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: 001-42121

Rapport Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**88-0724208**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
99 High Street, Suite 2100 **Boston, MA**	**02110**
(Address of principal executive offices)	**(Zip Code)**

(857) 321-8020

(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, $0.001 par value per share	RAPP	The Nasdaq Global 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, a 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 2, 2026, the registrant had 59,279,630 shares of common stock, $0.001 par value per share, outstanding.

Table of Contents

		Page
PART I.	FINANCIAL INFORMATION	5
Item 1.	Financial Statements (Unaudited)	5
	Condensed Consolidated Balance Sheets	5
	Condensed Consolidated Statements of Operations and Comprehensive Loss	6
	Condensed Consolidated Statements of Stockholders' Equity	7
	Condensed Consolidated Statements of Cash Flows	9
	Notes to Unaudited Condensed Consolidated Financial Statements	10
Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations	28
Item 3.	Quantitative and Qualitative Disclosures About Market Risk	43
Item 4.	Controls and Procedures	43
PART II.	OTHER INFORMATION	44
Item 1.	Legal Proceedings	44
Item 1A.	Risk Factors	44
Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	106
Item 3.	Defaults Upon Senior Securities	106
Item 4.	Mine Safety Disclosures	106
Item 5.	Other Information	106
Item 6.	Exhibits	107
Signatures		108

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (this "Quarterly Report") contains forward-looking statements. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in 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"). All statements other than statements of historical facts, including statements regarding our future results of operations and financial position, business strategy, product candidates, planned nonclinical studies and clinical trials, results of nonclinical studies, clinical trials, research and development costs, regulatory approvals, commercial strategy, timing and likelihood of success, as well as plans and objectives of management for future operations, are forward-looking statements. These statements involve known and unknown risks, uncertainties, and other important factors that are in some cases beyond our control and may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

In some cases, forward-looking statements can be identified by terms such as "may," "will," "should," "would," "expect," "plan," "anticipate," "could," "intend," "target," "project," "believe," "estimate," "predict," "potential," or "continue" or the negative of these terms or other similar expressions. Forward-looking statements contained in this Quarterly Report may include, but are not limited to, statements about:

- our ability to identify, develop, and commercialize current and future product candidates based on our RAP technology platform;
- the initiation, timing, progress, and results of our research and development programs, nonclinical studies and clinical trials;
- the translation of endpoints in our current and planned clinical trials to planned and future registrational trials;
- our ability to replicate positive results from earlier nonclinical studies or clinical trials conducted by us or third parties in current or future clinical trials;
- our ability to demonstrate that our current and future product candidates are safe and effective for their proposed indications;
- the number of patients with the diseases or disorders we elect to pursue with our product candidates, and the willingness of those patient populations to use and adhere to our product candidates if approved in the future;
- the implementation of our business model, and strategic plans for our business, programs, future product candidates, platform, and technology;
- our ability to advance any product candidates through applicable regulatory approval processes;
- our ability to obtain additional cash and the sufficiency of our existing cash, cash equivalents and short-term investments to fund our future operating expenses and capital expenditure requirements;
- the accuracy of our estimates regarding expenses, future revenue, capital requirements, and needs for additional financing;
- our ability to comply with our obligations under our intellectual property licenses with third parties, including Janssen Pharmaceutical NV;
- our ability to maintain, expand and protect our intellectual property portfolio;
- developments relating to our competitors and our industry;
- existing regulations and regulatory developments in the United States and other jurisdictions;
- our ability to identify and enter into future license agreements and collaborations;
- the Company's outstanding indebtedness;
- market volatility in the debt and equity capital markets;
- general economic, industry, and market conditions, including rising interest rates and inflation;
- our ability to attract, hire, and retain our key personnel and additional qualified personnel;
- our ability to achieve any milestones in connection with our license agreement with Tenacia Biotechnology (Hong Kong) Co., Ltd.; and
- our anticipated use of our existing cash, cash equivalents and short-term investments.

We have based these forward-looking statements largely on our current expectations and projections about our business, the industry in which we operate and financial trends that we believe may affect our business, financial condition, results of operations and prospects, and these forward-looking statements are not guarantees of future performance or development. These forward-looking statements speak only as of the date of this Quarterly Report and are subject to a number of risks, uncertainties and assumptions described in the section titled "Risk Factors" and elsewhere in this Quarterly Report. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified, 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. 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 or otherwise.

You should read this Quarterly Report, the documents that we reference in this Quarterly Report and the other documents that we file with the Securities and Exchange Commission ("SEC") with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

SUMMARY RISK FACTORS

Our business is subject to numerous risks and uncertainties, which include, but are not limited to, the following:

- We are a clinical-stage biotechnology company with a limited operating history, which may make it difficult to evaluate our current business and predict our future success and viability. We have incurred significant financial losses since our inception and anticipate that we will continue to incur significant financial losses for the foreseeable future.
- We will require additional funding in order to finance operations. If we are unable to raise capital when needed, or on acceptable terms, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.
- Our business is highly dependent on the success of our product candidates, particularly RAP-219 for focal onset seizures, bipolar mania, primary generalized tonic-clonic seizures ("PGTCS"), or other indications. If we are unable to successfully complete clinical development, obtain regulatory approval for or commercialize one or more of our product candidates, or if we experience delays in doing so, our business will be materially harmed.
- The successful development of pharmaceutical products involves a lengthy and expensive process and is highly uncertain.
- Due to the significant resources required for the development of our pipeline, and depending on our ability to access capital, we must prioritize the development of certain product candidates over others. Moreover, we may fail to expend our limited resources on product candidates or indications that may have been more profitable or for which there is a greater likelihood of success.
- The regulatory approval processes of the Food and Drug Administration, European Medicines Agency, Medicines and Healthcare products Regulatory Agency and other comparable regulatory authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.
- We are dependent on a third party having accurately generated, collected, interpreted and reported data from certain nonclinical studies and clinical trials that were previously conducted for our product candidates.
- If our clinical trials fail to replicate positive results from earlier nonclinical studies or clinical trials conducted by us or third parties, we may be unable to successfully develop, obtain regulatory approval for or commercialize our product candidates.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following regulatory approval, if obtained.
- If we do not achieve our projected development and commercialization goals in the timeframes we announce and expect, the development and commercialization of our product candidates may be delayed, and our business and results of operations may be harmed.
- We have concentrated our research and development efforts on the treatment of disorders of the nervous system, a field that faces certain challenges in drug development.
- Even if any of our product candidates receives regulatory approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.
- The number of patients with the diseases and disorders for which we are developing our product candidates has not been established with precision. If the actual number of patients with the diseases or disorders we elect to pursue with our product candidates is smaller than we anticipate, we may have difficulties in enrolling patients in our clinical trials, which may delay or prevent development of our product candidates. Even if such product candidates are successfully developed and approved, the markets for our product candidates may be smaller than we expect and our revenue potential and ability to achieve profitability may be materially adversely affected.
- We rely on third parties to assist in conducting our clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or commercialize our product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.

- We depend on in-licensed intellectual property. If we fail to comply with our obligations under our intellectual property licenses with third parties, we could lose license rights that are important to our business.
- If we or our licensors are unable to obtain and maintain patent protection for our product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to our product candidates and our ability to successfully commercialize our product candidates may be adversely affected.
- Our indebtedness and liabilities could limit the cash flow available for our operations, expose us to risks that could adversely affect our business, financial condition and results of operations and impair our ability to satisfy our obligations under the $172.5 million aggregate principal amount of 2.25% convertible senior notes due 2032 (the "Notes").
- Conversion of the Notes will dilute the ownership interest of our stockholders and may otherwise depress the price of our common stock.

The summary risk factors described above should be read together with the text of the full risk factors in the section titled "Risk Factors" and the other information set forth in this Quarterly Report, as well as in other documents that we file with the SEC. The risks summarized above or described in full elsewhere in this Quarterly Report are not the only risks that we face. Additional risks and uncertainties not presently known to us, or that we currently deem to be immaterial may also materially adversely affect our business, financial condition, results of operations, and future growth prospects.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

Rapport Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(In thousands, except share data)
(Unaudited)

	September 30, 2026	December 31, 2025
Assets		
Current assets		
Cash and cash equivalents	$ 74,754	$ 52,645
Accounts receivable	171	—
Short-term investments	320,275	437,894
Restricted cash	105	105
Prepaid expenses and other current assets	12,076	7,917
Total current assets	407,381	498,561
Property and equipment, net	1,957	2,976
Operating lease right of use asset, net	8,125	9,909
Other assets	1,095	985
Total assets	$ 418,558	$ 512,431
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 1,718	$ 4,190
Accrued expenses and other current liabilities	16,259	12,104
Operating lease liability	2,325	2,755
Total current liabilities	20,302	19,049
Operating lease liability, net of current portion	7,066	8,729
Total liabilities	27,368	27,778
Commitments and contingencies (Note 10)		
Stockholders' equity		
Undesignated preferred stock, $0.001 par value; 10,000,000 shares authorized at September 30, 2026 and December 31, 2025, respectively; zero shares issued and outstanding at September 30, 2026 and December 31, 2025	—	—
Common stock, $0.001 par value; 500,000,000 shares authorized at September 30, 2026 and December 31, 2025; 48,088,413 shares and 47,772,567 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	48	48
Additional paid-in capital	745,192	719,287
Accumulated other comprehensive income (loss)	(2,115)	546
Accumulated deficit	(351,935)	(235,228)
Total stockholders' equity	391,190	484,653
Total liabilities and stockholders' equity	$ 418,558	$ 512,431

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

Rapport Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share data)
(Unaudited)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
Collaboration revenue	$ —	$ —	$ 20,000	$ —
Operating expenses				
Research and development	34,316	22,279	118,446	64,531
Selling, general and administrative	9,770	7,707	30,661	22,059
Total operating expenses	44,086	29,986	149,107	86,590
Loss from operations	(44,086)	(29,986)	(129,107)	(86,590)
Other income:				
Interest income	3,851	3,061	12,400	8,870
Total other income	3,851	3,061	12,400	8,870
Net loss	$ (40,235)	$ (26,925)	$ (116,707)	$ (77,720)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.84)	$ (0.71)	$ (2.46)	$ (2.15)
Weighted-average common shares outstanding, basic and diluted	47,768,633	37,931,170	47,494,757	36,223,887
Comprehensive loss:				
Net loss	$ (40,235)	$ (26,925)	$ (116,707)	$ (77,720)
Change in unrealized (losses) gains on investments, net of tax	(845)	221	(2,661)	667
Total other comprehensive income (loss)	(845)	221	(2,661)	667
Comprehensive loss	$ (41,080)	$ (26,704)	$ (119,368)	$ (77,053)

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

Rapport Therapeutics, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-in	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance at December 31, 2025	47,772,567	$ 48	$ 719,287	$ 546	$ (235,228)	$ 484,653
Stock-based compensation expense	—	—	6,963	—	—	6,963
Issuance of common stock upon exercise of stock options	80,867	—	809	—	—	809
Vesting of restricted stock units	6,994	—	—	—	—	—
Repurchase of unvested restricted common stock	(52,975)	—	(5)	—	—	(5)
Net loss	—	—	—	—	(19,857)	(19,857)
Change in unrealized gain (loss) on investments, net of tax	—	—	—	(1,051)	—	(1,051)
Balance at March 31, 2026	47,807,453	$ 48	$ 727,054	$ (505)	$ (255,085)	$ 471,512
Stock-based compensation expense	—	—	7,202	—	—	7,202
Issuance of common stock upon exercise of stock options	147,626	—	1,592	—	—	1,592
Net loss	—	—	—	—	(56,615)	(56,615)
Change in unrealized gain (loss) on investments, net of tax	—	—	—	(765)	—	(765)
Balance at June 30, 2026	47,955,079	$ 48	$ 735,848	$ (1,270)	$ (311,700)	$ 422,926
Stock-based compensation expense	—	—	7,634	—	—	7,634
Issuance of common stock upon exercise of stock options	133,334	—	1,710	—	—	1,710
Net loss	—	—	—	—	(40,235)	(40,235)
Change in unrealized gain (loss) on investments, net of tax	—	—	—	(845)	—	(845)
Balance at September 30, 2026	48,088,413	$ 48	$ 745,192	$ (2,115)	$ (351,935)	$ 391,190

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

Rapport Therapeutics, Inc.
Condensed Consolidated Statement of Stockholders' Equity
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-in	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance at December 31, 2024	36,580,202	$ 37	$ 429,657	$ (522)	$ (123,745)	$ 305,427
Stock-based compensation expense	—	—	4,040	—	—	4,040
Issuance of common stock upon exercise of stock options	3,240	—	6	—	—	6
Repurchase of unvested restricted common stock	(86,008)	—	(1)	—	—	(1)
Net loss	—	—	—	—	(24,063)	(24,063)
Change in unrealized gain (loss) on investments, net of tax	—	—	—	427	—	427
Balance at March 31, 2025	36,497,434	$ 37	$ 433,702	$ (95)	$ (147,808)	$ 285,836
Stock-based compensation expense	—	—	4,389	—	—	4,389
Issuance of common stock upon exercise of stock options	243	—	—	—	—	—
Net loss	—	—	—	—	(26,732)	(26,732)
Change in unrealized gain (loss) on investments, net of tax	—	—	—	19	—	19
Balance at June 30, 2025	36,497,677	$ 37	$ 438,091	$ (76)	$ (174,540)	$ 263,512
Issuance of common stock from underwritten public offering, net of issuance costs of $18,061	11,057,692	11	269,428	—		269,439
Stock-based compensation expense	—	—	4,735	—		4,735
Issuance of common stock upon exercise of stock options	94,847	—	635	—		635
Net loss	—	—	—	—	(26,925)	(26,925)
Change in unrealized gain (loss) on investments, net of tax	—	—	—	221		221
Balance at September 30, 2025	47,650,216	$ 48	$ 712,889	$ 145	$ (201,465)	$ 511,617

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

Rapport Therapeutics, Inc.
Condensed Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	For the Nine Months Ended September 30,	
	2026	2025
Cash flows from operating activities:		
Net loss	$ (116,707)	$ (77,720)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation and amortization	637	752
Non-cash loss on sale of property and equipment	137	—
Net (accretion) of investments in marketable securities	(370)	(324)
Non-cash lease expense	1,785	1,461
Stock-based compensation expense	21,799	13,164
Changes in operating assets and liabilities:		
Accounts receivable	(171)	—
Prepaid expenses and other current assets	(3,199)	(2,610)
Other assets	344	(910)
Accounts payable	(2,463)	1,037
Accrued expenses and other current liabilities	4,154	2,480
Operating lease liabilities	(2,095)	(102)
Net cash used in operating activities	(96,149)	(62,772)
Cash flows from investing activities		
Purchases of short-term investments	(84,439)	(157,713)
Sale of short-term investments	10,075	—
Maturities of short-term investments	188,732	145,011
Purchases of property and equipment	(164)	(447)
Proceeds from sales of equipment	401	—
Net cash provided by (used in) investing activities	114,605	(13,149)
Cash flows from financing activities		
Proceeds from underwritten public offering, net of underwriter discounts and commissions and deferred offering costs	—	269,834
Payment of deferred offering costs	(453)	—
Proceeds from exercise of stock options	4,111	642
Repurchase of unvested restricted common stock	(5)	(1)
Net cash provided by financing activities	3,653	270,475
Net increase in cash, cash equivalents, and restricted cash	22,109	194,554
Cash, cash equivalents, and restricted cash at beginning of period	52,750	56,910
Cash, cash equivalents, and restricted cash at end of period	$ 74,859	$ 251,464
Supplemental disclosure for noncash investing and financing activities:		
Right-of-use assets obtained in exchange for operating lease liabilities	$ —	$ 10,499
Deferred offering costs included in accounts payable and accrued expenses at period end	$ 36	$ 394
Purchases of property and equipment included in accounts payable and accrued expenses at period end	$ 11	$ 65
Reconciliation of cash, cash equivalents and restricted cash		
Cash and cash equivalents	$ 74,754	$ 251,359
Restricted cash	$ 105	$ 105
Total cash, cash equivalents and restricted cash shown in the statement of cash flows	$ 74,859	$ 251,464

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

1. Nature of the Business and Basis of Presentation

Rapport Therapeutics, Inc., together with its consolidated subsidiary (the "Company") is a clinical-stage biotechnology company dedicated to discovering and developing small molecule precision medicines for patients with neurological or psychiatric disorders. The Company was incorporated in the state of Delaware in February 2022 as Precision Neuroscience NewCo, Inc. In October 2022, the Company changed its name to Rapport Therapeutics, Inc. The Company is located in Boston, Massachusetts and San Diego, California.

The Company is subject to risks and uncertainties common to early stage companies in the biotechnology industry, including, but not limited to, completing nonclinical studies and clinical trials, obtaining regulatory approval for product candidates, market acceptance of products, development by competitors of new technological innovations, dependence on key personnel, the ability to attract and retain qualified employees, reliance on third-party organizations, protection of proprietary technology, compliance with government regulations, and the ability to raise additional capital to fund operations. The Company's product candidates currently under development will require significant additional research and development efforts, including extensive nonclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure, and extensive compliance-reporting capabilities. Even if the Company's development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Liquidity

The accompanying condensed consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. The Company has incurred recurring losses since its inception, including net losses of $116.7 million and $77.7 million for the nine months ended September 30, 2026 and 2025, respectively. As of September 30, 2026, the Company had an accumulated deficit of $351.9 million. The Company had cash, cash equivalents and short-term investments, excluding restricted cash, of $395.0 million as of September 30, 2026.

In September 2025, the Company completed an underwritten follow-on public offering of its common stock which resulted in net proceeds of $269.4 million (the "September 2025 Offering").

The Company expects to continue to generate operating losses for the foreseeable future. The Company expects that its cash, cash equivalents and short-term investments will be sufficient to fund its operating expenses and capital expenditure requirements through at least 12 months from the issuance of these condensed consolidated financial statements.

The Company will need additional financing to support its continuing operations and pursue its growth strategy. Until such time as the Company can generate significant revenue from product sales, if ever, it expects to finance its operations through equity offerings, debt financings or other capital sources, including collaborations with other companies or other strategic transactions. The Company may not be able to obtain funding on acceptable terms, or at all. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders.

The inability to raise capital as and when needed would have a negative impact on the Company's financial condition and its ability to pursue its business strategy. The Company will need to generate significant revenue to achieve profitability, and it may never do so.

If the Company is unable to obtain funding, it could be forced to delay, reduce or eliminate some or all of its research and development programs, which could adversely affect its business prospects, or it may be unable to continue operations. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements have been prepared by the Company in accordance with accounting principles generally accepted in the United States of America ("GAAP") for interim financial reporting and as required by Regulation S-X, Rule 10-01. The accompanying unaudited condensed consolidated financial statements reflect the operations of the Company. Intercompany balances and transactions have been eliminated in consolidation. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB").

The condensed consolidated interim financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for a fair statement of the Company's financial position as of September 30, 2026 and the results of operations for the three and nine months ended September 30, 2026 and 2025. The condensed consolidated balance sheet as of December 31, 2025 was derived from audited annual financial statements but does not include all disclosures required by GAAP. The results of operations for the interim periods are not necessarily indicative of results to be expected for the year ending December 31, 2026, any other interim periods, or any future year or period.

2. Summary of Significant Accounting Policies

There have been no significant changes from the significant accounting policies and estimates disclosed in Note 2 of the "Notes to Consolidated Financial Statements" in the audited consolidated financial statements for the year ended December 31, 2025 and notes thereto, included in the Company's Annual Report on Form 10-K that was filed with the SEC on March 10, 2026 except as detailed below regarding accounting for collaboration arrangements and revenue recognition.

Use of Estimates

The preparation of the Company's condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of revenues and expenses during the reporting periods. Significant estimates and assumptions reflected within these condensed consolidated financial statements include, but are not limited to, research and development expenses and accruals, and the valuation of the Company's stock-based awards. The Company bases its estimates on known trends and other market-specific or relevant factors that it believes to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates, as there are changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results may differ materially from those estimates or assumptions.

Concentrations of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents and short-term investments. The Company maintains its cash and cash equivalents at high-quality and accredited financial institutions in amounts that could exceed federally insured limits. Cash equivalents are invested in money market funds. However, the Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships. The Company's short-term investments consist of U.S. Treasury bills, government securities, and government agency securities and as a result, the Company believes represent minimal credit risk.

The Company is dependent on third-party vendors for its product candidates. In particular, the Company relies, and expects to continue to rely, on a small number of vendors and collaborations with third parties to manufacture supplies and process its product candidates for its development programs. These programs could be adversely affected by a significant interruption in the manufacturing process.

Restricted Cash

Restricted cash as of September 30, 2026 and December 31, 2025 was restricted as cash collateral for the Company's business credit card program.

Cash and Cash Equivalents

The Company considers all short-term, highly liquid investments, with original maturities of three months or less, to be cash equivalents. As of September 30, 2026 and December 31, 2025, cash equivalents include $62.2 million and $47.9 million held in money market funds, respectively.

Short-Term Investments

The Company's short-term investments consist of investments in debt securities, including U.S. Treasury bills, government securities, and government agency securities with remaining maturities beyond three months at the date of purchase that are available to be converted into cash to fund its current operations. As of September 30, 2026 and December 31, 2025, all of the Company's debt securities were classified as available-for-sale and were carried at fair market value (see Note 3). The unrealized gains and losses on the Company's available-for-sale debt securities are recorded in other comprehensive income (loss) in the condensed consolidated statements of operations and comprehensive loss. Realized gains and losses are included in other income in the consolidated statements of operations and comprehensive loss and are determined using the specific identification method with transactions recorded on a trade date basis.

Debt securities in an unrealized loss position are evaluated for impairment at least quarterly. For available-for-sale debt securities in an unrealized loss position, the Company first assesses whether or not it intends to sell, or it is more likely than not that it will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the investment security's amortized cost basis is written down to fair value through net loss.

