28, 2026, the Company completed its IPO, in which it issued and sold 26,250,000 shares of its common stock at a public offering price of $17.00 per share, for aggregate gross proceeds of $446.3 million. Concurrently with the closing of the IPO, the Company issued and sold 5,255,542 shares of its common stock to AbbVie Inc. in a private placement at $17.00 per share, for gross proceeds of $89.3 million (the "Concurrent Private Placement"). The Company received aggregate net proceeds of approximately $494.9 million, after deducting underwriting discounts and commissions and offering costs. The Company incurred $1.8 million of placement agent fees associated with the Concurrent Private Placement, all of which were unpaid and included in accounts payable and accrued expenses and other current liabilities as of September 30, 2026.

Immediately prior to the closing of the IPO, all outstanding shares of convertible preferred stock automatically converted into 65,648,483 shares of common stock, and the Company filed its amended and restated certificate of incorporation, which authorizes 999,999,999 shares of common stock and 10,000,000 shares of undesignated preferred stock.

Liquidity and capital resources

As of September 30, 2026, the Company had cash, cash equivalents, and short-term investments of $901.0 million. On October 1, 2026, the Company issued and sold an additional 3,937,500 shares of the Company's common stock pursuant to the underwriters' exercise in full of their option to purchase additional shares at the IPO price of $17.00 per share, for gross proceeds of $66.9 million. The Company received net proceeds of approximately $62.3 million, after deducting underwriting discounts and commissions of $4.6 million. Since its inception, the Company has incurred significant operating losses, and has historically incurred negative cash flows from operations. The Company has devoted substantially all of its resources to organizing and staffing the Company, business planning, raising capital, developing its technologies, establishing and maintaining its intellectual property portfolio, identifying and developing its therapeutic candidates, preparing for, initiating, and conducting its ongoing and planned preclinical studies and clinical trials, establishing and maintaining arrangements with third parties for the manufacturing of the Company's therapeutic candidates, and providing general and administrative support for these operations.

From inception to September 30, 2026, the Company has funded its operations primarily through proceeds from the sale of convertible preferred stock and convertible notes, the upfront payment under the AbbVie Agreement (as defined below in Note 7) and, in September 2026, net proceeds of $494.9 million from its IPO and Concurrent Private Placement. In May 2025 the Company entered into the AbbVie Agreement, receiving an upfront payment which was recorded as deferred revenue. The recognition of this deferred revenue is expected to occur in future periods but is not anticipated to fully offset operating losses in the near term. As such, the Company expects to continue to incur substantial losses for the foreseeable future due to its research and development activities. Additional funding will be required in the future to continue with the Company's planned research and development and other activities. The Company expects to finance its operations through equity offerings, debt financings, and other capital sources, including potential strategic collaborations, licensing, and other similar arrangements. However, there can be no assurance that any additional financing or strategic transactions will be available to the Company on acceptable terms, if at all. If the Company is unable to raise additional funds or enter into such arrangements when needed, the Company could be forced to delay, limit, reduce or terminate its research and development programs or potential future commercialization efforts, or grant rights to develop and market its therapeutic candidates even if it would otherwise prefer to develop and market such therapeutic candidates itself.

Based on the Company's current operating plan, it estimates that its existing cash, cash equivalents, and short-term investments as of September 30, 2026 will be sufficient to fund its projected operating expenses and capital expenditure requirements for at least 12 months following the date these condensed consolidated financial statements are available to be issued.

2. Basis of presentation and summary of significant accounting policies

Basis of presentation and principles of consolidation

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly owned subsidiary, ADARx Pharmaceuticals Australia Pty Ltd. All intercompany balances and transactions have been eliminated in consolidation. These condensed consolidated financial statements have been prepared in accordance with United States ("U.S.") generally accepted accounting principles ("GAAP") for interim financial information and as required by the rules and regulations of the Securities and Exchange Commission ("SEC") for Form 10-Q and Article 10 of Regulation S-X. Certain information and note disclosures normally included in the financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. As such, the information included in this Quarterly Report on Form 10-Q should be read in conjunction with the audited financial statements and accompanying notes included in the final prospectus filed with the SEC on September 28, 2026 pursuant to Rule 424(b)(4) under the Securities Act (the "Prospectus"). These condensed consolidated financial statements include all adjustments necessary for the fair presentation of the Company's financial position, results of operations and cash flows for the periods presented.

Reverse stock split

On September 17, 2026, the Company amended its amended and restated certificate of incorporation in order to effect a 1-for-1.1717 reverse stock split of its issued and outstanding shares of common stock, Series A, Series B, Series B-1, and Series C preferred stock, as well as stock option awards to purchase shares of common stock. Consequently, all issued and outstanding shares of stock, stock option awards, and per share data have been retroactively adjusted in these financial statements to reflect the reverse stock split for all periods presented. The authorized shares and par value of the common stock and preferred stock remain unchanged. No fractional shares were issued in connection with the reverse stock split.

Significant accounting policies

During the nine months ended September 30, 2026, there were no significant changes to the Company's significant accounting policies as described in Note 2 of the Company's audited consolidated financial statements for the year ended December 31, 2025 included in the Prospectus.

Emerging growth company status and smaller reporting company status

The Company is an "emerging growth company," as defined in the JOBS Act, and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies. The Company may take advantage of these exemptions until the Company is no longer an emerging growth company. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. The Company has elected to use the extended transition period for complying with new or revised accounting standards and as a result of this election, its financial statements may not be comparable to companies that comply with public company effective dates. The Company will continue to remain an emerging growth company and may take advantage of these exemptions until the earliest of the following: (i) the last day of the fiscal year following the fifth anniversary of the date of the completion of the IPO, or December 31, 2031; (ii) the last day of the fiscal year in which the Company's total annual gross revenue is equal to or more than $1.235 billion; (iii) the date on which the Company has issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which the Company is deemed to be a large accelerated filer as defined in Rule 12b-2 under the Exchange Act.

The Company is also a smaller reporting company as defined in the Exchange Act. The Company may continue to be a smaller reporting company even after it is no longer an emerging growth company. The Company may take advantage of certain scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as its voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of the Company's second fiscal quarter, or the Company's annual revenue is less than $100.0 million during the most recently completed fiscal year and its voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of the Company's second fiscal quarter.

Specifically, as a smaller reporting company the Company may choose to present only the two most recent fiscal years of audited

financial statements in its Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Recently issued and adopted accounting pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board (the "FASB") or other standard setting bodies and adopted by the Company as of the specified effective date.

In October 2023, the FASB issued Accounting Standards Update ("ASU") No. 2023-06, *Disclosure Improvements: Codification Amendments in Response to the SEC's Disclosure Update and Simplification Initiative*. The amendments in the ASU are expected to clarify or improve disclosure and presentation requirements of a variety of Accounting Standards Codification ("ASC") Topics, allow users to more easily compare entities subject to the SEC's existing disclosures with those entities that were not previously subject to the requirements, and align the requirements in the ASC with the SEC's regulations. The ASU has an unusual effective date and transition requirements since it is contingent on future SEC rule setting. If the SEC fails to enact the required changes by June 30, 2027, this ASU is not effective for any entities. Early adoption is not permitted. The Company is currently evaluating the impact this ASU will have on its consolidated financial statements and related disclosures.

In November 2023, the FASB issued ASU No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*. The ASU primarily will require enhanced disclosures about significant segment expenses. The improved disclosure requirements apply to all public entities that are required to report segment information, including those with only one reportable segment. The Company adopted the guidance in the fiscal year beginning January 1, 2025 and there was no impact on the Company's reportable segments identified. Additional required disclosures have been added within Note 14.

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*. The ASU requires disaggregated information about a reporting entity's effective tax rate reconciliation as well as an expansion of other income tax disclosures. The ASU is effective on a prospective basis for annual reporting periods beginning after December 15, 2024. The Company adopted the guidance in the fiscal year beginning January 1, 2025 and applied the disclosure requirements on a retrospective basis effective for the year ended December 31, 2025. Adoption of this ASU resulted in additional disclosures presented in Note 11 of the Company's audited consolidated financial statements for the year ended December 31, 2025 included in the Prospectus and did not have an impact on the Company's consolidated financial position, results of operations or cash flows.

In November 2024, the FASB issued ASU No. 2024-03, *Income Statement-Reporting Comprehensive Income-Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* to improve financial reporting by requiring entities to disclose additional information about specific expense categories in the notes to financial statements at interim and annual reporting periods. This ASU is effective for annual reporting periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company is currently evaluating the impact this ASU will have on its consolidated financial statements and related disclosures.

In September 2025, the FASB issued ASU No. 2025-07, *Derivatives and Hedging (Topic 815) and Revenue Contracts with Customers (Topic 606): Derivatives Scope Refinements and Scope Clarification for Share-Based Noncash Consideration from a Customer in a Revenue Contract* ("ASU 2025-07"). The amendment provides for a new scope exception to the derivatives guidance of ASC Topic 815, *Derivative and Hedging* ("ASC 815") for underlyings based on the operations or activities specific to one of the parties to the contract. The amendment also provides clarification on the accounting for share-based noncash consideration received from a customer in arrangements within the scope of ASC 606. The amendment is effective for annual reporting periods beginning after December 15, 2026, including interim periods within those fiscal years. Early adoption is permitted. The Company early-adopted the amendment in connection with the execution of the AbbVie Agreement and as a result, the new derivative scope exception was applied and the options granted to AbbVie fall within the scope of ASC 606. See Note 7 for additional details.

In December 2025, the FASB issued ASU No. 2025-11, *Interim Reporting (Topic 270): Narrow-Scope Improvements* ("Topic 270"). This ASU clarifies the applicability of Topic 270 and the form and content of interim financial statements and will be effective for interim periods beginning January 1, 2028, and can be applied on a prospective or retrospective basis. The Company is currently evaluating the impact this ASU will have on its consolidated financial statements and related disclosures.

In December 2025, the FASB issued ASU 2025-12, *Codification Improvements* ("ASU 2025-12"). ASU 2025-12 addresses suggestions received from stakeholders on the ASC and makes other incremental improvements to U.S. GAAP. The update represents changes to the ASC that (1) clarify, (2) correct errors, or (3) make minor improvements. The amendments make the ASC easier to understand and apply. The guidance is effective for fiscal years beginning after December 15, 2026, including interim periods within those fiscal years. The Company is currently evaluating the impact of this standard on its condensed consolidated financial statements.

3. Short-term investments

The Company's short-term investments consisted of the following positions for available-for-sale fixed-maturity debt securities, disaggregated by class of instrument (in thousands):

	As of September 30, 2026			
	Amortized cost basis	Gross unrealized losses	Gross unrealized gains	Fair value
Short-term investments				
U.S. Treasury securities	$ 332,729	$ (2,332)	$ —	$ 330,397
Total	$ 332,729	$ (2,332)	$ —	$ 330,397

	As of December 31, 2025			
	Amortized cost basis	Gross unrealized losses	Gross unrealized gains	Fair value
Short-term investments				
U.S. Treasury securities	$ 411,582	$ —	$ 1,897	$ 413,479
Total	$ 411,582	$ —	$ 1,897	$ 413,479

The following tables summarize the fair value and gross unrealized losses aggregated by category and the length of time that individual securities have been in a continuous unrealized loss position (in thousands):

	As of September 30, 2026	
All unrealized holding losses are less than twelve months at date of measurement	Fair value	Gross unrealized losses
U.S. Treasury securities	$ 330,397	$ (2,332)
Total	$ 330,397	$ (2,332)

	As of December 31, 2025	
All unrealized holding losses are less than twelve months at date of measurement	Fair value	Gross unrealized losses
U.S. Treasury securities	$ —	$ —
Total	$ —	$ —

As of September 30, 2026, of the Company's U.S. Treasury securities, $185.8 million had contractual maturities of less than one year and $144.6 million had contractual maturities of between one and three years. All such securities are classified as current assets in accordance with the Company's short-term investment policy described in Note 2 of the Company's audited consolidated financial statements for the year ended December 31, 2025 included in the Prospectus. As of September 30, 2026, there were no individual securities that were in a continuous unrealized loss position for more than twelve months, and the gross unrealized losses on the Company's available-for-sale debt securities were considered immaterial. The Company does not intend to sell these securities and is not likely to be required to sell them before recovering their amortized cost basis. Based on the nature of the securities, the temporary nature of the unrealized losses, and their credit quality, the Company determined no credit loss impairment existed as of September 30, 2026 and December 31, 2025, and no allowance for credit losses was recorded.

4. Fair value measurements

The tables below present information about the Company's financial assets measured at fair value and indicate the fair value hierarchy of the valuation technique utilized to determine such fair value (in thousands):

	As of September 30, 2026			
	Level 1	Level 2	Level 3	Total
Cash and cash equivalents				
Money market funds	$ 562,924	$ —	$ —	$ 562,924
Short-term investments				
U.S. Treasury securities	330,397	—	—	330,397
Total	$ 893,321	$ —	$ —	$ 893,321

	As of December 31, 2025			
	Level 1	Level 2	Level 3	Total
Cash and cash equivalents				
Money market funds	$ 58,809	$ —	$ —	$ 58,809
Short-term investments				
U.S. Treasury securities	413,479	—	—	413,479
Total	$ 472,288	$ —	$ —	$ 472,288

For the nine months ended September 30, 2026, there were no transfers between Level 1, Level 2, and Level 3.

5. Property and equipment, net

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

	September 30, 2026	December 31, 2025
Computers and office equipment	$ 180	$ 90
Furniture and fixtures	48	40
Laboratory equipment	8,232	7,757
Construction in progress	18	28
Leasehold improvements	358	358
Total property and equipment	8,836	8,273
Less: accumulated depreciation	$ (4,439)	(3,539)
Property and equipment, net	$ 4,397	$ 4,734

Depreciation expense related to property and equipment was $0.3 million for each of the three months ended September 30, 2026 and 2025, and $0.9 million and $0.7 million for the nine months ended September 30, 2026 and 2025, respectively.

6. Balance sheet components

Accrued expenses and other current liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Accrued compensation related expenses	$ 4,048	$ 2,877
Accrued professional fees and other expenses	4,269	920
Accrued research, development and manufacturing expenses	3,277	2,574
Total accrued expenses and other current liabilities	$ 11,594	$ 6,371

7. Collaboration and license option agreement

AbbVie collaboration and license option agreement

In May 2025, the Company entered into the Collaboration and License Option Agreement (the "AbbVie Agreement") with the Puerto Rico branch of AbbVie Biotechnology Ltd ("AbbVie"). Pursuant to the terms of the AbbVie Agreement, the Company received an initial upfront payment of $335.0 million. The key terms and accounting conclusions for the AbbVie Agreement are included within Note 6 of the Company's audited consolidated financial statements for the year ended December 31, 2025 included in the Prospectus. No new collaboration agreements were executed and there were no material modifications to existing agreements during the nine months ended September 30, 2026.

AbbVie revenue recognized

During the three months ended September 30, 2026 and 2025, the Company recognized $2.2 million and $1.6 million, respectively, of collaboration revenue under the AbbVie Agreement. During the nine months ended September 30, 2026 and 2025, the Company recognized $5.1 million and $1.8 million, respectively, of collaboration revenue under the AbbVie Agreement. In each case, such

revenue was recognized based on the Company's performance of research and development services completed during the respective periods.

As of September 30, 2026 and December 31, 2025, the Company had short-term deferred revenue of $20.9 million and $16.6 million, respectively, and long-term deferred revenue of $305.5 million and $315.0 million, respectively, related to the AbbVie Agreement. As of September 30, 2026, $297.8 million of consideration allocated to license option material rights was included in long-term deferred revenue. The remaining long-term deferred research and development services revenue is expected to be recognized proportionally to the completed obligations over an expected remaining contractual term of approximately 2.1 years.

Amounts due to the Company that have not yet been received are recorded as accounts receivable and amounts received that have not yet been recognized as revenue are recorded as deferred revenue on the Company's condensed consolidated balance sheets. Costs incurred pursuant to the AbbVie Agreement are recorded as research and development expense.

8. Commitments and contingencies

Lease commitments

Refer to Note 7 of the Company's audited consolidated financial statements for the year ended December 31, 2025 included in the Prospectus for details on the Company's leased facilities.

The Company's lease costs for the three months ended September 30, 2026 and 2025 and the nine months ended September 30, 2026 and 2025 consisted of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating lease cost[(1)]	$ 570	$ 567	$ 1,762	$ 1,612
Variable lease cost	260	255	737	703
Total lease cost	$ 830	$ 822	$ 2,499	$ 2,315

(1) Includes short-term lease costs of $0.1 million and less than $0.1 million for the three months ended September 30, 2026 and 2025, respectively, and $0.2 million and $0.1 million for the nine months ended September 30, 2026 and 2025, respectively. Short-term lease costs relate to leases with terms of twelve months or less for which the Company elected to apply the short-term lease recognition exemption.

Supplemental information related to the Company's leases was as follows (dollars in thousands):

	September 30, 2026	December 31, 2025
Weighted-average remaining lease term	3.32 years	4.01 years
Weighted-average discount rate	8.15%	8.15%

Cash paid for operating lease liabilities during the nine months ended September 30, 2026 and 2025 was $1.7 million and $1.4 million, respectively.

Maturities of operating lease liabilities under non-cancelable leases by fiscal year for the Company's operating leases, as of September 30, 2026, were as follows (in thousands):

Year ending December 31,	
2026 (3 months)	$ 556
2027	2,140
2028	2,204
2029	2,270
Thereafter	190
Total future minimum lease payments	7,360
Less: imputed interest	(901)
Total lease liabilities balance	6,459
Less: lease liabilities, current portion	(1,710)
Lease liabilities, net of current portion	$ 4,749

Employee benefit plan

The Company maintains a defined-contribution plan under Section 401(k) of the Internal Revenue Code of 1986, as amended ("IRC"). The plan covers substantially all employees and allows participating employees to defer a portion of their earnings, up to the IRS annual contribution limit. The plan allows for a discretionary match in an amount up to 100% of each participant's first 2% of compensation contributed plus 50% of each participant's next 4% of compensation contributed. The Company's total matching contributions were $0.1 million and $0.1 million for the three months ended September 30, 2026 and 2025, respectively, and $0.5 million and $0.4 million for the nine months ended September 30, 2026 and 2025, respectively.

Contingencies

From time to time, the Company may be subject to claims and litigation arising in the ordinary course of business. The Company is not a party to any material legal proceedings, nor is it aware of any material pending or threatened litigation.

9. Convertible preferred stock

Immediately prior to the closing of the IPO on September 28, 2026, all 65,648,483 outstanding shares of Series A, Series B, Series B-1 and Series C convertible preferred stock automatically converted into an equal number of shares of common stock, and the carrying value of $357.6 million was reclassified to common stock and additional paid-in capital. No shares of convertible preferred stock were outstanding as of September 30, 2026.

Under the amended and restated certificate of incorporation, the board of directors may issue up to 10,000,000 shares of undesignated preferred stock, $0.0001 par value, in one or more series and fix their rights, preferences and privileges without further stockholder action. No shares of preferred stock were issued or outstanding as of September 30, 2026.

10. Stockholders' equity (deficit)

Common stock

As of September 30, 2026 and December 31, 2025, the Company had 999,999,999 and 102,000,000 shares, respectively, of its common stock authorized for issuance, $0.0001 par value per share, with 107,255,963 and 9,659,572 shares issued and outstanding, respectively. Common stock reserved for future issuance as of September 30, 2026 and December 31, 2025, consisted of the following:

	September 30, 2026	December 31, 2025
Convertible preferred stock outstanding	—	65,648,483
Stock options issued and outstanding	13,325,434	10,402,879
Shares available for grant under 2020 Plan	—	3,553,329
Shares available for grant under 2026 Plan	8,131,054	—
Shares available for grant under the ESPP	1,026,556	—
Total	22,483,044	79,604,691

11. Stock-based compensation

2020 Equity Incentive Plan

On July 24, 2020, the board of directors adopted, and the Company's stockholders approved, the 2020 Equity Incentive Plan. The 2020 Equity Incentive Plan, as amended and restated (the "2020 Plan"), provides for the grant of incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, and other forms of stock awards to employees, directors, and consultants of the Company. Following the effectiveness of the 2026 Equity Incentive Plan (the "2026 Plan"), no further awards will be granted under the 2020 Plan; however, awards outstanding under the 2020 Plan continue to be governed by its terms.

No shares remain available under the 2020 Plan after the 2026 Plan became effective.

2026 Equity Incentive Plan

In September 2026, the board of directors adopted, and stockholders approved, the 2026 Plan, which became effective on September 24, 2026. The 2026 Plan provides for the grant of incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance awards and other awards. The number of shares initially reserved

under the 2026 Plan is 21,456,547, which includes 7,942,658 new shares plus shares remaining available, and shares subject to outstanding awards that are forfeited or expire, under the 2020 Plan. Shares reserved for issuance automatically increase each January 1 from 2027 through 2036 by 5% of shares of common stock outstanding on December 31 of the preceding year or a lesser amount determined by the board of directors.

2026 Employee Stock Purchase Plan

In September 2026, the board of directors adopted, and stockholders approved, the 2026 Employee Stock Purchase Plan (the "ESPP"), which became effective on September 24, 2026. The ESPP initially reserves 1,026,556 shares of common stock. Shares reserved for future issuance automatically increase each January 1 from 2027 through 2036 by the lesser of 1% of shares of common stock outstanding on December 31 of the preceding year, or 3,079,668 shares, or a lesser amount determined by the board of directors. Eligible employees may purchase shares at 85% of the lower of the fair market value on the first day of the offering period or the purchase date. As of September 30, 2026, no offering period had commenced.

Stock options

The following is a summary of the Company's stock option activity under its 2020 and 2026 Plans:

	Shares	Weighted-average exercise price per share	Weighted-average remaining contractual life (years)	Aggregate intrinsic value (in thousands)
Outstanding as of December 31, 2025	10,402,879	$ 3.00	6.5	$ 34,924
Granted	5,643,424	$ 10.79		
Exercised	(442,366)	$ 1.08		
Forfeited/canceled	(2,278,503)	$ 6.18		
Outstanding as of September 30, 2026	13,325,434	$ 5.82	6.6	$ 188,077
Vested and exercisable as of September 30, 2026	7,133,634	$ 1.92	5.2	$ 128,563

The weighted-average grant date fair value of options granted during the three months ended September 30, 2026 was $12.20. The weighted-average grant date fair value of options granted during the nine months ended September 30, 2026 was $10.36. The total intrinsic value of options exercised during the three and nine months ended September 30, 2026 was $3.3 million, in each period. As of September 30, 2026, the total unrecognized compensation expense related to unvested stock option awards was $48.8 million. The Company expects to recognize that cost over a weighted-average period of approximately 3.6 years.

Stock-based compensation

In determining the fair value of the employee and non-employee stock options, the Company uses the Black-Scholes option-pricing model. Prior to the IPO, the fair value of the shares of common stock underlying stock options was determined by the board of directors with input from third-party valuations. Following the IPO, the fair value is based on the closing price of the Company's common stock on the Nasdaq Global Select Market on the grant date. Refer to Note 10 of the Company's audited consolidated financial statements for the year ended December 31, 2025, included in the Prospectus for details regarding the estimates and assumptions made.

The following key inputs were used in the Black-Scholes option-pricing model on a weighted-average basis:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Fair value of common stock	$ 14.86	$ 4.15	$ 11.74	$ 4.27
Expected term	6.08 years	6.01 years	6.09 years	6.02 years
Expected volatility	91.90 %	94.61 %	92.09 %	95.43 %
Risk-free interest rate	4.91 %	3.78 %	4.63 %	4.00 %
Expected dividend yield	— %	— %	— %	— %

Stock-based compensation expense recorded in the condensed consolidated statements of operations and comprehensive loss was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	183	166	494	512
General and administrative	1,745	1,275	3,777	3,805
Total stock-based compensation expense	$ 1,928	$ 1,441	$ 4,271	$ 4,317

12. Income taxes

The tax provision for interim periods is determined using an estimate of the Company's effective tax rate for the full year adjusted for discrete items, if any, that are taken into account in the relevant period. Each quarter the Company updates its estimate of the annual effective tax rate, and if the estimated tax rate changes, the Company makes a cumulative adjustment. No provision for federal, state or foreign income taxes has been recorded for the nine months ended September 30, 2026 and 2025. Refer to Note 11 of the Company's audited consolidated financial statements for the year ended December 31, 2025 included in the Prospectus for additional details regarding the Company's tax policies.

At September 30, 2026 and December 31, 2025, the Company had a full valuation allowance against its deferred tax assets, net of expected reversals of existing deferred tax liabilities, as it believes it is more likely than not that these benefits will not be realized.

13. Net loss per share

The following table summarizes the computation of basic and diluted net loss per share (in thousands, except share and per share amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Numerator:				
Net loss	$ (28,117)	$ (17,883)	$ (76,543)	$ (51,527)
Denominator:				
Weighted-average common shares outstanding, basic and diluted	16,127,078	9,654,689	11,840,561	9,632,648
Net loss per share, basic and diluted	$ (1.74)	$ (1.85)	$ (6.46)	$ (5.35)

The following outstanding potentially dilutive common stock equivalents were excluded from the computation of diluted net loss per share for the periods presented because including them would have been antidilutive:

	As of September 30,	
	2026	2025
Convertible preferred stock	—	65,648,483
Common stock options	13,325,434	10,654,526
Total	13,325,434	76,303,009

14. Segment reporting

The Company identifies its operating segments based on the regular review of operating results by the Chief Executive Officer, who acts as the Chief Operating Decision Maker ("CODM"). The CODM allocates resources and assesses performance based on these results. The Company has one reportable segment focused on developing proprietary RNA targeting delivery platforms as well as technologies for silencing or editing target mRNA. This segment encompasses the Company's pipeline of RNA therapeutics for treating diseases across various therapeutic areas, including complement-mediated, hereditary angioedema, thrombotic diseases, and extrahepatic programs targeting adipose (obesity) and neurons (neurodegenerative diseases including Alzheimer's).