For available-for-sale debt securities that do not meet the aforementioned criteria, the Company evaluates whether the decline in fair value has resulted from credit losses or other factors. In conducting this assessment for debt securities in an unrealized loss position, management evaluates the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security, among other factors.

If this assessment indicates that a credit loss exists, the present value of cash flows expected to be collected from the investment security are compared to the amortized cost basis of the security. If the present value of cash flows expected to be collected is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded for the credit loss, limited by the amount that the fair value is less than the amortized cost basis. Any unrealized loss that has not been recorded through an allowance for credit loss is recognized in other comprehensive income (loss). As of September 30, 2026 and December 31, 2025, there was no allowance for credit losses recorded on the Company's condensed consolidated balance sheet.

The Company's interest income consists of interest earned from cash, cash equivalents, and short-term investments.

Deferred Offering Costs

The Company capitalizes certain legal, professional, accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of an equity financing, these costs are recorded as a reduction of the proceeds from the offering, either as a reduction of the carrying value of the convertible preferred stock or in stockholders' equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering. Should the in-process equity financing be abandoned, the deferred offering costs would be expensed immediately as a charge to operating expenses in the condensed consolidated statements of operations and comprehensive loss. In connection with the 2026 Registration Statement (as defined below), there were $0.5 million of deferred offering costs recorded.

As of September 30, 2026 and December 31, 2025, the Company recorded deferred offering costs of $0.5 million and $35 thousand, respectively, on its condensed consolidated balance sheet.

Collaboration Arrangements

The Company analyzes its license agreements at contract inception to assess whether they are collaborative arrangements as defined by ASC No. 808, *Collaborative Arrangements* (ASC 808). Such determination is made based on whether the arrangements involve a joint operating activity in which both parties are active participants and exposed to significant risks and rewards that are dependent on the commercial success of the activity. To the extent an arrangement is within the scope of ASC 808, the Company evaluates whether any aspect of the arrangement between the Company and its partner is subject to other accounting literature. Transactions in a collaborative arrangement associated with vendor-customer exchanges between the parties are accounted for in accordance with ASC No. 606, *Revenue from Contracts with Customers* (ASC 606). Any aspect of a collaborative arrangement that is within the scope of other literature is accounted for pursuant to the applicable guidance. Transactions between collaborative partners that are outside the scope of other guidance are accounted for based on an analogy to authoritative accounting literature, or a reasonable, rational and consistently applied accounting policy election if there is no appropriate analogy. The Company considers the nature of the arrangement, nature of its business operations and contractual terms of the arrangement in determining the treatment of transactions not addressed within other accounting literature. Payments or reimbursements from collaborators related to shared research and development costs are recorded as a reduction to research and development expense, as such amounts represent cost-sharing in the context of a collaborative arrangement rather than consideration for goods or services provided to a customer.

Revenue Recognition

Revenue recognized to date has been generated exclusively from the Company's license arrangement with Tenacia Biotechnology (Hong Kong) Co., Ltd. ("Tenacia") pursuant to which it licensed the rights to certain named compounds for exploitation in specified territories (the "Tenacia License Agreement"). Through September 30, 2026, the Company has no products approved for commercial use and has not generated any revenue from product sales. Accordingly, the Company's revenue has been presented as collaboration revenue within the accompanying condensed consolidated statement of operations and comprehensive loss.

For transactions and contracts in which the counterparty is a customer, the Company recognizes revenue in accordance with ASC 606. Under ASC 606, the Company recognizes revenue when its customer obtains control of the promised goods and/or services in an amount that reflects the consideration it expects to receive in exchange for those goods and/or services. To determine the appropriate amount of revenue to be recognized for agreements and elements of contracts determined to be within the scope of ASC 606, the Company performs the following steps: (i) Identify the contract(s) with the customer, (ii) Identify the promised goods and/or services in the contract and determine which promised goods and/or services represent performance obligations, (iii) Measure the transaction price, (iv) Allocate the transaction price to the performance obligations in the contract and (v) Recognize revenue when (or as) each performance obligation is satisfied.

Pursuant to the guidance in ASC 606, the Company accounts for a contract or elements of a contract with a customer that is within the scope of ASC 606, when all of the following criteria are met: (i) The arrangement has been approved by the parties and the parties are committed to perform their respective obligations, (ii) Each party's rights regarding the goods and/or services to be transferred can be identified, (iii) The payment terms for the goods and/or services to be transferred can be identified, (iv) The arrangement has commercial substance and (v) Collection of substantially all of the consideration to which the Company will be entitled in exchange for the goods and/or services that will be transferred to the customer is probable.

The Company assesses the goods and/or services promised within a contract or elements of a contract that contains multiple promises to evaluate which promises are distinct. Promises are considered to be distinct and therefore, accounted for as separate performance obligations, provided that: (i) The customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer and (ii) The promise to transfer the good or service to the customer is separately identifiable from the other promises in the contract or elements of a contract. The Company determines that a customer can benefit from a good or service if it could be used, consumed, sold or otherwise held in a way that generates economic benefits. Factors that are considered in determining whether or not two or more promises are not separately identifiable include, but are not limited to, the following: (i) The Company provides a significant service of integrating goods and/or services with other goods and/or services promised, (ii) One or more of the goods and/or services significantly modifies or customizes, or are significantly modified or customized by, one or more of the other goods and/or services promised and (iii) The goods and/or services are highly interdependent or highly interrelated. In assessing whether promised goods and/or services are distinct from the other promises, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the collaborative partner and the availability of the associated expertise in the marketplace. The Company also considers whether the customer can benefit from a promise for its intended purpose without the receipt of the remaining promises and whether the value of a promise is dependent on the unsatisfied promises. Individual goods or services (or bundles of goods and/or services) that meet both criteria for being distinct are accounted for as separate performance obligations. Promises that are not distinct at contract inception are combined into a single performance obligation. Significant judgments made in accounting for contracts with customers primarily relate to the identification of performance obligations in the arrangement.

The Company considers a customer's right to elect to obtain additional goods and/or services at such customer's discretion to be an option if it is not presently obligated to provide the goods and/or services and it is not entitled to compensation in exchange for the associated goods and/or services. Options to acquire additional goods and/or services are evaluated to determine if the option provides a material right to the customer that it would not have received without entering into the contract. If so, the option is accounted for as a separate performance obligation. If not, the option is considered a marketing offer which would be accounted for as a separate contract upon the customer's exercise. Situations in which a customer has an ability to acquire additional goods and/or services for free or at significantly discounted rates would be considered to provide the customer with a material right. Options to purchase goods and/or services at prices that reflect the standalone selling prices of the associated goods and/or services are accounted for as marketing offers.

The Company measures the transaction price in reference to the amount of consideration to which it expects to be entitled in exchange for transferring the promised goods and/or services to the customer. Accordingly, the transaction price at inception is comprised entirely of a fixed fee due a specified number of days from contract execution. With respect to royalties, including milestone payments triggered upon the first commercial sale of a licensed product or based upon the achievement of a certain level of product sales, wherein the license is deemed to be the sole or predominant item to which the payments relate, the Company recognizes revenue upon the later of: (i) When the related sales occur or (ii) When the performance obligation to which some or all of the payment has been allocated has been satisfied (or partially satisfied). Amounts to be received with respect to customer options will be included in the transaction price for the associated contract upon exercise.

The Company allocates the transaction price to each performance obligation identified in the contract on a relative standalone selling price basis. The Company's contract with Tenacia is comprised of a single performance obligation. Therefore, the transaction price has been allocated in its entirety to the sole performance obligation identified in the arrangement. Option exercise fees are allocated to the goods and/or services underlying the associated option.

Revenue is recognized based on the amount of the transaction price that is allocated to each respective performance obligation when or as the performance obligation is satisfied by transferring a promised good and/or service to the customer. For performance obligations that are satisfied at a point in time, the Company recognizes revenue when control of the goods and/or services are transferred to the customer. For performance obligations that are satisfied over time, the Company recognizes revenue by measuring the progress toward complete satisfaction of the performance obligation using a single method of measuring progress which depicts the performance in transferring control of the associated goods and/or services to the customer. With respect to promises related to licenses to intellectual property that are determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenue from amounts allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license.

The Company receives payments from its licensee based on billing schedules established in the contract. Upfront payments are recorded as contract liabilities within deferred revenue until the Company performs its obligations under the associated agreement. No portion of any upfront payments remains deferred as of September 30, 2026. Amounts payable to the Company are recorded as accounts receivable when the Company's right to the consideration is unconditional.

Net Loss Per Share

The Company only has one class of shares outstanding, and basic net loss per common share is computed by dividing the net loss by the weighted average number of shares of common stock outstanding for the period. Diluted net loss per common share is computed by dividing net loss by the weighted average number of shares of common stock outstanding for the period, including potential dilutive common shares assuming the dilutive effect of outstanding stock awards. For periods in which the Company reports a net loss, diluted net loss per common share is the same as basic net loss per common share, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders. As per the statements of operations and comprehensive loss for the three and nine months ended September 30, 2026, comprehensive loss includes unrealized loss on short-term investments. For the three and nine months ended September 30, 2025, comprehensive loss was partially offset by unrealized gains on short-term investments.

Recently Issued Accounting Pronouncements Not Yet Adopted

In December 2025, the FASB issued ASU 2025-11 - Interim Reporting (Topic 270): Narrow-Scope Improvements ("ASU 2025-11"). ASU 2025-11 is intended to clarify and improve certain aspects of interim financial reporting, including the requirements for interim disclosures and the application of recognition and measurement guidance in interim periods. ASU 2025-11 is effective for interim reporting periods within annual reporting periods beginning after December 15, 2027. The Company is currently evaluating the potential impact of the guidance and potential additional disclosures required.

In November 2024, the FASB issued ASU 2024-03, Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses, which requires disclosures about specific types of expenses included in the expense captions presented on the face of the income statement as well as disclosures about selling expenses. The requirements of the ASU are effective for annual periods beginning after December 15, 2026, and for interim periods beginning after December 15, 2027, with early adoption permitted. The requirements will be applied prospectively with the option for retrospective application. The Company is currently in the process of evaluating the effects of this pronouncement on its related disclosures.

3. Fair Value Measurements

The following tables present the Company's fair value hierarchy for its assets and liabilities that are measured at fair value on a recurring basis and indicate the level within the fair value hierarchy of the valuation techniques the Company utilized to determine such fair value (in thousands):

	Fair Value Measurements at September 30, 2026			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	$ 62,213	$ —	$ —	$ 62,213
Short-term investments:				
U.S. Treasury bills	—	9,773	—	9,773
Government securities	—	199,972	—	199,972
Government agency securities	—	110,530	—	110,530
	$ 62,213	$ 320,275	$ —	$ 382,488

	Fair Value Measurements at December 31, 2025			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market funds	$ 47,947	$ —	$ —	$ 47,947
Short-term investments:				
U.S. Treasury bills	—	79,359	—	79,359
Government securities	—	248,398	—	248,398
Government agency securities	—	110,137	—	110,137
	$ 47,947	$ 437,894	$ —	$ 485,841

Money market funds are highly liquid and actively traded marketable securities that generally transact at a stable $1.00 net asset value representing its estimated fair value. There were no transfers between Level 1, Level 2 and Level 3 during the periods presented.

The Company classifies its marketable securities as short-term because they are available to be converted into cash to fund current operations. The fair value of the Company's U.S. Treasury bills, government securities, and government agency securities are classified as Level 2 because they are valued using observable inputs to quoted market prices, benchmark yields, reported trades, broker/dealer quotes or alternative pricing sources with reasonable levels of price transparency.

The underlying securities held in the money market funds held by the Company are all government backed securities.

Short-term investments consisted of the following (in thousands):

	September 30, 2026			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Short-term investments:				
U.S. Treasury bills	$ 9,807	$ —	$ (34)	$ 9,773
Government securities	201,117	—	(1,145)	199,972
Government agency securities	111,466	—	(936)	110,530
	$ 322,390	$ —	$ (2,115)	$ 320,275

	December 31, 2025			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Short-term investments:				
U.S. Treasury bills	$ 79,334	$ 25	$ —	$ 79,359
Government securities	247,968	430	—	248,398
Government agency securities	110,046	91	—	110,137
	$ 437,348	$ 546	$ —	$ 437,894

The contractual maturities of the Company's short-term investments in available-for-sale debt securities held were as follows (in thousands):

	September 30, 2026	December 31, 2025
Due within one year	$ 228,312	$ 243,613
Due between one and two years	91,963	194,281
	$ 320,275	$ 437,894

As of September 30, 2026, all investments in an unrealized loss position were in this position for less than 12 months. The Company evaluated its securities for potential other-than-temporary impairment and considered the decline in market value to be primarily attributable to current economic and market conditions. Additionally, the Company does not intend to sell the securities in

an unrealized loss position and does not expect it will be required to sell the securities before recovery of the unamortized cost basis. Given the Company's intent and ability to hold such securities until recovery, and the lack of a significant change in credit risk for these investments, the Company does not consider these investments to be impaired as of September 30, 2026. The Company did not recognize any credit losses during the three and nine months ended September 30, 2026.

4. Property and Equipment, Net

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

	September 30, 2026	December 31, 2025
Lab equipment	$ 3,641	$ 4,419
Furniture and Fixtures	35	34
Computer equipment	93	92
Leasehold improvements	295	295
Construction in process	11	118
Total property and equipment	4,075	4,958
Less: Accumulated depreciation	(2,118)	(1,982)
	$ 1,957	$ 2,976

Depreciation expense of property and equipment for the nine months ended September 30, 2026 and 2025 was $0.6 million and $0.8 million, respectively.

5. Accrued Expenses and Other Current Liabilities

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

	September 30, 2026	December 31, 2025
Research and development	$ 8,598	$ 3,749
Professional fees	1,794	1,229
Employee related	5,653	7,120
Accrued other	214	6
	$ 16,259	$ 12,104

6. Preferred Stock

On May 30, 2024, the Company's stockholders approved the third amended and restated certificate of incorporation, which was filed upon the closing of the initial public offering ("IPO") on June 10, 2024, and which, among other things, created 10,000,000 undesignated shares of authorized preferred stock, $0.001 par value per share.

7. Common Stock

Each share of common stock entitles the holder to one vote on all matters submitted to the stockholders for a vote. The holders of common stock are entitled to receive dividends, if any, as declared by the Company's board of directors. To date, no dividends have been declared or paid.

In July 2025, the Company filed a shelf registration statement on Form S-3 (the "2025 Registration Statement") with the U.S. Securities and Exchange Commission (the "SEC") with an offering amount of up to $400.0 million in common stock, preferred stock, debt securities, warrants and/or units. Concurrent with the filing of the 2025 Registration Statement, the Company entered into a common stock sales agreement (the "Sales Agreement"), dated July 1, 2025, by and between the Company and Leerink Partners LLC and Cantor Fitzgerald & Co., acting as sales agents, which established an at-the-market offering program pursuant to which the Company may offer and sell shares of its common stock from time to time (the "ATM Program"). In connection with the ATM Program, the Company also filed a prospectus with the Registration Statement for the offer and sale of up to $150.0 million of shares of common stock from time to time through the sales agents (the "July 2025 ATM Prospectus"). In September 2025, the Company terminated the July 2025 ATM Prospectus.

In September 2025, the Company completed an underwritten public offering of 11,057,692 shares of common stock (inclusive of 1,442,307 shares of common stock sold pursuant to the underwriters' full exercise of their option to purchase additional shares) at a public offering price of $26.00 per share (the "September 2025 Offering") pursuant to an effective shelf registration statement (File No. 333-288444) and a related prospectus supplement filed with the SEC. The net proceeds from the September 2025 Offering were approximately $269.4 million, after deducting underwriter discounts, commissions, and other offering costs of $18.1 million.

In connection with the ATM Program, the Company filed a prospectus supplement with the SEC in January 2026, for the offer and sale of up to $110.0 million of shares of common stock from time to time through the sales agents, to be issued pursuant to the Sales Agreement (the "January 2026 ATM Prospectus").

In March 2026, the Company filed an automatic shelf registration statement on Form S-3ASR (the "2026 Registration Statement"), containing (i) a base prospectus, which covers the offering, issuance and sale from time to time in one or more offerings of an indeterminate amount of common stock, preferred stock, debt securities, warrants and/or units; and (ii) a sales agreement prospectus for the offering and issuance and sale of up to a maximum aggregate offering price of $150.0 million of common stock, to be issued pursuant to the Sales Agreement. The 2025 Registration Statement was terminated automatically upon the filing of the 2026 Registration Statement. As of September 30, 2026, the Company has not sold any shares pursuant to the Sales Agreement.

As of September 30, 2026, the Company had reserved 10,898,775 shares of common stock, of which 2,250,270 shares were reserved for issuance under the 2022 Plan, 7,958,460 shares under the 2024 Stock Option and Grant Plan (the "2024 Plan") and 690,045 shares under the 2024 Employee Stock Purchase Plan (the "2024 ESPP") (see Note 8).

8. Stock-Based Compensation

2022 Plan

The 2022 Plan provides for the Company to grant incentive stock options ("ISO") or non-qualified stock options, unrestricted stock awards, restricted stock awards and restricted stock units (collectively, the "Awards") to the employees, directors, and consultants of the Company. The 2022 Plan is administered by the board of directors, or at the discretion of the board of directors, by a committee of the board of directors. The exercise prices, vesting and other restrictions are determined at the discretion of the board of directors, or its committee if so delegated.

The remaining shares reserved for issuance under the 2022 Plan ceased to be available for issuance at the time that the 2024 Plan became effective. There will be no further awards granted under the 2022 Plan, but all outstanding awards under the 2022 Plan will continue to be governed by their existing terms.

Subsequent to the effectiveness of the 2024 Plan, the shares of common stock underlying any awards under the 2022 Plan that are forfeited, cancelled, reacquired by the Company prior to vesting, satisfied without the issuance of stock or otherwise terminated (other than by exercise) and shares withheld upon exercise of an option or settlement of an award to cover the exercise price or tax withholding will be added to the shares of common stock available for issuance under the 2024 Plan.

2024 Plan

In May 2024, the Company's board of directors adopted, and its stockholders approved, the 2024 Plan, which became effective in June 2024. The 2024 Plan allows the Company to make equity-based and cash-based incentive awards to its officers, employees, directors, and consultants. The 2024 plan provides for the grant of incentive stock options, non-qualified stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. In addition, the number of shares reserved and available for issuance under the 2024 Plan will automatically increase on each January 1, by five percent of the outstanding number of shares of common stock on the immediately preceding December 31 or such lesser number of shares as determined by the compensation committee. On January 1, 2026, the annual increase resulted in an additional 2,388,628 shares being added to the 2024 Plan. As of September 30, 2026, there have been 134,373 stock-based awards cancelled under the 2022 Plan from which the underlying shares were added to the shares reserved under the 2024 Plan. As of September 30, 2026, the Company had 2,189,049 shares remaining available for future grants.

The shares of common stock underlying any awards under the 2024 Plan and the 2022 Plan that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by the Company prior to vesting, satisfied without the issuance of stock, expire, or are otherwise terminated (other than by exercise) will be added back to the shares of common stock available for issuance under the 2024 Plan.

2024 ESPP

In May 2024, the Company's board of directors adopted, and its stockholders approved, the 2024 ESPP, which became effective in June 2024. The 2024 ESPP initially reserved and authorized the issuance of up to a total of 324,243 shares of the Company's common stock to participating employees. The 2024 ESPP provides that the number of shares reserved and available for issuance will automatically increase on each January 1 thereafter through January 1, 2034, by the lesser of (i) 648,486 shares of common stock, (ii) one percent of the outstanding number of shares of common stock on the immediately preceding December 31, or (iii) such lesser number of shares of common stock as determined by the administrator of the 2024 ESPP. On January 1, 2026, the administrator determined that no additional shares would be reserved and authorized pursuant to the 2024 ESPP. The number of shares reserved under the 2024 ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization. As of September 30, 2026, the Company had 690,045 shares reserved for the 2024 ESPP.

No shares were issued during the nine months ended September 30, 2026 relating to the 2024 ESPP.

Stock Options

The Company has granted stock options with service-based vesting conditions. Stock options generally vest over four years and have a maximum term of ten years. The Company typically grants stock options to employees and non-employees at exercise prices deemed by the Board to be equal to the fair value of the common stock at the time of grant. The following table summarizes the Company's stock option activity for the nine months ended September 30, 2026:

	Number of Shares	Weighted-Average Exercise Price per share	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Balance at December 31, 2025	6,287,477	$ 12.23	8.62	$ 113,839
Granted	2,230,527	28.92		
Exercised	(361,827)	11.36		
Forfeited	(201,239)	21.27		
Options outstanding at September 30, 2026	7,954,938	$ 16.72	8.28	$ 135,181
Options vested and exercisable at September 30, 2026	3,340,172	12.34	7.89	71,006
Options vested and expected to vest at September 30, 2026	7,954,938	$ 16.72	8.28	$ 135,181

The weighted-average grant-date fair value of stock options granted during the nine months ended September 30, 2026 and 2025 was $20.39 and $10.88 per share, respectively. As of September 30, 2026, there was $64.8 million of total unrecognized compensation cost related to unvested stock options, which is expected to be recognized over a remaining weighted average period of 2.4 years.

Restricted Stock Awards ("RSA")

The Company has awarded RSAs both under the 2022 Plan as well as outside of the 2022 Plan. To date, the Company has not awarded any RSAs under the 2024 Plan.

Service-Based RSAs

The majority of the RSAs have service-based vesting conditions and vest over a period from immediately to four years. Compensation expense is recognized on a straight-line basis over the requisite service period.