Segment assets are reported on the consolidated balance sheets as total consolidated assets, encompassing all assets, liabilities, cash flows, and expenses within the Company's single reportable segment. When evaluating the Company's financial performance and making strategic decisions, the CODM focuses their review of expenses incurred based on the nature of those expenses.

The table below is a summary of the segment profit or loss, including significant segment expenses (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Collaboration revenue	$ 2,217	$ 1,555	$ 5,103	$ 1,763
Operating expenses:				
Research and development:				
External research and development:				
Agazisiran	3,780	2,967	13,797	7,198
Onvuzosiran	6,214	6,035	17,179	13,217
ADX-626	1,300	726	4,296	2,608
Preclinical and other costs	8,353	2,717	18,156	6,777
Total external research and development costs	19,647	12,445	53,428	29,800
Internal research and development costs	7,066	6,971	21,604	19,581
Total research and development	26,713	19,416	75,032	49,381
General and administrative	7,681	5,146	19,195	14,399
Total operating expenses	34,394	24,562	94,227	63,780
Loss from operations	(32,177)	(23,007)	(89,124)	(62,017)
Other income, net:				
Interest income	3,479	4,622	11,234	9,363
Other income	581	502	1,347	1,127
Total other income, net	4,060	5,124	12,581	10,490
Consolidated net loss	$ (28,117)	$ (17,883)	$ (76,543)	$ (51,527)

15. Subsequent events

In connection with the preparation of the condensed consolidated financial statements as of September 30, 2026 and December 31, 2025 and for the nine months ended September 30, 2026 and 2025, the Company has evaluated all events and transactions through November 12, 2026, the date on which these condensed consolidated financial statements were issued.

Exercise of underwriters' option

On October 1, 2026, the Company issued and sold an additional 3,937,500 shares of common stock pursuant to the underwriters' exercise in full of their option to purchase additional shares at the IPO price of $17.00 per share. The Company received net proceeds of approximately $62.3 million, after deducting underwriting discounts and commissions of approximately $4.6 million. The issuance of the additional shares is not reflected in the accompanying condensed consolidated financial statements as of and for the three and nine months ended September 30, 2026.

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

You should read the following discussion and analysis of our financial condition and results of operations together with our unaudited condensed consolidated financial statements and related notes included elsewhere in this Quarterly Report on Form 10-Q, as well as our audited consolidated financial statements and related notes and the section titled "Management's discussion and analysis of financial condition and results of operations" included in the Prospectus. 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 related financing, includes forward-looking statements that involve risks and uncertainties. You should read the sections titled "Risk Factors" and "Special Note Regarding Forward-Looking Statements" for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a late-clinical stage biotechnology company focused on developing next-generation RNA therapeutics designed to treat a broad spectrum of diseases. We are focused on advancing and expanding our pipeline of highly potent, selective and durable siRNA therapeutic candidates, including three clinical-stage programs and two advanced preclinical programs. Our goal is to control the expression of specific disease drivers with highly selective RNA targeted therapies, delivering life-changing treatments for patients with urgent unmet medical needs. Our team is united by a shared vision to build a leading next-generation siRNA therapeutics company.

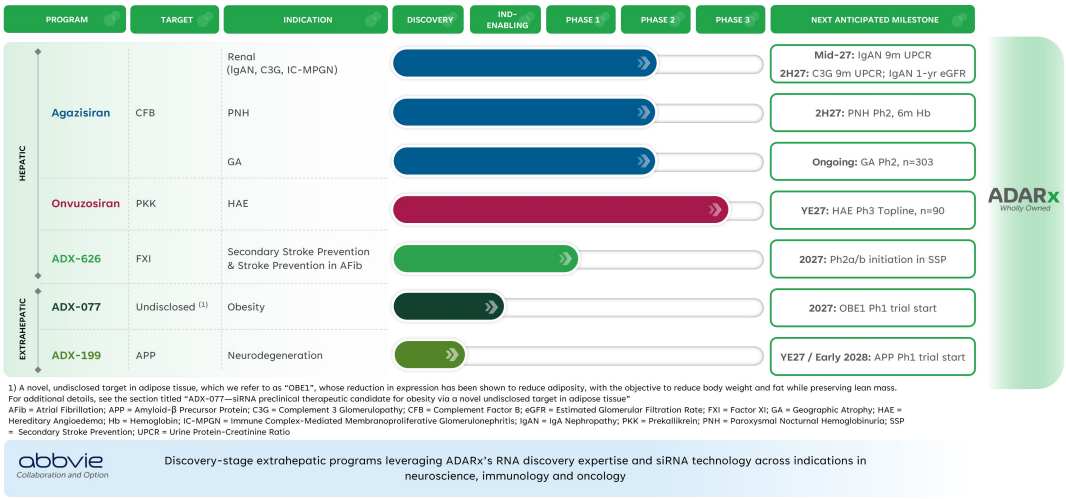
Our hepatic-targeted programs include:

- Agazisiran: Our complement factor B (CFB) program, agazisiran, represents a pipeline-in-a-product opportunity, where a single therapeutic candidate has the potential to address multiple indications. Our Phase 1 clinical trial evaluated the safety and tolerability, pharmacokinetic (PK) activity and pharmacodynamic (PD) activity of agazisiran. We are conducting three Phase 2 clinical trials to evaluate the efficacy and safety of agazisiran in patients with complement-mediated diseases: (1) renal diseases, including IgA nephropathy (IgAN), complement 3 glomerulopathy (C3G), and immune complex membranoproliferative glomerulonephritis (IC-MPGN); (2) paroxysmal nocturnal hemoglobinuria (PNH); and (3) geographic atrophy (GA) secondary to age-related macular degeneration (AMD).
- Onvuzosiran: Our most advanced clinical program, onvuzosiran, targets prekallikrein (PKK) for the prevention of hereditary angioedema (HAE) attacks. Our Phase 1/2 clinical trial evaluated the safety and tolerability, PK activity and PD activity of onvuzosiran in healthy volunteers and HAE patients. We believe our durability results support the potential for once every six months (Q6M) and once every three months (Q3M) dosing via subcutaneous (SC) injection. We are conducting our randomized, double-blind, placebo-controlled Phase 3 STOP-HAE clinical trial designed to evaluate the efficacy and safety of onvuzosiran in 90 adults with Type I or Type II HAE. In August 2026, we received Fast Track designation for onvuzosiran for prophylaxis to prevent attacks of HAE from the U.S. Food and Drug Administration (FDA). We have also previously received orphan drug designation for onvuzosiran for the treatment of HAE from the FDA.
- ADX-626: Our Factor XI (FXI) program, ADX-626, is in development for secondary stroke prevention with additional clinical potential for stroke prevention in atrial fibrillation.

Our extrahepatic-targeted programs include:

- ADX-077: Our adipose-targeted obesity program, ADX-077, is designed to reduce body weight and fat while preserving lean mass. Our obese non-human primate (NHP) study evaluated the preclinical activity and safety of ADX-077. We have initiated IND-enabling studies and plan to initiate a Phase 1 clinical trial in 2027, subject to receipt of regulatory clearance. Initial clinical data from this planned Phase 1 clinical trial are anticipated in 2028.
- ADX-199: Our neuron-targeted program, ADX-199, is designed to selectively silence and degrade amyloid-β precursor protein (APP) mRNA in neurons, which is genetically linked to Alzheimer's disease. Our NHP studies evaluated the preclinical activity and safety of ADX-199. We are advancing ADX-199 into IND-enabling studies and plan to initiate a Phase 1 clinical trial by the end of 2027 or in early 2028. Initial clinical data from this planned Phase 1 clinical trial are anticipated in 2028.

Beyond our named programs, we plan to continue to leverage our proprietary technologies to strategically expand our pipeline of next-generation siRNA therapeutics. Our therapeutics pipeline is summarized below:



Recent developments

- Agazisiran - Advancing three Phase 2 clinical trials for the treatment of complement-mediated diseases: We continued to advance a broad clinical development program including three ongoing Phase 2 clinical trials in (1) renal diseases, including IgAN, C3G and IC-MPGN; (2) PNH; and (3) GA secondary to AMD, to support agazisiran's potential as a pipeline-in-a-product opportunity. We intend to explore additional dosing regimens in our Phase 2 IgAN and PNH clinical trials.
- Onvuzosiran - Received Fast Track designation for HAE: In August 2026, the FDA granted Fast Track designation to onvuzosiran for prophylaxis to prevent attacks of HAE. Onvuzosiran is currently being evaluated in the Phase 3 STOP-HAE clinical trial (NCT06960213).
- ADX-626 - Advanced clinical development and Phase 2 planning in secondary stroke prevention (SSP) and stroke prevention in atrial fibrillation (AFib): Phase 2 planning is underway for development in SSP and for exploratory studies in stroke prevention in AFib patients. Determination has been made to not evaluate higher doses after the third dose cohort (400 mg) in the ongoing Phase 1 trial, and we intend to evaluate higher doses in our planned Phase 2 clinical trial.
- On October 1, 2026, we issued and sold an additional 3,937,500 shares of our common stock pursuant to the underwriters' exercise in full of their option to purchase additional shares at the public offering price. We received net proceeds of approximately $62.3 million, after deducting underwriting discounts and commissions of approximately $4.6 million. Aggregate net proceeds from the IPO, the concurrent private placement and the option exercise were approximately $557.2 million, after deducting underwriting discounts and commissions, placement agent fees and estimated offering expenses payable by us. See "—Liquidity and capital resources" and Notes 1 and 15 to our unaudited condensed consolidated financial statements.
- Expanded leadership team with board and executive appointments: Executive leader and current K2 Therapeutics CEO, Ying Huang, Ph.D., was appointed to the board of directors and veteran human resources leader Robert Ackles was appointed as Chief People Officer to help guide our strategic growth.

Risks and uncertainties

Since we commenced operations in 2019, we have devoted substantially all of our resources to organizing and staffing our company, business planning, raising capital, developing our technologies, establishing and maintaining our intellectual property portfolio, identifying and developing our therapeutic candidates, preparing for, initiating and conducting our ongoing and planned preclinical studies and clinical trials, establishing and maintaining arrangements with third parties for the manufacturing of our therapeutic candidates and providing general and administrative support for these operations.

We have incurred significant operating losses since our inception. As of September 30, 2026, we had an accumulated deficit of $297.2 million. Substantially all of our net losses have resulted from costs related to our research and development programs and to a lesser extent, from general and administrative expenses. As described in greater detail in the subsection titled "Collaboration and license option agreement" below, we entered into the AbbVie Agreement in May 2025 and recorded $335.0 million in deferred revenue from the receipt of the related upfront payment. However, the recognition of this deferred revenue is expected to occur in future periods and

is not anticipated to outweigh potential operating losses. As such, we expect to continue incurring significant expenses and operating losses for the foreseeable future and we anticipate these losses will increase substantially as we continue our development of, seek regulatory approval for and potentially commercialize our therapeutic candidates, seek to discover and develop additional therapeutic candidates, utilize third party manufacturers, hire additional personnel, expand and protect our intellectual property portfolio and incur additional costs associated with being a public company. If we obtain regulatory approval for our therapeutic candidates, we anticipate incurring significant expenses related to developing our commercialization capabilities for product sales, marketing and distribution. Our net losses may fluctuate significantly from quarter to quarter and year to year, depending on the timing of our clinical trials, preclinical studies and other research and development activities and capital expenditures.

Since our inception, we have primarily funded our operations through the sale of shares of our convertible preferred stock and convertible notes, payments received under the AbbVie Agreement, and more recently, our IPO and the concurrent private placement. As of September 30, 2026, we had cash, cash equivalents, and short-term investments of $901.0 million. Based on our current operating plans, we believe our existing cash, cash equivalents and short-term investments as of September 30, 2026, together with the net proceeds received in October 2026 from the exercise in full of the option granted to the underwriters of our IPO to purchase additional shares of our common stock, will be sufficient to fund our projected operating expenses and capital expenditure requirements into 2030. However, this estimate is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. We have based this estimate on assumptions that may prove to be incorrect, and we could use our available capital resources sooner than anticipated.

We do not have any products approved for sale and have not generated any revenue from product sales to date. We do not expect to generate any revenue from product sales until we successfully complete development and obtain regulatory approval for one or more of our current or any future therapeutic candidates, which we expect will take a number of years and may never occur. As a result, we will need substantial additional funding in addition to the net proceeds from the IPO and the concurrent private placement to support our continuing operations and pursue our business plan. Until such time we can generate significant revenue from product sales, if ever, we expect to finance our operations through public or private equity or debt financings or other capital sources, which may include strategic collaborations and other strategic arrangements with third parties. However, we may not be able to secure additional financing or enter into such other arrangements in a timely manner or on favorable terms, if at all. If we raise funds through collaboration or licensing arrangements with third parties or other strategic transactions, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs, or therapeutic candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. If we are unable to raise additional funds or enter into such arrangements when needed, we could be forced to delay, limit, reduce or terminate our research and development programs or future commercialization efforts, or grant rights to develop and market our therapeutic candidates even if we would otherwise prefer to develop and market such therapeutic candidates ourselves.

We do not own or operate manufacturing facilities for the production of our therapeutic candidates and currently have no plans to build our own clinical or commercial-scale manufacturing capabilities. We currently engage third-party manufacturers for the manufacturing of our therapeutic candidates, and we intend to continue to do so in the future. In addition, we rely on third parties to package, label, store, and distribute our therapeutic candidates, and we intend to rely on third parties for our commercial products if marketing approval is obtained. We believe that this strategy allows us to maintain a more efficient infrastructure by eliminating the need for us to invest in our own manufacturing facilities, equipment, and personnel while also enabling us to focus our expertise and resources on the discovery and development of our therapeutic candidates.

Collaboration and license option agreement

In May 2025, we entered into the Collaboration and License Option Agreement (the "AbbVie Agreement") with the Puerto Rico branch of AbbVie Biotechnology Ltd ("AbbVie"), pursuant to which we will use our proprietary platform, and AbbVie may contribute certain technology, to develop therapeutic agents that contain siRNA directed to certain agreed upon collaboration targets, in multiple disease areas including neuroscience, immunology and oncology, which AbbVie may select for further development.

With respect to each collaboration target, we granted to AbbVie an exclusive option to obtain an exclusive (even as to us), sublicensable license under certain intellectual property rights to develop, commercialize and otherwise exploit the siRNA-containing therapeutic agents (and products containing such therapeutic agents) directed to such collaboration target on a worldwide basis in all human and non-human diagnostic, prophylactic and therapeutic uses.

Under the terms of the AbbVie Agreement, we received a $335.0 million upfront payment from AbbVie. We are eligible to receive option extension payments upon AbbVie's selection of a development candidate for each collaboration target totaling up to $150.0 million in the aggregate and one-time payments upon each option exercise with respect to each licensed target totaling up to $235.0 million. In addition, contingent upon AbbVie exercising the option to acquire a target license, we are eligible to receive development and commercial milestone payments up to $3.5 billion, sales milestone payments up to $3.95 billion for all licensed targets, and tiered

royalty earnout payments on net sales of licensed products sold by AbbVie, its affiliates and sublicensees at rates in the high single digits to mid-teens subject to reduction in specified circumstances. AbbVie's royalty earnout payment obligation will commence, on a country-by-country and licensed product-by-licensed product basis, on the date of the first sale of such licensed product in such country that is counted among net sales, and shall expire on the last to occur of (i) expiration of the last eligible valid claim for such licensed product in such country and (ii)(a) if such licensed product is approved under a new drug application ("NDA") or its foreign equivalent, 10 years from first net sale or (b) if such licensed product is approved under a biologics license application ("BLA") or its foreign equivalent, 12 years from first net sale, and (iii) expiration of regulatory exclusivity in such country for such licensed product.

The parties have formed a joint governance committee, composed of an equal number of representatives from each of the Company and AbbVie, to manage the collaboration during the research phase.

Components of our results of operations

Collaboration revenue

Our revenue consists of collaboration revenue, including amounts recognized as payments for licenses, research funding and/or milestone payments earned under our collaboration and license agreements.

In the future, we may generate revenue from additional collaboration, licensing, or other similar arrangements that we may enter into with respect to our therapeutic candidates, as well as product sales from any approved product, if ever. Our ability to generate revenues will depend on the successful development, regulatory approval and eventual commercialization of any current or future therapeutic candidate. If we fail to complete the development of any current or future therapeutic candidate in a timely manner or to obtain regulatory approval for such therapeutic candidate, our ability to generate future revenue and our results of operations and financial position would be materially adversely affected.

Operating expenses

Our operating expenses consist of (i) research and development expenses and (ii) general and administrative expenses.

Research and development

Research and development ("R&D") expenses primarily consist of costs associated with our R&D activities, including drug discovery efforts, preclinical and clinical development of our therapeutic candidates and manufacturing.

External costs include:

- external R&D costs incurred with the development of our therapeutic candidates under agreements with third parties, such as contract research organizations ("CROs"), consultants, advisors, third party manufacturers and other third parties to conduct and support our clinical trials, preclinical studies and manufacturing; and
- costs associated with required regulatory filings, compliance and fees.

Internal costs include:

- personnel-related costs, including salaries, bonuses, benefits, travel and stock-based compensation expenses for personnel engaged in R&D functions; and
- costs related to general overhead expenses such as allocated facilities, depreciation, insurance and other allocated expenses, including rent expenses, lab supplies and equipment associated with our R&D activities.

We expense R&D costs as incurred. We account for non-refundable advance payments for goods and services that will be used in future R&D activities as an expense when the service has been performed or when the goods have been received.

We track external costs by therapeutic candidate. We do not track internal costs separately for each of our therapeutic candidates because these costs are associated with multiple programs and, as such, are not separately classified.

Although R&D activities are central to our business model, the successful development of our current and any future therapeutic candidates is highly uncertain. The timelines and costs associated with R&D activities are uncertain, can vary significantly for each therapeutic candidate and are difficult to predict. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to preclinical and clinical results, regulatory developments,

ongoing assessments as to each program's commercial potential, and our ability to maintain or enter into new collaborations, to the extent we determine the resources or expertise of a collaborator would be beneficial for any given program.

There are numerous factors associated with the successful development of any product, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. In addition, future regulatory factors beyond our control may impact our clinical development programs. Therapeutic candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect our R&D expenses to increase substantially for the foreseeable future in connection with our ongoing and planned clinical trials, as well as preclinical development activities. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any current and future therapeutic candidates.

Our future R&D expenses may vary significantly based on a wide variety of factors, such as:

- the initiation, type, number, scope, progress, expansions, results, costs and timing of clinical trials and preclinical studies of our therapeutic candidates and any future therapeutic candidates we may choose to pursue, including the costs of modification to clinical development plans based on feedback that we may receive from regulatory authorities and any third-party products used as combination agents in our clinical trials;
- per patient trial costs;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of patients that participate in the trials;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- the potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the cost and timing of manufacturing our therapeutic candidates;
- the phase of development of our therapeutic candidates;
- the efficacy and safety profile of our therapeutic candidates;
- the timing, receipt and terms of any approvals from applicable regulatory authorities; and
- the extent to which we establish additional collaboration, license, or other arrangements.

A change in the outcome of any of these variables with respect to the development of our therapeutic candidates may significantly impact the cost and timing associated with the development of our therapeutic candidates. We may never succeed in obtaining regulatory approval for any of our therapeutic candidates or successfully commercialize our products, even if approved.

General and administrative

General and administrative expenses consist primarily of personnel-related costs, including salaries, bonuses, benefits, travel and stock-based compensation expenses for personnel engaged in executive, accounting and finance, business development, legal and other administrative functions. Other significant costs include legal fees relating to intellectual property and corporate matters, professional fees for accounting, auditing, consulting and tax services, insurance, facility costs not otherwise included in R&D expenses and non-income taxes.

We expect our general and administrative expenses to increase substantially for the foreseeable future as we continue to increase our headcount to support our operations, including to support our continued R&D activities and preparing for potential commercialization. We also expect to incur increased expenses related to audit, legal, regulatory and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance premiums and investor relations costs associated

with operating as a public company. In addition, if we obtain regulatory approval for any product candidate, we expect to incur expenses associated with building the infrastructure and capabilities to commercialize such product. However, the timing of any such approval is highly uncertain, and it may be several years, if ever, that we receive any such regulatory approval.

Other income, net

Other income, net consists of (i) interest income and (ii) other income.

Interest income

Interest income consists primarily of accretion and interest income on available-for-sale marketable debt securities.

Other income

Other income consists primarily of realized gains and losses on available-for-sale marketable debt securities, unrealized gains and losses on foreign currency transactions, and dividend income.

Results of operations

Comparison of the three months ended September 30, 2026 and 2025

The following table sets forth our condensed consolidated results of operations for the periods presented (in thousands, except percentages):

	Three Months Ended September 30,			
	2026	2025	$ Change	% Change
	(unaudited)			
Revenue:				
Collaboration revenue	$ 2,217	$ 1,555	$ 662	43 %
Operating expenses:				
Research and development	26,713	19,416	7,297	38 %
General and administrative	7,681	5,146	2,535	49 %
Total operating expenses	34,394	24,562	9,832	40 %
Loss from operations	(32,177)	(23,007)	(9,170)	40 %
Other income, net:				
Interest income	3,479	4,622	(1,143)	(25)%
Other income	581	502	79	16 %
Total other income, net	4,060	5,124	(1,064)	(21)%
Loss before income tax	(28,117)	(17,883)	(10,234)	57 %
Net loss	$ (28,117)	$ (17,883)	$ (10,234)	57 %

Collaboration revenue

Collaboration revenue was $2.2 million and $1.6 million for the three months ended September 30, 2026 and 2025, respectively. The increase of $0.6 million was due to our performance of research and development services under the AbbVie Agreement. For additional information on the AbbVie Agreement and the accounting thereunder, see Note 7 to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

Research and development expenses

The following table sets forth our research and development expenses for the periods presented (in thousands):

	Three Months Ended September 30,	
	2026	2025
	(unaudited)	
External research and development costs:		
Agazisiran	$ 3,780	$ 2,967
Onvuzosiran	6,214	6,035
ADX-626	1,300	726
Preclinical and other costs	8,353	2,717
Total external research and development costs	19,647	12,445
Internal research and development costs:		
Personnel-related	4,179	3,670
Other	2,887	3,301
Total internal research and development costs	7,066	6,971
Total research and development expense	$ 26,713	$ 19,416

Research and development expenses were $26.7 million and $19.4 million for the three months ended September 30, 2026 and 2025, respectively. The increase of $7.3 million was primarily due to an increase of $1.6 million in external research and development expenses attributable to therapeutic candidates, an increase of $5.6 million in preclinical and other external research and development expenses, and an increase of $0.5 million in personnel-related expenses, partially offset by a decrease of $0.4 million in other internal research and development expenses.

General and administrative expenses

General and administrative expenses were $7.7 million and $5.1 million for the three months ended September 30, 2026 and 2025, respectively. The increase of $2.6 million was primarily due to an increase of $1.2 million in personnel-related costs, an increase of $0.7 million in professional fees and other expenses, an increase of $0.2 million in other general and administrative-related costs, and an increase of $0.5 million in stock-based compensation expenses.

Interest income

Interest income was $3.5 million and $4.6 million for the three months ended September 30, 2026 and 2025, respectively. The decrease of $1.1 million was primarily due to lower average cash balances held in short-term investments during the three months ended September 30, 2026 compared to the three months ended September 30, 2025.

Other income

Other income was $0.6 million and $0.5 million for the three months ended September 30, 2026 and 2025, respectively. The increase of $0.1 million was primarily due to an increase in dividend income on money market funds.

Comparison of the nine months ended September 30, 2026 and 2025

The following table sets forth our condensed consolidated results of operations for the periods presented (in thousands, except percentages):

	Nine Months Ended September 30,			
	2026	2025	$ Change	% Change
	(unaudited)			
Revenue:				
Collaboration revenue	$ 5,103	$ 1,763	$ 3,340	189 %
Operating expenses:				
Research and development	75,032	49,381	25,651	52 %
General and administrative	19,195	14,399	4,796	33 %
Total operating expenses	94,227	63,780	30,447	48 %
Loss from operations	(89,124)	(62,017)	(27,107)	44 %
Other income, net:				
Interest income	11,234	9,363	1,871	20 %
Other income	1,347	1,127	220	20 %
Total other income, net	12,581	10,490	2,091	20 %
Loss before income tax	(76,543)	(51,527)	(25,016)	49 %
Net loss	$ (76,543)	$ (51,527)	$ (25,016)	49 %

Collaboration revenue

Collaboration revenue was $5.1 million and $1.8 million for the nine months ended September 30, 2026 and 2025, respectively. The increase of $3.3 million was due to our performance of research and development services under the AbbVie Agreement. For additional information on the AbbVie Agreement and the accounting thereunder, see Note 7 to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

Research and development expenses

The following table sets forth our research and development expenses for the periods presented (in thousands):

	Nine Months Ended September 30,	
	2026	2025
	(unaudited)	
External research and development costs:		
Agazisiran	$ 13,797	$ 7,198
Onvuzosiran	17,179	13,217
ADX-626	4,296	2,608
Preclinical and other costs	18,156	6,777
Total external research and development costs	53,428	29,800
Internal research and development costs:		
Personnel-related	12,546	9,808
Other	9,058	9,773
Total internal research and development costs	21,604	19,581
Total research and development expense	$ 75,032	$ 49,381

Research and development expenses were $75.0 million and $49.4 million for the nine months ended September 30, 2026 and 2025, respectively. The increase of $25.6 million was primarily due to an increase of $12.2 million in external research and development expenses attributable to therapeutic candidates, an increase of $11.4 million in preclinical and other external research and development expenses, and an increase of $2.7 million in personnel-related expenses, partially offset by a decrease of $0.7 million in other internal research and development expenses.

General and administrative expenses

General and administrative expenses were $19.2 million and $14.4 million for the nine months ended September 30, 2026 and 2025, respectively. The increase of $4.8 million was primarily due to an increase of $2.4 million in personnel-related costs, an increase of $2.1 million in professional fees and other expenses and an increase of $0.4 million in other general and administrative-related costs, partially offset by a decrease of less than $0.1 million in stock-based compensation expenses.

Interest income

Interest income was $11.2 million and $9.4 million for the nine months ended September 30, 2026 and 2025, respectively. The increase of $1.8 million was primarily due to higher average cash balances held in short-term investments during the period.

Other income

Other income was $1.3 million and $1.1 million for the nine months ended September 30, 2026 and 2025, respectively. The increase of $0.2 million is primarily due to an increase in dividend income on money market funds.

Liquidity and capital resources

Sources of liquidity

Since our inception, we have primarily funded our operations through the sale of shares of our convertible preferred stock and convertible notes, payments received under the AbbVie Agreement, and more recently, our IPO and the concurrent private placement. We have not generated any revenue from product sales, and our revenue to date has consisted solely of amounts for research and development services under the AbbVie Agreement. We have incurred significant operating losses and negative cash flows from operations since our inception.

On September 28, 2026, we closed our IPO and issued 26,250,000 shares of our common stock at a price of $17.00 per share for net proceeds of approximately $415.0 million, after deducting underwriting discounts and commissions of $31.2 million. Concurrently with the closing of our IPO, we sold 5,255,542 shares of our common stock at a price of $17.00 per share in a concurrent private placement, for net proceeds of approximately $87.6 million, after deducting placement agent fees of $1.8 million which were unpaid as of September 30, 2026.

As of September 30, 2026, we had cash, cash equivalents and short-term investments of $901.0 million. On October 1, 2026, we received net proceeds of approximately $62.3 million from the issuance and sale of an additional 3,937,500 shares of common stock pursuant to the underwriters' exercise in full of their option to purchase additional shares.

Future funding requirements

Based on our current operating plans, we estimate that our existing cash, cash equivalents and short-term investments as of September 30, 2026, together with the net proceeds received in October 2026 from the exercise in full of the option granted to the underwriters of our IPO to purchase additional shares of our common stock, will be sufficient to fund our projected operating expenses and capital expenditure requirements into 2030. However, this estimate is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. We have based this estimate on assumptions that may prove to be incorrect, and we could use our available capital resources sooner than anticipated.

We have incurred significant operating losses since our inception. As of September 30, 2026, we had an accumulated deficit of $297.2 million. Substantially all of our net losses have resulted from costs related to our R&D programs, and to a lesser extent, from general and administrative expenses. As mentioned above, we entered into the AbbVie Agreement in May 2025 and recorded $335.0 million in deferred revenue from the receipt of a related upfront payment. However, the recognition of this deferred revenue is expected to occur in future periods and is not anticipated to outweigh potential operating losses. As such, we expect to continue incurring significant expenses and operating losses for the foreseeable future, and we anticipate these losses will increase substantially as we continue our development of, seek regulatory approval for and potentially commercialize our therapeutic candidates, seek to discover and develop additional therapeutic candidates, utilize third party manufacturers, hire additional personnel, expand and protect our intellectual property portfolio and incur additional costs associated with being a public company. If we obtain regulatory approval for our therapeutic candidates, we anticipate incurring significant expenses related to developing our commercialization capabilities for product sales, marketing and distribution. Our net losses may fluctuate significantly from quarter to quarter and year to year, depending on the timing of our clinical trials, preclinical studies and other R&D activities and capital expenditures.

Our future capital requirements will depend on many factors, including:

- the initiation, type, number, scope, progress, expansions, results, costs and timing of clinical trials and preclinical studies of our therapeutic candidates and any future therapeutic candidates we may choose to pursue, including the costs of modification to clinical development plans based on feedback that we may receive from regulatory authorities and any third-party products used as combination agents in our clinical trials;
- the costs and timing of manufacturing for our therapeutic candidates and commercial manufacturing if any therapeutic candidate is approved;
- the costs and timing of establishing or securing sales and marketing capabilities if any therapeutic candidate is approved;
- our ability to achieve sufficient market acceptance, coverage and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- our research and development costs and the receipt of milestone and other payments under the AbbVie Agreement;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of any such arrangements, including the timing and amount of any future milestone, royalty or other payments due under any such agreement;
- the costs associated with hiring and retaining skilled personnel and consultants as our preclinical and clinical activities increase;
- our investments in our operational, financial and management information systems;
- the costs associated with operating as a public company;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing our intellectual property portfolio; and
- costs associated with any products or technologies that we may in-license or acquire.

We do not have any products approved for sale and have not generated any product revenue to date. We do not expect to generate any revenue from product sales until we successfully complete development and obtain regulatory approval for one or more of our therapeutic candidates or any future therapeutic candidates. This process is expected to take several years and may never occur. Consequently, we will need substantial additional funding, beyond the net proceeds from the IPO, to support our ongoing operations and pursue our business plan. Until such time we can generate significant revenue from product sales, if ever, we expect to finance our operations through public or private equity or debt financings or other capital sources, which include existing strategic collaborations and may include other potential strategic arrangements with third parties. However, we may not be able to secure additional financing or enter into such other strategic arrangements in a timely manner or on favorable terms, if at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise funds through collaboration or licensing arrangements with third parties or other strategic transactions, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs, or therapeutic candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. If we are unable to raise additional funds or enter into such arrangements when needed, we could be forced to delay, limit, reduce or terminate our R&D programs or future commercialization efforts, or grant rights to develop and market our therapeutic candidates even if we would otherwise prefer to develop and market such therapeutic candidates ourselves.

Cash flows

The following table summarizes our cash flows for the periods presented (in thousands):

	Nine Months Ended September 30,	
	2026	2025
	(unaudited)	
Net cash (used in) provided by operating activities	$ (76,748)	$ 275,118
Net cash provided by (used in) investing activities	78,608	(230,077)
Net cash provided by financing activities	502,098	91
Effect of exchange rate changes on cash and cash equivalents	135	11
Net increase in cash and cash equivalents	$ 504,093	$ 45,143

Operating activities

Net cash used in operating activities was $76.7 million for the nine months ended September 30, 2026, primarily due to a net loss of $76.5 million and accretion of short-term investments of $0.4 million. This was partially offset by noncash charges for stock-based compensation of $4.3 million, depreciation expense of $0.9 million and amortization of right-of-use assets of $1.3 million. Changes in our operating assets and liabilities were $6.3 million, which primarily consisted of a decrease in deferred revenue of $5.1 million, an increase in prepaid expenses and other current assets of $4.1 million and a decrease in operating lease liabilities of $1.3 million, partially offset by a decrease in other non-current assets of $2.1 million, an increase in accounts payable of $2.0 million, and an increase in accrued expenses and other current liabilities of $0.1 million.

Net cash provided by operating activities was $275.1 million for the nine months ended September 30, 2025, primarily due to changes in our operating assets and liabilities of $323.0 million, partially offset by our net loss of $51.5 million. The changes in our operating assets and liabilities primarily consisted of an increase in deferred revenue of $333.2 million related to the upfront payment received under the AbbVie Agreement and an increase in accounts payable of $1.8 million, partially offset by an increase in prepaid expenses and other current assets of $9.4 million, an increase in other non-current assets of $1.5 million, a decrease in operating lease liabilities of $0.9 million, and a decrease in accrued expenses and other current liabilities of $0.2 million. Our net loss included noncash charges for stock-based compensation of $4.3 million, amortization of right-of-use assets of $1.1 million, and depreciation expense of $0.7 million, partially offset by accretion on investments of $2.5 million.

Investing activities

Net cash provided by investing activities was $78.6 million for the nine months ended September 30, 2026, primarily due to proceeds from the maturity of short-term investments of $131.0 million, partially offset by purchases of short-term investments of $51.8 million and purchases of property and equipment of $0.6 million.

Net cash used in investing activities was $230.1 million for the nine months ended September 30, 2025, primarily due to purchases of short-term investments of $369.9 million and purchases of property and equipment of $2.2 million, partially offset by proceeds from the maturity of short-term investments of $142.0 million.

Financing activities

Net cash provided by financing activities for the nine months ended September 30, 2026 was $502.1 million, consisting of net proceeds of $415.0 million from our IPO, gross proceeds of $89.3 million from the concurrent private placement, and proceeds from stock option exercises of $0.5 million, partially offset by payments of deferred offering costs of $2.7 million related to the IPO.

Cash flows from financing activities were immaterial for the nine months ended September 30, 2025.

Contractual obligations and other commitments

In March 2024, we entered into a non-cancelable lease amendment to extend the lease term of the existing office and laboratory space and to expand the original leased premises. The amendment commenced on February 1, 2025, and includes additional minimum lease payments aggregating to $5.4 million, as well as charges for common area maintenance and property taxes. As of September 30, 2026, total minimum lease payments for all our leases were $7.4 million. For additional information, see Note 7 of our audited consolidated financial statements for the year ended December 31, 2025 included in the Prospectus.

During the normal course of our business, we enter into contracts for research and professional services, and for the purchase of lab supplies used in our research activities. These contracts generally provide for termination after a notice period, and, therefore, are cancelable contracts and not separately presented. Since our inception, we have not had any off-balance sheet arrangements as defined under the rules and regulations of the SEC.

Recent accounting pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position, results of operations and cash flows is included in Note 2 to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

Critical accounting policies and estimates

Our unaudited condensed consolidated financial statements and the related notes included elsewhere in this Quarterly Report on Form 10-Q are prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make estimates and assumptions that impact the reported amounts of assets, liabilities, expenses and related disclosures. We continually evaluate these estimates and assumptions, basing them on historical experience and various other factors we consider reasonable under the circumstances. Actual results may differ from these estimates due to different assumptions or conditions.

There have been no material changes to our critical accounting policies and estimates from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations - Critical Accounting Policies and Estimates" which are included in the Prospectus.

Emerging growth company and smaller reporting company status

As an emerging growth company under the JOBS Act, we can take advantage of an extended transition period for complying with new or revised accounting standards. We have elected to avail ourselves of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we can adopt the new or revised standard at the time private companies adopt the new or revised standard and may do so until such time that we either irrevocably elect to opt out of such extended transition period or no longer qualify as an emerging growth company. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

Subject to certain conditions set forth in the JOBS Act, if, as an "emerging growth company," we choose to rely on such exemptions we may not be required to, among other things, (i) provide an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act, (ii) provide all of the compensation disclosure that may be required of non-emerging growth public companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act, (iii) comply with any requirement that may be adopted by the Public Company Accounting Oversight Board ("PCAOB") regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the consolidated financial statements (auditor discussion and analysis), or (iv) disclose certain executive compensation related items such as the correlation between executive compensation and performance and comparisons of the CEO's compensation to median employee compensation. We will continue to remain an emerging growth company until the earliest of the following: (i) the last day of the fiscal year following the fifth anniversary of the date of the completion of the IPO, or December 31, 2031; (ii) the last day of the fiscal year in which our total annual gross revenue is equal to or more than $1.235 billion; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer as defined in Rule 12b-2 under the Exchange Act.

We are also a smaller reporting company as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company, as defined by Rule 12b-2 under the Exchange Act and in Item 10(f)(1) of Regulation S-K, and are not required to provide the information under this item.

Item 4. Controls and Procedures.

Management's Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act, is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Our disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file under the Exchange Act is accumulated and communicated

to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating the disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. As required by Rule 13a-15(b) or Rule 15d-15(b) promulgated by the SEC under the Exchange Act, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the period covered by this Quarterly Report on Form 10-Q. Based on the foregoing, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective as of the end of the period covered by this Quarterly Report on Form 10-Q at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended September 30, 2026 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.

From time to time, we may become involved in litigation or other legal proceedings. As of the date of this Quarterly Report on Form 10-Q, we are not a party to any litigation or legal proceedings that, in the opinion of our management, are likely to have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below, together with all of the other information in this Quarterly Report on Form 10-Q, including our unaudited condensed consolidated financial statements and related notes and the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" in Part I, Item 2 of this Quarterly Report on Form 10-Q before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could materially and adversely affect our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline, and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently believe are not material also may impair our business operations.

Summary of risk factors

Our business is subject to a number of risks that you should carefully consider before making a decision to invest in our common stock. These risks are more fully described below and include, among others, the following:

- We have a limited operating history, have not completed any clinical programs and have no products approved for commercial sale, which may make it difficult for you to evaluate our current business and predict our future success and viability.
- We expect to incur significant losses for the foreseeable future and may never achieve or maintain profitability.
- We will need to obtain substantial additional funding to complete the development and any commercialization of our current and any future therapeutic candidates, which may cause dilution to our stockholders. If we are unable to raise this capital when needed, we may be forced to delay, reduce or eliminate our research and development programs or other operations.
- If we are unable to successfully develop, obtain regulatory approval for, and ultimately commercialize our therapeutic candidates, or experience significant delays in doing so, our business will be materially harmed.
- Interim, topline and preliminary data from our clinical trials and preclinical studies that we announce or publish from time to time may not be predictive of future results and may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.
- Preclinical and clinical development involves a lengthy and expensive process with an uncertain outcome, and the results of preclinical studies and early clinical trials are not necessarily predictive of future results.
- Certain of our programs target rare diseases, which have low prevalence, and it may be difficult to identify and enroll patients with these diseases.
- Our approach to the discovery and development of therapeutic candidates is unproven, and we do not know whether we will be able to develop any products of commercial value.
- Use of our therapeutic candidates could be associated with side effects, adverse events or other properties or safety risks, which could delay or preclude approval, cause us to suspend or discontinue clinical trials, abandon a therapeutic candidate, limit the commercial profile of an approved label or result in other significant negative consequences.
- As an organization, we have never completed any pivotal clinical trials or submitted an NDA for regulatory approval and may be unable to do so for any of our therapeutic candidates.
- Our therapeutic candidates are subject to extensive regulation and compliance, which is costly and time consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize our therapeutic candidates.
- We have conducted and will continue to conduct certain of our clinical trials for our therapeutic candidates outside of the United States. However, the FDA and other foreign equivalents may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

- We rely on third parties to conduct our preclinical studies and clinical trials and for the manufacture of our therapeutic candidates. If these third parties do not successfully carry out their contractual duties, comply with applicable regulatory requirements or meet expected deadlines, our development programs and our ability to seek or obtain regulatory approval for or commercialize our therapeutic candidates may be delayed or prevented.
- We have and in the future may seek to enter into additional collaborations, licenses and other similar arrangements and may not be successful in establishing or maintaining such collaborations, and even if we are, we may relinquish valuable rights and may not realize the benefits of such relationships.
- We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.
- Even if we receive regulatory approval for any therapeutic candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. Additionally, our therapeutic candidates, if approved, could be subject to labeling and other restrictions on marketing or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our therapeutic candidates, when and if any of them are approved.
- The successful commercialization of our therapeutic candidates, if approved, will depend in part on the extent to which governmental authorities and health insurers establish coverage, adequate reimbursement levels and favorable pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our products could limit our ability to market those products and decrease our ability to generate revenue.
- We currently have no marketing and sales organization and have no experience as a company in commercializing products, and we may have to invest significant resources to develop these capabilities. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our products, we may not be able to generate product revenue.
- If we are unable to obtain and maintain patent protection for our technology and therapeutic candidates or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.
- Patent terms may be inadequate to protect our competitive position on our therapeutic candidates for an adequate amount of time.
- We are subject to various federal, state and foreign healthcare laws and regulations, which could increase compliance costs, and our failure to comply with these laws and regulations could harm our results of operations and financial condition.
- We will need to expand our organization, and we may experience challenges in managing this growth as we build our capabilities, which could disrupt our operations.
- Our future success depends on our ability to retain our key personnel and to attract, retain and motivate qualified personnel.

Risks related to our limited operating history, financial position and need for additional capital

We have a limited operating history, have not completed any clinical programs and have no products approved for commercial sale, which may make it difficult for you to evaluate our current business and predict our future success and viability.

We are a late-clinical stage biotechnology company with a limited operating history. We were incorporated in December 2019, and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, developing our technologies, establishing and maintaining our intellectual property portfolio, identifying and developing our therapeutic candidates, preparing for, initiating and conducting our ongoing and planned preclinical studies and clinical trials, establishing and maintaining arrangements with third parties for the manufacturing of our therapeutic candidates and providing general and administrative support for these operations.

Our therapeutic candidates are in various stages of development, with three programs in clinical trials (agazisiran, onvuzosiran, and ADX-626) and two advanced preclinical programs. We have not yet demonstrated an ability to successfully complete clinical programs, including large-scale, pivotal clinical trials, obtain marketing approval, manufacture therapeutics at a commercial scale or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. This may make it difficult to evaluate the success of our business to date and assess our future viability.

We expect to incur significant losses for the foreseeable future and may never achieve or maintain profitability.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and the significant risk that therapeutic candidates will fail to demonstrate adequate efficacy or an acceptable safety profile, gain regulatory approval or become commercially viable. For the nine months ended September 30, 2026 and 2025, our net losses were $76.5 million and $51.5 million, respectively. As of September 30, 2026, we had an accumulated deficit of $297.2 million. Substantially all

of our losses have resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We expect to continue to incur significant expenses and additional operating losses for the foreseeable future as we seek to advance our therapeutic candidates through preclinical and clinical development, expand our research and development activities, develop new therapeutic candidates, complete clinical trials, seek regulatory approval and, if we receive regulatory approval, commercialize our products. Furthermore, the costs of advancing therapeutic candidates into each succeeding clinical phase tend to increase substantially over time. The total costs to advance any of our therapeutic candidates to regulatory approval in even a single jurisdiction would be substantial. Because of the numerous risks and uncertainties associated with biopharmaceutical product development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will be able to generate any revenue from the commercialization of any approved products or achieve or maintain profitability. Our expenses will also increase substantially as we operate as a public company and add clinical, scientific, operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Before we generate any revenue from product sales, each of our therapeutic candidates will require additional preclinical and/or clinical development, potential regulatory approval in multiple jurisdictions, manufacturing, building of a commercial organization, substantial investment and significant marketing efforts. Our expenses could increase beyond expectations if we are required by the FDA, the European Medicines Agency ("EMA"), the competent authorities of European Union ("EU") Member States, or comparable foreign regulatory authorities to perform preclinical studies and clinical trials in addition to those that we currently anticipate, and/or to modify any of our manufacturing processes or make other changes to our therapeutic candidates or development programs. As a result, we expect to continue to incur net losses and negative cash flows for the foreseeable future. These net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

As we continue to build our business, we expect our financial condition and operating results may fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any particular quarterly or annual period as indications of future operating performance. If we are unable to develop and commercialize one or more of our therapeutic candidates either alone or with collaborators, or if revenues from any therapeutic candidate that receives regulatory approval are insufficient, we will not achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability. If we are unable to achieve and then maintain profitability, the value of our securities will be adversely affected.

We will need to obtain substantial additional funding to complete the development and any commercialization of our current and any future therapeutic candidates, which may cause dilution to our stockholders. If we are unable to raise this capital when needed, we may be forced to delay, reduce or eliminate our research and development programs or other operations.

The development of biopharmaceutical therapeutic candidates is capital-intensive. We expect to spend substantial amounts to advance our therapeutic candidates into clinical development, to complete the clinical development of, and seek regulatory approvals for and commercialize our therapeutic candidates, if approved. We will require additional capital which we may raise through public or private equity or debt financings or other capital sources, which may include strategic collaborations and other strategic arrangements with third parties, to enable us to complete the development and potential commercialization of our therapeutic candidates. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative effect on our financial condition and our ability to pursue our business strategy. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities and harm our development efforts. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Our operations have consumed significant amounts of cash since inception. As of September 30, 2026, our cash and cash equivalents and short-term investments were $901.0 million, which we believe, together with the net proceeds of approximately $62.3 million received in October 2026 from the issuance and sale of an additional 3,937,500 shares of common stock pursuant to the underwriters' exercise in full of their option to purchase additional shares, will be sufficient to fund our projected operating expenses and capital expenditure requirements into 2030. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances could cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more than currently expected because of circumstances beyond our control. Because the length of time and activities associated with successful development of our therapeutic candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any marketing and commercialization activities.

Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the type, number, scope, progress, expansions, results, costs and timing of, discovery, preclinical studies and clinical trials of our current and future therapeutic candidates;
- the costs and timing of manufacturing for our current and future therapeutic candidates;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA or other comparable foreign regulatory authorities;
- the cost of obtaining, maintaining and protecting our intellectual property portfolio, including filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost associated with maintaining and enhancing our proprietary technologies;
- the cost of establishing a sales, marketing and distribution infrastructure to commercialize any therapeutic candidates for which we may obtain regulatory approval;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the cost of making royalty, milestone or other payments under any future in-license agreements;
- costs associated with growing our workforce and retaining and motivating our employees;
- the timing and amount of the milestone or other payments made to us under any current or future collaboration agreements;
- the initiation, progress, timing and results of our commercialization of our therapeutic candidates, if approved for commercial sale;
- costs associated with any products or technologies that we may in-license or acquire;
- the costs associated with being a public company; and
- our implementation of additional internal systems and infrastructure, including operational, financial and management information systems.

To the extent that we raise additional capital through the sale of our common stock, convertible securities or other equity securities, your ownership interest may be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. In addition, securing financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities and harm our development, manufacturing and commercialization efforts. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or therapeutic 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 or other arrangements 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 therapeutic candidates that we would otherwise prefer to develop and market ourselves.

Risks related to the discovery, development and regulatory approval of our therapeutic candidates

We currently have three therapeutic candidates in clinical development. All of our other development programs are in the preclinical or discovery stage. If we are unable to successfully develop, obtain regulatory approval for, and ultimately commercialize our therapeutic candidates, or experience significant delays in doing so, our business will be materially harmed.

We currently have three therapeutic candidates in clinical development. All of our other development programs are in the preclinical or drug discovery stage. We have invested substantially all of our efforts in developing our proprietary mRNA Silencing Technology ("MST") and Cell Targeted Delivery ("CTD") technologies, identifying potential therapeutic candidates and conducting preclinical studies and clinical trials. We are progressing our preclinical-stage therapeutic candidates through IND-enabling studies and will need to receive authorization from the FDA and other comparable foreign regulatory authorities, to proceed under an IND application, or its equivalent, prior to initiating their clinical development. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our therapeutic candidates. The success of our therapeutic candidates will depend on several factors, including the following:

- successful enrollment in clinical trials and completion of clinical trials with favorable results;

- successful completion of preclinical studies with favorable results, including those compliant with good laboratory practices ("GLP"), toxicology studies, biodistribution studies and minimum effective dose studies;
- allowance to proceed with clinical trials under INDs by the FDA, or under similar regulatory submissions by comparable foreign regulatory authorities for the conduct of clinical trials of our therapeutic candidates and our proposed design of future clinical trials;
- demonstrating safety and efficacy of our therapeutic candidates to the satisfaction of the FDA and other applicable regulatory authorities;
- receipt of regulatory approvals from applicable regulatory authorities, including NDAs from the FDA and maintaining such approvals;
- making arrangements with our third-party manufacturers for, or establishing manufacturing capabilities to manufacture sufficient quantities of our therapeutic candidates for clinical and, if approved, commercial use;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- establishing and maintaining patent and trade secret protection and regulatory exclusivity for our therapeutic candidates;
- maintaining an acceptable safety profile for any of our products following approval; and
- maintaining and growing an organization of people who can develop and commercialize our products and technology.

If we are unable to develop, obtain regulatory approval for, or, if approved, successfully commercialize our therapeutic candidates, we may not be able to generate sufficient revenue to continue our business.

Interim, topline and preliminary data from our clinical trials and preclinical studies that we announce or publish from time to time may not be predictive of future results and may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose interim, topline or preliminary data from our clinical trials and preclinical studies, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. Interim, topline and preliminary data from clinical trials that we may complete are also subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments for their disease. As a result, the interim, topline or preliminary results that we report may differ from future results of the same studies or trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Interim, topline and preliminary data further remain subject to audit and verification procedures that may result in the final data being materially different from the data we previously published. As a result, interim, topline and preliminary data should be viewed with caution until the final data are available. Adverse differences between interim, topline or preliminary data and final data could significantly harm our business prospects.

Further, others, including regulatory authorities, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular therapeutic candidate or product and the value of our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product, therapeutic candidate or our business. If the interim, topline or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our therapeutic candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Preclinical and clinical development involves a lengthy and expensive process with an uncertain outcome, and the results of preclinical studies and early clinical trials are not necessarily predictive of future results. Our therapeutic candidates may not have favorable results in clinical trials or preclinical studies or receive regulatory approval on a timely basis, if at all.

Preclinical and clinical development is expensive and can take many years to complete, and its outcome is inherently uncertain. We cannot guarantee that any clinical trials or preclinical studies will be conducted as planned or completed on schedule, if at all, and failure can occur at any time during the preclinical study or clinical trial process. For example, we may not be able to meet expected timeframes

for data readouts from our clinical trials. Despite promising clinical or preclinical results, any therapeutic candidate can unexpectedly fail at any stage of preclinical or clinical development. The historical failure rate for therapeutic candidates in our industry is high.