The following table summarizes the Company's service-based RSA grant activity for the nine months ended September 30, 2026:

	RSAs	Weighted-Average Grant Date Fair Value
Unvested shares at December 31, 2025	411,833	$ 3.94
Granted	—	—
Vested	(252,684)	3.87
Forfeited	(28,846)	2.92
Unvested shares at September 30, 2026	130,303	$ 4.28

The aggregate intrinsic fair value of service-based RSAs that vested during the nine months ended September 30, 2026 and 2025, was $9.1 million and $4.4 million, respectively. As of September 30, 2026, there was $0.5 million of total unrecognized compensation cost related to unvested service-based RSAs, which is expected to be recognized over a remaining weighted average period of 0.6 years.

Performance-Based RSAs

The Company has also granted performance-based RSAs to certain employees and directors with a vesting commencement date contingent upon the subsequent closing of the Company's Series A convertible preferred stock financing. The Company has determined that it has met all the conditions to establish the grant date for these performance-based RSAs at the original issuance date. Therefore, these awards are deemed to contain an implied performance condition. The vesting of the performance-based RSAs is also subject to grantees' continued service until the 4th anniversary date of the closing of a subsequent financing.

Share-based compensation expense associated with the performance-based RSAs is recognized if the performance condition is considered probable of achievement. In February 2023, the existing Series A convertible preferred stock investors waived the second and third tranche milestones and the Company closed on the sale of its second and third tranches of Series A convertible preferred stock. As a result, the performance condition was deemed to be met.

The following table summarizes the Company's performance-based RSA grant activity for the nine months ended September 30, 2026:

	RSAs	Weighted-Average Grant Date Fair Value
Unvested shares at December 31, 2025	212,977	$ 3.72
Granted	—	—
Vested	(123,274)	3.81
Forfeited	(24,129)	2.92
Unvested shares at September 30, 2026	65,574	$ 3.85

The aggregate intrinsic fair value of performance-based RSAs that vested during the nine months ended September 30, 2026 and 2025 was $4.4 million and $2.0 million, respectively. As of September 30, 2026, there was $37 thousand of total unrecognized compensation cost related to unvested performance-based restricted common stock, which is expected to be recognized over a remaining weighted average period of 0.3 years.

Restricted Stock Units ("RSUs")

Each RSU represents a right to receive one share of the Company's common stock when it becomes vested. The RSUs vest in two equal tranches over a two year period starting on the grant date subject to the satisfaction of a service relationship with the Company.

The following table summarizes restricted stock unit activity for the nine months ended September 30, 2026 and provides information for unvested shares as of September 30, 2026.

	RSUs	Weighted-Average Grant Date Fair Value
Unvested shares at December 31, 2025	13,987	$ 7.15
Granted	10,000	31.67
Vested	(6,994)	7.15
Forfeited	—	—
Unvested shares at September 30, 2026	16,993	$ 21.58

As of September 30, 2026, there was $0.3 million of total unrecognized compensation cost related to unvested RSUs, which is expected to be recognized over a remaining weighted average period of 0.9 years.

Performance-Based Restricted Stock Units ("PSUs")

In December 2024, the Company granted 95,500 performance-based restricted stock units ("PSUs") to certain employees. Each PSU represents a right to receive one share of the Company's common stock when it becomes vested.

The PSUs vest in two equal tranches over two performance periods starting on the grant date and ending, respectively, on December 31, 2025, and December 31, 2026, subject to the satisfaction of both service and performance conditions specifically defined for each performance period and each PSU award. The performance conditions are related to the achievement of certain program milestones in the Company's drug discovery and development process.

In December 2025, the Compensation Committee confirmed the achievement of certain performance milestones for the first tranche of the PSUs related to the achievement of certain drug discovery and development milestones in 2025, with 34,750 PSU shares vesting on December 31, 2025, and also confirmed there were certain milestones for the first tranche that were not achieved, resulting in the forfeiture of 13,000 PSU awards. In connection with the December 31, 2025 PSU vesting tranche, the Company recognized $0.9 million compensation expense.

During the three and nine months ended September 30, 2026, the Company did not recognize any additional compensation expense for the PSUs, as no additional performance conditions were considered probable of being met.

There were no PSUs granted in the nine months ended September 30, 2026 and 2025. As of both September 30, 2026 and December 31, 2025, the unrecognized compensation expenses associated with the PSUs were $1.0 million, with 47,750 shares unvested as of September 30, 2026.

Stock-Based Compensation

The Company recorded stock-based compensation expense for stock options of $7.2 million and $4.2 million in the three months ended September 30, 2026 and 2025, respectively, and $20.7 million and $11.4 million in the nine months ended September 30, 2026 and 2025, respectively. The Company recorded stock-based compensation expense for RSAs of $0.3 million and $0.5 million in the three months ended September 30, 2026 and 2025, respectively, and $1.0 million and $1.7 million in the nine months ended September 30, 2026 and 2025, respectively.

The following table summarizes the classification of the Company's stock-based compensation expense in the condensed consolidated statements of operations and comprehensive loss (in thousands):

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
Selling, general and administrative	$ 4,665	$ 3,037	$ 13,136	$ 8,536
Research and development	2,969	1,698	8,663	4,628
	$ 7,634	$ 4,735	$ 21,799	$ 13,164

9. Leases

Operating Lease

In June 2023, the Company entered into a lease in Boston, Massachusetts commencing August 31, 2023 with an initial term of 40 months. The monthly lease payments are $66 thousand for the first 12 months, with 2% escalation each year. In conjunction with the lease, the Company paid a security deposit of $0.1 million that is recorded on the Company's condensed consolidated balance sheet in prepaid expenses and other current assets as of September 30, 2026.

In February 2024, the Company entered into a lease in San Diego, California to lease a total of 20,626 square feet for laboratory and office space. The lease commenced January 11, 2025 with an initial term of 60 months with no option to extend for an additional term. The lease provides for annual base rent for the premises of approximately $1.9 million for the first year of the lease. Thereafter, the annual base rent will increase at an adjustment rate of 3.0% each year until the end of the term. The Company is also obligated to pay the landlord certain fees, taxes, and operating expenses, subject to certain exclusions.

In November 2024, the Company entered into a sublease for a new corporate headquarters space in Boston, Massachusetts for 15,275 square feet of office space. The lease commenced June 1, 2025 with a lease term of 6.5 years with no option to extend for an additional term. The lease provides for annual base rent for the premise of approximately $0.8 million for the first year of the lease. Thereafter, the annual base rent will increase at an adjustment of 2.0% each year until the end of the term. The Company is also obligated to pay the landlord certain fees, taxes, and operating expenses, subject to certain exclusions. In conjunction with the lease commencement, the Company paid a security deposit of $0.4 million that is recorded on the Company's condensed consolidated balance sheet in other assets as of September 30, 2026.

10. Commitments and Contingencies

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scopes and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with the board of directors that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company is not aware of any indemnification arrangements that could have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its condensed consolidated financial statements as of September 30, 2026 and December 31, 2025.

Legal Proceedings

From time to time, the Company may become involved in legal proceedings or other litigation relating to claims arising in the ordinary course of business. The Company accrues a liability for such matters when it is probable that future expenditures will be made and that such expenditures can be reasonably estimated. Significant judgment is required to determine both probability and estimated exposure amount. Legal fees and other costs associated with such proceedings are expensed as incurred. As of September 30, 2026 and December 31, 2025, the Company was not a party to any material legal proceedings or claims.

NeuroPace Master Services Agreement and Statement of Work

In November 2023, the Company entered into a master services agreement (the "NeuroPace Agreement") with NeuroPace Inc. ("NeuroPace"), the manufacturer and distributor of the RNS system. Pursuant to the NeuroPace Agreement and in accordance with statement of work agreements entered into from time to time, NeuroPace provides the Company with certain services with respect to data from the RNS systems used in the Company's clinical trials of RAP-219 in drug-resistant focal onset seizures ("FOS"). The NeuroPace Agreement also grants the Company a royalty-free, worldwide, exclusive, non-transferable license to all data collected by the RNS systems in its RAP-219 clinical trials in drug-resistant FOS and the outcomes of algorithms that are applied to such data, as well as the ability to publish the outcomes of algorithms, subject to certain conditions. The consideration the Company will pay to NeuroPace for such services is set out in each statement of work agreement.

Concurrently with the execution of the NeuroPace Agreement, the parties also entered into an initial statement of work, as amended in March 2024, and a second statement of work, as amended in February 2026, (collectively, the "NeuroPace SOWs") under the NeuroPace Agreement, pursuant to which NeuroPace agreed to provide services related to the RAP-219 Phase 2a clinical trial and open-label long term safety trial in RAP-219 for FOS, including, among other things, clinical trial readiness support, identification of potential patients satisfying the enrollment criteria and RNS system data reporting and data analysis. Pursuant to the payment schedule set out in the NeuroPace SOWs, the Company will pay NeuroPace an aggregate of up to $5.8 million over a period of approximately four years in connection with NeuroPace's provision of services and achievement of certain patient enrollment and deliverable milestones. As of September 30, 2026, the Company had incurred cumulative expenses of $4.9 million on both NeuroPace SOWs.

11. License and Collaboration Agreement

Tenacia License Agreement

Agreement Summary

On March 6, 2026, the Company entered into the Tenacia License Agreement. The arrangement provides Tenacia with the exclusive rights to develop and commercialize the Company's product candidate, RAP-219, in mainland China, Hong Kong, Macau and Taiwan (each, a "Market" and collectively, the "Tenacia Territory"). The Company retains exclusive rights to RAP-219 outside the Tenacia Territory.

Under the terms of the Tenacia License Agreement, the Company granted to Tenacia a license to use certain intellectual property for the development and commercialization of RAP-219 and products containing RAP-219 across indications, including FOS and bipolar mania. The license conveyed by the Company also provides Tenacia with the ability to use the covered intellectual property on a non-exclusive basis for manufacturing and supply purposes.

Pursuant to the terms of the Tenacia License Agreement, Tenacia is responsible for conducting all development and commercialization activities with respect to the Tenacia Territory, with the exception of the associated manufacturing. The Tenacia License Agreement requires Tenacia to participate in the Company's planned global clinical trials for RAP-219 and products containing RAP-219 for the FOS and bipolar mania indications, including long-acting injectable formulations, by conducting the part of the global clinical trials in the Tenacia Territory. Tenacia will cover development costs for the Tenacia Territory and share the corresponding global trial costs at the percentage specified in the Agreement. In connection with the Tenacia License Agreement, the Company entered into a clinical supply agreement with Tenacia in June 2026. Pursuant to that supply agreement, the Company will provide manufacturing and supply for clinical and commercial purposes at agreed upon pricing until such time when Tenacia takes over manufacturing responsibilities.

Under the terms of the Tenacia License Agreement, the Company received a $20.0 million non-refundable upfront payment. Additionally, to the extent Tenacia commercializes any licensed products containing RAP-219, the Company is eligible to receive up

to $308.0 million in contingent sales-based milestone payments and tiered royalty payments ranging from the mid-single digits to the mid-teens based on a percentage of net sales, subject to certain customary reductions and offsets.

Unless earlier terminated, the Tenacia License Agreement will expire on a Market-by-Market basis at the end of the applicable royalty term. The Tenacia License Agreement contains customary termination rights, including for uncured material breach, insolvency and certain other specified circumstances. Any expiration or termination of the Tenacia License Agreement does not affect the rights and obligations of the parties that accrued prior to the expiration or termination date.

Accounting Analysis

The Company determined that the Tenacia License Agreement is a collaborative arrangement as defined by ASC 808 because it involves a joint operating activity comprised of the development of RAP-219 and products containing RAP-219 wherein both parties are: (i) Active participants via participation in the global clinical trials for their respective territory and (ii) Exposed to significant risks and rewards dependent on the commercial success of the development efforts since recovery of costs incurred is contingent upon receipt of regulatory approval. The Company further determined that the contract is partially within the scope of ASC 606 since it contains transactions in which Tenacia is a customer for a good or service that is a distinct unit of account. More specifically, the Company concluded that the Tenacia License Agreement is composed of the following components: (i) License granted to Tenacia to exploit RAP-219 and products containing RAP-219, including manufacturing rights and (ii) Parties' participation in the global development of RAP-219 and products containing RAP-219. For each of these components, the Company determined the following: (i) Unit of account associated with license granted to Tenacia is within the scope of ASC 606 since the transaction is in the context of a vendor-customer exchange and (ii) Parties' conduct of development activities related to the global clinical trials is outside the scope of ASC 606 since the parties are operating in the capacity of collaborative partners.

The Company's obligations under the Tenacia License Agreement comprise a single promise related to the exclusive license that was granted to Tenacia to use certain intellectual property in the development and commercialization of RAP-219 and products containing RAP-219, including manufacturing rights. Accordingly, the Company determined that the Tenacia License Agreement has only one performance obligation consisting of the sole promise in the contract. As it relates to Tenacia's option to purchase clinical and commercial supply from the Company, the Tenacia License Agreement does not obligate Tenacia to purchase any minimum amount or quantities. As such, the Company concluded that the provision of manufacturing activities related to clinical and commercial supply represents customer options. Such options do not give rise to any performance obligations because the applicable terms, including the anticipated pricing, do not incorporate a material right. Instead, the options were determined to be marketing offers as their pricing is indicative of the standalone selling prices of the underlying goods and/or services. Therefore, the manufacturing activities were excluded as a performance obligation at the outset of the Tenacia License Agreement. Any purchases will be accounted for as a separate arrangement. The Company entered into a clinical supply agreement with Tenacia in June 2026. The Company will recognize revenue under the clinical supply agreement upon transfer of control to Tenacia. No other customer options had been exercised as of September 30, 2026.

The Company's license arrangement with Tenacia provides for the payment of the following: (i) Non-refundable upfront fee, (ii) First commercial sale milestones, (iii) Sales-based milestones and (iv) Royalties on net sales of licensed products. The Company measured the transaction price solely in reference to the $20.0 million upfront payment. None of the variable consideration payable under the arrangement is included in the transaction price. All potential milestone and royalty payments are subject to the royalty recognition constraint whereby such amounts will be recognized as revenue upon the later of: (i) When the related sales occur or (ii) When the performance obligation to which some or all of the payment has been allocated has been satisfied (or partially satisfied) because the exclusive license is deemed to be the sole or predominant item to which the payments relate. Because the payments wholly relate to the exclusive license which was conveyed at inception of the arrangement, the Company will recognize revenue associated with the milestones and royalties from the sales of licensed products when the respective triggering event or related sales occur, as applicable. Through September 30, 2026, no milestones have been achieved and no royalties have been earned.

The Company allocated the transaction price of $20.0 million to the sole identified performance obligation in its entirety. Amounts allocated to the performance obligation comprised entirely of the exclusive license promise are recognized as revenue at a point in time since the underlying license represents functional intellectual property. Control of the license which granted the right to use the Company's intellectual property was transferred to Tenacia simultaneously with contract effectiveness on March 6, 2026 consistent with when the intellectual property was made available for Tenacia's use and benefit. As a result, the Company has recognized $20.0 million of collaboration revenue during the nine months ended September 30, 2026 upon satisfaction of the associated performance obligation.

Conversely, the global development activities under the Tenacia License Agreement do not represent a transaction with a customer. Accordingly, payments received by the Company resulting from reimbursements for Tenacia's share of development costs will be accounted for as a reduction to the associated expense in the period the costs are incurred in the condensed consolidated statement of operations and comprehensive loss. As of September 30, 2026, the Company recorded $0.2 million in accounts receivable on the condensed consolidated balance sheet for reimbursable costs incurred under the Tenacia License Agreement. During the three and nine months ended September 30, 2026, the Company recognized $0.1 million and $0.4 million, respectively, as a reduction of research and development expense.

As of September 30, 2026, the Company did not have any deferred revenue related to the Tenacia License Agreement. The Company incurred approximately $2.0 million of incremental costs for financial advisory fees to obtain the arrangement with Tenacia. Such amount was expensed in full during the nine months ended September 30, 2026 consistent with the timing of the transfer of the associated performance obligation. The amount is classified as selling, general and administrative expense in the accompanying condensed consolidated statement of operations and comprehensive loss for the nine months ended September 30, 2026. No contract assets related to costs to obtain a contract with a customer or costs to fulfill a contract with a customer are capitalized at September 30, 2026.

12. Segment

The Company is currently developing medicines for patients with neurological or psychiatric disorders in the United States. The Company does not have any revenue generating products, and revenue will not be generated from any other current or future product candidates until regulatory approval is obtained and products are commercialized.

For the three and nine months ended September 30, 2026 and 2025, the Company identified one operating and reportable segment. The Company defines its operating segments based on internally reported financial information that is regularly reviewed by the Chief Operating Decision Maker ("CODM") to analyze financial performance, make decisions, and allocate resources. The Company's Chief Executive Officer is the CODM.

The CODM reviews the segment's profit or loss based on net (loss) income reported on the condensed consolidated statement of operations and comprehensive loss and considers forecast-to-actuals variances on a quarterly basis for expenses that are deemed significant. Further, the CODM reviews the segment's assets based on total assets reported on the condensed consolidated balance sheet. In addition, the CODM is regularly provided information on total cash, which is inclusive of cash, cash equivalents and short-term investments, as a measure of segment assets. As of September 30, 2026, the Company's cash, cash equivalents and short-term investments excluding restricted cash, were $395.0 million. All long-lived assets are held in the United States.

The Company's CODM views specific categories within research and development expenses and selling, general and administrative expenses as significant given the direct correlation between cash burn and profitability as a pre-revenue company. The following table reconciles reported revenues to net loss under the significant expense principle for the three and nine months ended September 30, 2026 and 2025 (in thousands):

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
Collaboration revenue	$ —	$ —	$ 20,000	$ —
Research and development:				
RAP-219 program external expenses	17,481	8,733	67,390	26,381
Preclinical programs external expenses	4,473	4,474	13,632	12,442
R&D personnel-related costs (including stock-based compensation)	10,559	7,381	31,836	20,833
Other costs	1,803	1,691	5,588	4,875
Selling, general and administrative:				
G&A personnel-related costs (including stock-based compensation)	7,486	5,487	21,554	15,491
Professional and consulting costs	1,459	1,322	6,594	3,906
Facility related and other	825	898	2,513	2,662
Loss from operations	$ (44,086)	$ (29,986)	$ (129,107)	$ (86,590)
Interest income	3,851	3,061	12,400	8,870
Net loss	$ (40,235)	$ (26,925)	$ (116,707)	$ (77,720)

Accordingly, the Company consists of a single operating and reportable segment and the condensed consolidated financial statements and notes thereto are presented as a single reportable segment.

13. Net Loss per Share

Basic and diluted net loss per common share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
Numerator:				
Net loss attributable to common stockholders	$ (40,235)	$ (26,925)	$ (116,707)	$ (77,720)
Denominator:				
Weighted average common shares outstanding, basic and diluted	47,768,633	37,931,170	47,494,757	36,223,887
Net loss per share attributable to common stockholders, basic and diluted	$ (0.84)	$ (0.71)	$ (2.46)	$ (2.15)

For purposes of this calculation, the Company excluded the following potential common shares, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share available to common shareholders for the periods indicated because including them would have had an anti-dilutive effect:

	As of September 30,	
	2026	2025
Options to purchase common stock	7,954,938	6,296,672
Unvested performance-based restricted stock units	47,750	95,500
Unvested restricted stock units	16,993	13,987
Unvested restricted common stock—service based	130,303	544,235
Unvested restricted common stock—performance based	65,574	258,613

14. Subsequent Events

Underwritten Offerings of Common Stock, Pre-Funded Warrants and Convertible Notes and Concurrent Private Placement

In October 2026, the Company issued and sold in separate underwritten offerings with Morgan Stanley & Co. LLC, Leerink Partners LLC and Evercore Group L.L.C. (the "Underwriters"): (i) 9,761,905 shares of common stock at a public offering price of $21.00 per share (the "Offering Price"), and, in lieu of shares of common stock to certain investors, pre-funded warrants to purchase up to 1,190,476 shares of common stock at a public offering price of $20.999 per share, which represents the Offering Price for the common stock less the $0.001 per share exercise price for each pre-funded warrant, which included the Underwriters' exercise of their option to purchase additional shares of common stock in full (the "2026 Equity Offering") and (ii) $172.5 million aggregate principal amount of 2.25% convertible senior notes due 2032, which included the Underwriters' exercise of their option to purchase additional Notes in full (the "Notes") (the "2026 Notes Offering" and together with the 2026 Equity Offering, the "2026 Underwritten Offerings").

Concurrently with the 2026 Underwritten Offerings, the Company issued 1,428,571 shares of the Company's common stock at a price per share equal to the Offering Price to certain investors in a private placement (the "2026 Private Placement" and together with the 2026 Underwritten Offerings, the "October 2026 Offerings"). The October 2026 Offerings closed on October 30, 2026, subject to customary closing conditions.

The net proceeds from the 2026 Equity Offering are approximately $215.7 million, after deducting underwriting discounts and commissions and estimated offering expenses. The net proceeds from the 2026 Notes Offering are approximately $166.8 million, after deducting underwriting discounts and commissions and estimated offering expenses. The net proceeds from the 2026 Private Placement are approximately $28.2 million, after deducting placement agent discounts and commissions and estimated offering expenses.

The Notes are senior, unsecured obligations of the Company, bear interest at a rate of 2.25% per annum, payable semi-annually in arrears on June 1 and December 1 of each year beginning on June 1, 2027, and will mature on December 1, 2032, unless earlier repurchased, redeemed or converted. The initial conversion rate is 37.3483 shares of common stock per $1,000 principal amount of Notes, equivalent to an initial conversion price of approximately $26.7750 per share, a premium of approximately 27.50% over the Offering Price per share of the Company's common stock in the 2026 Equity Offering. Upon conversion, the Company will pay or deliver cash, shares of common stock or a combination, at the Company's election.