The results from clinical trials or preclinical studies of a therapeutic candidate may not predict the results of later clinical trials of the therapeutic candidate, and interim, topline, or preliminary results of a clinical trial are not necessarily indicative of final results. Therapeutic candidates in later stages of clinical trials may fail to show the desired safety and efficacy characteristics despite having progressed through preclinical studies and initial clinical trials. The positive results we have observed for our therapeutic candidates in preclinical animal models, and in certain therapeutic candidates, clinical trials, may not be predictive of our ongoing and future clinical trials in humans. Conversely, our determination that rat is not an appropriate species for the evaluation of the long-term safety of onvuzosiran may be incorrect, and the off-target effects and lack of pharmacologic activity that we observed in our preclinical rat studies may prove to be relevant to our ongoing and future onvuzosiran clinical trials. We continue to engage with the FDA regarding our nonclinical program for onvuzosiran, including the relevance of the rat as a species for evaluating its long-term safety. It is not uncommon to observe results in clinical trials that are unexpected based on preclinical studies and early clinical trials, and many therapeutic candidates fail in clinical trials despite very promising early results. Unexpected observations or toxicities observed in these studies, or in IND-enabling studies for any of our development programs, will delay clinical trials for such development programs. Moreover, preclinical and clinical data may be susceptible to varying interpretations and analyses. A number of companies in the biopharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving promising results in earlier studies. Such setbacks have occurred and may occur for many reasons, including, but not limited to: clinical sites and investigators may deviate from clinical trial protocols, whether due to lack of training or otherwise, and we may fail to detect any such deviations in a timely manner; patients may fail to adhere to any required clinical trial procedures, including any requirements for post-treatment follow-up; our therapeutic candidates may fail to demonstrate effectiveness or safety in certain patient subpopulations, which has not been observed in earlier trials due to limited sample size, lack of analysis, or otherwise; or our clinical trials may not adequately represent the patient populations we intend to treat, whether due to limitations in our trial designs or otherwise, such as where one patient subgroup is overrepresented in the clinical trial. There can be no assurance that we will not suffer similar setbacks despite the data we observed in earlier or ongoing studies. Based upon negative or inconclusive results, we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials, which would cause us to incur additional operating expenses and delays and may not be sufficient to support regulatory approval on a timely basis or at all. For the foregoing reasons, we cannot be certain that our ongoing and planned preclinical studies and planned clinical trials will be successful. Further, any safety concerns observed in any of our preclinical studies or clinical trials in our targeted indications could limit the prospects for regulatory approval of our therapeutic candidates in those and other indications, which could have a material adverse effect on our business, financial condition and results of operations.

Any difficulties or delays in the commencement or completion, or the termination or suspension, of our current or planned clinical trials could result in increased costs to us, or delay or limit our ability to generate revenue and adversely affect our commercial prospects.

Before we can initiate clinical trials for a therapeutic candidate, we must submit the results of preclinical studies to the FDA or comparable foreign regulatory authorities along with other information, including information about therapeutic candidate chemistry, manufacturing and controls and our proposed clinical trial protocol, as part of an IND or similar regulatory filing required for authorization or allowance to proceed with clinical development. The FDA or comparable foreign regulatory authorities may determine that our IND or comparable submission is inadequate or incomplete and place the proposed studies on a clinical hold, which is an order issued by the FDA or comparable foreign regulatory authorities to delay or suspend a clinical investigation. An IND and comparable submission may be deemed inadequate for a variety of reasons, including, but not limited to:

- deficiencies in preclinical data, including insufficient pharmacology or toxicology data to support the safety of initiating human trials, inadequate study design, failure to follow GLP standards, or lack of appropriate animal models;
- chemistry, manufacturing, and controls (“CMC”) issues, such as inadequate characterization of the drug substance or product, insufficient information regarding manufacturing processes, controls, or stability, or failure to demonstrate product quality and consistency;
- clinical protocol deficiencies, including flawed study design, inappropriate endpoints, inadequate statistical analysis plans, insufficient risk mitigation measures, or failure to adequately protect trial participants;
- investigator or site concerns, including lack of qualifications, inadequate monitoring plans, or concerns regarding compliance with good clinical practices (“GCP”);
- safety concerns, including unresolved toxicological signals, potential for serious adverse events, or inadequate safety monitoring procedures;

- inadequate prior human experience data, where available data do not sufficiently support the proposed dosing, route of administration, or patient population; and
- regulatory or administrative deficiencies, including incomplete submissions, failure to respond adequately to FDA information requests, or inconsistencies across submitted materials.

If the FDA or comparable foreign regulatory authorities place any of our studies on clinical hold, we may be required to generate additional data, revise our protocols, amend manufacturing processes, or address other FDA or comparable concerns before the hold is lifted. The process for resolving a clinical hold can be time-consuming, costly, and uncertain. In some cases, the FDA or comparable foreign regulatory authorities may require significant additional clinical work, which may not be feasible within our resources or timelines. There can be no assurance that we will be able to resolve any clinical hold in a timely manner, or at all. A prolonged or unresolved clinical hold could delay or prevent the development and commercialization of our therapeutic candidates, materially harm our business, and adversely affect our financial condition.

The FDA or comparable foreign regulatory authorities may require us to conduct additional preclinical studies for any therapeutic candidate before it allows us to initiate clinical trials under any IND or similar regulatory filing, which may lead to delays and increase the costs of our preclinical development programs.

Moreover, even if these trials begin, issues may arise that could cause regulatory authorities to suspend or terminate such clinical trials. Any delays in the commencement or completion of our ongoing and planned clinical trials for our current and any future therapeutic candidates could significantly affect our product development timelines and product development costs.

We do not know whether our planned trials will begin on time or if our ongoing or future clinical trials will be completed on schedule, if at all. The commencement, data readouts and completion of clinical trials can be delayed for a number of reasons, including delays related to:

- inability to obtain animals or materials to initiate and generate sufficient preclinical, toxicology, or other in vivo or in vitro data to support the initiation or continuation of clinical trials;
- obtaining regulatory authorizations or allowances to commence a trial or reaching a consensus with regulatory authorities on trial design;
- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical studies;
- any failure or delay in reaching an agreement with contract research organizations ("CROs") and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining approval from one or more institutional review boards ("IRBs"), or independent ethics committees;
- IRBs refusing to approve, suspending or terminating the trial at an investigational site, precluding enrollment of additional patients, or withdrawing their approval of the trial;
- changes to clinical trial protocol;
- clinical sites deviating from trial protocol or dropping out of a trial;
- manufacturing sufficient quantities of a therapeutic candidate for use in clinical trials;
- delays or failure to recruit suitable patients to participate in a clinical trial, or patients failing to enroll or remain in our trials at the rate we expect, or failing to return for post-treatment follow-up;
- patients choosing an alternative treatment for the indications for which we are developing our therapeutic candidates, or participating in competing clinical trials;
- lack of adequate funding to continue the clinical trial;
- patients experiencing severe or serious unexpected drug or non-drug related adverse effects;
- the serious, life-threatening diseases of the patients enrolled in our clinical trials, who may die or suffer adverse medical events during the course of the trials for reasons that may not be related to our therapeutic candidates;
- occurrence of serious adverse events in trials of the same class of agents conducted by other companies, or other third party developments that may impact the safety profile of our drugs;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;

- a facility manufacturing our therapeutic candidates or any of their components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current good manufacturing practice ("cGMP") and similar foreign requirements, regulations or other applicable requirements, or infections or cross-contaminations of therapeutic candidates in the manufacturing process;
- any changes to our manufacturing process that may be necessary or desired;
- drug and material shortages or other factors that may prevent our manufacturers from timely producing the necessary drug products in sufficient quantities;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, good clinical practices ("GCP"), or other regulatory requirements;
- third-party contractors not performing data collection or analysis in a compliant, timely or accurate manner (including in compliance with cGMP and similar foreign requirements); or
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications.

We could also encounter delays if a clinical trial is suspended or terminated by us, or by requirement or request of the IRBs or ethics committees of the institutions in which such trials are being conducted, by a Data Safety Monitoring Board for such trial or by the FDA or comparable foreign regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or comparable foreign regulatory authorities resulting in the imposition of a full or partial clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. In addition, changes in regulatory requirements and policies may occur at any time, and we may need to amend clinical trial protocols to comply with these changes. Amendments may require us to resubmit our clinical trial protocols to the FDA or comparable foreign regulatory authorities, or to IRBs and ethics committees for reexamination, which may impact the costs, timing, ability to proceed, or successful completion of a clinical trial.

Further, conducting clinical trials in foreign countries, as we currently do and may do in the future, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks, including war, relevant to such foreign countries. It is also possible that the FDA or other regulatory authorities may not accept the clinical data generated in research conducted in a foreign country.

If we experience delays in the completion of, or the termination of, any clinical trial of our therapeutic candidates, the commercial prospects of our therapeutic candidates will be harmed, and our ability to generate product revenues from any of these therapeutic candidates will be delayed. Moreover, any delays in completing our clinical trials will increase our costs, slow down our therapeutic candidate development and regulatory approval process and jeopardize our ability to commence product sales and generate revenues.

We may find it difficult to enroll patients in our clinical trials. Certain of our programs target rare diseases, such as IgAN, C3G, IC-MPGN, PNH and HAE, which have low prevalence, and it may be difficult to identify and enroll patients with these diseases. If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may not be able to initiate or continue clinical trials for our therapeutic candidates if we are unable to identify and enroll a sufficient number of eligible patients to participate in these trials as may be required by the FDA or similar regulatory authorities outside the United States. For example, we are developing agazisiran for the treatment of IgAN, C3G, IC-MPGN and PNH, among other complement-mediated diseases, and onvuzosiran for HAE attack prevention. IgAN, C3G, IC-MPGN, PNH and HAE are rare diseases with limited patient pools from which to draw. Subject enrollment, a significant factor in the timing of clinical trials, is affected by many factors including, among other things, the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility and exclusion criteria for the trial, the design of the clinical trial, the risk that enrolled patients will not complete a clinical trial, our ability to recruit clinical trial investigators with the appropriate competencies and experience, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages and risks of the therapeutic candidate being studied in relation to other available therapies, including any new products that may be approved for the indications we are investigating as well as any therapeutic candidates under development. In order to collect data sufficient to support a marketing authorization, we will be required to identify and enroll a sufficient number of patients for each of our clinical trials. Potential patients for any planned clinical trials may

not be adequately diagnosed or identified with the diseases which we are targeting or may not meet the entry criteria for such trials. We also may encounter difficulties in identifying and enrolling patients with a stage of disease appropriate for our planned clinical trials and monitoring such patients adequately during and after treatment. We may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible patients to participate in the clinical trials required by the FDA or comparable foreign regulatory authorities. In addition, the process of finding and diagnosing patients may prove costly.

Competing with numerous ongoing trials and established therapies poses a challenge in recruiting patients. Our clinical trials may also compete with other clinical trials of therapeutic candidates that target similar indications to our therapeutic candidates, and this competition could reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors.

The timing of our clinical trials depends, in part, on the speed at which we can recruit patients to participate in our trials, as well as completion of required follow-up periods. The eligibility criteria of our clinical trials, once established, will further limit the pool of available trial participants. If patients are unwilling to participate in our trials for any reason, including the existence of concurrent clinical trials for similar patient populations or the availability of approved therapies, or we otherwise have difficulty enrolling a sufficient number of patients, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of our therapeutic candidates may be delayed. Our inability to enroll a sufficient number of patients for any of our ongoing or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. In addition, we expect to rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will have limited influence over their actual performance.

We cannot assure you that our assumptions used in determining expected clinical trial timelines are correct or that we will not experience delays in enrollment, which would result in the delay of completion of such trials beyond our expected timelines.

Our approach to the discovery and development of therapeutic candidates based on our MST and CTD technology is unproven, and we do not know whether we will be able to develop any products of commercial value, or if competing technological approaches will limit the commercial value of our therapeutic candidates or render our technology obsolete.

The success of our business depends primarily upon our ability to identify, develop and commercialize products based on our proprietary MST and CTD technologies, which leverage a novel and unproven approach. While we have had encouraging preclinical and early clinical trial results based on our technologies, we have not yet succeeded and may not succeed in demonstrating safety and efficacy for any therapeutic candidates in clinical trials or in obtaining regulatory approval thereafter. Our research methodology and novel approach to siRNA therapy may be unsuccessful in identifying additional therapeutic candidates, and any therapeutic candidates based on our technologies may be shown to have harmful side effects or may have other characteristics that may necessitate additional clinical testing or make the therapeutic candidates unmarketable or unlikely to receive regulatory approval. Further, because all of our therapeutic candidates and development programs are based on our proprietary technologies and siRNA approach, adverse developments with respect to one of our programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other programs. In addition, certain of our therapeutic candidates target pathways that are unproven, such as our CFB program, agazisiran, which targets CFB in a pathway that, while it is validated in other diseases we are pursuing, is unproven for the treatment of GA secondary to AMD.

In addition, the biotechnology and biopharmaceutical industries are characterized by rapidly advancing technologies. Our future success will depend in part on our ability to maintain a competitive position with our siRNA approach. If we fail to stay at the forefront of technological change in utilizing our proprietary technologies to create and develop therapeutic candidates, we may be unable to compete effectively. Our competitors may render our siRNA approach obsolete, or limit the commercial value of our therapeutic candidates, by advances in existing technological approaches or the development of new or different approaches, potentially eliminating the advantages in our drug discovery process that we believe we derive from our research approach and proprietary technologies. Adverse developments with respect to other companies that attempt to use a similar approach to our approach may also adversely impact the actual or perceived value of our technologies and potential of our therapeutic candidates.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations.

Use of our therapeutic candidates could be associated with side effects, adverse events or other properties or safety risks, which could delay or preclude approval, cause us to suspend or discontinue clinical trials, abandon a therapeutic candidate, limit the commercial profile of an approved label or result in other significant negative consequences that could severely harm our business, prospects, operating results and financial condition.

As is the case with biopharmaceuticals generally, it is likely that there may be side effects and adverse events associated with our therapeutic candidates' use. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Although other oligonucleotide therapeutics have received regulatory approval, our siRNAs, which rely on a novel approach to oligonucleotide therapies, may present enhanced risk and uncertainty for our therapeutic candidates compared to more well-established classes of therapies. Moreover, there have been only a limited number of clinical trials involving the use of oligonucleotide therapeutics. It is impossible to predict when or if any therapeutic candidates we may develop will prove safe in humans. Undesirable side effects caused by our therapeutic candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. Drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Moreover, if our therapeutic candidates are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may elect to abandon their development or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, which may limit the commercial expectations for the therapeutic candidate if approved. We may also be required to modify our study plans based on findings after we commence our clinical trials. Many therapeutic candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the compound. In addition, regulatory authorities may draw different conclusions or require additional testing to confirm these determinations.

It is possible that as we test our therapeutic candidates in larger, longer and more extensive clinical trials, or as the use of these therapeutic candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, may be reported by patients. If such side effects become known later in development or upon approval, such findings may harm our business, financial condition and prospects significantly.

Patients treated with our products, if approved, may experience previously unreported adverse reactions, and it is possible that the FDA or other regulatory authorities may ask for additional safety data as a condition of, or in connection with, our efforts to obtain approval of our therapeutic candidates. If safety problems occur or are identified after our products, if any, reach the market, we may make the decision or be required by regulatory authorities to amend the labeling of our products, recall our products or even withdraw approval for our products.

In addition, if one or more of our therapeutic candidates receives regulatory approval, and we or others later identify undesirable side effects caused by such product, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw, suspend or limit approvals of such product, or seek an injunction against its manufacture or distribution;
- we may be required to recall a product or change the way such product is administered to patients;
- regulatory authorities may require additional warnings on the label, such as a "black box" warning or a contraindication;
- we may be required to implement a Risk Evaluation and Mitigation Strategy ("REMS") or comparable foreign risk mitigation measures, or create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way a product is distributed, conduct additional clinical trials or change the labeling of a product or be required to conduct additional post-marketing studies or surveillance;
- we could be sued and held liable for harm caused to patients;
- sales of the product may decrease significantly, or the product could become less competitive; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular therapeutic candidate, if approved, and could significantly harm our business, results of operations and prospects.

As an organization, we have never completed any pivotal clinical trials or submitted an NDA for regulatory approval and may be unable to do so for any of our therapeutic candidates.

We are early in our development efforts for most of our therapeutic candidates and we will need to successfully complete our ongoing clinical trials and later-stage and pivotal clinical trials in order to obtain FDA or comparable foreign regulatory approval to market any of our therapeutic candidates, as well as complete IND-enabling studies for certain of our preclinical therapeutic candidates. Carrying out clinical trials and the submission of a successful NDA is a complicated process. As an organization, we have not completed any pivotal clinical trials, have limited experience as a company in preparing, submitting and prosecuting regulatory filings and have not previously submitted an NDA or other comparable foreign regulatory submission for any therapeutic candidate. As interactions with the FDA may not be comprehensive, we cannot be certain how many clinical trials of any of our therapeutic candidates will be required or how such trials should be designed. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to regulatory submission and approval of any of our therapeutic candidates. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of therapeutic candidates that we develop. Failure to commence or complete, or delays in, our planned clinical trials, could prevent us from or delay us in submitting NDAs for and commercializing our therapeutic candidates.

Our therapeutic candidates are subject to extensive regulation and compliance, which is costly and time consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize our therapeutic candidates.

The clinical development, manufacturing, labeling, packaging, storage, record-keeping, advertising, promotion, import, export, marketing, distribution and adverse event reporting, including the submission of safety and other information, of our therapeutic candidates are subject to extensive regulation by the FDA in the United States and by comparable foreign regulatory authorities in foreign markets. In the United States, we are not permitted to market our therapeutic candidates until we receive regulatory approval from the FDA. The process of obtaining regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity and novelty of the therapeutic candidates involved, as well as the target indications and patient population. Approval policies or regulations may change, and the FDA has substantial discretion in the drug approval process, including the ability to delay, limit or deny approval of a therapeutic candidate for many reasons. Despite the time and expense invested in clinical development of therapeutic candidates, regulatory approval is never guaranteed. Neither we nor any current or future collaborator is permitted to market any of our therapeutic candidates in the United States until we receive approval from the FDA.

The FDA or comparable foreign regulatory authorities can delay, limit or deny approval of a therapeutic candidate for many reasons, including:

- such authorities may disagree with the design or implementation of our or our collaborators' current or future clinical trials;
- negative or ambiguous results from our clinical trials or results may not meet the level of statistical significance required by the FDA or comparable foreign regulatory agencies for approval;
- serious and unexpected drug-related side effects may be experienced by participants in our clinical trials or by individuals using drugs similar to our therapeutic candidates;
- such authorities may not accept clinical data from trials which are conducted at clinical facilities outside of the United States, including those countries with a potentially different standard of care;
- we or any of our current or future collaborators may be unable to demonstrate that a therapeutic candidate is safe and effective, and that therapeutic candidate's clinical and other benefits outweigh its safety risks;
- such authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- such authorities may not agree that the data collected from clinical trials of our therapeutic candidates are acceptable or sufficient to support the submission of an NDA or other submission or to obtain regulatory approval in the United States or elsewhere, and such authorities may impose requirements for additional preclinical studies or clinical trials;
- such authorities may disagree regarding the formulation, labeling and/or the specifications of our therapeutic candidates;
- approval may be granted only for indications that are significantly more limited than what we apply for and/or with other significant restrictions on distribution and use;
- such authorities may find deficiencies in the manufacturing processes, approval policies or facilities of our third-party manufacturers with which we or any of our current or future collaborators contract for clinical and commercial supplies;

- regulations of such authorities may significantly change in a manner rendering our or any of our current or future collaborators' clinical data insufficient for approval; or
- such authorities may not accept a submission due to, among other reasons, the content or formatting of the submission.

With respect to foreign markets, approval procedures vary among countries and, in addition to the foregoing risks, may involve additional product testing, administrative review periods and agreements with pricing authorities. In addition, events raising questions about the safety of certain marketed biopharmaceuticals may result in increased cautiousness by the FDA and comparable foreign regulatory authorities in reviewing new drugs and biologics based on safety, efficacy or other regulatory considerations and may result in significant delays in obtaining regulatory approvals. Any delay in obtaining, or inability to obtain, applicable regulatory approvals could prevent us or any of our current or future collaborators from commercializing our therapeutic candidates.

We have conducted and will continue to conduct certain of our clinical trials for our therapeutic candidates outside of the United States. However, the FDA and other foreign equivalents may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

We have conducted and will continue to conduct one or more of our clinical trials for our therapeutic candidates outside the United States. For example, we have conducted Phase 1 clinical trials in Australia and will rely on the data from these clinical trials to support INDs submitted in the United States to the FDA. Although the FDA may accept data from clinical trials conducted outside the United States in support of an IND or NDA, acceptance of this data is subject to certain conditions imposed by the FDA. For example, where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, regardless of whether the applicable clinical trials were conducted under an IND, the FDA will not approve the application on the basis of foreign data alone, unless such data are applicable to the U.S. population and U.S. medical practice; the studies were performed by clinical investigators of recognized competence; and the data are considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. For studies that are conducted only at sites outside of the United States and not otherwise subject to an IND, the FDA requires the clinical trial to have been conducted in accordance with GCPs, and the FDA must be able to validate the data from the clinical trial through an on-site inspection if it deems such inspection necessary. For such studies that are not otherwise subject to an IND, the FDA generally does not provide advance comment on the clinical protocols for the studies, and therefore there is an additional potential risk that the FDA could determine that the study design or protocol for a non-U.S. clinical trial was inadequate, which could require us to conduct additional clinical trials, which could delay our development timelines, increase costs, or adversely affect our ability to obtain regulatory approval and commercialize our therapeutic candidates.

Conducting clinical trials outside the United States also exposes us to additional risks, including risks associated with:

- additional foreign regulatory requirements;
- foreign exchange fluctuations;
- compliance with foreign manufacturing, customs, shipment and storage requirements;
- cultural differences in medical practice and clinical research; and
- diminished protection of intellectual property in some countries.

Disruptions at the FDA and other government agencies or comparable foreign regulatory authorities caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

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, statutory, regulatory and policy changes, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs and biologics or modifications to approved drugs and biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, in recent years the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough FDA employees causing delays with internal agency activities. In addition, the current U.S. Presidential administration has issued certain policies and Executive Orders directed towards reducing the employee headcount and costs associated with U.S. administrative agencies, including the FDA,

which have led to substantial personnel changes, and it remains unclear the degree to which these efforts or the resulting changes in FDA personnel or priorities may limit or otherwise adversely affect the FDA's ability to conduct routine activities.

If a prolonged government shutdown occurs, or if funding shortages, staffing limitations or similar factors hinder or prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, such events could significantly impact the ability of the FDA or other such regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

We may expend our limited resources to pursue a particular therapeutic candidate and fail to capitalize on therapeutic candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on specific therapeutic candidates and specific indications. As a result, we may forgo or delay pursuit of opportunities with other therapeutic candidates, or opportunities for our therapeutic candidates in other indications, that could have had greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and therapeutic candidates for specific indications may not yield any commercially viable therapeutic candidates. If we do not accurately evaluate the commercial potential or target market for a particular therapeutic candidate, we may relinquish valuable rights to that therapeutic candidate through collaborations, licenses and other similar arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such therapeutic candidate.

We may not be able to obtain or maintain orphan drug designations for any of our therapeutic candidates, and we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs or biologics for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a drug or biologic as an orphan product if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population of greater than 200,000 individuals in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the United States, orphan designation entitles a sponsor to financial incentives such as opportunities for grant funding toward clinical trial costs, tax advantages and user-fee waivers. In addition, if a therapeutic candidate that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications including an NDA, to market the same drug for the same approved indication or use within the relevant disease or condition for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity within the relevant indication or use, or where the manufacturer is unable to assure sufficient product quantity to meet the needs relating to the orphan-protected indication or use. Similarly, if a product is approved within a disease or condition broader than the orphan-designated disease or indication, it may not be eligible for orphan exclusivity.

In October 2025, the FDA granted orphan drug designation to onvuzosiran for the treatment of patients with HAE. In addition, we may seek orphan drug designations for our other therapeutic candidates, but there can be no assurances that we will be able to obtain such designations. Even if we obtain orphan drug designation for another therapeutic candidate, we may not be able to obtain or maintain orphan drug exclusivity for that therapeutic candidate. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same indications or uses. Even after an orphan drug is approved, the FDA or comparable foreign regulatory authority can subsequently approve the same drug for the same disease or condition if such regulatory authority concludes that the later drug is clinically superior because it is shown to be safer, more effective or makes a major contribution to patient care within the relevant indication or use. Orphan drug exclusivity in the United States may also be lost if the FDA later determines that the initial request for designation was materially defective. In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs containing different active ingredients for the same or similar indications or uses. In addition, if a subsequent drug is approved for marketing for the same or a similar indication or use as any of our therapeutic candidates that receive regulatory approval, we may face increased competition and lose market share regardless of orphan drug exclusivity. Orphan drug designation neither shortens the development time nor regulatory review time of a drug nor does it change the regulatory requirements for approval.

Risks related to third parties

We rely on third parties to conduct our preclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties, comply with applicable regulatory requirements or meet expected deadlines, our development programs and our ability to seek or obtain regulatory approval for or commercialize our therapeutic candidates may be delayed or prevented.

We are dependent on third parties to conduct our preclinical studies and clinical trials. Specifically, we have used and relied on, and intend to continue to use and rely on, medical institutions, clinical investigators, CROs and consultants to conduct our preclinical studies and clinical trials in accordance with our clinical protocols and regulatory requirements. These CROs, investigators and other third parties play a significant role in the conduct and timing of these studies and trials and subsequent collection and analysis of data. While we have and will have agreements governing the activities of our third-party contractors, we have limited control over their actual performance. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on our CROs and other third parties does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCP requirements, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for all of our therapeutic candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs or trial sites fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. In addition, our clinical trials must be conducted with therapeutic candidates manufactured in accordance with cGMP and comparable foreign regulations. Any failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

There is no guarantee that any of our CROs, investigators or other third parties will devote adequate time and resources to such trials or perform as contractually required. If any of these third parties fails to meet expected deadlines, adhere to our clinical protocols or meet regulatory requirements, or otherwise performs in a substandard manner, our clinical trials may be extended, delayed or terminated. In addition, many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other development activities that could harm our competitive position. In addition, principal investigators for our clinical trials may also serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA concludes that the financial relationship may have affected the interpretation of the study, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection by the FDA of any NDA we submit. Any such delay or rejection could prevent us from commercializing our therapeutic candidates.

If any of our relationships with these third parties terminates, we may not be able to enter into arrangements with alternative third parties on commercially reasonable terms, in a timely manner or at all. Switching or adding additional CROs, investigators and other third parties involves additional cost and requires our management's time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we work to carefully manage our relationships with our CROs, investigators and other third parties, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

We rely on third parties for the manufacture of our therapeutic candidates for preclinical and clinical development. This reliance on third parties increases the risk that we will not have sufficient quantities of our therapeutic candidates or products or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not own or operate manufacturing facilities and have no plans to develop our own clinical or commercial- scale manufacturing capabilities. We rely, and expect to continue to rely, on third parties for the manufacture of our therapeutic candidates and related raw materials for preclinical and clinical development, as well as for commercial manufacture if any of our therapeutic candidates receive marketing approval. The facilities used by third-party manufacturers to manufacture our therapeutic candidates must be approved by the FDA and any comparable foreign regulatory authority, as applicable, following inspections that may be conducted after we submit an NDA to the FDA or any comparable application to a foreign regulatory authority. We do not control the manufacturing process of, and are dependent on, third-party manufacturers for compliance with cGMP requirements. If these third-party manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or any comparable foreign regulatory authority, they may not be able to secure or maintain regulatory approval for their manufacturing facilities. In addition, we have limited control over the ability of third-party manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any comparable foreign regulatory authority does not approve these facilities for the manufacture of our therapeutic candidates or if it withdraws any such approval in the future, we may need to identify alternative manufacturing facilities, which could significantly impact our ability to develop, obtain regulatory approval for, or market our therapeutic candidates, if approved. Our failure, or the failure of our third- party manufacturers, to comply with applicable regulations

could result in sanctions being imposed on us or them, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, seizures or recalls of therapeutic candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Our or a third party's failure to execute on our manufacturing requirements on commercially reasonable terms, in a timely manner and in compliance with cGMP or other regulatory requirements could adversely affect our business in a number of ways, including:

- an inability to initiate or continue clinical trials of our therapeutic candidates under development;
- delay in submitting regulatory applications or receiving marketing approvals for our therapeutic candidates;
- additional inspections of third-party manufacturing facilities by regulatory authorities;
- requirements to cease development or to recall batches of our therapeutic candidates; and
- if any of our therapeutic candidates are approved, an inability to meet commercial demands for our therapeutic candidates or any other future therapeutic candidates.

In addition, we do not have any long-term commitments or supply agreements with our third-party manufacturers. We may be unable to establish any long-term agreements with third-party manufacturers on acceptable terms, or at all. Even if we are able to establish such agreements, reliance on third-party manufacturers entails additional risks, including:

- failure of third-party manufacturers to comply with regulatory requirements and maintain quality assurance;
- breach of the manufacturing agreement by the third party;
- failure to manufacture our product according to our specifications;
- failure to manufacture our product according to our schedule or at all;
- misappropriation of our proprietary information, including our trade secrets and know-how; and
- termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Our therapeutic candidates and any products that we may develop may compete with other therapeutic candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval, and any related remedial measures may be costly or time consuming to implement. We do not currently have arrangements in place for redundant supply or a second source for all required raw materials used in the manufacture of our therapeutic candidates. If our existing or future third-party manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may be unable to replace them on a timely basis or at all.

Certain biotechnology companies, CROs and contract development and manufacturing organizations ("CDMOs"), including companies based in China, may become subject to trade restrictions, sanctions, other regulatory requirements, or proposed legislation by the U.S. government, which could potentially impact services available for our research and development or our ability to secure the materials we need for our drug candidates. For example, on December 18, 2025, the United States passed legislation, namely the BIOSECURE Act ("BIOSECURE Act") as an amendment to the National Defense Authorization Act (NDAA) for Fiscal Year 2026, which implicates U.S. government contracts, grants and loans to entities that use equipment and services from certain "biotechnology companies of concern" ("BCC"). Specifically, the BIOSECURE Act prohibits the U.S. Government from procuring or obtaining biotechnology equipment or services produced or provided by a BCC; entering into, extending, or renewing government contracts with an entity that directly or indirectly uses biotechnology equipment or services from a BCC in performance of that federal contract; and/or issuing grants or loans to purchase, obtain, or use biotechnology equipment or services produced or provided by a BCC.

The BIOSECURE Act does not itself name specific entities as BCCs. Instead, BCCs include entities that are identified by the U.S. Department of War ("Department of Defense") Section 1260H list of "Chinese military companies". BCCs can also be designated by the Office of Management and Budget ("OMB") through a criteria-based pathway administered with an interagency process.

If certain biotechnology companies are added to the 1260H list or otherwise designated by OMB pursuant to the mechanisms described above, the BIOSECURE Act would have the potential to severely restrict the ability of U.S. biopharmaceutical companies like us to purchase services or products from, collaborate with, or otherwise work with BCC without losing the ability to contract with, or otherwise receive funding from, the U.S. government. Like many other biotechnology companies, we do business with companies in

China (while these Chinese companies are not currently listed on the 1260H list or otherwise designated by OMB, it remains possible that some of our contractual counterparties could be designated as a BCC in the future). If any of our current or future CROs, CDMOs, or other suppliers were so designated, we could be required to terminate, suspend, materially modify or transition those relationships. There can be no assurance that we would be able to identify, qualify, and onboard alternative third parties on acceptable terms, in a timely manner or at all. Transitioning the manufacturing of our drug candidates to an alternative manufacturer would require a complex technology transfer process and, in some cases, regulatory clearance or approval, which could take 9 to 12 months or longer. In addition, in October 2026, the U.S. Department of the Treasury proposed regulations implementing the Comprehensive Outbound Investment National Security Act of 2025 that would, among other things, prohibit U.S. persons from investing in, or entering into joint ventures with, companies in China and certain other countries of concern that engage in a broad range of biotechnology activities. If adopted as proposed, these regulations could limit our ability to establish or expand operations in China and require us to modify or forgo our current or future activities in China.

Such counterparties may also be subject to U.S. legislation, sanctions, trade restrictions and other foreign regulatory requirements which could increase the cost of, or reduce the supply of, or delay the procurement or supply of materials or services available to us. Any such disruption could adversely affect the development of our therapeutic candidates. Our current and anticipated future dependence on third parties for the manufacture of our therapeutic candidates or products may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis. If we are unable to successfully transition manufacturing or other activities away from affected manufacturers, CROs, CDMOs, or other suppliers within the required timeframes, our business, financial condition, and prospects could be materially and adversely affected.

We have and in the future may seek to enter into additional collaborations, licenses and other similar arrangements and may not be successful in establishing or maintaining such collaborations, and even if we are, we may relinquish valuable rights and may not realize the benefits of such relationships.

We have and in the future may seek to enter into additional collaborations, joint ventures, licenses and other similar arrangements for the development or commercialization of our therapeutic candidates, if approved, due to capital costs required to develop or commercialize the therapeutic candidates or manufacturing constraints. For example, we entered into the AbbVie Agreement pursuant to which we granted to AbbVie, with respect to each collaboration target, an exclusive option to obtain an exclusive (even as to us), sublicensable license under certain intellectual property rights to develop, commercialize and otherwise exploit the siRNA-containing therapeutic agents (and products containing such therapeutic agents) directed to such collaboration target on a worldwide basis in all human and non-human diagnostic, prophylactic and therapeutic uses. See the section titled "Business—Collaboration and license option agreement" in the Prospectus for more information. We may not be successful in our efforts to establish or maintain such collaborations for our therapeutic candidates because our research and development pipeline may be insufficient, our therapeutic candidates may be deemed to be at too early of a stage of development for collaborative effort, or third parties may not view our therapeutic candidates as having the requisite potential to demonstrate safety and efficacy or significant commercial opportunity. In addition, we face significant competition in seeking appropriate strategic partners, and the negotiation process can be time-consuming and complex. Even if we are successful in our efforts to establish or maintain such collaborations, the terms that we agree upon may not be favorable to us. We may need to relinquish valuable rights to our future revenue streams, research programs, intellectual property, or therapeutic candidates or grant licenses on terms that may not be favorable to us, as part of any such arrangement, and such arrangements may restrict us from entering into additional agreements with other potential collaborators. For example, under the AbbVie Agreement, we are prohibited under certain circumstances from (i) exploiting any siRNA directed to a collaboration target during the applicable option period, (ii) commercializing or manufacturing commercial supply of any siRNA directed to a licensed target in any country, or (iii) researching, developing or manufacturing pre-clinical or clinical supply of any siRNA directed to a licensed target in the United States or, for a specified time period, outside the United States, in each case, subject to certain change of control and acquisition-related exceptions. See the section titled "Business—Collaboration and license option agreement" in the Prospectus for more information.

In addition, we have in our current collaboration with AbbVie, and if we enter into additional collaborations in the future we will have, limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our therapeutic candidates. Our ability to generate revenue from these arrangements will depend on any current or future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot be certain that, following a collaboration, license or strategic transaction, we will achieve an economic benefit that justifies such transaction, and such transaction may not yield additional development or therapeutic candidates for our pipeline. Furthermore, we may not be able to maintain such collaborations if, for example, the development or approval of a therapeutic candidate is delayed, the safety of a therapeutic candidate is questioned or the sales of an approved product are unsatisfactory.

In addition, any current or future collaborations may be terminable by our strategic partners, and we may not be able to adequately protect our rights under these collaborations. For example, under the AbbVie Agreement, AbbVie may terminate the AbbVie Agreement in its entirety or on a target-by-target or country-by-country basis immediately or subject to certain notice periods depending on the circumstances or, upon a material breach or insolvency of the company, AbbVie may elect to continue the AbbVie Agreement with

certain reduced payments to us, termination of AbbVie's diligence obligations, and disbanding of the joint governance committee. Furthermore, strategic partners may negotiate for certain rights to control decisions regarding the development and commercialization of our therapeutic candidates, if approved, and may not conduct those activities in the same manner as we do or would. Any termination of collaborations we have entered into or may enter into in the future, or any delay in entering into collaborations related to our therapeutic candidates, could delay the development and commercialization of our therapeutic candidates, if approved, and reduce their competitiveness if they reach the market, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

If conflicts arise between us and our current or future collaborators, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.

If conflicts arise between us and our current or future collaborators, such collaborators may act in a manner adverse to us and could limit our ability to implement our strategies. Our collaborators may develop, either alone or with others, products in related fields that are competitive with the therapeutic candidates we may develop that are the subject of these collaborations with us. Competing products, either developed by the collaborators or to which the collaborators have rights, may result in the withdrawal of support for our therapeutic candidates.

Our current or future collaborators could also become our competitors. Our collaborators could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, fail to devote sufficient resources to the development and commercialization of products or merge with or be acquired by a third party who may do any of these things. Any of these developments could harm our product development efforts.

If we are not able to establish additional collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our research programs and therapeutic candidates and the potential commercialization of any therapeutic candidates that we may develop will require substantial additional cash. For some therapeutic candidates, we may decide to collaborate with additional pharmaceutical or biotechnology companies for the development and potential commercialization of those therapeutic candidates.

We face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Whether we reach a definitive agreement for any additional collaboration will depend, among other things, on our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA, the EMA or similar regulatory authorities outside the United States, the potential market for the subject therapeutic candidate, the costs and complexities of manufacturing and delivering such therapeutic candidate to patients, the potential of competing products, the uncertainty with respect to our ownership of technology, and industry and market conditions generally. The collaborator may also consider alternative therapeutic candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms or at all. If we are unable to do so, we may have to curtail the development of the therapeutic candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization, reduce the scope of any sales or marketing activities, or increase our own expenditures on the development of the therapeutic candidate.

We are dependent on third-party vendors to provide certain licenses, products and services and our business and operations, including clinical trials, could be disrupted by any problems with our significant third-party vendors.

We engage a number of third-party service providers to supply critical services, such as contract research services, contract manufacturing services and information technology services. Disruptions to the business, financial stability or operations of these service providers, including due to strikes, labor disputes or other disruptions to the workforce or to their willingness and ability to produce or deliver such products or provide such services in a manner that satisfies the requirements put forth by the authorities, or in a manner that satisfies our own requirements, could affect our ability to develop and market our future therapeutic candidates on a timely basis. If these service providers are unable or unwilling to continue to provide their products or services in the manner expected, or at all, we could encounter difficulty finding alternative service providers. Even if we are able to secure appropriate alternative service providers

in a timely manner, costs for such products or services could increase significantly. Any of these events could adversely affect our results of operations and our business.

Risks related to commercialization of our therapeutic candidates

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

The biopharmaceutical industry in general, and the RNA therapy field in particular, is characterized by rapidly advancing and changing technologies, intense competition and a strong emphasis on intellectual property. We face substantial and increasing competition from large and specialty biopharmaceutical companies, as well as public and private medical research institutions and governmental agencies. Competitors may compete with us in hiring scientific and management personnel, establishing clinical trial sites, recruiting patients to participate in clinical trials and acquiring technologies complementary to, or necessary for, our programs. Our known biopharmaceutical competitors in the RNA therapy space include, but may not be limited to, the following: Alnylam Pharmaceuticals, Inc., Argo Biopharmaceutical Co., Ltd., Aro Biotherapeutics Co., Arrowhead Pharmaceuticals, Inc., Avidity Biosciences, Inc. (acquired by Novartis AG), City Therapeutics, Inc., Dicerna Pharmaceuticals, Inc. (acquired by Novo Nordisk), Dyne Therapeutics, Inc., Ionis Pharmaceuticals, Inc., SanegeneBio Inc., Sarepta Therapeutics, Inc., Silence Therapeutics PLC, Sirius Therapeutics, Inc., Sirnaomics Ltd., Suzhou Ribo Life Science Co., Ltd, and Wave Life Sciences Ltd. In addition, we will compete with the many companies targeting the same indications as our therapeutic candidates, as well as more generally with other companies developing alternative scientific and technological approaches. Our competitors in the different indications include but are not limited to: Arrowhead, Biogen, Novartis AG, Otsuka Pharmaceutical Co., Ltd., RemeGen Co., Ltd., Takeda Pharmaceutical Co., Ltd., Vera Therapeutics, Inc., and Vertex Pharmaceuticals Inc. in renal indications; AstraZeneca PLC (US), Biogen Inc., Novartis AG, Regeneron Pharmaceuticals Inc. and Roche Holding AG in paroxysmal nocturnal hemoglobinuria; Annexon, Inc., Astellas Pharma Inc., Biogen Inc., and Regeneron Pharmaceuticals Inc., in GA; Argo Biopharmaceutical Co., Ltd., BioCryst Pharmaceuticals Inc., CSL Behring, Intellia Therapeutics, KalVista (acquired by Chiesi), Pharvaris N.V., and Takeda Pharmaceutical Co. Ltd. in HAE; Bayer, Bristol-Myers Squibb Company, Johnson & Johnson, Novartis AG, Regeneron, Suzhou Ribo Life Science Co., Sirius Therapeutics, and Sirnaomics Ltd. in thrombotic diseases; Alnylam Pharmaceuticals, Inc., Arrowhead Pharmaceuticals, Inc., Eli Lilly, Novo Nordisk, Regeneron, Suzhou Ribo Life Science, and Wave Life Sciences in obesity; and Alector, Inc. and Alnylam Pharmaceuticals, Inc. in neurodegeneration.

Many of our current or potential competitors have significantly greater financial, technical and human resources, as well as more expertise in research and development, manufacturing, preclinical testing, conducting clinical studies and trials and commercializing and marketing approved products, than us. Mergers and acquisitions in the biopharmaceutical industry may result in even greater resource concentration among a smaller number of competitors. Smaller or early-stage companies may also prove to be significant competitors, either alone or through collaborative arrangements with large and established companies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other comparable foreign regulatory authority approval for their products more rapidly than us, which could result in our competitors establishing a strong market position before we are able to enter the market. Key competitive factors affecting the success of all of our programs are likely to be their efficacy, safety, convenience, price and degree of reimbursement.

Even if we receive regulatory approval for any therapeutic candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. Additionally, our therapeutic candidates, if approved, could be subject to labeling and other restrictions on marketing or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our therapeutic candidates, when and if any of them are approved.

Following potential approval of any of our therapeutic candidates, the FDA may impose significant restrictions on a product's indicated uses or marketing or impose ongoing requirements for potentially costly and time-consuming post-approval studies, post-market surveillance or clinical trials to monitor the safety and efficacy of the product. The FDA may also require a REMS as a condition of approval of our therapeutic candidates, which could include requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our therapeutic candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our products will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, listing and registration requirements, as well as continued compliance with cGMPs and GCP requirements for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with our products,

including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of our products, withdrawal of the product from the market or voluntary or mandatory product recalls;
- restrictions on product distribution or use, or requirements to conduct post-marketing studies or clinical trials;
- fines, restitutions, disgorgement of profits or revenues, warning letters, untitled letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of approvals;
- product seizure or detention, or refusal to permit the import or export of our products; and
- injunctions or the imposition of administrative, civil or criminal penalties.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our therapeutic candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

In addition, the FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could adversely affect the commercialization of our therapeutic candidates, if approved. We cannot predict the likelihood, nature or extent of such changes. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability.

The commercial success of our therapeutic candidates will depend upon the degree of market acceptance of such therapeutic candidates by physicians, patients, healthcare payors and others in the medical community.

Our therapeutic candidates may not be commercially successful. Even if any of our therapeutic candidates receive regulatory approval, they may not gain market acceptance among physicians, patients, healthcare payors or the medical community. The commercial success of any of our current or future therapeutic candidates will depend significantly on the broad adoption and use of the resulting product by physicians and patients for approved indications. The degree of market acceptance of our products will depend on a number of factors, including:

- demonstration of clinical efficacy and safety, including as compared to more-established products;
- the indications for which our therapeutic candidates are approved;
- the limitation of our targeted patient population and other limitations or warnings contained in any labeling approved by the FDA or other regulatory authority;
- acceptance of a new drug for the relevant indication by healthcare providers and their patients;
- the relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies;
- the pricing and cost-effectiveness of our products, as well as the cost of treatment with our products in relation to alternative treatments and therapies;
- our ability to obtain and maintain sufficient third-party coverage and adequate reimbursement from government healthcare programs, including Medicare and Medicaid, private health insurers and other third-party payors;
- the willingness of patients to pay all, or a portion of, out-of-pocket costs associated with our products in the absence of sufficient third-party coverage and adequate reimbursement;
- any restrictions on the use of our products, and the prevalence and severity of any adverse effects;
- potential product liability claims;
- the timing of market introduction of our products as well as competitive drugs;
- the effectiveness of our or any collaborators' sales and marketing strategies; and
- unfavorable publicity relating to the product.

If any therapeutic candidate is approved but does not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors or patients, we may not generate sufficient revenue from that product and may not become or remain profitable. Our efforts to

educate the medical community and third-party payors regarding the benefits of our products may require significant resources and may never be successful.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found or alleged to have improperly promoted off-label uses, we may become subject to enforcement action and significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, as our therapeutic candidates would be, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. If we cannot successfully manage the promotion of our therapeutic candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

The successful commercialization of our therapeutic candidates, if approved, will depend in part on the extent to which governmental authorities and health insurers establish coverage, adequate reimbursement levels and favorable pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our products could limit our ability to market those products and decrease our ability to generate revenue.

The availability of coverage and the adequacy of reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford prescription medications such as our therapeutic candidates, if approved. Our ability to achieve coverage and acceptable levels of reimbursement for our products by third-party payors will have an effect on our ability to successfully commercialize those products. Moreover, we are initially developing therapeutic candidates targeting rare disorders with small patient populations. In order for products that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such products must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved therapeutic candidate with a smaller patient population that accounts for the smaller potential market size. Even if we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization.

We cannot be sure that coverage and reimbursement in the United States, the European Union or elsewhere will be available for any product that we may develop, and any reimbursement that may become available may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for biopharmaceutical products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs when an equivalent generic drug or a less expensive therapy is available. It is possible that a third-party payor may consider our products as substitutable and only offer to reimburse patients for the less expensive product. Even if we are successful in demonstrating improved efficacy or improved convenience of administration with our products, pricing of existing drugs may limit the amount we will be able to charge for our products. These payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in product development. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our products and may not be able to obtain a satisfactory financial return on products that we may develop. Further, coverage policies and third-party payor reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs will be covered. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our products.

Obtaining and maintaining reimbursement status is time consuming, costly and uncertain. The Medicare and Medicaid programs often are used as models for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs. However, no uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination

process is often a time consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases at short notice, and we believe that changes in these rules and regulations are likely.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost containment initiatives in Europe and other countries has and will continue to put pressure on the pricing and usage of our products. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our products. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our products. We expect to experience pricing pressures in connection with the sale of any of our products due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. For example, the U.S. Department of Health and Human Services ("HHS") imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation. In addition, HHS has been empowered to negotiate the price of certain single-source drugs that have been on the market for at least seven (7) years without being the listed product for a generic, or certain BLA-licensed biological products on the market for at least eleven (11) years without being the reference biological product for a biosimilar covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

The affected populations for our therapeutic candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our therapeutic candidates.

We select the targets for development of our therapeutic candidates based on a number of factors, including the estimated patient populations where we believe there is a meaningful addressable market opportunity. However, our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our therapeutic candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our therapeutic candidates will ultimately depend upon a number of factors, including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access, alternative therapies and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included in this Quarterly Report on Form 10-Q should be viewed with caution. Further, the data and statistical information used in this Quarterly Report on Form 10-Q, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

We currently have no marketing and sales organization and have no experience as a company in commercializing products, and we may have to invest significant resources to develop these capabilities. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our products, we may not be able to generate product revenue.

We have no internal sales, marketing or distribution capabilities, nor have we commercialized a product. If any of our therapeutic candidates ultimately receives regulatory approval, we must build a marketing and sales organization with technical expertise and supporting distribution capabilities to commercialize each such product in major markets, which will be expensive and time consuming, or collaborate with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. We have no prior experience as a company in the marketing, sale and distribution of biopharmaceutical products and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. We may not be able to enter into collaborations or hire consultants or external service providers to assist us in sales,

marketing and distribution functions on acceptable financial terms, or at all. In addition, our product revenues and our profitability, if any, may be lower if we rely on third parties for these functions than if we were to market, sell and distribute any products that we develop ourselves. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we are not successful in commercializing our products, either on our own or through arrangements with one or more third parties, we may not be able to generate any future product revenue and we would incur significant additional losses.

Our future growth may depend, in part, on our ability to operate in foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future growth may depend, in part, on our ability to develop and commercialize our therapeutic candidates in foreign markets. We are not permitted to market or promote any of our therapeutic candidates before we receive regulatory approval from applicable regulatory authorities in foreign markets, and we may never receive such regulatory approvals for any of our therapeutic candidates. To obtain separate regulatory approval in many other countries we must comply with numerous and varying regulatory requirements regarding safety and efficacy and governing, among other things, clinical trials, commercial sales, pricing and distribution of our therapeutic candidates. If we obtain regulatory approval of our therapeutic candidates and ultimately commercialize our products in foreign markets, we would be subject to additional risks and uncertainties, including:

- different regulatory requirements for approval of drugs in foreign countries;
- reduced protection for intellectual property rights;
- the existence of additional third-party patent rights of potential relevance to our business;
- compliance with export control and import laws and regulations and unexpected changes in or uncertainty with respect to the enforcement of tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is common;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, trade disputes, public health pandemics or epidemics, or natural disasters including earthquakes, typhoons, floods and fires.

Risks related to our intellectual property

If we are unable to obtain and maintain patent protection for our technology and therapeutic candidates or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely, and will continue to rely, upon a combination of patents, trade secret protection, assignments, and confidentiality agreements to protect the intellectual property related to our technologies, therapeutic candidate development programs and therapeutic candidates. Our success depends in large part on our ability to secure and maintain patent protection in the United States and other countries with respect to our current therapeutic candidates and any future therapeutic candidates we may develop. We seek to protect our position by filing patent applications in the United States and abroad related to our technologies, development programs and therapeutic candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Moreover, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent applications that we own may fail to result in issued patents with claims that cover our products and technology, including current therapeutic candidates and any future therapeutic candidates we may develop, in the United States or in other foreign countries, in whole or in part. Alternately, our existing patent and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can prevent a patent from issuing from a pending patent application or later invalidate or narrow the scope of an issued patent. For example, publications of discoveries

in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Even if patents are successfully issued to us and even if such patents cover our current therapeutic candidates or any future therapeutic candidates we may develop, third parties may challenge their validity, ownership, enforceability or scope thereof, which may result in such patents being narrowed, invalidated, or held unenforceable or circumvented. Any successful challenge to these patents or any other patents owned by us could deprive us of rights necessary for the successful commercialization of any of our therapeutic candidates. In addition, the issuance of a patent does not give us the right to practice the patented invention. Third parties may have blocking patents that could prevent us from marketing our therapeutic candidate, if approved, or practicing our own patented technology. Our competitors may be able to circumvent our patents by developing similar or alternative therapeutic candidates in a non-infringing manner. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a therapeutic candidate under patent protection could be reduced. If any of our patents expire or are challenged, invalidated, circumvented or otherwise limited by third parties prior to the commercialization of our therapeutic candidate, and if we do not own or have exclusive rights to other enforceable patents protecting our therapeutic candidate or technologies, competitors and other third parties could market products and use processes that are substantially similar, or superior, to ours and our business would suffer.

If the patent applications we hold with respect to our development programs and therapeutic candidates fail to issue, if their validity, breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for any of our current or future therapeutic candidates or technology, it could dissuade companies from collaborating with us to develop therapeutic candidates, encourage competitors to develop competing products or technologies and threaten our ability to commercialize future therapeutic candidates. Any such outcome could harm our business.