ATM Program Sale

Subsequent to September 30, 2026, the Company sold 399,201 shares of common stock under the ATM Program for gross proceeds of approximately $10 million.

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 in conjunction with our condensed consolidated financial statements and related notes and other financial information included elsewhere in this Quarterly Report on Form 10-Q (this "Quarterly Report") and with our audited financial statements and the notes thereto for the year ended December 31, 2025 included in our Annual Report on Form 10-K for the year ended December 31, 2025 (our "Annual Report"). This discussion and analysis and other parts of this Quarterly Report contain forward-looking statements based upon our current plans and expectations that involve risks, uncertainties and assumptions, such as statements regarding our plans, strategies, objectives, expectations, intentions and beliefs. Our actual results and the timing of events could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth under "*Risk Factors*" and elsewhere in this Quarterly Report. You should carefully read the "Risk Factors" section of this Quarterly Report to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see "Special Note Regarding Forward-Looking Statements." Our historical results are not necessarily indicative of the results that may be expected for any period in the future.*

Overview

We are a clinical-stage biotechnology company dedicated to the discovery and development of small molecule precision medicines for patients with neurological or psychiatric disorders. Our foundational science has elucidated complexities of neuronal receptor biology and enables us to map and target certain neuronal receptor complexes. Neuronal receptors are complex assemblies of proteins, comprising receptor principal subunits and their receptor associated proteins ("RAPs"), the latter of which play crucial roles in regulating receptor expression and function. We believe that our deep expertise in RAP biology provides an opportunity for us to interrogate previously inaccessible targets and develop neurological and psychiatric drugs that are specific for receptor variants and neuroanatomical regions associated with certain diseases. Most neuroactive drugs lack this specificity, often resulting in undesired and intolerable side effects. Leveraging our expertise, we are developing a portfolio of precision product candidates that we believe has the potential to transform the standard of care of many neurological and psychiatric disorders.

Our founders have made pioneering discoveries related to the function of RAPs in the brain. Their findings form the basis of our RAP technology platform, which enables a differentiated approach to generate precision small molecule product candidates with the potential to overcome many limitations of conventional neurology drug discovery. RAP-219, our most advanced product candidate, is an AMPA receptor ("AMPAR") negative allosteric modulator ("NAM"). RAP-219 is designed to achieve neuroanatomical specificity through its selective targeting of a RAP known as TARPγ8, which is associated with the neuronal AMPARs. Whereas AMPARs are distributed widely in the central nervous system ("CNS"), TARPγ8 is expressed only in discrete regions, including the neocortex and mesial temporal lobe, where focal onset seizures ("FOS") often originate. By contrast, TARPγ8 has minimal expression in the hindbrain, where drug effects are often associated with adverse events. As such, we believe RAP-219 has the potential for a differentiated profile as compared to traditional neuroscience medications. Due to the role of AMPA biology in various neurological disorders and our precision approach of selectively targeting TARPγ8, we believe RAP-219 has pipeline-in-a-product potential and we are evaluating it as a potentially transformational treatment for patients with FOS, primary generalized tonic-clonic seizures ("PGTCS"), and bipolar mania.

Several Phase 1 trials in RAP-219 have been conducted in healthy adult volunteers, including a single ascending dose ("SAD") trial; a multiple ascending dose ("MAD") trial; a second MAD trial ("MAD-2"), to assess alternative dosing regimens that may accelerate time to reach therapeutic exposure; and a positron emission tomography ("PET") trial, which utilized a companion PET radiotracer to confirm brain target receptor occupancy ("RO") and brain region specificity across a range of dosing and exposure levels.

In September 2025, we announced positive topline results from our Phase 2a proof-of-concept trial of RAP-219 in adult patients with drug-resistant FOS. The trial met its primary and secondary endpoints. The trial demonstrated a statistically significant reduction in long episodes ("LEs") – an objective electrographic biomarker for clinical seizure reduction – compared with baseline over the 8-week treatment period. In the trial, RAP-219 also demonstrated a statistically significant and clinically meaningful reduction in clinical seizures compared with baseline. RAP-219 was generally well tolerated. In December 2025, we presented post-hoc analysis from the Phase 2a proof-of-concept trial showing treatment with RAP-219 had consistent effects in the first (weeks 1-4) and second (weeks 5-9) four-week segments of the treatment period. This demonstrates that there was a rapid onset of efficacy, and a consistent reduction in LEs, and a consistent, clinically meaningful reduction in clinical seizures throughout the 8-week treatment period. Following 8-weeks of RAP-219 treatment in the Phase 2a trial, patients entered an 8-week follow-up period to assess the durability effect on LEs and clinical seizures. In April 2026, we presented results from the 8-week follow-up period. Based on pharmacokinetic ("PK") data collected across the Company's Phase 1 and Phase 2 trials and further supported by population PK modeling, RAP-219 is now estimated to have a 22-day half-life, compared to the prior reported estimate of 14 days. Due to the long half-life, plasma concentrations of RAP-219 remained within the receptor occupancy therapeutic levels over the 8-week follow-up period, resulting in sustained biomarker and clinical responses consistent with the 8-week treatment period. In the first four weeks of follow-up (weeks 9-12), RAP-219 showed even greater reductions in LEs and clinical seizures than were observed during the 8-week treatment period. Clinically meaningful improvements over baseline continued through the second four weeks of follow-up (weeks 13-16).

In late 2025, we initiated an open-label long term safety trial to allow patients enrolled in our Phase 2a proof-of-concept trial in drug-resistant FOS to resume taking RAP-219. Initial data from the open-label trial are expected in the fourth quarter of 2026. In December 2025, we received U.S. Food and Drug Administration ("FDA") feedback from an end-of-Phase 2 meeting supporting advancement into two Phase 3 trials of RAP-219 in patients with drug-resistant FOS. We initiated the Phase 3 program in the second quarter of 2026, with two parallel pivotal trials, FOCUS 1 and FOCUS 2, currently enrolling patients.

We are also expanding our epilepsy portfolio into PGTCS, the most common type of generalized seizure and an important next step in addressing unmet need in patients with seizure disorders. With proof-of-concept established in FOS, we plan to initiate a Phase 3 trial in PGTCS in the first half of 2027.

We believe RAP-219 also has therapeutic potential in bipolar disorder. In October 2026, we announced positive topline results from our Phase 2 trial of RAP-219 in adults experiencing acute mania associated with bipolar I disorder ("BD"). The trial met its primary endpoint, with RAP-219 achieving a statistically significant 3.1-point least squares mean improvement over placebo in the Young Mania Rating Scale total score at Week 3 (mean reduction from baseline of 15.4 points with RAP-219 versus 12.3 points with placebo ($p=0.045$)), and RAP-219 was generally well-tolerated. All treatment emergent adverse events (during the treatment period) were mild or moderate in severity, and there were no serious adverse events. These results establish clinical proof-of-concept for RAP-219 and its novel TARPγ8-selective mechanism in bipolar mania. We plan to have a meeting with the FDA in the first quarter of 2027 to discuss the Phase 2 data and registrational trial design for RAP-219 in adults experiencing acute mania associated with BD. Initiation of two Phase 3 trials of RAP-219 in acute mania is expected in the second half of 2027. Additional data from the Phase 2 trial will be presented at a future medical meeting.

We also previously submitted an Investigational New Drug ("IND") application to the FDA for initiation of a Phase 2 proof-of-concept trial in RAP-219 for the treatment of diabetic peripheral neuropathic pain ("DPNP"), which was placed on clinical hold by the FDA in the fourth quarter of 2024. Following further interactions with the FDA, the FDA removed its clinical hold on the DPNP IND in December 2025. We are deferring further investment in the RAP-219 DPNP program at this time to prioritize our α6ß4 program, which we believe has significant potential in chronic pain and migraine, as described below.

Additionally, we continue to develop a long-acting injectable ("LAI") formulation of RAP-219. We believe an LAI formulation has the potential to improve patient adherence and expand the potential clinical utility across all of the RAP-219 indications. We expect to initiate a Phase 1 study, with initial 1 PK data from the study expected in the third quarter of 2027.

We also have two advanced discovery-stage nicotinic acetylcholine receptor ("nAChR") programs stemming from our RAP technology platform. nAChRs have been clinically validated in patient-reported neuropathic pain and our first advanced discovery-stage nAChR program comprises agonists of the α6ß4 nAChR. α6ß4 nAChRs are selectively expressed in sensory neurons, and the α6 subunit has human genetic validation in chronic pain. Our α6ß4 nAChR agonist development candidate, RAP-641, is directed at a genetically validated precision target that we are pursuing as a potential novel non-opiate, non-CNS approach for chronic pain and migraine. We expect to initiate a Phase 1 study, with preliminary results expected in the second half of 2027.

The second advanced discovery-stage nAChR program comprises modulators of the α9α10 nAChR. Third-party nonclinical genetic data suggest that this nAChR could be an attractive target in treating hearing and vestibular disorders. We continue to leverage our RAP technology platform to discover additional product candidates that we believe have the potential to provide transformative benefits for large patient populations with neurological or psychiatric diseases.

We have incurred significant operating losses in each year since our inception. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of any product candidates we may develop. Our net losses were $40.2 million and $26.9 million for the three months ended September 30, 2026 and 2025, respectively, and $116.7 million and $77.7 million for the nine months ended September 30, 2026 and 2025, respectively. As of September 30, 2026, we had an accumulated deficit of $351.9 million. We expect our expenses and operating losses will increase substantially as we:

- continue to conduct our ongoing clinical trials of RAP-219, including advancement into late-stage global clinical trials, as well as initiate and complete additional clinical trials of future product candidates or current product candidates in new indications or patient populations;
- conduct our ongoing nonclinical studies and ongoing and planned clinical trials;
- utilize third parties to manufacture our potential future product candidates and related raw materials;
- continue our early research and development activities;
- seek to identify additional research programs and program candidates to expand our pipeline;
- hire additional research and development, clinical, commercial, and operational personnel;
- maintain, expand, enforce, defend and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- seek regulatory approvals for any potential future product candidates for which we successfully complete clinical trials;
- acquire or in-license product candidates, intellectual property and technologies;
- establish and maintain collaborations;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any therapies for which we may obtain regulatory approval; and
- continue to incur costs associated with being a public company, including audit, legal, regulatory, and tax-related services associated with maintaining compliance with an exchange listing and Securities Exchange Commission ("SEC") requirements, director and officer insurance premiums and investor relations costs.

In addition, we have several preclinical and clinical development, regulatory, and commercial milestone payment obligations under our licensing arrangements. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our nonclinical studies, clinical trials and our expenditures on other research and development activities.

Additionally, in October 2026, we issued and sold in separate underwritten offerings with Morgan Stanley & Co. LLC, Leerink Partners LLC and Evercore Group L.L.C. (the "Underwriters"): (i) 9,761,905 shares of common stock at a public offering price of $21.00 per share (the "Offering Price"), and, in lieu of shares of common stock to certain investors, pre-funded warrants to purchase up to 1,190,476 shares of common stock at a public offering price of $20.999 per share, which represents the Offering Price for the common stock less the $0.001 per share exercise price for each pre-funded warrant, which included the Underwriters' exercise of their option to purchase additional shares of common stock in full (the "2026 Equity Offering") and (ii) $172.5 million aggregate principal amount of 2.25% convertible senior notes due 2032, which included the Underwriters' exercise of their option to purchase additional Notes in full (the "Notes") (the "2026 Notes Offering" and together with the 2026 Equity Offering, the "2026 Underwritten Offerings").

Concurrently with the 2026 Underwritten Offerings, we issued 1,428,571 shares of our common stock (the "PIPE Shares") at a price per share equal to the Offering Price to certain investors in a private placement (the "2026 Private Placement" and together with the 2026 Underwritten Offerings, the "October 2026 Offerings"). The October 2026 Offerings closed on October 30, 2026, subject to customary closing conditions.

The net proceeds from the 2026 Equity Offering are approximately $215.7 million, after deducting underwriting discounts and commissions and estimated offering expenses. The net proceeds from the 2026 Notes Offering are approximately $166.8 million, after deducting underwriting discounts and commissions and estimated offering expenses. The net proceeds from the 2026 Private Placement are approximately $28.2 million, after deducting placement agent discounts and commissions and estimated offering expenses.

The Notes will be our senior, unsecured obligations, will bear interest at a rate of 2.25% per annum, payable semi-annually in arrears on June 1 and December 1 of each year beginning June 1, 2027, and will mature on December 1, 2032, unless earlier repurchased, redeemed or converted. The initial conversion rate is 37.3483 shares of common stock per $1,000 principal amount of Notes, equivalent to an initial conversion price of approximately $26.7750 per share, a premium of approximately 27.50% over the Offering Price per share of the common stock in the 2026 Equity Offering. Upon conversion, we will pay or deliver cash, shares of common stock or a combination, at our election.

Subsequent to September 30, 2026, we sold 399,201 shares of common stock under the ATM Program (as defined below) for gross proceeds of approximately $10 million.

We do not expect to generate any revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our potential future product candidates, which will not be for at least the next several years, if ever. If we obtain regulatory approval for any of our potential future product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, until such time as we can generate significant revenue from sales of our potential future product candidates, if ever, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, including potential collaborations, licenses and other similar arrangements. See the section titled "*—Liquidity and Capital Resources*" included elsewhere in this Quarterly Report. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements when needed would have a negative impact on our financial condition and could force us to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market potential future product candidates that we would otherwise prefer to develop and market ourselves.

We believe that our existing cash, cash equivalents, and short-term investments will enable us to fund our operating expenses and capital expenditure requirements through the end of 2029. See the sections titled "*—Liquidity and Capital Resources" and "Risk Factors—Risks Related to Our Limited Operating History, Financial Condition and Need for Additional Capital*" included elsewhere in this Quarterly Report.

License and Collaboration Agreements

Option and License Agreement with Janssen Pharmaceutical NV

In August 2022, we entered into an option and license agreement with Janssen Pharmaceutical NV, as amended on April 3, 2023, April 18, 2023, May 2, 2023, October 2, 2023, and April 9, 2024 (collectively, the "Janssen License"), under which we received an exclusive option to obtain from Janssen (a) a worldwide exclusive license for the research, development, and commercialization of transmembrane TARPγ8 AMPAR products for the diagnosis, treatment, prophylaxis or palliation of any disease or condition in humans or other animals (the "Field") and (b) an assignment of certain patents related to TARPγ8, in each case of (a)-(b), subject to certain retained rights by Janssen. Pursuant to the Janssen License, we also received a worldwide, royalty-free, non-exclusive license (exclusive under certain joint patents) for the research, development, and commercialization of certain neuronal nicotinic acetylcholine ("nACh") products in the Field.

We made a non-refundable, non-creditable upfront payment of $1.0 million to Janssen after we entered into the Janssen License. In October 2022, we exercised the option and paid a non-refundable, non-creditable option fee of $4.0 million to Janssen. If we succeed in developing and commercializing TARPγ8 products, Janssen will be eligible to receive (i) up to $76.0 million in development milestone payments and up to $40.0 million sales milestone payments for the product containing the lead TARPγ8 development candidate, and (ii) up to $25.0 million in development milestone payments and up to $42.0 million sales milestone payments for other TARPγ8 products containing a non-lead TARPγ8 development candidate.

Janssen is also eligible to receive (a) royalties ranging from mid to high-single digit percentages on worldwide net sales of any products containing a TARPγ8 development candidate and (b) royalties ranging from low to mid-single digit percentages for other TARPγ8 products that do not contain a TARPγ8 development candidate, in each case of (a) and (b), subject to potential reductions following the expiration of valid claims and regulatory exclusivity covering such TARPγ8 products, the launch of certain generic products and the application of certain anti-stacking reductions for third party intellectual property payments, subject to a customary reduction floor. The royalties for any TARPγ8 product will expire on a country-by-country basis upon the latest to occur of (i) the expiration of all valid patent claims covering such product in such country, (ii) the expiration of all regulatory exclusivities in such country, and (iii) a specified number of years following the first commercial sale of such product in such country. The Janssen License provides us with certain other exclusive rights with respect to small molecules with activity against TARPγ8 and nACh.

We have the right to terminate the Janssen License for any or no reason upon providing prior written notice to Janssen upon ninety (90) days' prior written notice to Janssen. Either party may terminate the license agreement in its entirety for the other party's material breach if such party fails to cure the breach or upon certain insolvency events involving the other party.

NeuroPace Master Services Agreement and Statements of Work

In November 2023, we entered into a master services agreement (the "NeuroPace Agreement") with NeuroPace Inc. ("NeuroPace"), the manufacturer and distributor of the responsive neurostimulation ("RNS") system. Pursuant to the NeuroPace Agreement and in accordance with statement of work agreements entered into from time to time, NeuroPace provides us with certain services with respect to data from the RNS systems used in our clinical trials. The NeuroPace Agreement also grants us a royalty-free, worldwide, exclusive, non-transferable license to all data collected by the RNS systems in our RAP-219 clinical trials in drug-resistant focal onset seizures and the outcomes of algorithms that are applied to such data, as well as the ability to publish the outcomes of algorithms, subject to certain conditions. The consideration we will pay to NeuroPace for such services is set out in each statement of work agreement.

Concurrently with the execution of the NeuroPace Agreement, the parties also entered into an initial statement of work, as amended in March 2024, and a second statement of work, as amended in February 2026 (collectively, the "NeuroPace SOWs"), under the NeuroPace Agreement, pursuant to which NeuroPace agreed to provide services related to our RAP-219 Phase 2a clinical trial and open-label long term safety trial in RAP-219 for FOS, including, among other things, clinical trial readiness support, identification of potential patients satisfying the enrollment criteria and RNS system data reporting and data analysis. Pursuant to the payment schedule set out in the NeuroPace SOWs, we will pay NeuroPace an aggregate of up to $5.8 million over a period of approximately four years in connection with NeuroPace's provision of services and achievement of certain patient enrollment and deliverable milestones. As of September 30, 2026, we have incurred cumulative expenses of $4.9 million on both NeuroPace SOWs.

Tenacia Collaboration and License Agreement

On March 6, 2026, we entered into a License Agreement (the "Tenacia License Agreement") with Tenacia Biotechnology (Hong Kong) Co., Ltd. ("Tenacia"). The arrangement provides Tenacia with the exclusive rights to develop and commercialize our product candidate, RAP-219 in mainland China, Hong Kong, Macau and Taiwan (each, a "Market" and collectively, the "Tenacia Territory"). We retain exclusive rights to RAP-219 outside the Tenacia Territory.

Under the terms of the Tenacia License Agreement, we granted to Tenacia an exclusive license to use certain intellectual property for the development and commercialization of RAP-219 and products containing RAP-219 across indications, including focal onset seizures ("FOS") and bipolar mania. See Note 11 - *License and Collaboration Agreement* for a description of the key terms of the Tenacia License Agreement.

Under the terms of the Tenacia License Agreement, we received a $20.0 million non-refundable upfront payment. Additionally, to the extent Tenacia commercializes any licensed products containing RAP-219, we are eligible to receive up to $308.0 million in contingent sales-based milestone payments and tiered royalty payments ranging from the mid-single digits to the mid-teens based on a percentage of net sales, subject to customary reductions and offsets.

Components of Results of Operations

Revenue

We have not generated any revenue from the sale of products since inception and do not expect to generate any revenue from the sale of products for several years, if ever. As discussed above and in Note 11 - *License and Collaboration Agreement* to our condensed consolidated financial statements, we entered into the Tenacia License Agreement in March 2026. We recognized $20.0 million of collaboration revenue from the Tenacia License Agreement during the nine months ended September 30, 2026.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the development and research of our clinical and pre-clinical potential future product candidates. Our research and development expenses include:

- personnel-related costs, including salaries, bonuses, benefits, and stock-based compensation for employees engaged in manufacturing, research and development functions;

- the costs to acquire in-process research and development with no alternative future use acquired in an asset acquisition;
- external expenses, including expenses incurred under arrangements with third parties, such as contract research organizations, contract manufacturing organizations, consultants and our clinical and scientific advisors;
- the cost of developing and validating our outsourced manufacturing process for use in our nonclinical studies and future clinical trials;
- the cost to obtain licenses to intellectual property and related future payments should certain development milestones be achieved;
- costs for laboratory supplies, research materials, and reagents; and
- facility costs, depreciation, and other expenses related to research and development activities, which include direct or allocated expenses for rent, maintenance of facilities, and utilities.

Our primary focus since inception has been the development of RAP-219. Our research and development costs consist primarily of personnel-related costs and external costs, such as fees paid to Contract Manufacturing Organizations ("CMOs"), Contract Research Organizations ("CROs") and consultants in connection with our nonclinical studies and clinical trials. We expense all research and development costs in the periods in which they are incurred. Because we are working on multiple research and development programs at one time, we track many of our external expenses on a program-by-program basis. We do not allocate personnel-related costs or other indirect costs to specific product development programs because these costs are deployed across multiple programs and, as such, are not separately classified.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher and more variable development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will increase substantially in the near term as we advance RAP-219 through clinical development, pursue regulatory approval of RAP-219, continue to discover and develop additional product candidates and incur expenses associated with hiring additional personnel to support our research and development efforts, including the associated manufacturing activities.

Upfront and milestone payments made are accrued for and expensed when the achievement of the milestone is probable up to the point of regulatory approval. Milestone payments made upon regulatory approval will be capitalized and amortized over the remaining useful life of the related product.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of any of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from sales or licensing of our product candidates. This is due to the numerous risks and uncertainties associated with drug development, including the uncertainty of:

- the timing and progress of preclinical and clinical development activities;
- timely and successful completion of nonclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable;
- effective INDs or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for any product candidates we may develop;
- successful enrollment and completion of clinical trials, including under the U.S. FDA's current Good Clinical Practices ("GCPs"), current Good Laboratory Practices ("GLPs"), and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our future clinical trials that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended populations;
- receipt of marketing approvals from applicable regulatory authorities;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense and enforcement of patent, trademark, trade secret and other intellectual property protection or regulatory exclusivity for any product candidates we may develop; and
- maintenance of a continued acceptable safety, tolerability and efficacy profile of any product candidates we may develop following approval.