The patent position of biotechnology and pharmaceutical companies is generally highly uncertain, involves complex legal, scientific and factual questions, and is characterized by the existence of large numbers of patents and frequent litigation based on allegations of patent or other intellectual property infringement or violation. The standards that the U.S. Patent and Trademark Office ("USPTO") and its foreign counterparts use to grant patents are not always applied predictably or uniformly. In addition, the laws of jurisdictions outside the United States may not protect our rights to the same extent as the laws of the United States, and many companies have encountered significant problems in protecting and defending such rights in foreign jurisdictions. For example, European patent law is more restrictive to the patentability of methods of treatment of the human body than U.S. patent law. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. Since patent applications in the United States and other jurisdictions are confidential for a period of time after filing, we cannot be certain that we were the first to file for patents covering our inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in the issuance of patents or may result in the issuance of patents which fail to protect our technology or products, in whole or in part, or which fail to effectively prevent others from commercializing competitive technologies and products.

The issuance of a patent is not conclusive as to its inventorship, ownership, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. We may become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Thus, even if our patent applications are issued as patents, they may not issue in a form that will provide us with meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future therapeutic candidates, we may be open to competition from generic versions of such products. Given the amount of time required for the development, testing and regulatory review of new therapeutic candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Third parties may assert claims against us alleging infringement of their patents and proprietary rights, or we may need to become involved in lawsuits to defend or enforce our patents, either of which could result in substantial costs or loss of productivity, delay or prevent the development and commercialization of our therapeutic candidates, prohibit our use of proprietary technology or sale of products or put our patents and other proprietary rights at risk.

Competitors with greater resources than we do may be able to sustain the costs of complex intellectual property litigation to a greater degree and for longer periods of time than we could. In addition, patent holding companies that focus solely on extracting royalties and settlements by enforcing patent rights may target us.

We may be subject to third-party claims including infringement, interference or derivation proceedings, post-grant review and inter partes review before the USPTO or similar adversarial proceedings or litigation in other jurisdictions. Even if such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, and the holders of such patents may be able to block our ability to commercialize the applicable therapeutic candidate unless we obtained a license under the applicable patents, or until such patents expire or are finally determined to be invalid or unenforceable. There may be third-party patents or patent applications with claims to compositions, formulations, or methods of treatment, prevention use, or manufacture of our therapeutic candidates or technologies. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our therapeutic candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover aspects of our compositions, formulations, or methods of treatment, prevention or use, the holders of any such patents may be able to prohibit our use of those compositions, formulations, methods of treatment, prevention or use or other technologies, effectively blocking our ability to develop and commercialize the applicable therapeutic candidate until such patent expires or is finally determined to be invalid or unenforceable or unless we obtained a license.

For example, we are aware of issued patents by third parties in the United States and Europe, which are directed to oligonucleotide therapeutics including for the treatment of HAE. These patents could be alleged to cover our onvuzosiran or its use to treat HAE. We are also aware of an issued patent by a third party in the United States, directed to the GalNAc ligand conjugation technology, which could be alleged to cover one or more our therapeutic candidates. While we believe that these patents are subject to invalidity challenges, and if enforced against us, we believe we would have valid defenses to any claims of patent infringement, we cannot be certain, however, that we would prevail if a dispute were to arise. As such, we cannot be certain how an adverse determination would affect our business.

We may in the future pursue invalidity proceedings with respect to third party patents. The outcome following legal assertions of invalidity is unpredictable. Even if resolved in our favor, these legal proceedings may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such proceedings adequately. Some of these third parties may be able to sustain the costs of such proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent proceedings could compromise our ability to compete in the marketplace. If we do not prevail in the invalidity proceedings the third parties may assert a claim of patent infringement directed at our therapeutic candidates.

In addition, defending such claims would cause us to incur substantial expenses and, if such claims are successful, could cause us to pay substantial damages if we are found to be infringing a third party's patent rights. These damages potentially include increased damages (possibly treble damages) and attorneys' fees if we are found to have infringed such rights willfully. Further, if a patent infringement suit is brought against us or our third-party service providers, our development, manufacturing or sales activities relating to the product or therapeutic candidate that is the subject of the suit may be delayed or terminated, as parties making claims against us may obtain injunctive or other equitable relief. As a result of patent infringement claims, or in order to avoid potential infringement claims, we may choose to seek, or be required to seek, a license from the third party, which may require payment of substantial royalties or fees, or require us to grant a cross-license under our intellectual property rights. These licenses may not be available on reasonable terms or at all. Even if a license can be obtained on reasonable terms, the rights may be nonexclusive, which would give our competitors access to the same intellectual property rights. If we are unable to enter into a license on acceptable terms, we could be prevented from commercializing one or more of our therapeutic candidates, or forced to modify such therapeutic candidates, or to cease some aspect of our business operations, which could harm our business significantly.

We might also be forced to redesign or modify our therapeutic candidates so that we no longer infringe the third-party intellectual property rights, which may result in significant cost or delay to us, or which redesign or modification could be impossible or technically infeasible. Even if we were ultimately to prevail, any of these events could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business. Intellectual property litigation, regardless of its outcome, may cause

negative publicity, adversely impact prospective customers, cause product shipment delays, or prohibit us from manufacturing, importing, marketing or otherwise commercializing our products, services and technology. In addition, if the breadth or strength of protection provided the patents and patent applications we own is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future therapeutic candidates.

Competitors may infringe our patents or other intellectual property. If we were to initiate legal proceedings against a third party to enforce a patent covering one of our therapeutic candidates, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the United States and in Europe, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness, lack of written description, or non-enablement. Third parties might allege unenforceability of our patents because during prosecution of the patent, an individual connected with such prosecution withheld relevant information or made a misleading statement. Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. The outcome of proceedings involving assertions of invalidity and unenforceability during patent litigation is unpredictable. A court may decide that a patent right of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. With respect to the validity of patents, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution, but that an adverse third party may identify and submit in support of such assertions of invalidity. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our therapeutic candidate(s). Our patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without infringing our patents or other intellectual property rights.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors view these announcements in a negative light, the price of our common stock could be adversely affected. Such litigation or proceedings could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have an adverse effect on our ability to compete in the marketplace.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop, manufacture and market our therapeutic candidates.

We cannot guarantee that any of our patent searches or analyses, including but not limited to the identification of relevant patents, analysis of the scope of relevant patent claims or determination of the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States, Europe and elsewhere that is relevant to or necessary for the commercialization of our therapeutic candidates in any jurisdiction. For example, in the United States, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States, the European Union and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our therapeutic candidates could be filed by others without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our therapeutic candidates or the use of our therapeutic candidates. After issuance, the scope of patent claims remains subject to construction as determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to market our therapeutic candidates. We may incorrectly determine that our therapeutic candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States, the European Union or elsewhere that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our therapeutic candidates. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our therapeutic candidates, if approved.

If we fail to identify or correctly interpret relevant patents, we may be subject to infringement claims. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we fail in any such dispute, in addition to being forced to pay monetary damages, we may be temporarily or permanently prohibited from commercializing our therapeutic candidates.

If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing our therapeutic candidates and technology. Under any such license, we would most likely be required to pay various types of fees, milestones, royalties or other amounts. Moreover, we may not be able to obtain any required license on commercially reasonable terms or at all, and if such an instance arises, our ability to commercialize our therapeutic candidates may be impaired or delayed, which could in turn significantly harm our business. Parties making claims against us may also seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our therapeutic candidates. We might, if possible, also be forced to redesign our therapeutic candidates in a manner that no longer infringes third-party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

Changes in patent laws or patent jurisprudence could diminish the value of patents in general, thereby impairing our ability to protect our therapeutic candidates.

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and pharmaceutical industries involve both technological and legal complexity. Therefore, obtaining and enforcing biotechnology and pharmaceutical patents is costly, time-consuming and inherently uncertain. In addition, the Leahy-Smith America Invents Act ("AIA"), which was passed in September 2011, resulted in significant changes to the U.S. patent system.

An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned from a "first-to-invent" to a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application and be diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions.

Among some of the other changes introduced by the AIA are changes that limit where a patentee may file a patent infringement suit and provide opportunities for third parties to challenge any issued patent in the USPTO. This applies to all U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action.

Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. It is not clear what, if any, impact the AIA will have on the operation of our business. However, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

We may become involved in opposition, interference, derivation, inter partes review, post-grant review or other proceedings challenging our patent rights, and the outcome of any proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

In addition, on June 1, 2023, the European Union Patent Package ("EU Patent Package") regulations were implemented with the goal of providing a single pan-European Unitary Patent and a new European Unified Patent Court ("UPC") for litigation involving European patents. As a result, all European patents, including those issued prior to ratification of the EU Patent Package, now by default automatically fall under the jurisdiction of the UPC, unless otherwise opted out. It is uncertain how the UPC will impact granted European patents in the biotechnology and pharmaceutical industries. Our European patent applications, if issued, could be challenged in the UPC. During the first seven years of the UPC's existence, the UPC legislation allows a patent owner to opt its European patents out of the jurisdiction of the UPC. We may decide to opt out our future European patents from the UPC, but doing so may preclude us from realizing the benefits of the UPC. Moreover, if we do not meet all of the formalities and requirements for opt-out under the UPC, our current or future European patents could remain under the jurisdiction of the UPC. The UPC will provide our competitors with a new forum to centrally revoke our European patents, and allow for the possibility of a competitor to obtain pan-European injunction. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize our technology and our therapeutic candidates due to increased competition and, resultantly, on our business, financial condition, results of operations

and prospects. The UPC and Unitary Patent are significant changes in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation in the UPC.

Additionally, the U.S. Supreme Court has ruled on several patent cases in recent years either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations, and there are other open questions under patent law that courts have yet to decisively address. We cannot predict how decisions by the federal courts, the U.S. Congress or the USPTO may impact the value of our patent rights. For example, the Supreme Court of the United States held in *Amgen v. Sanofi* (2023) that a functionally claimed genus was invalid for failing to comply with the enablement requirement of the Patent Act. In addition, the Federal Circuit issued a decision, *In re Cellect, LLC* (2023), involving the interaction of patent term adjustment ("PTA"), terminal disclaimers, and obvious-type double patenting which may affect the patent term of any issued patents that rely on any PTA. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. In the 2013 case Assoc. for Molecular Pathology v. Myriad Genetics, Inc., for instance, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents. For example, the Inflation Reduction Act ("IRA") passed by Congress authorizes the Secretary of the Department of HHS to negotiate prices directly with participating manufacturers for selected medicines covered by Medicare even if these medicines are protected by an existing patent. For small molecule medicines, the process begins seven years after initial approval by the FDA. While we do not believe that the IRA or its effects will impact our ability to obtain patents in the near future, we cannot be certain whether it will affect our patent strategy in the long run. Additionally, in July 2025, the FDA began publicly releasing portions of Complete Response Letters ("CRLs") issued to drug and biologic sponsors, and in September 2025 the FDA announced that it would publish CRLs promptly after they are issued, including for applications that have not been approved. While the FDA has stated that confidential information will be protected, it remains unclear how the FDA will apply this practice over time. Because CRLs often contain specific observations about study design, clinical endpoints, chemistry, manufacturing, and controls ("CMC") data, or other proprietary information, any public release could unintentionally disclose information that competitors may use to infer proprietary aspects of our therapeutic candidates or platform technologies. This could compromise the confidentiality of our trade secrets and know-how or facilitate third-party efforts to design around or challenge the validity, enforceability, or scope of our patents, or accelerate the development of generics or biosimilars. If we are required to modify or limit the information shared with the FDA to mitigate such risks, it could increase costs, slow our regulatory interactions, or delay product approval timelines. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. In addition, the European patent system is relatively stringent in the type of amendments that are allowed during prosecution, but the complexity and uncertainty of European patent laws has also increased in recent years. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business.

In addition, the U.S. federal government retains certain rights in inventions produced with its financial assistance under the Patent and Trademark Law Amendments Act ("Bayh-Dole Act"). The federal government retains a "nonexclusive, nontransferable, irrevocable, paid-up license" for its own benefit. The Bayh-Dole Act also provides federal agencies with "march-in rights." March-in rights allow the government, in specified circumstances, to require the contractor or successors in title to the patent to grant a "nonexclusive, partially exclusive, or exclusive license" to a "responsible applicant or applicants." If the patent owner refuses to do so, the government may grant the license itself. If, in the future, we co-own or license in technology which is critical to our business that is developed in whole or in part with federal funds subject to the Bayh-Dole Act, our ability to enforce or otherwise exploit patents covering such technology may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO, European and other patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In addition, periodic maintenance and annuity fees on any issued patent are due to be paid to the USPTO, European and other patent agencies over the lifetime of the patent. While an inadvertent failure to make payment of such fees or to comply with such provisions can in many cases be cured by additional payment of a late fee or by other means in accordance with the applicable rules, there are situations in which non-compliance with such provisions will result in the abandonment or lapse of the patent or patent application, and the partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents within prescribed time limits. If we fail to maintain the patents and patent applications covering our therapeutic candidates or if we otherwise allow our patents or patent applications to be abandoned or lapse, it can create opportunities for competitors to enter the market, which

would hurt our competitive position and could impair our ability to successfully commercialize our therapeutic candidates in any indication for which they are approved.

We enjoy only limited geographical protection with respect to certain patents, and we may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents covering our therapeutic candidates in all countries throughout the world would be prohibitively expensive, and even in countries where we have sought protection for our intellectual property, such protection can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly developing countries, and the breadth of patent claims allowed can be inconsistent. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. In-licensing patents covering our therapeutic candidates in all countries throughout the world may similarly be prohibitively expensive, if such opportunities are available at all. And in-licensing or filing, prosecuting and defending patents even in only those jurisdictions in which we develop or commercialize our therapeutic candidates may be prohibitively expensive or impractical. Competitors may use our technologies in jurisdictions where we have not obtained patent protection or licensed patents to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as that in the United States or the European Union. These products may compete with our therapeutic candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications while they are still pending. The grant proceeding of each national or regional patent is an independent proceeding which may lead to situations in which applications may be rejected by the relevant patent office, while substantively similar applications are granted by others. For example, relative to other countries, China has a heightened requirement for patentability and specifically requires a detailed description of medical uses of a claimed drug. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our patents, requiring us to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for and launch generic versions of our products. It is also quite common that depending on the country, the scope of patent protection may vary for the same therapeutic candidate or technology.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or regulations in the United States and the European Union, and many companies have encountered significant difficulties in protecting and defending proprietary rights in such jurisdictions. Moreover, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets or other forms of intellectual property, particularly those relating to biotechnology products, which could make it difficult for us to prevent competitors in some jurisdictions from marketing competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, are likely to result in substantial costs and divert our efforts and attention from other aspects of our business, and additionally could put at risk our patents of being invalidated or interpreted narrowly, could increase the risk of our patent applications not issuing, or could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, while damages or other remedies may be awarded to the adverse party, which may be commercially significant. If we prevail, damages or other remedies awarded to us, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop. Furthermore, while we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our therapeutic candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our therapeutic candidates in all of our expected significant foreign markets. If we encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished and we may face additional competition in those jurisdictions.

In some jurisdictions including European Union countries, compulsory licensing laws compel patent owners to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we are forced to grant a license to third parties under patents relevant to our business, or if we are prevented from enforcing patent rights against third parties, our competitive position may be substantially impaired in such jurisdictions.

Moreover, geo-political actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. For example, the United States and foreign government actions related to Russia's conflict with Ukraine may limit or prevent filing, prosecution, and maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of any future patents or patent applications that we may file in Russia, resulting in partial or complete loss of patent rights in

Russia. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These products may compete with our therapeutic candidates and our patents or other IP rights may not be effective or sufficient to prevent them from competing.

Further, many foreign countries could threaten to impose retaliatory measures that may adversely impact our intellectual property rights in those countries. For example, on March 14, 2025, Brazil enacted Law No. 15. 122/2025 (known as the Economic Reciprocity Law), which provides a framework that allows for the suspension of obligations related to foreign entities' intellectual property rights. Additionally, changes in U.S. trade policy, including the imposition of new or increased tariffs as well as retaliatory measures by other countries, could adversely affect our patent strategy, such as where we choose to file, maintain, or enforce our patents. Also, if we are required to move our research or manufacturing activities to new regions, this may expose us to jurisdictions with weaker intellectual property enforcement, differing patent eligibility standards, or greater risk of compulsory licensing. These factors could compromise the protection or value of our proprietary technologies, including our core patents and related know-how.

Patent terms may not protect our competitive position on our therapeutic candidates for an adequate amount of time.

The term of any individual patent depends on applicable law in the country where the patent is granted. In the United States, provided all maintenance fees are timely paid, a patent generally has a term of 20 years from its application filing date or earliest claimed non-provisional filing date. Extensions may be available under certain circumstances, but the life of a patent and, correspondingly, the protection it affords is limited. We may also be required to disclaim a portion of a patent term in order to overcome double patenting rejections from the patent office, thus potentially shortening our exclusivity period. Even if we obtain patents covering our therapeutic candidates, when the terms of all patents covering a product expire, our business may become subject to competition from competitive products, including generic versions of our products. Given the amount of time required for the development, testing and regulatory review and approval of new therapeutic candidates, patents protecting such candidates may expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of marketing exclusivity for our therapeutic candidates, our business may be harmed.

In the United States, a patent that covers an FDA-approved drug or biologic may be eligible for a term extension designed to restore the period of the patent term that is lost during the premarket regulatory review process conducted by the FDA. Depending upon the timing, duration and conditions of FDA marketing approval of our therapeutic candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984 ("Hatch-Waxman Act"), which permits a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, and only claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. In the European Union, patents covering our therapeutic candidates may be eligible for term extensions based on similar legislation. In either jurisdiction, however, we may not receive a patent term extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Even if we are granted such extension, the duration of such extension may be less than our request. If we are unable to obtain a patent term extension, or if the length of any such extension is less than our request, the period during which we can enforce our patent rights for that product will be in effect shortened and our competitors may obtain approval to market competing products sooner. The resulting reduction in revenue from applicable products could be substantial.

Our proprietary rights may not adequately protect our technologies and therapeutic candidates, and do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are the same as or similar to our therapeutic candidates but that are not covered by the claims of the patents that we own, will own or will license;

- others, including inventors or developers of our patented technologies who may become involved with competitors, may independently develop similar technologies that function as alternatives or replacements for any of our technologies without infringing our intellectual property rights;
- we might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, will own, or will license;
- we might not have been the first to file patent applications covering certain inventions of the patents or patent applications that we own, or will own or will license;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our patents;
- issued patents that we own may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- ownership, validity or enforceability of our patents or patent applications may be challenged by third parties; and
- the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patent protection for certain aspects of our technology, we also consider trade secrets, including confidential and unpatented know-how, important to the maintenance of our competitive position. We protect trade secrets and confidential and unpatented know-how, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to such knowledge, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants that obligate them to maintain confidentiality and assign their inventions to us. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or technology and processes or that the assignment agreements that have been entered into are self-executing. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, or claim ownership in intellectual property that we believe is owned by us. Monitoring unauthorized uses and disclosures of our intellectual property is difficult, and we do not know whether the steps we have taken to protect our intellectual property will be effective. In addition, we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts in the United States and certain foreign jurisdictions are less willing or unwilling to protect trade secrets.

Moreover, our competitors or other third parties may independently develop knowledge, methods and know-how equivalent to our trade secrets or seek to reverse engineer our technology for which we do not have patent protection. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third parties, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed. We may need to share our proprietary information, including trade secrets, with our current and future business partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or foreign actors, and those affiliated with or controlled by state actors. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. The failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We are also subject both in the United States and outside the United States to various regulatory schemes regarding requests for the information we provide to regulatory authorities, which may include, in whole or in part, trade secrets or confidential commercial information. While we are likely to be notified in advance of any disclosure of such information and would likely object to such disclosure, there can be no assurance that our challenge to the request would be successful. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our unregistered trademarks or trade names. Over the long term, if we are unable to successfully register our trademarks and trade names and establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

We may need to license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

The growth of our business may depend in part on our ability to acquire or in-license proprietary rights. For example, our programs may involve therapeutic candidates that may require the use of proprietary rights held by third parties. Our therapeutic candidates may also require specific formulations to work effectively and efficiently.

These formulations may be covered by intellectual property rights held by others. We may develop products containing our compositions and pre-existing pharmaceutical compositions. These pharmaceutical products may be covered by intellectual property rights held by others. We may be required by the FDA, EMA or other foreign regulatory authorities to provide a companion diagnostic test or tests with our therapeutic candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights and may need to seek to develop alternative approaches that do not infringe on such intellectual property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license under such intellectual property rights, any such license may be non-exclusive, which may allow our competitors access to the same technologies licensed to us.

We may in the future enter into license agreements with others that may advance our existing or future research or allow commercialization of our existing or future therapeutic candidates. These licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future.

In addition, subject to the terms of any such license agreements, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. In such an event, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our future licensors fail to prosecute, maintain, enforce, and defend such patents or patent applications, or lose rights to those patents or patent applications, the rights we may license may be reduced or eliminated, and our right to develop and commercialize any of our therapeutic candidates that are subject of such licensed rights could be adversely affected.

Our future licensors may rely on third-party consultants or collaborators or on funds from third parties such that our future licensors are not the sole and exclusive owners of the patents we in-license. If other third parties have ownership rights to our future in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

It is possible that we may be unable to obtain licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, therapeutic candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected therapeutic candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, manufacturing methods, therapeutic candidates, or future

methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Disputes may arise between us and our future licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patents and other rights to third parties;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- our right to transfer or assign the license;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we may license intellectual property or technology from third parties may be complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we license in the future prevent or impair our ability to maintain our licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected therapeutic candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

In spite of our best efforts, our future licensors might conclude that we materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

From time to time, we may be required to license technologies relating to our therapeutic research programs from additional third parties to further develop or commercialize our therapeutic candidates. Should we be required to obtain licenses to any third-party technology, including any such patents required to manufacture, use or sell our therapeutic candidates, such licenses may not be available to us on commercially reasonable terms, or at all. The inability to obtain any third-party license required to develop or commercialize any of our therapeutic candidates could cause us to abandon any related efforts, which could seriously harm our business and operations.

Any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our products or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or therapeutic candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;

- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our therapeutic candidates or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable therapeutic candidates;
- collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and
- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third parties.

We do and may employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, consultants, collaborators, independent contractors and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us and to not use the know-how or confidential information of their former employer or other third parties, we may be subject to claims that we or our employees, consultants, collaborators or independent contractors have inadvertently or otherwise used or disclosed know-how or confidential information of their former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable personnel or intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property, which could result in customers seeking other sources for the technology, or in ceasing from doing business with us. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or therapeutic candidate. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful, litigation could result in substantial cost and reputational loss and be a distraction to our management and other employees. Moreover, any such litigation or the threat thereof may adversely affect our reputation, our ability to form strategic alliances or sublicense our rights to collaborators, engage with scientific advisors or hire employees or consultants, each of which would have an adverse effect on our business, results of operations and financial condition.

We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our therapeutic candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Our use of open source software could impose limitations on our ability to commercialize our therapeutic candidates.

We may use open source software in the future that contains modules licensed for use from third-party authors under open source licenses. Some of the software may be provided under license arrangements that allow use of the software for research or other

non-commercial purposes. As a result, in the future, as we seek to use our platform in connection with commercially available products, we may be required to license that software under different license terms, which may not be possible on commercially reasonable terms, if at all. If we are unable to license software components on terms that permit its use for commercial purposes, we may be required to replace those software components, which could result in delays, additional cost and/or additional regulatory approvals.

Use and distribution of open source software may entail greater risks than use of third-party commercial software, as open source licensors generally do not provide warranties or other contractual protections regarding infringement claims or the quality of the software code. Some open source licenses contain requirements that we make available source code for modifications or derivative works we create based upon the type of open source software we use. If we combine our proprietary software with open source software in a certain manner, we could, under certain of the open source licenses, be required to release the source code of our proprietary software to the public. This could allow our competitors to create similar products with lower development effort and time, and ultimately could result in a loss of product sales for us. Although we monitor our use of open source software, the terms of many open source licenses have not been interpreted by U.S. courts, and there is a risk that those licenses could be construed in a manner that could impose unanticipated conditions or restrictions on our ability to commercialize our therapeutic candidates. We could be required to seek licenses from third parties in order to continue offering our therapeutic candidates, to re-engineer our therapeutic candidates or to discontinue the sale of our therapeutic candidates in the event re-engineering cannot be accomplished on a timely basis, any of which could materially and adversely affect our business, financial condition, results of operations and prospects.

Risks related to our industry and business

We are subject to various federal, state and foreign healthcare laws and regulations, which could increase compliance costs, and our failure to comply with these laws and regulations could harm our results of operations and financial condition.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors and customers expose us to broadly applicable foreign, federal and state fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute any products for which we obtain marketing approval. Such laws include:

- the federal Anti-Kickback Statute prohibits, among other things, individuals or entities from knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, overtly or covertly, in cash or in kind to induce or in return for purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;
- the federal civil False Claims Act, prohibits, among other things, any individual or entity from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment of government funds, or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, a claim that includes items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”), created additional federal criminal statutes that prohibit, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA and its implementing regulations, impose obligations on covered entities, including certain healthcare providers, health plans, and healthcare clearinghouses, and their respective business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity as well as their covered subcontractors, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.
- the federal Civil Monetary Penalties Law, which prohibits, among other things, the submission of false or fraudulent claims, the employment or contracting of an excluded individual or entity, and offering or transferring remuneration to a federal healthcare beneficiary that a person knows or should know is likely to influence the beneficiary’s selection of a particular provider, practitioner, or supplier of reimbursable items or services.
- the federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services (“CMS”) information related to payments or other transfers of value made during the previous year to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors),

certain other health care providers including, among others, physician assistants and nurse practitioners, and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held during the previous year by such physicians and their immediate family members;

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers;
- some state laws require biotechnology companies to comply with the biotechnology industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and
- some state and local laws require identification or licensing of pharmaceutical sales representatives; and some state laws that require biotechnology companies to report information on the pricing of certain drug products.