A change in the outcome of any of these variables with respect to the development of any of our product candidates or potential future product candidate could mean a significant change in the costs and timing associated with the development of that product candidate or potential future product candidate. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials beyond those that we anticipate would be required for the completion of clinical development of a product candidate or potential future product candidate, or if we experience significant delays in our clinical trials due to slower than expected patient enrollment or other reasons, we would be required to expend significant additional financial resources and time on the completion of clinical development. We may never obtain regulatory approval for any of our product candidates, and, even if we do, drug commercialization takes several years and millions of dollars in development costs.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of personnel-related costs, including salaries, bonuses, benefits, and stock-based compensation charges for those individuals in executive, finance, human resources, facility operations, and other administrative functions. Other costs include legal fees relating to intellectual property and corporate matters, professional fees for auditing, accounting, tax and consulting services, office and information technology costs, insurance costs, and facilities, depreciation and other selling, general and administrative expenses, which include direct or allocated expenses for rent and maintenance of facilities and utilities.

We anticipate that our selling, general and administrative expenses will increase for the foreseeable future to support development of product candidates and our continued research activities. These increases will likely include additional costs related to the hiring of additional personnel and fees paid to outside consultants, among other expenses. We also anticipate increased expenses related to audit, accounting, legal, regulatory, and tax-related services associated with maintaining compliance with The Nasdaq Global Market and SEC requirements, director and officer insurance premiums, and investor relations costs associated with operating as a public company.

Other Income

Interest Income

Interest income consists of interest earned from our cash, cash equivalents and short-term and long term investments.

Income Taxes

For the three and nine months ended September 30, 2026 and 2025, we recorded no income tax provision. As of September 30, 2026 and December 31, 2025, we recorded a full valuation allowance of our net deferred tax assets, as we believed it was more likely than not we would not be able to utilize our deferred tax assets prior to their expiration.

Since our inception, we have not recorded any income tax benefits for the net losses we have incurred in each year or for our research and development tax credits, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our net operating losses ("NOLs"), carryforwards and tax credits will be not realized. As of December 31, 2025, we had federal NOL carryforwards of approximately $46.1 million and state NOL carryforwards of approximately $15.1 million, respectively. Federal losses have an indefinite carryforward period, but can only offset 80% of federal taxable income in a given year. Losses for state purposes begin to expire in 2043. As of December 31, 2025, we also had federal and state tax research and development credit carryforwards of approximately $8.0 million and $3.2 million, respectively, to offset future tax liabilities, which begin to expire in 2042 and 2037, respectively. As of December 31, 2025, we had no unrecognized tax benefits.

Results of Operations

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

The following table summarizes our results of operations for the three months ended September 30, 2026 and 2025:

	For the Three Months Ended September 30,		
	2026	2025	Change
	(in thousands)		
Operating expenses			
Research and development	$ 34,316	$ 22,279	$ 12,037
Selling, general and administrative	9,770	7,707	2,063
Total operating expenses	44,086	29,986	14,100
Loss from operations	(44,086)	(29,986)	(14,100)
Other income:			
Interest income	3,851	3,061	790
Total other income	3,851	3,061	790
Net loss	$ (40,235)	$ (26,925)	$ (13,310)

Operating Expenses

Research and Development Expenses

	For the Three Months Ended September 30,		
	2026	2025	Change
	(in thousands)		
Direct external program expenses:			
RAP-219 program	$ 17,481	$ 8,733	$ 8,748
Preclinical programs	4,473	4,474	(1)
Internal and unallocated expenses:			
Personnel-related costs (including stock-based compensation)	10,559	7,381	3,178
Other costs	1,803	1,691	112
Total research and development expenses	$ 34,316	$ 22,279	$ 12,037

Research and development expenses were $34.3 million for the three months ended September 30, 2026, as compared to $22.3 million for the three months ended September 30, 2025. The increase of $12.0 million consisted of the following:

- a $8.7 million increase in RAP-219 program costs, which consisted primarily of a $5.8 million increase in clinical trial costs, primarily driven by our Phase 3 pivotal programs in drug-resistant FOS, costs for our Phase 2 proof-of-concept trial in bipolar mania, IND-enabling costs for our LAI program, and start-up costs for our Phase 3 trial in PGTCS, a $2.3 million increase in contract manufacturing costs related to the production of clinical supply, a $0.1 million increase in nonclinical toxicology studies related to long-term toxicology work, a $0.4 million increase in medical affairs costs, and a $0.1 million increase in IT costs related to clinical software;
- a $3.2 million increase in personnel-related costs due to an increase in headcount, which consisted primarily of salaries, bonuses, and other compensation-related costs of $1.9 million and stock-based compensation of $1.3 million; and
- a $0.1 million increase in other costs, consisting of research and development facility costs and information technology costs related to increased headcount and continued expansion of our Boston and San Diego sites.

Selling, General and Administrative Expenses

	For the Three Months Ended September 30,		
	2026	2025	Change
		(in thousands)	
Personnel-related (including stock-based compensation)	$ 7,486	$ 5,487	$ 1,999
Professional and consulting costs	1,459	1,322	137
Facility related and other	825	898	(73)
Total selling, general and administrative expense	$ 9,770	$ 7,707	$ 2,063

Selling, general and administrative expenses were $9.8 million for the three months ended September 30, 2026, as compared to $7.7 million for the three months ended September 30, 2025. The increase of $2.1 million consisted of the following:

- a $2.0 million increase in workforce expense due to an increase in headcount, consisting primarily of salaries, bonuses, and other compensation-related costs of $0.4 million and stock-based compensation of $1.6 million; and
- a $0.1 million increase in professional and consulting costs primarily associated with an increase in legal costs.

Other Income

	For the Three Months Ended September 30,		
	2026	2025	Change
		(in thousands)	
Other income:			
Interest income	$ 3,851	$ 3,061	$ 790
Total other income	$ 3,851	$ 3,061	$ 790

Other income was $3.9 million for the three months ended September 30, 2026, as compared to $3.1 million for the three months ended September 30, 2025. The $0.8 million increase was driven by increased interest income due to the increased cash, cash equivalent and short-term investment balances for the three months ended September 30, 2026 due to proceeds from the September 2025 Offering.

Income Taxes

For each of the three months ended September 30, 2026 and 2025, we recorded an income tax provision of zero.

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

The following table summarizes our results of operations for the nine months ended September 30, 2026 and 2025:

	For the nine months ended September 30,		
	2026	2025	Change
		(in thousands)	
Collaboration Revenue	$ 20,000	$ —	$ 20,000
Operating expenses			
Research and development	118,446	64,531	53,915
Selling, general and administrative	30,661	22,059	8,602
Total operating expenses	149,107	86,590	62,517
Loss from operations	(129,107)	(86,590)	(42,517)
Other income:			
Interest income	12,400	8,870	3,530
Total other income	12,400	8,870	3,530
Net loss	$ (116,707)	$ (77,720)	$ (38,987)

Collaboration Revenue

Collaboration revenue was $20.0 million for the nine months ended September 30, 2026 as a result of revenue recognized related to the non-refundable upfront fee received pursuant to the Tenacia License Agreement. Through September 30, 2026, no milestones have been achieved and no royalties have been earned.

Operating Expenses

Research and Development Expenses

	For the nine months ended September 30,		
	2026	2025	Change
		(in thousands)	
Direct external program expenses:			
RAP-219 program	$ 67,390	$ 26,381	$ 41,009
Preclinical programs	13,632	12,442	1,190
Internal and unallocated expenses:			
Personnel-related costs (including stock-based compensation)	31,836	20,833	11,003
Other costs	5,588	4,875	713
Total research and development expenses	$ 118,446	$ 64,531	$ 53,915

Research and development expenses were $118.4 million for the nine months ended September 30, 2026, as compared to $64.5 million for the nine months ended September 30, 2025. The increase of $53.9 million consisted of the following:

- a $41.0 million increase in RAP-219 program costs, which consisted primarily of a $34.9 million increase in clinical trial costs, primarily driven by start-up costs for our Phase 3 trials in drug-resistant FOS, costs for our open-label long term safety trial for RAP-219 in FOS, costs for our Phase 2 proof-of-concept trial in bipolar mania, costs for IND-enabling activities for our LAI program, start-up costs for our Phase 3 trial in PGTCS, a $5.1 million increase in contract manufacturing costs related to the production of clinical supply, a $0.4 million increase in consulting costs, a $0.4 million increase in medical affairs costs, and a $0.1 million increase in nonclinical toxicology studies related to long-term toxicology work;
- an $11.0 million increase in personnel-related costs due to an increase in headcount, which consisted primarily of salaries, bonuses, and other compensation-related costs of $7.0 million, and stock-based compensation of $4.0 million;
- a $1.2 million increase in preclinical program costs, which consisted primarily of a $1.4 million increase in toxicology and animal studies related to our discovery programs, and a $0.1 million increase in external chemistry efforts. These increases were offset by a $0.4 million decrease in lab supplies costs;
- a $0.7 million increase in other costs, consisting of research and development facility costs and information technology costs related to increased headcount and continued expansion of our Boston and San Diego sites.

Selling, General and Administrative Expenses

	For the nine months ended September 30,		
	2026	2025	Change
		(in thousands)	
Personnel-related (including stock-based compensation)	$ 21,554	$ 15,491	$ 6,063
Professional and consulting costs	6,594	3,906	2,688
Facility related and other	2,513	2,662	(149)
Total selling, general and administrative expense	$ 30,661	$ 22,059	$ 8,602

Selling, general and administrative expenses were $30.7 million for the nine months ended September 30, 2026, as compared to $22.1 million for the nine months ended September 30, 2025. The increase of $8.6 million consisted of the following:

- a $6.1 million increase in workforce expense due to an increase in headcount, consisting primarily of salaries, bonuses, and other compensation-related costs of $1.5 million and stock-based compensation of $4.6 million; and
- a $2.7 million increase in professional and consulting costs primarily associated with increases of $2.8 million in consulting costs and $0.7 million in legal costs primarily due to the execution of the Tenacia License Agreement, offset by a $0.8 million decrease in accounting consulting costs.

Other Income

	For the nine months ended September 30,		
	2026	2025	Change
		(in thousands)	
Other income:			
Interest income	$ 12,400	$ 8,870	$ 3,530
Total other income	$ 12,400	$ 8,870	$ 3,530

Other income was $12.4 million for the nine months ended September 30, 2026, as compared to $8.9 million for the nine months ended September 30, 2025. The $3.5 million increase in interest income was due to the increased cash, cash equivalent and short-term investment balances for the nine months ended September 30, 2026 due to proceeds from the September 2025 Offering.

Income Taxes

For each of the nine months ended September 30, 2026 and 2025, we recorded an income tax provision of zero.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception in February 2022, our revenue has been generated from payments received pursuant to collaboration agreements and we have incurred significant operating losses and negative cash flows from operations. We expect to incur significant expenses and operating losses for the foreseeable future as we advance the clinical development of our product candidates and pipeline. Further, we expect to continue to incur additional costs associated with operating as a public company. We have funded our operations with proceeds from the sale of convertible notes, convertible preferred stock, the issuance of common stock in our IPO, concurrent private placement, September 2025 Offering, and October 2026 Offerings, and collaboration agreements. As of September 30, 2026, we had cash, cash equivalents and short-term investments of $395.0 million, excluding our restricted cash.

We previously entered into a common stock sales agreement (the "Sales Agreement"), dated July 1, 2025, by and between the Company and Leerink Partners LLC and Cantor Fitzgerald & Co., acting as sales agents, which established an at-the-market offering program pursuant to which we may offer and sell shares of our common stock from time to time (the "ATM Program").

In March 2026, we filed an automatic shelf registration statement on Form S-3ASR (the "2026 Registration Statement"), containing (i) a base prospectus, which covers the offering, issuance and sale from time to time in one or more offerings of an indeterminate amount of common stock, preferred stock, debt securities, warrants and/or units; and (ii) a sales agreement prospectus for the offering and issuance and sale of up to a maximum aggregate offering price of $150.0 million of common stock, to be issued pursuant to the Sales Agreement. As of September 30, 2026, we have not sold any shares pursuant to our ATM program. As market conditions permit, we may offer and sell securities under the 2026 Registration Statement, including through the ATM Program, in order to fund our operations or provide additional liquidity.

In October 2026, we completed the October 2026 Offerings in which we issued and sold (i) 9,761,905 shares of common stock at the Offering Price and, in lieu of shares of common stock to certain investors, pre-funded warrants to purchase up to 1,190,476 shares of common stock at a public offering price of $20.999 per share, (ii) $172.5 million Notes and (iii) 1,428,571 PIPE Shares. The net proceeds from the October 2026 Offerings are approximately $410.7 million, after deducting underwriting discounts and commissions and estimated offering expenses.

In addition to our existing cash, cash equivalents and marketable securities, we expect to receive research and development reimbursements and are eligible to earn development and commercial milestone payments and royalties under our license agreement with Tenacia. Our ability to earn these milestone payments and the timing of earning these payments is dependent upon the outcome of agreed upon research and development and commercialization activities and is uncertain at this time.

Cash Flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	For the nine months ended September 30,	
	2026	2025
	(in thousands)	
Net cash used in operating activities	$ (96,149)	$ (62,772)
Net cash provided by (used in) investing activities	114,605	(13,149)
Net cash provided by financing activities	3,653	270,475
Net increase in cash, cash equivalents and restricted cash	$ 22,109	$ 194,554

Operating Activities

During the nine months ended September 30, 2026, operating activities used $96.1 million of cash, resulting primarily from our net loss of $116.7 million and changes in operating assets and liabilities of $3.4 million, partially offset by $21.8 million of non-cash stock-based compensation expense, $1.8 million of non-cash lease expense, $0.6 million of non-cash depreciation expense, and non-cash accretion of investments in marketable securities of $0.4 million. The $3.4 million change in operating assets and liabilities is primarily driven by a $2.1 million decrease in operating lease liabilities, a decrease in accounts payable of $2.5 million due to timing of payments to vendors, an increase in prepaid expenses and other current assets of $3.2 million primarily due to recognition of advanced payments on contracts related to our clinical trials and an increase to accounts receivable of $0.2 million for reimbursable costs related to the Tenacia License Agreement, partially offset by increases in accrued expenses of $4.2 million due to increased clinical trial costs and a decrease in other assets of $0.3 million due to the movement of an upfront long-term prepaid clinical trial payment from long-term to short term.

During the nine months ended September 30, 2025, operating activities used $62.8 million of cash, resulting primarily from our net loss of $77.7 million, non-cash accretion of investments in marketable securities of $0.3 million, and changes in operating assets and liabilities of $0.1 million, partially offset by $13.2 million of non-cash stock-based compensation expense, $0.8 million of non-cash depreciation expense, and $1.5 million of non-cash lease expense. The $0.1 million change in operating assets and liabilities is primarily driven by an increase in prepaid expenses and other current assets of $2.6 million, primarily due to advanced payments on new contracts related to our clinical trials, a $0.1 million decrease in operating lease liabilities, and an increase in other assets of $0.9 million due to the payment of the security deposit for our new corporate headquarters lease and an up-front long-term clinical trial payment, partially offset by increases in accounts payable of $1.0 million and accrued expenses of $2.5 million due to increased expenditures and timing of payments to vendors.

Investing Activities

During the nine months ended September 30, 2026, net cash provided by investing activities was $114.6 million, primarily consisting of maturities of short-term investments of $188.7 million, sales of short-term investments of $10.1 million, and proceeds from sales of equipment of $0.4 million, partially offset by purchases of short-term investments of $84.4 million and purchases of property and equipment of $0.2 million.

During the nine months ended September 30, 2025, net cash used in investing activities was $13.1 million, primarily consisting of maturities of short-term investments of $145.0 million, partially offset by purchases of short-term investments of $157.7 million and purchases of property and equipment of $0.4 million.

Financing Activities

During the nine months ended September 30, 2026, net cash provided by financing activities was $3.7 million, primarily consisting of proceeds from exercise of stock options of $4.1 million, offset by payment of deferred offering costs of $0.5 million in connection with the preparation and filing of the 2026 Registration Statement.

During the nine months ended September 30, 2025, net cash provided by financing activities was $270.5 million, primarily consisting of net proceeds from the September 2025 Offering in the aggregate amount of $269.8 million and proceeds from exercise of stock options of $0.6 million.

Future Funding Requirements

As of September 30, 2026, we had cash, cash equivalents and short-term investments of $395.0 million, excluding our restricted cash. As of the issuance date of the condensed consolidated financial statements for the nine months ended September 30, 2026, we expect that our cash, cash equivalents and short-term investments will be sufficient to fund our operating expenses and capital expenditure requirements through at least 12 months from the issuance of the condensed consolidated financial statements. We believe that our existing cash, cash equivalents and short-term investments will enable us to fund our operating expenses and capital expenditure requirements through the end of, 2029. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. However, our forecast for the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. Additionally, the process of conducting nonclinical studies and testing potential future product candidates in clinical trials is costly, and the timing of progress and expenses in these studies and trials is uncertain. We will need to raise substantial additional capital in the future.

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance our nonclinical studies and the current and future clinical trials of our product candidates. Our funding requirements and timing and amount of our operating expenditures will depend on many factors, including:

- the rate of progress in the development of RAP-219 and our other product candidates;
- the type, number, scope, progress, expansions, results, costs, and timing of, discovery efforts, nonclinical studies and clinical trials of RAP-219 and potential future product candidates;
- the costs and timing of manufacturing for RAP-219 and our potential future product candidates and commercial manufacturing;
- the costs, timing, and outcome of regulatory review of our product candidates;
- the terms and timing of establishing and maintaining licenses and other similar arrangements;
- the legal costs of obtaining, maintaining, and enforcing our patents and other intellectual property rights;
- our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company;
- the costs associated with hiring additional personnel and consultants as our nonclinical and future clinical activities increase;
- the costs and timing of establishing or securing sales and marketing capabilities if any potential future product 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 ability to achieve any milestones in connection with the Tenacia License Agreement;

- costs associated with any products or technologies that we may in-license or acquire; and
- costs associated with being a public company.

Until such time, if ever, as we can generate substantial product revenue to support our cost structure, we expect to finance our cash needs through equity offerings, debt financings, or other capital sources, potentially including collaborations, licenses, and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Additional 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 collaborations, license arrangements, or other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or potential future product candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. Our failure to raise capital or enter into such other arrangements when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise additional funds through equity or debt financings, or through other sources 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 our product candidates and potential future product candidates even if we would otherwise prefer to develop and market such potential future product candidates ourselves.

Contractual Obligations and Commitments

Leases

As of September 30, 2026, we had future minimum operating lease payments under non-cancelable leases of $10.8 million related to leases we have recognized on our condensed consolidated balance sheet, which included existing laboratory and office leases and the new corporate headquarters lease that commenced June 1, 2025 in Boston, Massachusetts. The future minimum operating lease payments under these non-cancelable leases are due over a weighted average remaining lease term of 4.0 years.

Option and License Agreement with Janssen Pharmaceutical NV

We made an upfront non-refundable, non-creditable payment of $1.0 million to Janssen after we entered into the Janssen License. In October 2022, we exercised the option and made a non-refundable, non-creditable option fee of $4.0 million to Janssen. If we succeed in developing and commercializing TARPγ8 products, Janssen will be eligible to receive (i) up to $76.0 million in development milestone payments and up to $40.0 million sales milestone payments for the product containing the lead TARPγ8 development candidate and (ii) up to $25.0 million in development milestone payments and up to $42.0 million sales milestone payments for the other products containing a non-lead TARPγ8 development candidate. We are also required to pay tiered royalties related to the TARPγ8 development candidate of a mid to high single-digit percentage on worldwide net sales and tiered royalties related to the TARPγ8 products that do not contain a TARPγ8 development candidate of low to mid single-digit percentages on annual net sales of the products covered by the license.

NeuroPace Master Services Agreement and Statements of Work

In connection with the execution of the NeuroPace Agreement, the parties also entered into an initial statement of work, as amended in March 2024, and a second statement of work, as amended in February 2026, (collectively, the "NeuroPace SOWs") under the NeuroPace Agreement, pursuant to which NeuroPace agreed to provide services related to the RAP-219 Phase 2a proof-of-concept clinical trial and planned open-label long term safety trial in RAP-219 for FOS, including, among other things, clinical trial readiness support, identification of potential patients satisfying the enrollment criteria and RNS system data reporting and data analysis. Pursuant to the payment schedule set out in the NeuroPace SOWs, the Company will pay NeuroPace an aggregate of up to $5.8 million over a period of approximately four years in connection with NeuroPace's provision of services and achievement of certain patient enrollment and deliverable milestones. The Company has incurred cumulative expenses of $4.9 million on the NeuroPace SOWs through September 30, 2026.

Apart from the contracts with payment commitments that we have documented above, we have entered into contracts in the normal course of business with CROs, CMOs and other third parties for nonclinical research studies and testing, clinical trials and manufacturing services. These contracts do not contain any minimum purchase commitments and are cancelable by us upon prior notice and, as a result, are not included in the table of contractual obligations and commitments above. Payments due upon cancellation consist only of payments for services provided and expenses incurred, including non-cancelable obligations of our service providers, up to the date of cancellation.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our condensed consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States ("GAAP"). The preparation of our condensed consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements, as well as the reported amounts of expenses incurred during the reporting periods. We base our estimates on historical experience, known trends and events, and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and expenses that are not readily apparent from other sources. We evaluate our estimates and judgments on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

For information regarding our critical accounting policies and estimates, please refer to Note 2 — "Summary of Significant Accounting Policies" contained in our Annual Report. The critical accounting policy below supplements the critical accounting policies discussed in our Annual Report.

Revenue Recognition

We recognize revenue in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC"), Topic 606, *Revenue Recognition* ("ASC 606"). Accordingly, we recognize revenue following the five step model prescribed under Accounting Standards Updates No. 2014-09, *Revenue from Contracts with Customers*: (i) Identify the contract(s) with the customer, (ii) Identify the promised goods and/or services in the contract and determine which promised goods and/or services represent performance obligations, (iii) Measure the transaction price, (iv) Allocate the transaction price to the performance obligations in the contract and (v) Recognize revenue when (or as) each performance obligation is satisfied. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations, and whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

As part of the accounting for these arrangements, we must develop assumptions that require judgment to determine the standalone selling price for each performance obligation identified in the contract and use judgment in the determination of the transaction price and the application of the constraint. The determination of standalone selling price has not had a significant impact on the accounting for our revenue arrangements given the nature of the performance obligations. We have also not been required to apply significant judgment in determining the transaction price given the nature of the variable consideration and the application of the constraint.

Emerging Growth Company and Smaller Reporting Company Status

The Jumpstart Our Business Startups Act of 2012 permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to "opt out" 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 will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company. As a result of this election, our condensed consolidated financial statements may not be comparable to other public companies that comply with new or revised accounting pronouncements as of public company effective dates. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

A company remains an emerging growth company until as late as the end of the fiscal year following the fifth anniversary of its initial public offering, although circumstances could cause a company to lose that status earlier, including if it is deemed to be a "large accelerated filer," which occurs when the market value of its common stock that is held by non-affiliates exceeds $700 million as of the prior June 30 unless it remains eligible to use the "smaller reporting company" requirements under the revenue test in Rule 12b-2 of the Exchange Act, or if it has total annual gross revenue of $1.235 billion or more during any fiscal year before that time, in which cases it would no longer be an emerging growth company as of the following December 31, or if it issues more than $1.0 billion in non-convertible debt during any three-year period before that time, in which case it would no longer be an emerging growth company immediately. We could be an emerging growth company until as late as December 31, 2029, but we expect to lose that status earlier since the aggregate market value of our shares of common stock held by non-affiliates exceeded $700 million on the last business day of our second quarter in 2026.

We are also a "smaller reporting company" as defined in the Exchange Act, and have elected to take advantage of certain of the scaled disclosures available to smaller reporting companies. However, since the aggregate market value of our shares of common stock held by non-affiliates exceeded $700 million on the last business day of our second quarter in 2026, beginning with our Quarterly Report on Form 10-Q for the quarter ended March 31, 2027, we will no longer be eligible to use the scaled disclosure accommodations available to smaller reporting companies and will become subject to additional disclosure requirements.

Recent Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2—"Summary of Significant Accounting Policies" to our condensed consolidated financial statements included elsewhere in this Quarterly Report.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and are not required to provide the information required by 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. 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 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. We are not currently a party to any litigation or legal proceedings that, in the opinion of our management, are probable to have material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on our business, financial condition, results of operations and prospects because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors.

Our business involves significant risks. Stockholders should carefully consider the risks and uncertainties described below and the other information in this Quarterly Report on Form 10-Q (this "Quarterly Report") and in the other documents that we file with the SEC. Our business, financial condition, results of operations, or prospects could be materially and adversely affected if any of these risks occur, and as a result, the market price of our common stock could decline and stockholders could lose all or part of their investment. This Quarterly Report also contains forward-looking statements that involve risks and uncertainties not presently known to us or that we currently deem to be immaterial. See "Special Note Regarding Forward-Looking Statements" on page 1 for more information. Our actual results could differ materially and adversely from those anticipated in these forward-looking statements as a result of certain important factors, including those set forth below.

Risks Related to Our Limited Operating History, Financial Condition and Need for Additional Capital

We are a clinical-stage biotechnology company with a limited operating history, which may make it difficult to evaluate our current business and predict our future success and viability. We have incurred significant financial losses since our inception and anticipate that we will continue to incur significant financial losses for the foreseeable future.

We are a clinical-stage biotechnology company with a limited operating history. We were formed in February 2022 and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, developing our receptor associated protein ("RAP") technology platform and technology, identifying potential product candidates, securing intellectual property rights, and planning and undertaking nonclinical studies and clinical trials. Substantially all of our product candidates were initially developed by Janssen Pharmaceutical NV ("Janssen"), which we in-licensed pursuant to the option and license agreement with Janssen (the "Janssen License"), entered into shortly after our formation. We have not yet demonstrated an ability to generate product revenues, obtain regulatory approvals, manufacture any product on 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. Our limited operating history as a company makes any assessment of our future success and viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early-stage biotechnology companies in rapidly evolving fields, and we have not yet demonstrated an ability to successfully overcome such risks and difficulties. If we do not address these risks and difficulties successfully, our business will suffer.

The success of our business depends primarily upon our ability to identify, develop, and commercialize product candidates based on our RAP technology platform. We do not know whether we will be able to develop any product candidates that succeed through preclinical and clinical development or products of commercial value. We have no products approved for commercial sale and have not generated any revenue from product sales to date. We will continue to incur significant research and development and other expenses related to our preclinical and clinical development and ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. Our net losses totaled $116.7 million and $77.7 million for the nine months ended September 30, 2026 and 2025, respectively. As of September 30, 2026, we have not yet generated revenues from product sales and had an accumulated deficit of $351.9 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our product candidates.

We anticipate that our expenses will increase substantially if, and as, we:

- advance our product candidates through clinical development, including as we continue to advance RAP-219 into and through later-stage clinical trials;
- seek regulatory approvals for our product candidates that successfully complete clinical trials;
- hire additional clinical, quality control, medical, scientific and other technical personnel to support the clinical development of our product candidates;

- experience an increase in headcount as we expand our research and development organization and market development and pre-commercial planning activities;
- undertake any pre-commercial or commercial activities to establish sales, marketing and distribution capabilities;
- advance our preclinical-stage product candidates into clinical development;
- seek to identify, acquire and develop additional product candidates, including through business development efforts to invest in or in-license other technologies or product candidates;
- maintain, expand and protect our intellectual property portfolio;
- make milestone, royalty or other payments due under the Janssen License and any future in-license or collaboration agreements; and
- make milestone, royalty, interest or other payments due under any future financing or other arrangements with third parties.

Biotechnology product development entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate efficacy or an acceptable safety profile, gain regulatory approval, secure market access and reimbursement and become commercially viable, and therefore any investment in us is highly speculative. Accordingly, before making an investment in us, our prospects, factoring in the costs, uncertainties, delays and difficulties frequently encountered by companies in clinical development, especially clinical-stage biotechnology companies such as ours, should be carefully considered. Any predictions about our future success or viability may not be as accurate as they would otherwise be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products. We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives.

Additionally, our expenses could increase beyond our expectations if we are required by the U.S. Food and Drug Administration (the "FDA"), European Medicines Agency ("EMA"), Medicines and Healthcare products Regulatory Agency ("MHRA") or other comparable regulatory authorities to perform clinical trials in addition to those that we currently expect, or if there are any delays in establishing appropriate manufacturing arrangements for or in completing our clinical trials or the development of any of our product candidates.

We will require additional funding in order to finance operations. If we are unable to raise capital when needed, or on acceptable terms, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

Developing biotechnology products, including conducting nonclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. We expect our expenses to continue to increase in connection with our ongoing activities, particularly as we conduct clinical trials of, and seek regulatory and marketing approval for, our product candidates. Even if our current or future product candidates are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. To date, we have funded our operations principally through private financings, our initial public offering ("IPO") and concurrent private placement, which closed in June 2024, our underwritten public offering in September 2025, our at-the-market offering program pursuant to which we may offer and sell shares of our common stock from time to time (the "ATM Program") under which, subsequent to September 30, 2026, we have sold 399,201 shares for gross proceeds of approximately $10 million, and our separate underwritten public offerings and concurrent private placement in which we issued and sold (i) 9,761,905 shares of common stock at a public offering price of $21.00 per share (the "Offering Price") and, in lieu of shares of common stock to certain investors, pre-funded warrants to purchase up to 1,190,476 shares of common stock at a public offering price of $20.999 per share, including full exercise of the underwriters' option to purchase additional shares (the "2026 Equity Offering"), (ii) $172.5 million aggregate principal amount of 2.25% convertible senior notes due 2032 (the "Notes"), including full exercise of the underwriters' option to purchase additional Notes, (the "2026 Notes Offering" and together with the 2026 Equity Offering, the "2026 Underwritten Offerings") and (iii) 1,428,571 shares of our common stock (the "PIPE Shares") at a price per share equal to the Offering Price to certain investors in a private placement (the "2026 Private Placement" and together with the 2026 Underwritten Offerings, the "October 2026 Offerings"). We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the clinical and preclinical development of our product candidates, continue to identify additional targets, commence additional nonclinical studies and clinical trials, and continue to identify and develop additional product candidates either through internal development or through acquisitions or in-licensing product candidates.

As of September 30, 2026, we had $395.0 million of cash, cash equivalents and short-term investments, excluding restricted cash. Based upon our current operating plan, we believe that our existing cash, cash equivalents and short-term investments, together with the net proceeds from the October 2026 Offerings will enable us to fund our operating expenses and capital expenditure requirements through the end of 2029. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. We may also raise additional financing on an opportunistic basis in the future.

For example, we may seek additional capital due to favorable market conditions, including through our at-the-market program, or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. Attempting to secure additional financing may divert our management from our day-to-day activities, which may adversely affect our ability to develop our product candidates. Our future capital requirements will depend on many factors, including but not limited to:

- the scope, timing, progress, costs and results of discovery, preclinical development and clinical trials for our current or future product candidates;
- the number of nonclinical studies and clinical trials required for regulatory approval of our current or future product candidates;
- the costs, timing and outcome of regulatory review of any of our current or future product candidates;
- the costs associated with acquiring or licensing additional product candidates, technologies or assets, including the timing and amount of any milestones, royalties or other payments due in connection with our acquisitions and licenses;
- the cost of manufacturing clinical and commercial supplies of our current or future product candidates;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims, including any claims by third parties that we are infringing upon their intellectual property rights;
- the effectiveness of our precision neuroscience approach at identifying target patient populations and utilizing our approach to enrich our patient population in our clinical trials;
- our ability to maintain existing, and establish new, strategic collaborations or other arrangements and the financial terms of any such agreements, including the timing and amount of any future milestone, royalty or other payments due under any such agreement;
- the costs and timing of future commercialization activities, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval;
- expenses to attract, hire and retain skilled personnel;
- the costs of operating as a public company;
- our ability to establish a commercially viable pricing structure and obtain approval for coverage and adequate reimbursement from third-party and government payors;
- the effect of macroeconomic trends including inflation and rising interest rates;
- addressing any potential supply chain interruptions or delays;
- the effect of competing technological and market developments; and
- the extent to which we acquire or invest in business, products and technologies.

Because of the numerous risks and uncertainties associated with research and development of product candidates, we are unable to predict the timing or amount of our working capital requirements. In addition, if we obtain regulatory approval for our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution which make it difficult to predict when or if we will be able to achieve or maintain profitability. Furthermore, we have incurred and expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in order to support our continuing operations. Our ability to raise additional funds will depend on financial, economic, political and market conditions and other factors, over which we may have no or limited control. Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If we fail to obtain necessary capital when needed on acceptable terms, or at all, it could force us to delay, limit, reduce or terminate our product development programs, future commercialization efforts or other operations.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our product candidates.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our operations with our existing cash, cash equivalents and short-term investments, any future equity or debt financings and upfront, milestone or royalty payments, if any, received under our current or future licenses, collaborations or royalty agreements. If we raise additional capital through the sale

of equity or convertible debt securities, or issue any equity or convertible debt securities in connection with a collaboration agreement or other contractual arrangement, the ownership interests of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. In addition, the possibility of such issuance may cause the market price of our common stock to decline.

We previously entered into a common stock sales agreement (the "Sales Agreement"), dated July 1, 2025, by and between the Company and Leerink Partners LLC and Cantor Fitzgerald & Co., acting as sales agents, which established an at-the-market offering program pursuant to which we may offer and sell shares of our common stock from time to time (the "ATM Program"). In March 2026, we filed an automatic shelf registration statement on Form S-3ASR (the "2026 Registration Statement"), containing (i) a base prospectus, which covers the offering, issuance and sale from time to time in one or more offerings of common stock, preferred stock, debt securities, warrants and/or units; and (ii) a sales agreement prospectus for the offering and issuance and sale of up to a maximum aggregate offering price of $150.0 million of common stock, to be issued pursuant to the Sales Agreement. As of September 30, 2026, we have not sold any shares pursuant to our ATM program.

Debt financing, if available, may result in increased fixed payment obligations and involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, declaring dividends or acquiring, selling or licensing intellectual property rights or assets, which could adversely impact our ability to conduct our business.

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 intellectual property, technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. We could also be required to seek funds through arrangements with collaborators or others at an earlier stage than otherwise would be desirable. Any of these occurrences may have a material adverse effect on our business, operating results and prospects.

We maintain the majority of our cash and cash equivalents in accounts with major U.S. and multi-national financial institutions, and our deposits at certain of these institutions exceed insured limits. Market conditions and changes in financial regulations and policies can impact the viability of these institutions. In the event of failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position. In addition, changes in regulations governing financial institutions are beyond our control and difficult to predict; consequently, the impact of such changes on our business and results of operations is difficult to predict and may have an adverse effect on us.

The obligations from our license agreement with Janssen may be a drain on our cash resources, or may cause us to incur debt obligations or equity dilution to satisfy the payment obligations.

Under the terms of the Janssen License, Janssen is entitled to substantial contingent payments upon the occurrence of certain events. For example, we will be required to pay Janssen up to $76.0 million in development milestone payments and up to $40.0 million sales milestone payments for products containing RAP-219. In order to satisfy our obligations to make these payments, if and when they are triggered, we may need to issue equity or convertible debt securities that may cause dilution to our stockholders, or we may use our existing cash and cash equivalents or incur debt obligations to satisfy the payment obligations in cash, which may adversely affect our financial position. In addition, these obligations may impede our ability to raise money in future public offerings of debt or equity securities or to obtain a third-party line of credit.

Risks Related to Our Business

Our business is highly dependent on the success of our product candidates, particularly RAP-219 for focal onset seizures, primary generalized tonic-clonic seizures (PGTCS), and bipolar mania. If we are unable to successfully complete clinical development, obtain regulatory approval for or commercialize one or more of our product candidates, or if we experience delays in doing so, our business will be materially harmed.

To date, as an organization, we have not completed the development of any product candidates. We have initiated the Phase 3 program for RAP-219 in drug-resistant focal onset seizures ("FOS") and anticipate initiating Phase 3 trials of RAP-219 in adults experiencing acute mania associated with bipolar I disorder in the second half of 2027 and of RAP-219 in primary generalized tonic-clonic seizures ("PGTCS") in the first half of 2027. However, our other product candidates remain approaching early-stage clinical or preclinical development. Our future success and ability to generate revenue from our product candidates is dependent on our ability to successfully develop, obtain regulatory approval for and commercialize one or more of our product candidates. All of our product candidates will require substantial additional investment for clinical development, regulatory review and approval in one or more

jurisdictions. If any of our product candidates, particularly RAP-219, encounter safety or efficacy problems, development delays or regulatory issues or other problems, our development plans and business would be materially harmed.

We may not have the financial resources to continue development of our product candidates if we experience any issues that delay or prevent regulatory approval of, or our ability to commercialize, our product candidates, including:

- our inability to demonstrate to the satisfaction of the FDA, EMA, MHRA or other comparable regulatory authorities that our product candidates are safe and effective;
- insufficiency of our financial and other resources to complete the necessary clinical trials and nonclinical studies;
- negative or inconclusive results from our clinical trials, nonclinical studies or the clinical trials of others for product candidates similar to ours, leading to a decision or requirement to conduct additional clinical trials or nonclinical studies or abandon a program;
- product-related adverse events ("AEs") experienced by subjects in our clinical trials, including unexpected toxicity results, or by individuals using drugs or therapeutic biologics similar to our product candidates;
- delays in submitting an Investigational New Drug ("IND") application or other regulatory submission to the FDA, EMA, MHRA or other comparable regulatory authorities, or delays or failure in obtaining the necessary approvals from regulators to commence a clinical trial or a suspension or termination, or hold, of a clinical trial once commenced;
- conditions imposed by the FDA, EMA, MHRA or other comparable regulatory authorities regarding the scope or design of our clinical trials;
- poor effectiveness of our product candidates during clinical trials;
- better than expected performance of control arms, such as placebo groups, which could lead to negative or inconclusive results from our clinical trials;
- delays in enrolling subjects in our clinical trials;
- high drop-out rates of subjects from our clinical trials;
- inadequate supply or quality of product candidates or other materials necessary for the conduct of our clinical trials;
- higher than anticipated clinical trial or manufacturing costs;
- unfavorable FDA, EMA, MHRA or comparable regulatory authority inspection and review of our clinical trial sites;
- an evolving geopolitical landscape limiting or restricting access to foreign clinical trial sites and/or foreign clinical trial data and documentation;
- failure of our third-party contractors or investigators to comply with regulatory requirements or the clinical trial protocol or otherwise meet their contractual obligations in a timely manner, or at all;
- delays and changes in regulatory requirements, policies and guidelines, including the imposition of additional regulatory oversight around clinical testing generally or with respect to our therapies in particular; or
- varying interpretations of data by the FDA, EMA, MHRA or other comparable regulatory authorities.

In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of this data is subject to conditions imposed by the FDA, and there can be no assurance that the FDA will accept data from trials conducted outside of the United States. Additionally, recent policy proposals in the U.S., if enacted in the future, may make acceptance by the FDA or inclusion in a marketing application of foreign data more difficult or costly. In addition, the FDA recently signaled increased scrutiny of foreign clinical trial data submitted in support of marketing applications, indicating plans for increased foreign site inspections and increased transparency of inspection results. If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and could delay or permanently halt our development of the applicable product candidates.

The successful development of pharmaceutical products involves a lengthy and expensive process and is highly uncertain.

Successful development of pharmaceutical products involves a lengthy and expensive process, is highly uncertain and is dependent on numerous factors, many of which are beyond our control. Product candidates that appear promising in the early phases of development may fail to reach the market for several reasons, including:

- clinical trial results may show the product candidates to be less effective than expected (for example, a clinical trial could fail to meet its primary or key secondary endpoint(s)) or have an unacceptable safety or tolerability profile;
- failure to receive the necessary regulatory approvals or a delay in receiving such approvals, which, among other things, may be caused by patients who fail the trial screening process, slow enrollment in clinical trials, patients dropping out of trials, patients lost to follow-up, length of time to achieve trial endpoints, additional time requirements for data analysis or New Drug Application ("NDA") or similar foreign application preparation, discussions with the FDA, EMA, MHRA or other comparable regulatory authority or other comparable regulatory request for additional nonclinical or clinical data (such as long-term toxicology studies) or unexpected safety or manufacturing issues;
- nonclinical study results may show the product candidate to be less effective than desired or to have harmful side effects;
- post-marketing approval requirements; or
- the proprietary rights of others and their competing products and technologies that may prevent our product candidates from being commercialized.

For example, in the fourth quarter of 2024, we were notified by the FDA that the IND submitted by us for the initiation of a Phase 2 proof-of-concept trial of RAP-219 in diabetic peripheral neuropathic pain ("DPNP") was placed on clinical hold. The FDA requested additional information and amendments specific to the protocol design. Following further interactions with the FDA, the FDA removed its clinical hold on the DPNP IND in December 2025 and we deferred further investment in the program.

Furthermore, the length of time necessary to complete clinical trials and submit an application for marketing approval for a final decision by a regulatory authority varies significantly from one product candidate to the next and from one country or jurisdiction to the next and may be difficult to predict.

Even if we are successful in obtaining marketing approval, commercial success of any approved products will also depend in large part on the availability of coverage and adequate reimbursement from third-party payors, including government payors such as the Medicare and Medicaid programs and managed care organizations in the United States or country-specific governmental organizations in foreign countries, which may be affected by existing and future healthcare reform measures designed to reduce the cost of healthcare. Third-party payors could require us to conduct additional studies, including post-marketing studies related to the cost effectiveness of a product, to qualify for reimbursement, which could be costly and divert our resources. If government and other healthcare payors were not to provide coverage and adequate reimbursement for our products once approved, market acceptance and commercial success would be reduced. Even if we are able to obtain coverage and adequate reimbursement for our products once approved, there may be features or characteristics of our products, such as dose preparation requirements, that prevent our products from achieving market acceptance by the healthcare or patient communities.

In addition, if any of our product candidates receive marketing approval, we will be subject to significant regulatory obligations regarding the submission of safety and other post-marketing information and reports and registration, and will need to continue to comply (or ensure that our third-party providers comply) with current Good Manufacturing Practices ("cGMPs") in manufacturing our products and Good Clinical Practices ("GCPs") for any clinical trials that we conduct post-approval. In addition, there is always the risk that we, a regulatory authority or a third party might identify previously unknown problems with a product post-approval, such as AEs of unanticipated severity or frequency. Compliance with these requirements is costly, and any failure to comply or other issues with our product candidates post-approval could adversely affect our business, financial condition and results of operations.

Due to the significant resources required for the development of our pipeline, and depending on our ability to access capital, we must prioritize the development of certain product candidates over others. Moreover, we may fail to expend our limited resources on product candidates or indications that may have been more profitable or for which there is a greater likelihood of success.