A more detailed description of these laws can be found in the "Other U.S. healthcare laws and compliance requirements" section of "Business—Government regulation" in the Prospectus. Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare laws and regulations will involve ongoing substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings and the curtailment or restructuring of our operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending ourselves against any such actions that may be brought against us, our business may be impaired. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare program.

Recently enacted legislation, future legislation and healthcare reform measures may increase the difficulty and cost for us to commercialize our therapeutic candidates and may affect the prices we may set.

In the United States and some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system, including cost-containment measures that may reduce or limit coverage and reimbursement for newly approved drugs and affect our ability to profitably sell any therapeutic candidates for which we obtain marketing approval. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare.

For example, in March 2010, the Patient Protection and Affordable Care Act ("ACA") was enacted in the United States, which substantially changed the way healthcare is financed by both governmental and private insurers, expanded access to health insurance, and significantly affected the pharmaceutical industry.

Since its enactment, there have been executive, judicial and Congressional challenges and amendments to certain aspects of the ACA. For example, on July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law, which narrowed access to ACA marketplace exchange enrollment and declined to extend the ACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired ACA subsidies. It is possible that the ACA will be subject to additional challenges in the future. It is unclear how such challenges and the healthcare reform measures of the current administration will impact the ACA.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2032. The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at HHS, the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with pharmaceutical companies that require the drug manufacturers to offer, through a direct-to-consumer platform (TrumpRx), U.S. patients and Medicaid programs prescription drug

Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives; (3) imposing tariffs on certain imported pharmaceutical products, including an additional duty of up to 100% on many imported patented pharmaceutical products and their ingredients under Section 232 of the Trade Expansion Act of 1962, which became fully effective on September 29, 2026, subject to exclusions, including for articles imported solely for use in clinical trials or research and development; and (4) as part of the Make America Healthy Again Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager payment methodologies, among other things. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In June 2024, in Loper Bright Enterprises v. Raimondo, the U.S. Supreme Court greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

Individual states in the United States have also become increasingly active in implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Some states have also enacted legislation creating so-called prescription drug affordability boards, which ultimately may attempt to impose price limits on certain drugs in these states. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine which drugs and suppliers will be included in their healthcare programs. Furthermore, there has been increased interest by third party payors and governmental authorities in reference pricing systems and publication of discounts and list prices.

We expect that these new laws and other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare reimbursement and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our therapeutic candidates, if approved.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our products.

We face an inherent risk of product liability as a result of the clinical trials of our therapeutic candidates and will face an even greater risk if we commercialize our therapeutic candidates for which we obtain marketing approval. For example, we may be sued if our therapeutic candidates allegedly cause injury or are found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the therapeutic candidate, negligence, strict liability and a breach of warranties. Claims may be brought against us by clinical trial participants, patients or others using, administering or selling products that may be approved in the future. Claims could also be asserted under state consumer protection acts.

If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit or cease the commercialization of our products. Even a successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our products;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- a diversion of our management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;

- significant negative financial impact;
- the inability to commercialize our therapeutic candidates; and
- a decline in our stock price.

We currently hold approximately $5.0 million in product liability insurance coverage in the aggregate. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our therapeutic candidates. Insurance coverage is increasingly expensive. Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of our therapeutic candidates. Although we will maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies will also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

We and any of our current or future collaborators will be required to report to regulatory authorities if any of our approved products cause or contribute to adverse medical events, and any failure to do so would result in sanctions that would materially harm our business.

If we or any of our current or future collaborators are successful in commercializing our products, the FDA and foreign regulatory authorities would require that we and such collaborators report certain information about adverse medical events if those products may have caused or contributed to those adverse events. The timing of our obligation to report would be triggered by the date we become aware of the adverse event as well as the nature of the event. We, our CROs and any of our current or future collaborators may fail to report adverse events within the prescribed timeframe. If we, our CROs or any of our current or future collaborators fail to comply with such reporting obligations, the FDA or a foreign regulatory authority could take action, including criminal prosecution, the imposition of civil monetary penalties, seizure of our products or delay in approval or clearance of future products.

We will need to expand our organization, and we may experience challenges in managing this growth as we build our capabilities, which could disrupt our operations.

As of September 30, 2026, we had 106 full-time employees. We will need to expand our organization, and we may have difficulty identifying, hiring and integrating new personnel. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of therapeutic candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our therapeutic candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Our future success depends on our ability to retain our key personnel and to attract, retain and motivate qualified personnel.

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on our executive officers, as well as the other members of our management, scientific and clinical teams. Although we have formal employment agreements with our executive officers and key employees, these agreements do not prevent them from terminating their employment with us at any time and, for certain of our executive officers, entitle them to receive severance payments in connection with their voluntary resignation of employment for good reason, as defined in the employment agreements. Additional details regarding these arrangements can be found in the section titled “Executive and director compensation—Employment arrangements with our named executive officers” in the Prospectus.

If we lose one or more of our executive officers or key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing our executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of highly specialized skills and experience required to develop, gain regulatory approval of and commercialize our therapeutic candidates successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel on acceptable terms given the

competition among numerous biopharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be engaged by entities other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to advance the clinical development of and commercialize therapeutic candidates will be limited.

We and the third parties with whom we work are subject to stringent and evolving U.S. and foreign laws, regulations, and rules, contractual obligations, industry standards, policies and other obligations related to data privacy and security. Our (or the third parties with whom we work) actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation (including class claims) and mass arbitration demands; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences.

In the ordinary course of business, we, and third parties with whom we work, collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive information, including data we collect about trial participants in connection with clinical trials, biometric information, and other sensitive data (collectively, sensitive information). The global data protection landscape is rapidly evolving, and our data processing activities subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). For example, at the federal level, HIPAA imposes specific requirements relating to the privacy, security, and transmission of individually identifiable protected health information. We may obtain health information from third parties, such as research institutions with which we collaborate, that are subject to privacy and security requirements under HIPAA. Although we are not directly subject to HIPAA, we could potentially be subject to criminal penalties if we, our affiliates, or our agents knowingly receive individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA, and subject to other civil and/or criminal penalties if we obtain, use, or disclose information in a manner not permitted by other privacy and data security and consumer protection laws.

Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices, affording residents with certain rights concerning their personal data and requiring covered business to enter into contracts with their service providers that process personal data. As applicable, such rights may include the right to know, access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, certain profiling, and certain automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments or requiring opt-in consent from a consumer. These state laws may allow for statutory fines for noncompliance and empower the state attorneys general to enforce. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act (collectively, the "CCPA"), applies to personal information of consumers, business representatives, and employees who are California residents, and requires regulated businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. The CCPA provides for significant fines for intentional violation and allows private litigants affected by certain data breaches to recover significant statutory damages, allows for enforcement by the California attorney general, and created the California Privacy Protection Agency, which can bring administrative enforcement actions. The CCPA and other comprehensive U.S. state privacy laws exempt some data processed in the context of clinical trials, but these developments may further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties with whom we work. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future. Health-specific consumer privacy laws were also passed in multiple states, including Washington and Nevada. For example, Washington State enacted the Washington My Health My Data Act, which broadly defines consumer health data, creates a private right of action to allow individuals to sue for violations of the law, imposes stringent consent requirements, and grants consumers certain rights with respect to their health data, including to request deletion of their information. These laws and regulations are constantly evolving and may impose limitations on our business activities. We use identity verification technologies in connection with employee timekeeping that may subject us to biometric privacy laws.

Outside the United States, an increasing number of laws, regulations, and industry standards govern data privacy and security. For example, the European Union's General Data Protection Regulation ("EU GDPR"), the United Kingdom's Data Protection Act 2018 and the EU GDPR as retained in UK law pursuant to the European Union (Withdrawal) Act 2018 (UK GDPR) (collectively, GDPR), Australia's Privacy Act, and China's Personal Information Protection Law ("PIPL") impose strict requirements for processing personal data. For example, under the GDPR, companies may face temporary or definitive bans on data processing and other corrective actions;

fines of up to 20 million euros under the EU GDPR, 17.5 million pounds sterling under the UK GDPR or, in each case, 4% of worldwide annual turnover of the preceding financial year, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests. In Canada, the Personal Information Protection and Electronic Documents Act ("PIPEDA") and various related provincial laws, as well as Canada's Anti-Spam Legislation ("CASL"), apply to our operations. The Swiss Federal Act on Data Protection ("FADP") also applies to the collection and processing of personal data, including health-related information, by companies located in Switzerland, or in certain circumstances, by companies located outside of Switzerland.

In the EU, there is a strong focus on cyber resilience with multiple laws regulating resilience and incident response capabilities that directly impact pharmaceutical companies, including the EU Network and Information Security Directive ("NIS2") which applies to entities carrying out research and development activities of medicinal products and other healthcare sector activities. This imposes obligations to have in place cybersecurity risk management measures, including incident response planning, supply chain security, encryption, and access controls, and timely reporting of significant cyber incidents to national authorities. Non-compliance with NIS2 may result in administrative fines up to at least €10 million or 2% of the total worldwide annual turnover of the preceding fiscal year (whichever is higher) for entities classified as "essential," and up to at least €7 million or 1.4% of such turnover (whichever is higher) for entities classified as "important." Notably, senior management can be held personally liable for cybersecurity failures.

Our employees and personnel use artificial intelligence ("AI") to perform their work, and the use of personal data in AI technologies is subject to evolving AI-specific regulations and various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws and regulations regulating AI and/or automated decision-making technologies. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. Compliance with AI laws and regulations will likely be an increasing and substantial cost in the future.

In the ordinary course of business, we transfer personal data from Europe and other jurisdictions to the United States or other countries. The EU and other jurisdictions have enacted laws requiring safeguards for the transfer of personal data to other countries. In particular, the EU and other member states of the European Economic Area ("EEA") and the United Kingdom ("UK") require such safeguards for the transfer of personal data to the United States and other countries whose privacy laws they generally believe are inadequate. Other jurisdictions may adopt or have already adopted similarly stringent cross-border data transfer laws.

There are currently various mechanisms that we could use to lawfully transfer personal data from the EEA and UK to the United States in compliance with law, such as the EU standard contractual clauses, the UK's International Data Transfer Agreement / Addendum. We may also in the future use the EU-U.S. Data Privacy Framework and the UK extension thereto (which allow for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework). These mechanisms can be subject to legal challenges, and there is no assurance that we can satisfy or continue to rely on these measures to lawfully transfer personal data to the United States.

If there is no lawful manner for us to transfer personal data from the EEA, the UK or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as in the EEA or UK) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from European regulators, individual litigants, and activist groups. Some EU regulators have ordered certain companies to suspend or permanently cease certain transfers out of the EU for allegedly violating the GDPR's cross-border data transfer limitations.

Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons ("DSP"), which places additional restrictions on certain data transactions involving access to bulk U.S. sensitive personal data or government-related data by countries of concern (i.e., China, Russia, Iran, North Korea, Venezuela and Cuba) and covered persons that may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to transfer data in connection with certain transactions or agreements. Non-compliance with the DSP could result in significant civil or criminal penalties, which could materially adversely affect our business, results of operations, and financial condition.

The Federal Trade Commission ("FTC") also sets expectations for taking appropriate steps to safeguard consumers' personal information, and providing a level of privacy or security commensurate to promises made to individuals. The FTC expects a company's data privacy and security measures to be reasonable and appropriate in light of the sensitivity and volume of customer information it

holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Failure to meet these standards, or failure to comply with a company's own privacy commitments to individuals, may constitute unfair or deceptive acts or practices in violation of Section 5 of the FTC Act. The FTC also has the power to enforce the Health Breach Notification Rule, which imposes notification obligations on companies for breaches of certain health information contained in personal health records. Enforcement by the FTC under the FTC Act and Health Breach Notification Rule can result in civil penalties or enforcement actions.

In addition to data privacy and security laws, we are subject to industry standards and, we are, and may become in the future, subject to additional obligations. We are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. For example, certain privacy laws, such as the GDPR and the CCPA, to the extent they apply, require us to impose specific contractual restrictions on companies that process data at our direction (e.g., our service providers). We also publish privacy policies, marketing materials, whitepapers, and other statements concerning data privacy and security. Regulators in the United States and Europe are increasingly scrutinizing these statements, and if these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, or misrepresentative of our practices, we may be subject to investigation or enforcement actions by regulators or experience other adverse consequences.

Obligations related to data privacy and security are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources, which may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf. Our business model materially depends on our ability to process personal data through our CROs, so we are particularly exposed to the risks associated with the rapidly changing legal landscape. For example, we may be at heightened risk of regulatory scrutiny, and any changes in the regulatory framework could require us to fundamentally change our business model.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties with whom we work fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans or restrictions on processing personal data; orders to destroy or not use personal data; and imprisonment of company officials.

Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: loss of customers; interruptions or stoppages in our business operations (including, as relevant, clinical trials); inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations.

If our information technology systems, the third parties with whom we work or our data, are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse consequences.

In the ordinary course of our business, we and the third parties with whom we work process personal information, sensitive information, and other confidential information, including proprietary and confidential business data, trade secrets, intellectual property, and sensitive third-party data. Cyber-attacks, malicious internet-based activity, online and offline fraud, and other similar activities threaten the confidentiality, integrity, availability, and security of our personal, sensitive, and confidential information and information technology systems, and those of the third parties with whom we work. Such threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer "hackers," threat actors, "hacktivists," organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors.

Some actors now engage and are expected to continue to engage in cyber-attacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties with whom we work may be vulnerable to a heightened risk of these attacks, including retaliatory cyber-attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services.

We and the third parties with whom we work are subject to a variety of evolving threats, including but not limited to social-engineering attacks (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks, credential stuffing attacks, credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks,

software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, earthquakes, fires, floods, attacks enhanced or facilitated by AI, and other similar threats.

In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

It may be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks. There can also be no assurance that our and our third-party service providers', strategic partners', contractors', consultants', CROs' and collaborators' cybersecurity risk management program and processes, including policies, controls or procedures, will be fully implemented, complied with or effective in protecting our systems, networks and Confidential Information.

Remote work has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations. Additionally, any integration of artificial intelligence in our or any third party's operations, products or services is expected to pose new or unknown cybersecurity risks and challenges.

Future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

We rely on third parties to operate critical business systems to process personal, sensitive, and confidential information in a variety of contexts, including, without limitation, cloud-based infrastructure, data center facilities, encryption and authentication technology, employee email, content delivery to customers, and other functions, including in connection with our clinical trial activities. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If the third parties with whom we work experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if the third parties with whom we work fail to satisfy their privacy or security-related obligations owed to us, any award may be insufficient to cover our damages, we may be unable to recover such award, or we may be alleged to have failed to monitor such parties in ways or manners that comply with applicable data privacy laws. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or that of the third parties with whom we work have not been compromised.

While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties with whom we work). We have not and may not in the future, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we may experience delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident.

Any of the previously identified or similar threats have in the past and may in the future cause a security incident or other interruption that have in the past and may in the future result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our personal, sensitive, and confidential information or our information technology systems, or those of the third parties with whom we work. A security incident or other interruption could disrupt our ability (and that of third parties with whom we work) to provide our products.

We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive information.

In the EU, the NIS2 regulates resilience and incident response capabilities of entities operating in a number of sectors, including the health sector. Non-compliance with NIS2 may result in administrative fines up to at least €10 million or 2% of the total worldwide annual turnover of the preceding fiscal year (whichever is higher) for entities classified as "essential," and up to at least €7 million or

1.4% of such turnover (whichever is higher) for entities classified as "important." Notably, senior management can be held personally liable for cybersecurity failures.

Applicable data privacy and security obligations may require us, or we may voluntarily choose, to notify (or cause our third party service provides to notify) relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents, or to take other actions, such as providing credit monitoring and identity theft protection services. Such disclosures and related actions can be costly, and the disclosure or the failure to comply with such applicable requirements could lead to adverse consequences.

If we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, we may experience material adverse consequences, such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing personal data, including sensitive information; litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; diversion of management attention; interruptions in our operations (including availability of data); financial loss; and other similar harms.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

In addition to experiencing a security incident, third parties may gather, collect, or infer information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, our personal sensitive or confidential information could be leaked, disclosed, or revealed as a result of or in connection with our employees', personnel's, or vendors' use of generative AI technologies.

Our business could be affected by litigation, government investigations, and enforcement actions.

We currently operate in a number of jurisdictions in a highly regulated industry and we could be subject to litigation, government investigation and enforcement actions on a variety of matters in the United States or foreign jurisdictions, including, without limitation, intellectual property, regulatory, product liability, environmental, whistleblower, false claims, privacy, anti-kickback, anti-bribery, securities, commercial, breach of contract, employment and other claims and legal proceedings which may arise from conducting our business. Any determination that our operations or activities are not in compliance with existing laws or regulations could result in the imposition of fines, civil and criminal penalties, equitable remedies, including disgorgement, injunctive relief and/or other sanctions against us, and remediation of any such findings could have an adverse effect on our business operations.

Defense from and participation in legal proceedings, government investigations and enforcement actions can be expensive and time consuming. An adverse outcome resulting from any such proceeding, investigations or enforcement actions could result in significant damages awards, fines, penalties, exclusion from the federal healthcare programs, debarment, injunctive relief, product recalls, reputational damage and modifications of our business practices, which could have a material adverse effect on our business and results of operations.

We are subject to U.S. and certain foreign laws and regulations regarding anti-corruption, anti-bribery, export and import controls, sanctions and embargoes. We could face liability and other serious consequences for violations which can harm our business.

We are subject to anti-corruption laws and regulations, including the U.S. Foreign Corrupt Practices Act of 1977, as amended ("FCPA"), the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act and other state and national anti-bribery laws in the countries in which we may conduct activities in the future. Anti-corruption laws are interpreted broadly and generally prohibit companies and their employees, agents, contractors and other third-party collaborators from offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly through third parties, to any person in the public or private sector to obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. We may engage third parties to sell our products or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals outside the United States. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violation of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

We are also subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls. Compliance with applicable regulatory requirements regarding the export of our products may create delays in the introduction of our products in international markets or, in some cases, prevent the export of our products to some countries altogether.

Furthermore, U.S. export control laws and economic sanctions prohibit the provision of certain products and services to countries, governments and persons targeted by U.S. sanctions.

There is no certainty that all of our employees, agents, suppliers, manufacturers, contractors or collaborators will comply with all applicable anti-corruption, export and import control, and sanctions laws and regulations. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers, or our employees, the closing down of facilities, including those of our suppliers and manufacturers, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries as well as difficulties in manufacturing or continuing to develop our products, and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, and our business, prospects, operating results and financial condition.

Our employees and independent contractors, including principal investigators, CROs, consultants and vendors, may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk that our employees and independent contractors, including principal investigators, CROs, consultants and vendors may engage in misconduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violate: (i) the laws and regulations of the FDA and other similar regulatory requirements, including those laws that require the reporting of true, complete and accurate information to such authorities, (ii) manufacturing standards, including cGMP requirements, (iii) federal and state data privacy, security, fraud and abuse and other healthcare laws and regulations in the United States and abroad or (iv) laws that require the true, complete and accurate reporting of financial information or data. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, the creation of fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in clinical holds, denial of marketing applications, withdrawal of regulatory approvals, regulatory sanctions, and criminal penalties, and could cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a noncompliance with applicable laws or regulations. In addition, we are subject to the risk that a person or government could allege or suspect fraud or other misconduct, even if none occurred. If any allegations or resulting investigations are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and financial results, including, without limitation, the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Some of our manufacturers and suppliers are located in China, and U.S.-China trade tensions and restrictions on certain biotechnology companies could adversely affect our supply chain and operations.

Some of our manufacturers, suppliers and other third-party service providers are located in China. Trade tensions and geopolitical conflicts between the United States and China have escalated in recent years, and changes in laws, regulations, sanctions, export controls, tariffs (including uncertainty related to the enforceability thereof), import restrictions or other policies adopted by the United States, China or other jurisdictions could disrupt our supply chain, increase our costs, delay our development or manufacturing activities, or otherwise adversely affect our operations.

In addition, certain Chinese biotechnology companies and related service providers may become subject to U.S. trade restrictions, sanctions, or other regulatory limitations. For example, the U.S. BIOSECURE Act, enacted as part of the Fiscal Year 2026 National Defense Authorization Act, restricts U.S. government agencies from procuring or obtaining certain biotechnology equipment or services from designated BCCs, and from entering into, extending or renewing certain contracts with entities that use such equipment or services in the performance of U.S. government contracts. The BIOSECURE Act also restricts the use of certain federal funds for biotechnology equipment or services provided by BCCs. In addition, in October 2026, the U.S. Department of the Treasury proposed regulations implementing the Comprehensive Outbound Investment National Security Act of 2025 that would, among other things, prohibit U.S.

persons from investing in, or entering into joint ventures with, companies in China and certain other countries of concern that engage in a broad range of biotechnology activities. If adopted as proposed, these regulations could limit our ability to establish or expand operations in China and require us to modify or forgo our current or future activities in China.

It is possible some of our manufacturers, suppliers and other third-party service providers could be impacted by the legislation described above. If this occurs, we may be required to qualify alternative vendors, transfer activities to other facilities or service providers, amend or terminate existing arrangements, conduct additional development or validation work, or implement additional compliance controls, any of which could increase our costs, disrupt our operations, delay our development, and adversely affect our business and operations.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

Our ability to utilize our net operating loss ("NOL") carryforwards and certain other tax attributes depends on many factors, including our future income, which cannot be assured, and the impact of any tax reform legislation or proposals. Under current law, U.S. federal NOLs generated in tax years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such NOL carryforwards is limited to 80% of taxable income. It is uncertain if and to what extent various states will conform to U.S. federal income tax law.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the "Code"), and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50 percentage point change, by value, in its equity ownership by "5-percent shareholders" over a three-year period, the corporation's ability to use its pre-change NOL carryforwards, research and development credits and other tax attributes to offset its post-change income or taxes, as applicable, may be limited. We have not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. We may experience ownership changes as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our NOL carryforwards or research and development credits is materially limited, it would harm our future results of operations by effectively increasing our future tax obligations.

We have recorded a full valuation allowance related to our NOL carryforwards and other deferred tax assets due to the uncertainty of the ultimate realization of the future benefits of those assets.

We may be subject to adverse legislative or regulatory changes in tax laws, and there are uncertainties in the interpretation and application of existing, new and proposed tax laws and regulations, any of which could materially affect our tax obligations and effective tax rate.

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the U.S. Internal Revenue Service ("IRS") and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect our stockholders or us. In recent years, the Tax Cuts and Jobs Act, the Coronavirus Aid, Relief, and Economic Security Act, the Inflation Reduction Act and the OBBBA made many significant changes to U.S. tax laws. For example, the Tax Cuts and Jobs Act amended the Code to require that certain research and experimental expenditures be capitalized and amortized over five years if incurred in the United States or fifteen years if incurred in foreign jurisdictions for tax years beginning after December 31, 2021. However, the OBBBA eliminated the requirement to capitalize and amortize research and experimental expenditures incurred in the United States. Although the U.S. Congress has considered legislation that would defer, modify, or repeal the capitalization and amortization requirement for foreign research or experimental expenditures, there is no assurance that such changes will be made. If the requirement is not deferred, repealed, or otherwise modified, it may increase our cash taxes and effective tax rate. In addition, it is uncertain if and to what extent various states will conform to current federal tax law, or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of NOL carryforwards and other deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses could have a material impact on the value of our deferred tax assets and could increase our future tax expense.

Investors' expectations of our performance relating to environmental, social and governance factors may impose additional costs and expose us to new risks.

There is an increasing focus from certain investors, employees and other stakeholders concerning corporate responsibility, specifically related to environmental, social and governance factors. Some investors may use these factors to guide their investment strategies and, in some cases, may choose not to invest in us if they believe our policies relating to corporate responsibility are inadequate. Third-party providers of corporate responsibility ratings and reports on companies have increased to meet growing investor demand for measurement of corporate responsibility performance. The criteria by which companies' corporate responsibility practices are assessed may change, which could result in greater expectations of us and cause us to undertake costly initiatives to satisfy such new criteria. If

we elect not to or are unable to satisfy such new criteria, investors may conclude that our policies with respect to corporate responsibility are inadequate. We may face reputational damage in the event that our corporate responsibility procedures or standards do not meet the standards set by various constituencies.

Furthermore, if our competitors' corporate responsibility performance is perceived to be greater than ours, potential or current investors may elect to invest with our competitors instead. In addition, in the event that we communicate certain initiatives and goals regarding environmental, social and governance matters, we could fail, or be perceived to fail, in our achievement of such initiatives or goals, or we could be criticized for the scope of such initiatives or goals. If we fail to satisfy the expectations of investors, employees and other stakeholders or our initiatives are not executed as planned, our reputation and financial results could be materially and adversely affected.

Risks related to ownership of our common stock

An active trading market for our common stock may not develop or be sustained.

Our common stock has been listed on the Nasdaq Global Select Market only since September 25, 2026, and an active trading market for our common stock may never develop or be sustained. The lack of an active market may impair the value of your shares, your ability to sell your shares at the time you wish to sell them and the prices that you may obtain for your shares. Further, an inactive trading market for our common stock may also impair our ability to raise capital by selling shares of our common stock or enter into strategic partnerships and transactions by issuing our shares of our common stock as consideration. If an active trading market for our common stock does not develop, or is not sustained, you may not be able to sell your shares quickly or at the market price, or at all, and it may be difficult for you to sell your shares without depressing the market price for our common stock.