Our lead product candidate, RAP-219, is in clinical development for FOS, PGTCS, and bipolar mania, and we continue to develop a long-acting injectable ("LAI") formulation of RAP-219. Additionally, we have two advanced discovery-stage nAChR programs stemming from our RAP technology platform. The first is our α6ß4 nAChR agonist development candidate, RAP-641, a genetically validated precision target that we are pursuing as a potential novel non-opiate, non-CNS approach for chronic pain and migraine, and the second comprises modulators of the α9α10 nAChR which third-party nonclinical genetic data suggest could be an attractive target in treating hearing and vestibular disorders.

Due to the significant resources required for the development of our product candidates, we must decide which product candidates and indications to pursue and advance and the amount of resources to allocate to each. For example, we deferred further investment in our DPNP program for RAP-219. Our decisions concerning the allocation of research, development, collaboration, management and financial resources toward particular product candidates, therapeutic areas or indications may not lead to the development of viable commercial products and may divert resources away from better opportunities. If we make incorrect determinations regarding the viability or market potential of any of our product candidates or misread trends in the pharmaceutical industry, in particular for disorders of the nervous system, our business, financial condition and results of operations could be materially and adversely affected. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases and disease pathways that may later prove to have greater commercial potential than those we choose to pursue, or relinquish valuable rights to such product candidates through collaboration, licensing or royalty arrangements in cases in which it would have been advantageous for us to invest additional resources to retain sole development and commercialization rights.

We may seek to grow our business through acquisitions or investments in new or complementary businesses, products or technologies, through the licensing of products or technologies from third parties or other strategic alliances. The failure to manage acquisitions, investments, licenses or other strategic alliances, or the failure to integrate them with our existing business, could have a material adverse effect on our operating results, dilute our stockholders' ownership, increase our debt or cause us to incur significant expense.

Our success depends on our ability to continually enhance and broaden our product offerings in response to changing clinician and patients' needs, competitive technologies and market pressures. Accordingly, from time to time we may consider opportunities to acquire, make investments in or license other technologies, products and businesses that may enhance our capabilities, complement our existing products and technologies or expand the breadth of our markets or customer base. Potential and completed acquisitions, strategic investments, licenses and other alliances involve numerous risks, including:

- difficulty assimilating or integrating acquired or licensed technologies, products, employees or business operations;
- issues maintaining uniform standards, procedures, controls and policies;
- unanticipated costs associated with acquisitions or strategic alliances, including the assumption of unknown or contingent liabilities and the incurrence of debt or future write-offs of intangible assets or goodwill;
- diversion of management's attention from our core business and disruption of ongoing operations;
- adverse effects on existing business relationships with suppliers, sales agents, health care facilities, surgeons and other health care providers;
- risks associated with entering new markets in which we have limited or no experience;
- potential losses related to investments in other companies;
- potential loss of key employees of acquired businesses; and
- increased legal and accounting compliance costs.

We do not know if we will be able to identify acquisitions or strategic relationships we deem suitable, whether we will be able to successfully complete any such transactions on favorable terms, if at all, or whether we will be able to successfully integrate any acquired business, product or technology into our business or retain any key personnel, suppliers, sales agent, health care facilities, physicians or other health care providers. Our ability to successfully grow through strategic transactions depends upon our ability to identify, negotiate, complete and integrate suitable target businesses, technologies or products and to obtain any necessary financing. These efforts could be expensive and time-consuming and may disrupt our ongoing business and prevent management from focusing on our operations.

To finance any acquisitions, investments or strategic alliances, we may choose to issue shares of our securities as consideration, which could dilute the ownership of our stockholders. If the price of our securities is low or volatile, we may be unable to consummate any acquisitions, investments or strategic alliances using our securities as consideration. Additional funds may not be available on terms that are favorable to us, or at all.

We, our collaborators and our service providers are, or may become, subject to a variety of stringent and evolving privacy and data security laws, regulations, and rules, contractual obligations, industry standards, policies and other obligations related to privacy and data security. Any actual or perceived failure to comply with such obligations could expose us to significant fines or other penalties and otherwise harm our business and operations.

In the ordinary course of our business, we and the third parties upon which we rely (such as our third party contract research organizations ("CROs") and other contractors and consultants) 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 proprietary and confidential business data, trade secrets, intellectual property, sensitive third-party data, business plans, transactions, financial information and data we collect about trial participants in connection with clinical trials (collectively, sensitive data). Our data processing activities subject us to numerous evolving privacy and data security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to privacy and data security.

The legislative and regulatory framework for the processing of personal data worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. In the United States, numerous federal, state and local laws and regulations, including federal health information privacy laws, state information security and data breach notification laws, federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws) govern the processing of health-related and other personal data.

At the state level, numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, many of which differ from each other in significant ways, thus complicating compliance efforts, including providing specific disclosures in privacy notices and affording individuals certain rights concerning their personal data. For example, in California, the California Consumer Privacy Act ("CCPA") established a comprehensive privacy framework for covered businesses by creating an expanded definition of personal information, establishing data privacy rights for consumers in the State of California, imposing special rules on the collection of consumer data from minors, and creating a new and potentially severe statutory damages framework for violations of the CCPA and for businesses that fail to implement reasonable security procedures and practices to prevent data breaches. The liability framework includes civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. While clinical trial data and information governed by HIPAA are exempt from the CCPA, other personal information may be applicable and periodic changes to the CCPA continue to broaden its scope.

Similar laws have been passed and proposed in numerous states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future. Such proposed legislation, if enacted, may add additional complexity, variation in requirements, restrictions and potential legal risk, require additional investment of resources in compliance programs, impact strategies and the availability of previously useful data and could result in increased compliance costs and/or changes in business practices and policies. The existence of comprehensive privacy laws in different states in the country makes our compliance obligations more complex and costly and increases the likelihood that we may be subject to enforcement actions or otherwise incur liability for noncompliance. While these states exempt some data processed in the context of clinical trials, these developments may further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties upon whom we rely.

Additionally, we may be subject to new laws governing the privacy of consumer health data. For example, Washington's My Health My Data Act, which applies broadly to the processing of 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. Connecticut and Nevada have also passed similar laws regulating consumer health data. These various privacy and data security laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products. In addition, other states have proposed and/or passed legislation that regulates the privacy and/or security of certain other specific types of information. For example, a small number of states have passed laws that regulate biometric data specifically. State laws are changing rapidly and there have been a number of proposals in the U.S. Congress for a new comprehensive federal data privacy law to which we may likely become subject to, if enacted.

Regulators and legislators in the U.S. are increasingly scrutinizing and restricting certain personal data transfers and transactions involving foreign countries. For example, 28 CFR Part 202 (Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons), prohibits transfers of certain types of data, including health data, genetic data, and biospecimens, to countries of concern, including China. The rule also prohibits covered businesses from engaging in certain investment agreements, employment agreements and vendor agreements involving such data and countries of concern, absent specified cybersecurity controls and the rule does not exempt key-coded or otherwise anonymized, pseudonymized, de-identified, or encrypted data. Actual or alleged violations of such rules may be punishable by criminal and/or civil sanctions, result in exclusion from participation in federal and state programs, or restrict our ability to use certain vendors, sites, investigators, or service providers in global clinical trials.

Outside the United States, an increasing number of laws, regulations, and industry standards govern privacy and data security. For example, the European Union's General Data Protection Regulation ("EU GDPR") and the United Kingdom's GDPR ("UK GDPR") impose strict requirements for processing personal data. Also in Europe, the ePrivacy Directive in the EU and the Privacy

and Electronic Communications Regulations in the UK impose obligations regarding our use of cookies and tracking technologies on our website and set forth requirements related to direct marketing. Non-compliance with these requirements could expose us to regulatory fines, the maximum amounts of which vary by jurisdiction.

The EU GDPR and the UK GDPR (together, "GDPR") establish stringent requirements regarding the processing of personal data, including strict requirements relating to processing sensitive data (such as health data), ensuring there is a legal basis or condition to justify the processing of personal data, where required obtaining consent from individuals, transparent disclosures about how personal data is to be used, limitations on retention of information, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, maintaining records of processing activities and documenting data protection impact assessments where there is high risk processing and taking certain measures when engaging third-party processors.

Under GDPR, companies may face temporary or definitive bans on data processing and other corrective activities, fines of up to €20 million (approximately GBP 17.5 million) or 4% of annual global revenues, whichever is greater, and private litigation related to processing of personal data brought by data subjects or consumer protection organizations authorized at law to represent their interests. Non-compliance could also result in a material adverse effect on our business, financial position and results of operations.

In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the United States or other countries due to data localization requirements or limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area ("EEA") and the United Kingdom ("UK") have restricted the transfer of personal data to the United States and other countries based on whether those jurisdictions' privacy laws are considered to provide adequate protection. Other jurisdictions may adopt similarly stringent interpretations of their data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA's standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or 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 Europe) 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 regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data out of Europe for allegedly violating the GDPR's cross-border data transfer limitations.

Although the UK is regarded as a third country under the EU GDPR, the European Commission has issued an adequacy decision recognizing the UK as providing adequate data protection and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted. The UK government has confirmed that personal data transfers from the UK to the EEA remain free flowing. The respective provisions and enforcement of the EU GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties. This lack of clarity on future UK laws and regulations and their interaction with EU laws and regulations could add legal risk, complexity and cost to our handling of personal data and our privacy and data security compliance programs and could require us to implement different compliance measures for the UK and the EEA.

Additionally in the EU, the NIS 2 Directive ("NIS 2") is replacing the cybersecurity legal framework under the current NIS framework in the EU, aiming to ensure a high level of cybersecurity in the region. NIS 2 brings new medium and large organizations providing services in the EU within scope of the legal framework. It extends to additional sectors and expands the list of in-scope healthcare organizations, including to certain providers engaged in research and development of medicinal products. The new regime imposes direct obligations on management in respect of an in-scope organization's compliance with NIS 2, requires covered organizations to put in place certain cyber risk management measures, strengthens incident reporting requirements and provides supervisory authorities with a greater supervision ability compliance. The majority of obligations will come into force when national legislation implementing NIS 2 becomes effective in the relevant EU Member State. EU Member States had until October 17, 2024 to transpose NIS 2 into national legislation, although some countries have still not completed the transposition. As such, the cybersecurity regulatory landscape in the EU is currently fragmented and uncertain. To the extent we are subject to NIS 2, we will require additional investment of our resources in compliance programs. Under NIS 2, companies may be subject to administrative fines of up to the higher amount of €10 million or 2% of worldwide turnover.

Similarly, China's Personal Information Protection Law ("PIPL"), imposes comprehensive requirements on the processing of personal information, including restrictions on the export of sensitive personal information outside of China. PIPL requires data handlers to satisfy specified conditions, such as obtaining certification or entering into standard contractual arrangements prescribed by the CAC, before transferring personal information abroad. If our partners or collaborators operating in China, including Tenacia, are deemed to be in violation of PIPL's cross-border data transfer restrictions, the CAC or other competent authorities could order the suspension or cessation of such data transfers, which could prevent or materially delay the sharing of clinical trial data and other sensitive information with us and adversely affect our ability to advance the development and regulatory approval of our product candidates.

In addition to privacy and data security laws, we are contractually subject to industry standards adopted by industry groups and may become subject to such obligations in the future. We are also bound by other contractual obligations related to privacy and data security, and our efforts to comply with such obligations may not be successful.

We publish privacy policies, and we may publish marketing materials, and other statements, such as compliance with certain certifications or self-regulatory principles, regarding privacy and data security. If these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

Obligations related to privacy and data security (and consumers' data privacy expectations) 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 and 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. All of these evolving compliance and operational requirements impose significant costs, such as costs related to organizational changes, implementing additional protection technologies, training employees and engaging consultants and legal advisors, which are likely to increase over time. In addition, such requirements may require us to modify our data processing practices and policies, utilize management's time and/or divert resources from other initiatives and projects.

We may at times fail (or be perceived to have failed) in our efforts to comply with our privacy and data security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties on which we rely fail, or are perceived to have failed, to address or comply with applicable privacy and data 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 on processing personal data; and orders to destroy or not use personal data. For example, we rely on Tenacia to comply with relevant privacy and data requirements to enable sharing of clinical data collected in China with us, and if Tenacia fails to comply with requirements, we may not be able to access or utilize such data, or we could face increased compliance risks arising from such non-compliance. Further, there has been an increase in privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. 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.

Our information technology systems and infrastructure, or those of our collaborators and service providers, or our data, may be subject to cyber-attacks, cybersecurity incidents or breaches, compromises or other interruptions, which could result in additional costs, loss of revenue, significant liabilities, harm to our brand, material disruption of our development programs and operations, or other adverse consequences.

In the ordinary course of our business, we and the third parties upon which we rely process sensitive data, and, as a result, we and the third parties upon which we rely face a variety of evolving threats that could cause cyber-attacks, cybersecurity incidents, breaches, compromises, or other interruptions. Although we take steps to develop and maintain systems and controls designed to protect our sensitive data, systems and infrastructure, there can be no assurance that our internal technology systems and infrastructure, or those of third parties upon which we rely, will be sufficient to protect against a cyber-attack, cybersecurity breach, compromise or other incident such as an industrial espionage attack, ransomware, or insider threat attack, which may compromise our system infrastructure or lead to the loss, destruction, alteration or dissemination of, or damage to, our sensitive data. 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.

The risk of a cyber-attack, cybersecurity incident, breach, compromise, or other interruption has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. Such risks come from a variety of evolving threats, including but not limited to, social engineering attacks (including through deep fakes, which may be increasingly 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, 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, attacks enhanced or facilitated by artificial intelligence (“AI”), telecommunications failures, earthquakes, fires, floods, and other similar threats. Use of generative AI models in our internal or third-party systems may create new attack surfaces or methods for adversaries, which could impact us and our vendors. Malicious actors may use AI tools to identify and exploit these and other software vulnerabilities, as well as use AI technologies to launch more sophisticated, automated, targeted and coordinated attacks. Any cybersecurity incidents or data breaches related to our use of AI applications could adversely affect our reputation and results of operations.

Individuals engage in 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 upon which we rely, may be vulnerable to a heightened risk of cyber-attacks, including retaliatory cyber-attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our services.

We also face increased risks of a cyber-attack, cybersecurity incident, breach, compromise, or other interruption due to our reliance on internet technology and the number of our employees who work on a hybrid basis at home, in the office, or other public spaces. This may create additional opportunities for cyber criminals to exploit vulnerabilities. Additionally, 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 that were not found during due diligence of such acquired or integrated entities.

In addition, our reliance on third-party service provider software could introduce new cybersecurity risks and vulnerabilities, including supply-chain attacks. We rely on third-party service providers and technologies to operate critical business systems to process sensitive data in a variety of contexts and our ability to monitor these third parties’ information security practices is limited. These third parties may not have adequate information security measures in place and if our third-party service providers experience a cyber-attack, cybersecurity incident, breach, compromise, or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award.

We may be unable to detect vulnerabilities in our information technology systems and infrastructure on a timely basis or until after a cyber-attack, cybersecurity incident, breach, compromise, or other interruption has occurred. Further, we may experience delays in developing and deploying remedial measures designed to adequately address any such identified vulnerabilities.

We have in the past experienced threats and security incidents related to our data and systems, and we may in the future experience additional threats, compromises, breaches or incidents. If we, or a third party upon whom we rely, experience a cyber-attack, cybersecurity incident, breach, compromise, or other interruption, or are perceived to have experienced a cyber-attack, cybersecurity incident, breach, compromise, or other interruption, we may experience adverse consequences, such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including individual and group claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); financial loss; and other potentially significant harms. Such events may require us to fundamentally change our business activities and practices in response to such litigation or investigations. 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 privacy and data security obligations. Cyber liability insurance may not provide adequate coverage against potential liabilities related to any experienced cybersecurity incident or breach.

Further, applicable privacy and data security obligations may require us to notify relevant stakeholders of a cyber-attack, cybersecurity incident, breach, compromise, or other interruption. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences. In addition, cyber-attacks, cybersecurity incidents, breaches, compromises, or other interruptions may cause stakeholders (including investors and potential customers) to stop supporting our business, deter new customers from using our products, and negatively impact our ability to grow and operate our business.

If we were to experience a material cyber-attack, cybersecurity incident, breach, compromise, or other interruption that causes interruptions in our operations, it could result in a material disruption of our product development programs.

The use of new and evolving technologies, such as AI and machine learning ("ML"), in our operations, and the operations of third parties upon which we rely, may result in spending additional resources and present new risks and challenges that can impact our business including by posing security and other risks to our sensitive data. As a result, we may be exposed to reputational harm, other adverse consequences, and liability.

The use of new and evolving technologies, such as AI/ML, in our operations, and the operations of third parties upon which we rely presents new risks and challenges that could negatively impact our business, including cybersecurity, data privacy, information technology, intellectual property, regulatory, legal, operational, competitive, reputational, and other risks and challenges. Specifically, risks related to bias, AI hallucinations, discrimination, harmful content, misinformation, fraud, scams, targeted attacks such as model poisoning or data poisoning, surveillance, data leakage, loss of consensus reality, inequality, environmental harms, and other harms may flow from our development, use, or deployment of AI technologies. The use of certain AI/ML technologies can also give rise to intellectual property risks, including compromises to proprietary intellectual property and intellectual property infringement. Additionally, several jurisdictions around the globe, including Europe and certain U.S. states, have proposed, enacted, or are considering, laws governing the development and use of AI/ML. Such laws in the EU impose significant obligations on providers and deployers of high risk artificial intelligence systems, come with significant penalties for non-compliance, and encourage providers and deployers of artificial intelligence systems to account for EU ethical principles in their development and use of these systems. For instance, the EU's Artificial Intelligence Act ("AI Act") prohibits certain uses of AI based on their risk levels and imposes various governance, risk management and transparency obligations. The AI Act may require us to implement AI governance, human oversight, documentation, monitoring, data quality, cybersecurity and transparency measures.

Likewise, such legislative developments in the United States have introduced emerging compliance obligations for companies that develop or deploy AI technologies, which will impose novel risks on AI developers and users. Recently, states have advanced, and in some cases passed, dozens of laws focused on AI governance and regulation, including on deployment of AI in healthcare settings. These state-level initiatives reflect a growing trend toward AI regulation in the absence of federal legislation. As a result, we may face a fragmented and evolving compliance landscape that could increase operational complexity, regulatory scrutiny, and legal exposure associated with our use or development of AI technologies. If we develop or use AI systems governed by these laws or regulations, we will need to meet higher standards of data quality, transparency, monitoring and human oversight, and we would need to adhere to specific and potentially burdensome and costly ethical, accountability, and administrative requirements, with the potential for significant enforcement or litigation in the event of any perceived non-compliance. Additionally, certain privacy laws extend rights to consumers (such as the right to delete certain personal data) and regulate automated decision making, which may pose difficulties in the context of our use of AI/ML. These obligations may make it harder for us to conduct our business using AI/ML, lead to regulatory fines or penalties, require us to change our business practices, retrain our AI/ML, or prevent or limit our use of AI/ML. For example, the Federal Trade Commission has required other companies to turn over (or disgorge) valuable insights or trainings generated through the use of AI/ML where they allege the company has violated privacy and consumer protection laws. If we cannot use AI/ML or that use is restricted, our business may be less efficient, or we may be at a competitive disadvantage.

The rapid evolution of AI/ML will require the application of significant resources to design, develop, test and maintain our products and services to help ensure that AI/ML is implemented in accordance with applicable law and regulation and in a socially responsible manner and to minimize any real or perceived unintended harmful impacts. Our vendors may in turn incorporate AI/ML tools into their own offerings, and the providers of these AI/ML tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security.

Further, bad actors around the world use increasingly sophisticated methods, including the use of AI/ML, to engage in cyber-attacks, cyber espionage campaigns, exploitation of expanded attack surfaces, and other illegal activities involving the theft and misuse of sensitive data. Technological developments in AI, including advances in frontier artificial intelligence models, may increase the sophistication and speed of cybersecurity incidents and other malicious attacks that we experience. These technologies may enable threat actors to better identify vulnerabilities, develop targeted exploits, conduct phishing or social engineering campaigns, or otherwise compromise or breach our information systems more efficiently. Vulnerabilities that affect us or our third-party service providers may be exploited more rapidly, and although we maintain cybersecurity risk management processes designed to address cybersecurity threats, evolving artificial intelligence capabilities may reduce these safeguards' effectiveness and require additional investments and resources. Any of these effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business.

Risks Related to the Discovery and Development of Our Current or Future Product Candidates

The regulatory approval processes of the FDA, EMA, MHRA and other comparable regulatory authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

We are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining regulatory approval from the FDA. Foreign regulatory authorities, such as the EMA and MHRA, impose similar requirements. The time required to obtain approval by the FDA, EMA, MHRA or other comparable regulatory authorities is inherently unpredictable, but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. The leadership changes at the FDA or other global regulatory authorities may compound this uncertainty. Jurisdictions outside of the United States, such as the European Union or Japan, may have different requirements for regulatory approval, which may require us to conduct additional clinical, nonclinical or chemistry, manufacturing and control studies. Moreover, the U.S. Supreme Court's July 2024 decision to overturn prior established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which FDA's regulations, policies, and decisions may become subject to increasing legal challenges, delays, and/or changes. To date, we have not submitted an NDA to the FDA or similar drug approval submissions to comparable foreign regulatory authorities for any product candidate. We must complete additional nonclinical studies and clinical trials to demonstrate the safety and efficacy of our product candidates in humans before we will be able to obtain these approvals.

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of our initial and potential additional product candidates is susceptible to the risk of failure inherent at any stage of development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of AEs that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements and determination by the FDA, EMA, MHRA or other comparable regulatory authorities that a product candidate may not continue development or is not approvable. It is possible that even if any of our product candidates have a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of such product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials we may fail to detect toxicity of, or intolerability caused by, such product candidate, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case. Serious AEs or other AEs, as well as tolerability issues, could hinder or prevent market acceptance of the product candidate at issue.