The trading price of our common stock may be volatile and you could lose all or part of your investment.

The trading price of our common stock is likely to be volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by those factors discussed in this "Risk Factors" section and many other factors, including:

- timing and results of our preclinical studies and clinical trials or those of our competitors;
- the costs and timing of manufacturing for our therapeutic candidates;
- the success of existing or new competitive therapies, products or technologies;
- development of new therapeutic candidates that may address our markets and make our therapeutic candidates less attractive;
- failure or discontinuation of any of our research or development programs;
- changes in the level of expenses related to any of our research or development programs;
- developments related to any existing or future collaborations;
- the recruitment or departure of key personnel;
- regulatory or legal developments in the United States and other countries;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- changes in the structure of healthcare payment systems;
- the results of our efforts to discover, develop, acquire or in-license additional therapeutic candidates or products;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- changes in or failure to meet or exceed financial estimates and projections of the investment community or that we provide to the public;
- actual or expected changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our executive officers, directors or principal stockholders, or others;
- variations in our financial results or those of companies that are perceived to be similar to us;

- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions;
- changes in accounting principles; and
- the other factors described in this "Risk Factors" section and elsewhere in this Quarterly Report on Form 10-Q.

In addition, the stock market in general and the market for biopharmaceutical companies in particular have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to operating performance of the particular companies affected. Following price volatility, holders of securities may institute securities class action litigation against the issuer. If any holders of our common stock were to bring such a lawsuit against us, we could incur substantial costs defending the lawsuit and the attention of our board of directors and senior management would be diverted from the operation of our business. Any adverse determination in litigation could also subject us to significant liabilities. Further, a decline in the financial markets and related factors beyond our control may cause the price of our common stock to decline rapidly and unexpectedly. Broad market and industry factors such as these could materially and adversely affect the market price of our common stock, regardless of our actual operating performance.

Our executive officers, directors and principal stockholders, if they choose to act together, have the ability to control or significantly influence all matters submitted to stockholders for approval.

Our executive officers, directors and greater than 5% stockholders and their respective affiliates have the ability, if they choose to act together, to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control or significantly influence the election of directors, the composition of our management and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination that other stockholders may desire. The interests of these stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily of those of other stockholders, including seeking a premium value for their shares of our common stock. Any of these actions could adversely affect the market price of our common stock.

A significant portion of our total outstanding shares are eligible to be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares of our common stock, could reduce the market price of our common stock.

In connection with our IPO, we, our directors and executive officers and holders of substantially all of our outstanding common stock agreed with the underwriters, subject to specified exceptions, not to sell, transfer or hedge any shares of our common stock or securities convertible into or exchangeable for our common stock for a period of 180 days after September 24, 2026, the date of our final prospectus, without the prior written consent of J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC, which may release any of these securities from the restrictions at any time without notice. Upon expiration of the lock-up period, shares of our common stock held by the applicable holders will become eligible for sale in the public market, subject in some cases to the volume, manner-of-sale and other limitations of Rule 144. The 5,255,542 shares we sold to AbbVie Inc. in the concurrent private placement are not subject to a lock-up agreement but are restricted securities under Rule 144 with a holding period that began on September 28, 2026.

Moreover, holders of an aggregate of 70,904,025 shares of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders, until such shares can otherwise be sold without restriction under Rule 144 under the Securities Act, or until the rights terminate pursuant to the terms of the investor rights agreement between us and such holders. We have also registered all shares of our common stock subject to equity awards issued or reserved for future issuance under our equity compensation plans on a registration statement on Form S-8 filed with the SEC on September 28, 2026. Shares registered on Form S-8 can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates under Rule 144 under the Securities Act and the market standoff provisions and lock-up agreements described above. Any sales of securities by these stockholders could have a negative impact on the trading price of our common stock.

Future sales and issuances of our securities, including pursuant to our equity incentive plans, may cause dilution to our stockholders or decrease our stock price.

We expect that significant additional capital may be necessary to continue our planned operations, including for expanding product development, conducting preclinical studies and clinical trials and commercializing our therapeutic candidates. We may seek additional capital through public or private equity or debt financings or other capital sources, which may include strategic collaborations and other

strategic arrangements with third parties, to enable us to complete the development and potential commercialization of our therapeutic candidates. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder.

Pursuant to the 2026 Plan, our management is authorized to grant stock option awards and other equity-based awards to our employees, directors and consultants. Additionally, the number of shares of our common stock reserved for issuance under the 2026 Plan will automatically increase on January 1 of each calendar year, beginning on January 1, 2027 and continuing through and including January 1, 2036, by 5% of the total number of shares of our capital stock outstanding on December 31 of the preceding year, or a lesser number of shares determined by our board of directors. In addition, pursuant to the ESPP, the number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, beginning on January 1, 2027 and continuing through and including January 1, 2036, by the lesser of (i) 1% of the total number of shares of our capital stock outstanding on December 31st of the preceding calendar year, and (ii) 3,079,668 shares; provided that before the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii).

Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

We are an emerging growth company and a smaller reporting company and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and may remain an emerging growth company until the last day of the fiscal year following the fifth anniversary of the closing of our IPO, or December 31, 2031. However, if certain events occur prior to the end of such five-year period, including if we become a "large accelerated filer," our annual gross revenues exceed $1.235 billion or we issue more than $1.0 billion of non-convertible debt in any three-year period, we will cease to be an emerging growth company prior to the end of such five-year period. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's discussion and analysis of financial condition and results of operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the PCAOB regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

In addition, the JOBS Act allows us as an "emerging growth company" to delay adoption of new or revised accounting pronouncements applicable to public companies until such pronouncements are made applicable to private companies.

As a result of our reliance on these exemptions, the information we provide to stockholders will be different than the information that is available with respect to other public companies that are not emerging growth companies. For example, in the Prospectus we only included two years of audited consolidated financial statements and have not included all of the executive compensation-related information that would be required if we were not an emerging growth company. It is possible that this may cause investors to find our common stock less attractive. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our share price may be reduced or more volatile.

Even following the termination of our status as an emerging growth company, we may be able to take advantage of the reduced disclosure requirements applicable to "smaller reporting companies," as that term is defined in Rule 12b-2 of the Exchange Act, and, in particular, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. To the extent that we are no longer eligible to use exemptions from various reporting requirements, we may be unable to realize our anticipated cost savings from these exemptions, which could have a material adverse impact on our operating results.

Future changes in financial accounting standards or practices may cause adverse and unexpected revenue fluctuations and adversely affect our reported results of operations.

Future changes in financial accounting standards may cause adverse, unexpected revenue fluctuations and affect our reported financial position or results of operations. Financial accounting standards in the United States are constantly under review and new pronouncements and varying interpretations of pronouncements have occurred with frequency in the past and are expected to occur again in the future. As a result, we may be required to make changes in our accounting policies. Those changes could affect our financial condition and results of operations or the way in which such financial condition and results of operations are reported. Compliance with new accounting standards may also result in additional expenses. As a result, we intend to invest all reasonably necessary resources to comply with evolving standards, and this investment may result in increased general and administrative expenses and a diversion of management time and attention from business activities to compliance activities. See the section titled "Management's discussion and analysis of financial condition and results of operations—Recent accounting pronouncements." As an emerging growth company, the JOBS Act allows us to delay adoption of new or revised accounting standards applicable to public companies until such pronouncements are made applicable to private companies. We have elected to use the extended transition period for complying with new or revised accounting standards and as a result of this election, our consolidated financial statements may not be comparable to companies that comply with public company effective dates. However, we may elect to early adopt any new or revised accounting standards whenever such early adoption is permitted for non-public companies. We may take advantage of these exemptions up until the time that we are no longer an emerging growth company.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, would be your sole source of gain.

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. As a result, capital appreciation, if any, of our common stock would be your sole source of gain on an investment in our common stock for the foreseeable future. See the section titled "Dividend policy" in the Prospectus for additional information.

Delaware law and provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Provisions of our amended and restated certificate of incorporation and amended and restated bylaws may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our common stock. Among other things, our amended and restated certificate of incorporation and amended and restated bylaws:

- permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate (including the right to approve an acquisition or other change in our control);
- provide that the authorized number of directors may be changed only by resolution of the board of directors;
- provide that the board of directors or any individual director may only be removed with cause and the affirmative vote of the holders of at least 66-2/3% of the voting power of all of our then outstanding common stock;
- provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- divide our board of directors into three classes, with each class serving staggered three-year terms;
- require that any action to be taken by our stockholders be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner and also specify requirements as to the form and content of a stockholder's notice;
- do not provide for cumulative voting rights, therefore allowing the holders of a majority of the shares of our common stock entitled to vote in any election of directors to elect all of the directors standing for election; and
- provide that special meetings of our stockholders may be called only by the Chairperson of the board of directors, our Chief Executive Officer, or by the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors.

The amendment of any of these provisions, with the exception of the ability of our board of directors to issue shares of preferred stock and designate any rights, preferences and privileges thereto, would require approval by the holders of at least 66-2/3% of our then-outstanding common stock. Such ability to issue preferred stock with voting or conversion rights could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in our control that may otherwise benefit holders of our common stock and may adversely affect the market price of the common stock and the voting and other rights of the holders of our common stock. We have no current plans to issue any shares of preferred stock.

In addition, as a Delaware corporation, we are subject to Section 203 of the Delaware General Corporation Law. These provisions may prohibit large stockholders, in particular those owning 15% or more of our outstanding voting stock, from merging or combining with us for a certain period of time. A Delaware corporation may opt out of this provision by express provision in its original certificate of incorporation or by amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision.

These and other provisions in our amended and restated certificate of incorporation, amended and restated bylaws and Delaware law could make it more difficult or costly for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by our then-current board of directors, including delay or impede a merger, tender offer or proxy contest involving our company. The existence of these provisions could negatively affect the price of our common stock and limit opportunities for you to realize value in a corporate transaction.

For information regarding these and other provisions, see the section titled “Description of capital stock” in the Prospectus.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and any appellate court therefrom are the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders’ ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware is the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (i) any derivative claim or cause of action brought on our behalf; (ii) any claim or cause of action that is based upon a violation of a duty owed by any current or former director, officer, other employee or stockholder, to us or our stockholders; (iii) any claim or cause of action against us or any current or former director, officer or other employee, arising out of or pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws; (iv) any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws (including any right, obligation, or remedy thereunder); (v) any claim or cause of action as to which the Delaware General Corporation Law confers jurisdiction on the Court of Chancery of the State of Delaware; and (vi) any claim or cause of action against us or any current or former director, officer or other employee, governed by the internal-affairs doctrine or otherwise related to our internal affairs, in all cases to the fullest extent permitted by applicable law and subject to the court having personal jurisdiction over the indispensable parties named as defendant; provided, however, that if the designation of such court as the sole and exclusive forum for a claim or action referred to in foregoing clauses (i) through (vi) would violate applicable law, then the United States District Court for the District of Delaware shall be the sole and exclusive forum for such claim or cause of action. These provisions would not apply to suits brought to enforce a duty or liability created by the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. Additionally, investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act, including all causes of action asserted against any defendant named in such complaint. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there is uncertainty as to whether the provisions will be enforced by a court in those other jurisdictions.

These choice of forum provisions may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees and may discourage these types of lawsuits and result in increased costs for investors to bring a claim. If a court were to find the choice of forum provisions contained in our amended and restated certificate of

incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

General risk factors

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we no longer qualify as an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the Nasdaq listing requirements and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors.

We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act ("Section 404") we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the SEC after we become a public company. However, while we remain an emerging growth company or smaller reporting company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing whether such controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. In addition, if we identify one or more material weaknesses as a result of this implementation and evaluation process, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our consolidated financial statements.

Unstable market and economic conditions, including any adverse macroeconomic conditions or geopolitical events, may have serious adverse consequences on our business, financial condition and stock price.

The global credit and financial markets have experienced extreme volatility and disruptions in the past several years, including severely diminished liquidity and credit availability, rising inflation and monetary supply shifts, rising interest rates, supply chain constraints, labor shortages, declines in consumer confidence, declines in economic growth, increases in unemployment rates, recession risks and uncertainty about economic stability. The financial markets and the global economy may also be adversely affected by the current or anticipated impact of public health crises, military conflict, including the conflict between Russia and Ukraine and the ongoing conflict in the Middle East, tensions with and economic uncertainty in China, terrorism, tariffs or other geopolitical events. For example, the COVID-19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the capital markets. Sanctions imposed by the United States and other countries in response to military conflicts, including in Ukraine and the Middle East, may also adversely impact the financial markets and the global economy, and any economic countermeasures by the affected countries or others could exacerbate market and economic instability. The extent of the impact of these conditions on our operational and financial performance, including our ability to execute our business strategies and initiatives in the expected timeframe, as well as that of third parties upon whom we rely, will depend on future developments which are uncertain and cannot be predicted. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon

clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive an economic downturn, which could directly affect our ability to attain our operating goals on schedule and on budget.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Section 404(a) of the Sarbanes-Oxley Act requires that beginning with our second annual report following our IPO, management assess and report annually on the effectiveness of our internal control over financial reporting and identify any material weaknesses in our internal control over financial reporting. Although Section 404(b) of the Sarbanes-Oxley Act requires our independent registered public accounting firm to issue an annual report that addresses the effectiveness of our internal control over financial reporting, we have opted to rely on the exemptions provided in the JOBS Act, and consequently will not be required to comply with SEC rules that implement Section 404(b) until such time as we are no longer an emerging growth company or smaller reporting company.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our consolidated financial statements or identify other areas for further attention or improvement. Inadequate internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. For example, our directors or executive officers could inadvertently fail to disclose a new relationship or arrangement causing us to fail to make any related party transaction disclosures. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

We and our directors and executive officers may be subject to litigation for a variety of claims, which could harm our reputation and adversely affect our business, results of operations and financial condition.

In the ordinary course of business, we may in the future be involved in and subject to litigation for a variety of claims or disputes and receive regulatory inquiries. These claims, lawsuits and proceedings could include labor and employment, wage and hour, commercial, allege securities law violations or other investor claims, claims that our employees have wrongfully disclosed or we have wrongfully used proprietary information of our employees' former employers and other matters. The number and significance of these potential claims and disputes may increase as our business expands. Further, our general liability insurance may not cover all potential claims made against us or be sufficient to indemnify us for all liability that may be imposed. Any claim against us, regardless of its merit, could be costly, divert management's attention and operational resources, and harm our reputation.

Our directors and executive officers may also be subject to litigation. Our amended and restated certificate of incorporation and our amended and restated bylaws authorize us to indemnify our directors, officers, employees and other agents to the fullest extent permitted by Delaware law. The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and our amended and restated bylaws may discourage stockholders from bringing a lawsuit against our directors for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions. We also maintain customary directors' and officers' liability insurance. See the section titled "Executive and director compensation—Limitations on liability and indemnification" in the Prospectus.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business. Additionally, the increase in the cost of directors' and officers' liability insurance may cause us to opt for lower overall policy limits or to forgo insurance that we may otherwise rely on to cover significant defense costs, settlements and damages awarded to plaintiffs.

If securities or industry analysts do not publish research, publish inaccurate or unfavorable research, or if they issue an adverse or misleading opinion regarding our business or our common stock, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no or only very few securities analysts commence coverage of us, or if industry analysts cease coverage of us, the trading price for our common stock would be negatively affected. If one or more of the analysts who cover us downgrade our common stock or publish inaccurate, misleading or unfavorable research about our business or if any of our preclinical studies or clinical trials and operating results fail to meet the expectations of analysts, our common stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our common stock price and trading volume to decline.

Our operations are concentrated in one location, and we or the third parties upon whom we depend may be adversely affected by a wildfire, earthquake or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Our current operations are predominantly located in California. Any unplanned event, such as a flood, wildfire, explosion, earthquake, extreme weather condition, epidemic or pandemic, power outage, telecommunications failure or other natural or manmade accidents or incidents that result in us being unable to fully utilize our facilities may have a material and adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Any similar impacts of natural or manmade disasters on our third-party manufacturers and CROs could cause delays in our clinical trials and may have a material and adverse effect on our ability to operate our business and have significant negative consequences on our financial and operating conditions. If a natural disaster, power outage or other event occurred that prevented us from using our clinical sites, impacted clinical supply or the conduct of our clinical trials, that damaged critical infrastructure, such as the manufacturing facilities of our third-party manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we and our manufacturers and CROs have in place may prove inadequate in the event of a serious disaster or similar event. In the event of an accident or incident at these facilities, we cannot assure you that the amounts of insurance we currently carry will be sufficient to satisfy any damages and losses. If our facilities, or the manufacturing facilities of our manufacturers or CROs, are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our development programs may be harmed. Any business interruption could adversely affect our business, financial condition, results of operations and prospects.

Our insurance policies may be inadequate, may not cover all of our potential liabilities and may potentially expose us to unrecoverable risks.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include property, general liability, employee benefits liability, business automobile, workers' compensation, clinical trials/products liability, cybersecurity liability, directors' and officers' and employment practices insurance. We do not know, however, if we will be able to maintain insurance with adequate levels of coverage. No assurance can be given that an insurance carrier will not seek to cancel or deny coverage after a claim has occurred. Any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our financial position and results of operations. For example, although we maintain product liability insurance coverage that also covers our clinical trials, this insurance may not be adequate to cover all liabilities that we may incur, and we may be required to increase our product liability insurance coverage. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and successfully commercialize any therapeutic candidate. Insurance availability, coverage terms and pricing continue to vary with market conditions. We endeavor to obtain appropriate insurance coverage for insurable risks that we identify. However, we may fail to correctly anticipate or quantify insurable risks, we may not be able to obtain appropriate insurance coverage and insurers may not respond as we intend to cover insurable events that may occur. Any significant uninsured liability may require us to pay substantial amounts, which would materially adversely affect our business, financial condition, results of operations and growth.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our clinical development programs and the diseases our therapeutic candidates are being developed to treat. We intend to utilize appropriate social media in connection with communicating about our development programs. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to report an alleged adverse event during a clinical trial. When such disclosures occur, we may fail to monitor and comply with applicable adverse event reporting obligations, or we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our investigational products. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website, or a risk that a post on a social networking website by any of our employees may be construed as inappropriate promotion. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business.

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

(a) ***Unregistered Sales of Equity Securities***

From July 1, 2026 through September 28, 2026, the date on which we filed our registration statement on Form S-8, we issued an aggregate of 432,360 shares of our common stock upon the exercise of stock options granted under the 2020 and 2026 Plans, for aggregate cash consideration of approximately $0.5 million, and granted options to purchase an aggregate of 3,052,151 shares of our common stock under the 2020 Plan, with a weighted-average exercise price of $14.55 per share, to our employees, directors and consultants. These issuances and grants were exempt from registration under the Securities Act in reliance on Rule 701 promulgated under the Securities Act, as transactions under compensatory benefit plans and contracts relating to compensation, or Section 4(a)(2) of the Securities Act, as transactions by an issuer not involving a public offering.

Concurrently with the closing of the IPO, we issued and sold 5,255,542 shares of our common stock to AbbVie Inc. in a private placement at a price of $17.00 per share, for aggregate gross proceeds of approximately $89.3 million (approximately $87.6 million after deducting placement agent fees payable). The shares were issued and sold in a private placement pursuant to Section 4(a)(2) of the Securities Act. The proceeds of the private placement are not included in the amounts described below under "Use of Proceeds".

(b) ***Use of Proceeds***

On September 24, 2026, our registration statement on Form S-1 (File No. 333-298782) for our IPO (the "Registration Statement") was declared effective by the SEC, and on the same date we filed a related registration statement on Form S-1 pursuant to Rule 462(b) under the Securities Act (File No. 333-299119), which became effective upon filing (together with the Registration Statement, the "Registration Statements"). The offering commenced on September 24, 2026. Pursuant to the Registration Statements, we registered an aggregate of 30,187,500 shares of our common stock, including 3,937,500 shares subject to the underwriters' option to purchase additional shares, at a public offering price of $17.00 per share, for an aggregate offering price of $513,187,500.

On September 28, 2026, we closed the IPO and issued and sold 26,250,000 shares of our common stock at the public offering price of $17.00 per share, for aggregate gross proceeds of approximately $446.3 million. On October 1, 2026, we issued and sold an additional 3,937,500 shares of our common stock pursuant to the underwriters' exercise in full of their option to purchase additional shares at the public offering price of $17.00 per share, for aggregate gross proceeds of approximately $66.9 million. Accordingly, we have sold all 30,187,500 shares registered under the Registration Statements, for aggregate gross proceeds of approximately $513.2 million, and the offering has terminated. J.P. Morgan Securities LLC, Morgan Stanley & Co. LLC, TD Securities (USA) LLC and UBS Securities LLC acted as representatives of the underwriters and, together with LifeSci Capital LLC, acted as book-running managers of the IPO.

We received aggregate net proceeds from the IPO, including from the sale of the option shares, of approximately $469.7 million, after deducting underwriting discounts and commissions of approximately $35.9 million and estimated offering expenses payable by us of approximately $7.6 million. No payments for such expenses were made, directly or indirectly, to (i) any of our directors or officers or their associates, (ii) any person owning 10% or more of any class of our equity securities or (iii) any of our affiliates, other than payments in the ordinary course for salaries and expense reimbursements.

There has been no material change in the expected use of the net proceeds from our IPO as described in the Prospectus.

(c) ***Issuer Repurchases of Equity Securities***

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

(a) None.

(b) None.

(c) During the quarter ended September 30, 2026, none of our directors or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement" (as those terms are defined in Item 408 of Regulation S-K).

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference				Filed / Furnished Herewith
		Form	File No.	Exhibit	Filing Date	
3.1	Amended and Restated Certificate of Incorporation of the Registrant.	8-K	001-43482	3.1	9/28/2026	
3.2	Amended and Restated Bylaws of the Registrant.	S-1	333-298782	3.4	9/4/2026	
10.1	Common Stock Purchase Agreement, dated as of September 13, 2026, by and between AbbVie Inc. and the Registrant.	S-1/A	333-298782	10.20	9/14/2026	
10.2+	Form of Indemnification Agreement, by and between the Registrant and each of its directors and executive officers.	S-1	333-298782	10.1	9/4/2026	
10.3+	ADARx Pharmaceuticals, Inc. 2026 Equity Incentive Plan.	S-1/A	333-298782	10.4	9/21/2026	
10.4+	Forms of Stock Option Grant Notice, Option Agreement and Notice of Exercise under the ADARx Pharmaceuticals, Inc. 2026 Equity Incentive Plan.	S-1/A	333-298782	10.5	9/21/2026	
10.5+	ADARx Pharmaceuticals, Inc. 2026 Employee Stock Purchase Plan.	S-1/A	333-298782	10.6	9/21/2026	
10.6+	Non-Employee Director Compensation Policy.	S-1/A	333-298782	10.7	9/21/2026	
10.7+	Confirmatory Offer Letter, between the Registrant and Zhen Li, Ph.D., dated September 10, 2026.	S-1/A	333-298782	10.8	9/21/2026	
10.8+	Confirmatory Offer Letter, between the Registrant and A. Robert MacLeod, Ph.D., dated September 9, 2026.	S-1/A	333-298782	10.9	9/21/2026	
10.9+	Confirmatory Offer Letter, between the Registrant and Ryan Fisk, M.B.A., dated September 11, 2026.	S-1/A	333-298782	10.12	9/21/2026	
10.10+	Confirmatory Offer Letter, between the Registrant and Rui Zhu, Ph.D., dated September 9, 2026.	S-1/A	333-298782	10.13	9/21/2026	
10.11+#	Board Member Agreement, dated July 21, 2026, by and between the Registrant and Ying Huang, Ph.D.	S-1	333-298782	10.17	9/4/2026	
10.12+	Confirmatory Offer Letter, between the Registrant and Robert Ackles, dated September 9, 2026.	S-1/A	333-298782	10.18	9/21/2026	
10.13+	Severance and Change of Control Plan.	S-1/A	333-298782	10.19	9/21/2026	
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X

32.1*	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	X
32.2*	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	X
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.	X
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents	X
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)	X

+ Indicates management contract or compensatory plan.

Pursuant to Item 601(b)(10)(iv) of Regulation S-K promulgated by the SEC, portions of this exhibit (indicated by [***]) have been omitted because the registrant has determined that the information is both not material and is the type that the registrant treats as private or confidential. The registrant hereby agrees to furnish supplementally to the SEC, upon its request, an unredacted copy of this exhibit.

* The certifications attached as Exhibit 32.1 and 32.2 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 ADARx Pharmaceuticals, Inc. 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 Quarterly Report on 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.

ADARx Pharmaceuticals, Inc.

By: /s/ Zhen Li, Ph.D.
Zhen Li, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 12, 2026

By: /s/ Ryan Fisk, M.B.A.
Ryan Fisk, M.B.A.
Chief Financial Officer and Chief Business Officer
(Principal Financial Officer and Principal Accounting Officer)

Date: November 12, 2026

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Zhen Li, Ph.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of ADARx Pharmaceuticals, Inc.;

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)) 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) 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

   (c) 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 12, 2026

By: /s/ Zhen Li, Ph.D.
Zhen Li, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Ryan Fisk, M.B.A., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of ADARx Pharmaceuticals, Inc.;

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)) 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) 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

   (c) 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 12, 2026

By: /s/ Ryan Fisk, M.B.A.
Ryan Fisk, M.B.A.
Chief Financial Officer and Chief Business Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

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 of ADARx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Zhen Li, Ph.D., certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(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.

Date: November 12, 2026

By: /s/ Zhen Li, Ph.D.
Zhen Li, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

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 of ADARx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Ryan Fisk, M.B.A., certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(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.

Date: November 12, 2026

By: /s/ Ryan Fisk, M.B.A.
Ryan Fisk, M.B.A.
Chief Financial Officer and Chief Business Officer
(Principal Financial Officer and Principal Accounting Officer)