Our current and future product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA, EMA, MHRA or other comparable regulatory authorities may disagree as to the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA, EMA, MHRA or other comparable regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA, EMA, MHRA or other comparable regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA, EMA, MHRA or other comparable regulatory authorities may disagree with our interpretation of data from clinical trials or nonclinical studies;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA to the FDA or other submission or to obtain regulatory approval in the United States, the European Union, the UK or elsewhere;
- the FDA, EMA, MHRA or other comparable regulatory authorities may find deficiencies with or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA, EMA, MHRA or other comparable regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of clinical trial results may result in our failing to obtain regulatory approval to market any product candidate we develop, which would substantially harm our business, results of operations and prospects. The FDA, EMA, MHRA and other comparable regulatory authorities have substantial discretion in the approval process and determining when or whether regulatory approval will be granted for any product candidate that we develop. Even if we believe the data collected from future clinical trials of our product candidates are promising, such data may not be sufficient to support approval by the FDA, EMA, MHRA or other comparable regulatory authorities.

In addition, even if we were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

The FDA, EMA, MHRA or comparable regulatory authorities may disagree with our regulatory plan for our product candidates.

The historical approach for FDA approval of a new drug is dispositive data from two or more adequate and well-controlled clinical trials of the product candidate in the relevant patient population. Adequate and well-controlled clinical trials typically involve a large number of patients, have significant costs and take years to complete. The FDA, EMA, MHRA or other comparable regulatory authorities may disagree with us about whether a clinical trial is adequate and well-controlled or may request that we conduct additional clinical trials prior to regulatory approval. In addition, there is no assurance that the doses, endpoints and trial designs that we intend to use for our planned clinical trials, including those that we have developed based on feedback from regulatory agencies or those that have been used for the approval of similar drugs, will be acceptable for future approvals. In June 2026, FDA issued revised draft guidance clarifying how sponsors can rely on one scientifically rigorous adequate and well-controlled clinical investigation with confirmatory evidence to satisfy the statutory substantial evidence of effectiveness standard. We may pursue marketing approval based on data from only one adequate and well-controlled clinical investigation. However, FDA retains broad discretion to require additional clinical data for any product candidate, including a second adequate and well-controlled clinical trial. The FDA may be unwilling to apply regulatory flexibility and our clinical trial results may not support approval of our product candidates. In addition, our product candidates could fail to receive regulatory approval, or regulatory approval could be delayed, for many reasons, including the following:

- the FDA, EMA, MHRA or comparable regulatory authorities may not file or accept our NDA or marketing application for substantive review;
- the FDA, EMA, MHRA or comparable regulatory authorities may disagree with the dosing regimen, design or implementation of our clinical trials;
- the FDA, EMA, MHRA or comparable regulatory authorities may determine there is not substantial evidence of effectiveness to support approval;
- we may be unable to demonstrate to the satisfaction of the FDA, EMA, MHRA or comparable regulatory authorities that our product candidates are safe and effective for any of their proposed indications;
- the results of our clinical trials may not meet the level of statistical significance required by the FDA, EMA, MHRA or comparable regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
- the FDA, EMA, MHRA or comparable regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of the FDA, EMA, MHRA or comparable regulatory authorities to support the submission of an NDA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- the FDA, EMA, MHRA or comparable regulatory authorities may find deficiencies with or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA, EMA, MHRA or comparable regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

We are dependent on a third party having accurately generated, collected, interpreted and reported data from certain nonclinical studies and clinical trials that were previously conducted for our product candidates.

Substantially all of our product candidates were initially developed by Janssen, which we in-licensed pursuant to the Janssen License shortly after our formation. We entered into this license on the basis of our interpretation of the medical and scientific meaningfulness of Janssen's initial data. Therefore, we are dependent on Janssen having designed certain nonclinical studies and clinical trials; conducted its research and development in accordance with the applicable protocols, legal and regulatory requirements, and scientific standards; having accurately reported the results of all nonclinical studies conducted with respect to such product candidates; and having correctly collected and interpreted the data from these studies and trials. These risks also apply to any additional product candidates that we may acquire or in-license in the future. If these activities were not compliant, accurate or correct, the clinical development, regulatory approval or commercialization of our product candidates will be adversely affected and the earlier-reported results may not support data that we generate in our own nonclinical or clinical work with those product candidates.

In addition, we rely in part on nonclinical data using earlier generation TARPγ8 NAMs and third-party published data with other TARPγ8 NAMs for support of RAP-219. While we believe it is an accepted scientific practice to reference nonclinical data generated using other molecules of the same class, it is possible that similar studies with RAP-219 would not have generated entirely consistent results and, as such, the studies performed with other molecules of the same class may not be reflective of the therapeutic potential of RAP-219.

If our clinical trials fail to replicate positive results from earlier nonclinical studies or clinical trials conducted by us or third parties, we may be unable to successfully develop, obtain regulatory approval for or commercialize our product candidates.

The results observed from nonclinical studies or early-stage clinical trials of our product candidates may not necessarily be predictive of the results of later-stage clinical trials that we currently plan to conduct or may conduct in the future. Similarly, positive results from such nonclinical studies or early-stage clinical trials may not be replicated in our subsequent nonclinical studies or clinical trials. Furthermore, our product candidates may not be able to demonstrate similar activity or adverse event profiles as other product candidates that we believe may have similar profiles.

In addition, in our planned future clinical trials, we may utilize clinical trial designs or dosing regimens that have not been tested in prior clinical trials. There can also be no assurance that any of our clinical trials will ultimately be successful or support further clinical development of any of our product candidates. There is a high failure rate for drugs proceeding through clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, nonclinical findings made while clinical trials were underway or safety or efficacy observations made in nonclinical studies and nonclinical trials, including previously unreported adverse effects or AEs.

Additionally, we have and will continue to utilize an "open-label" clinical trial design in some of our clinical trials. An "open-label" clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Our Phase 2a proof-of-concept trial in drug-resistant FOS was an open label trial. Most open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results of a product candidate when studied in a controlled environment with a placebo or active control.

Moreover, nonclinical and clinical data are often susceptible to varying interpretations and analyses and many companies that believed their product candidates performed satisfactorily in nonclinical studies and clinical trials nonetheless failed to obtain FDA, EMA, MHRA or comparable foreign regulatory authority approval.

We may incur unexpected costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

To obtain the requisite regulatory approvals to commercialize any of our product candidates, we must demonstrate through extensive nonclinical studies and clinical trials that our product candidates are safe and effective in humans. We may experience delays in completing our clinical trials or nonclinical studies and initiating or completing additional clinical trials or nonclinical studies, including as a result of regulators not allowing or delay in allowing clinical trials to proceed under an IND or similar foreign authorization, or not approving or delaying approval for any clinical trial grant or similar approval we need to initiate a clinical trial. We may also experience numerous unforeseen events during our clinical trials that could delay or prevent our ability to receive marketing approval or commercialize the product candidates we develop, including:

- regulators, institutional review boards ("IRBs"), ethics committees or other reviewing bodies may not authorize us or our investigators to commence a clinical trial, or to conduct or continue a clinical trial at a prospective or specific trial site;
- we may not reach agreement on acceptable terms with prospective 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;
- we may experience challenges or delays in recruiting principal investigators or study sites to lead our clinical trials;
- the number of subjects or patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be insufficient or slower than we anticipate, and the number of clinical trials being conducted at any given time may be high and result in fewer available patients for any given clinical trial, or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors, including those manufacturing our product candidates or conducting clinical trials on our behalf, may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may have to amend clinical trial protocols submitted to regulatory authorities or conduct additional studies to reflect changes in regulatory requirements or guidance, which may be required to re-submit to an IRB and regulatory authorities for re-examination;
- regulators or other reviewing bodies may find deficiencies with, fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we enter into agreements for clinical and commercial supplies, or the supply or quality of any product candidate or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and
- the potential for approval policies or regulations of the FDA, EMA, MHRA or other comparable regulatory authorities to significantly change in a manner rendering our clinical data insufficient for approval.

Regulators or IRBs of the institutions in which clinical trials are being conducted may suspend, limit or terminate a clinical trial, or data monitoring committees may recommend that we suspend or terminate a clinical trial, 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, EMA, MHRA or other comparable regulatory authorities resulting in the imposition of a clinical hold, 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. Negative or inconclusive results from our clinical trials or nonclinical studies could mandate repeated or additional clinical trials and, to the extent we choose to conduct clinical trials in other indications, could result in changes to or delays in clinical trials of our product candidates in such other indications. We do not know whether any clinical trials that we conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market our product candidates for the indications that we are pursuing. If later-stage clinical trials do not produce favorable results, our ability to obtain regulatory approval for our product candidates may be adversely impacted.

Our failure to successfully initiate and complete clinical trials and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market our product candidates would significantly harm our business. We expect that our product candidate development costs will also increase if we experience delays in testing or regulatory approvals and we may be required to obtain additional funds to complete clinical trials. There can be no assurance that our clinical trials will begin as planned or be completed on schedule, if at all, or that we will not need to restructure or otherwise modify our trials after they have begun. For example, in the fourth quarter of 2024, we were notified by the FDA that the IND submitted by us for the initiation of a Phase 2 proof-of-concept trial of RAP-219 in DPNP was placed on clinical hold. The FDA requested additional information and amendments specific to the protocol design. Following further discussions with the FDA, the FDA removed its clinical hold on the DPNP IND in December 2025 and we deferred further investment in the program. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates, which may harm our business and results of operations. In addition, many of the factors that cause, or lead to, delays of clinical trials may ultimately lead to the denial of regulatory approval of our product candidates. Finally, such delays may result in the consideration of reprioritization of company product candidate development programs.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following regulatory approval, if obtained.

Undesirable side effects caused by any of our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label, the inclusion of a Risk Evaluation and Mitigation Strategy, or the delay or denial of regulatory approval by the FDA, EMA, MHRA or other comparable regulatory authorities.

We may observe safety or tolerability issues beyond those we anticipate with our product candidates in ongoing or future RAP-219 clinical trials or in clinical programs for other product candidates. Additionally, it is possible that patients in our trials may experience greater side effects than observed in healthy volunteers. Additionally, adverse safety effects observed with AMPAR-inhibitors could adversely impact future labeling FDA may require for RAP-219. For example, perampanel's FDA-approved label is accompanied by a boxed warning for serious psychiatric and behavioral reactions, including aggression, hostility and homicidal ideation and threats. Furthermore, significant drug-drug interactions were reported for perampanel. The concomitant use with the other ASMs carbamazepine, phenytoin and oxcarbazepine decreased plasma levels of perampanel by approximately 50 to 67 percent. In addition, perampanel at a dose of 12 mg per day reduced exposure of levonorgestrel, an oral contraceptive, by approximately 40 percent. While we believe there are critical differences between perampanel and RAP-219, the FDA could impose similar warnings and labeling requirements on RAP-219, if approved, in the future.

Many compounds that initially showed promise in clinical or earlier-stage testing are later found to cause undesirable or unexpected side effects that prevented further development of the compound. Results of future clinical trials of our product candidates could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics, despite a favorable tolerability profile observed in earlier-stage testing. At any time, we may decide to terminate or greatly narrow the target population for a clinical development program due to unacceptable side effects or safety concerns.

If unacceptable side effects arise in the development of our product candidates, we, the FDA, EMA, MHRA or other comparable regulatory authorities, the IRBs, or the independent ethics committees at the institutions in which our trials are conducted, could suspend, limit or terminate our clinical trials, or the independent safety monitoring committee could recommend that we suspend, limit or terminate our trials, or the FDA, EMA, MHRA or other comparable regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. We may be unable to overcome any such suspensions or holds that are placed on our clinical trials. Treatment-emergent side effects that are deemed to be drug-related could delay recruitment of clinical trial subjects or may cause subjects that enroll in our clinical trials to discontinue participation in our clinical trials. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We may need to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in harm to patients that are administered our product candidates. Any of these occurrences may adversely affect our business, financial condition and prospects significantly.

Moreover, clinical trials of our product candidates are conducted in carefully defined sets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects.

Even if we complete the necessary nonclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of our product candidates.

Any product candidate we develop and the activities associated with its development and commercialization, including its design, testing, manufacture, safety, efficacy, record keeping, labeling, storage, approval, advertising, promotion, sale, and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the United States, and by the EMA, MHRA and other comparable regulatory authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction and it is possible that none of the product candidates we are developing or may seek to develop in the future will ever obtain regulatory approval.

We have no experience in submitting and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs or regulatory consultants to assist us in this process. Securing regulatory approval requires the submission of extensive nonclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we develop may not be effective, may be only moderately effective, or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude its obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity, and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, changes in leadership, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA, EMA, MHRA and other comparable regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional nonclinical, clinical or other studies. In addition, varying interpretations of the data obtained from nonclinical and clinical testing could delay, limit, or prevent marketing approval of a product candidate. Any marketing approval that we may ultimately obtain could be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

Interim, topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data becomes 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 publish interim, topline or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are 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. Preliminary or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our reputation and business prospects.

If we do not achieve our projected development and commercialization goals in the timeframes we announce and expect, the development and commercialization of our product candidates may be delayed, and our business and results of operations may be harmed.

For planning purposes, we sometimes estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development objectives. These milestones may include our expectations regarding the commencement or completion of scientific studies and clinical trials, the submission of regulatory filings or commercialization objectives. From time to time, we may publicly announce the expected timing of some of these milestones, such as the completion of an ongoing clinical trial, the initiation of other clinical programs, receipt of marketing approval or a commercial launch of a product. The achievement of many of these milestones may be outside of our control. All of these milestones are based on a variety of assumptions which, if not realized as expected, may cause the timing of achievement of the milestones to vary considerably from our estimates, including:

- our available capital resources or capital constraints we experience;

- the rate of progress, costs and results of our clinical trials and research and development activities, including the extent of scheduling conflicts with participating clinicians and collaborators;
- our ability to identify and enroll patients who meet clinical trial eligibility criteria;
- our receipt of approvals by the FDA, EMA, MHRA and other comparable regulatory authorities and the timing thereof;
- other actions, decisions or rules issued by regulators;
- our ability to access sufficient, reliable and affordable supplies of materials used to manufacture our product candidates;
- the efforts of our collaborators with respect to the commercialization of our product candidates; and
- the securing of, costs related to, and timing issues associated with, product manufacturing as well as sales and marketing activities.

If we fail to achieve announced milestones in the timeframes we expect, the development and commercialization of our product candidates may be delayed, and our business and results of operations may be harmed.

We have concentrated our research and development efforts on the treatment of disorders of the nervous system, a field that faces certain challenges in drug development.

We have focused our research and development efforts on addressing disorders of the brain and nervous system. Efforts by pharmaceutical companies in this field have faced certain challenges in drug development. In particular, many neurological and psychiatric disorders such as FOS and bipolar disorder rely on subjective patient-reported outcomes as key endpoints. This makes them more difficult to evaluate than indications with more objective endpoints. These indications are often subject to a placebo effect, which may make it more challenging to isolate the effects of our product candidates. There can be no guarantee that we will successfully overcome these challenges with RAP-219 in our ongoing and future trials for FOS, PGTCS, or BD or that we will not encounter other challenges in the development of our product candidates. Moreover, given the history of clinical failures in this field, future clinical or regulatory failures by us or others may have result in further negative perception of the likelihood of success in this field, which may significantly and adversely affect the market price of our securities.

We may be subject to additional risks because we intend to evaluate our product candidates in combination with the standard of care for the indications that we are pursuing.

We intend to evaluate our product candidates in combination with other compounds, specifically the standard of care for the indications that we are pursuing. The use of our product candidates in combination with such other compounds may subject us to risks that we would not face if our product candidates were being administered as a monotherapy. The outcome and cost of developing a product candidate to be used with other compounds is difficult to predict and dependent on a number of factors that are outside our control. If we experience efficacy or safety issues in our clinical trials in which our product candidates are being administered with other compounds, we may not receive regulatory approval for our product candidates, which could prevent us from ever generating revenue or achieving profitability.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The timely completion of clinical trials in accordance with our protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion.

Patient enrollment is affected by many factors, including:

- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary and secondary endpoints;
- the severity of the disease under investigation;
- the proximity of patients to study sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;

- patients that enroll in our clinical trials may misrepresent their eligibility or may otherwise not comply with the clinical trial protocol, resulting in the need to drop such patients from the clinical trial, increase the needed enrollment size for the clinical trial or extend the clinical trial's duration;
- approval of new indications for existing therapies or approval of new therapies in general;
- competing clinical trials and clinicians' and patients' perceptions as to the potential advantages and risks of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications that we are investigating;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in our clinical trials will drop out of the trials before completion.

We may experience challenges in recruiting principal investigators and patients to participate in ongoing and future clinical trials for such product candidates if we are unable to sufficiently demonstrate the potential of such product candidates to them. In addition, our clinical trials may compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will 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. Since the number of qualified clinical investigators is limited, we may conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial site. Furthermore, if significant AEs or other side effects are observed in any of our clinical trials, we may have difficulty recruiting patients to our trials and patients may drop out of our trials. Additionally, patients, including patients in any control groups, may withdraw from the clinical trial for various reasons, including but not limited to if they are not experiencing improvement in their underlying disease or condition, or if they experience other difficulties or issues relating to their underlying disease or condition. Participants with CNS disorders such as bipolar disorder constitute a vulnerable patient population and may withdraw from the clinical trial if they are not experiencing improvement in their underlying disease or condition or if they experience other difficulties or issues relating to their underlying disease or condition or otherwise, which may or may not be related to our product candidate in clinical trial. Withdrawal of patients from our clinical trials may compromise the quality of our data.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays or might require us to abandon one or more clinical trials or our development efforts altogether. Delays in patient enrollment may result in increased costs, affect the timing or outcome of the planned clinical trials, product candidate development and approval process and jeopardize our ability to seek and obtain the regulatory approval required to commence product sales and generate revenue, which could prevent completion of these trials, adversely affect our ability to advance the development of our product candidates, cause our value to decline and limit our ability to obtain additional financing if needed.

Even if any of our product candidates receives regulatory approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.

We have never commercialized a product, and even if any of our product candidates is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to achieve sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Many of the indications for our product candidates have well-established standards of care that physicians, patients and payors are familiar with and, in some cases, are available generically. Even if our product candidates are successful in registrational clinical trials, they may not be successful in displacing these current standards of care if we are unable to demonstrate superior efficacy, safety, ease of administration and/or cost-effectiveness. For example, physicians may be reluctant to take their patients off their current medications and switch their treatment regimen to our product candidates. Further, patients often acclimate to the treatment regimen that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch due to lack of coverage and adequate reimbursement. Even if we are able to demonstrate our product candidates' safety and efficacy to the FDA and other regulators, safety or efficacy concerns in the medical community may hinder market acceptance.

Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources, including management time and financial resources, and may not be successful. If any product candidate is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to competitive therapies;
- the prevalence and severity of any side effects;
- whether the product is designated under physician treatment guidelines as a first-, second- or third-line therapy;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- the product's acceptance into standard of care treatment algorithms by medical societies that could limit payor and physician uptake;
- limitations or warnings, including distribution or use restrictions contained in the product's approved labeling;
- the strength of sales, marketing and distribution support;
- changes in the standard of care for the targeted indications for the product; and
- availability and adequacy of coverage and reimbursement from government payors, managed care plans and other third-party payors.

Any failure by one or more of our product candidates that obtains regulatory approval to achieve market acceptance or commercial success would adversely affect our business prospects.

If we fail to discover, develop and commercialize other product candidates, we may be unable to grow our business and our ability to achieve our strategic objectives would be impaired.

Although the development and commercialization of our current product candidates are our initial focus, as part of our longer-term growth strategy, we plan to develop other product candidates. In addition to the product candidates in our clinical-stage pipeline, we have in-licensed additional assets that are in earlier stages of development. We intend to evaluate internal opportunities from our existing product candidates or other potential product candidates, and also may choose to in-license or acquire other product candidates to treat patients suffering from other disorders with significant unmet medical needs and limited treatment options. These other potential product candidates will require additional, time-consuming development efforts prior to commercial sale, including nonclinical studies, clinical trials and approval by the FDA, EMA, MHRA or other comparable regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, we cannot be certain that any such products that are approved will be manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives.

In addition, we intend to devote substantial capital and resources for basic research to discover and identify additional product candidates. These research programs require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render our product candidates obsolete;
- product candidates that we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

In the future, we may also seek to in-license or acquire product candidates or the underlying technology. The process of proposing, negotiating and implementing a license or acquisition is lengthy and complex. Other companies, including some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We may not be able to acquire the rights to additional product candidates on terms that we find acceptable, or at all.

In addition, future acquisitions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of management's time and attention to develop acquired products or technologies;
- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions;
- higher than expected acquisition and integration costs;
- difficulty in combining the operations and personnel of any acquired businesses with our operations and personnel;
- increased amortization expenses;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to motivate key employees of any acquired businesses.

If we are unsuccessful in identifying and developing additional product candidates, either through internal development or licensing or acquisition from third parties, our potential for growth and achieving our strategic objectives may be impaired.

The number of patients with the diseases and disorders for which we are developing our product candidates has not been established with precision. If the actual number of patients with the diseases or disorders we elect to pursue with our product candidates is smaller than we anticipate, we may have difficulties in enrolling patients in our clinical trials, which may delay or prevent development of our product candidates. Even if such product candidates are successfully developed and approved, the markets for our product candidates may be smaller than we expect and our revenue potential and ability to achieve profitability may be materially adversely affected.

Our pipeline includes product candidates for a variety of neuroscience diseases. There is no precise method of establishing the actual number of patients with any of these disorders in any geography over any time period. With respect to many of the indications in which we have developed, are developing, or plan to develop our product candidates, we have estimates of the prevalence of the disease or disorder. Our estimates as to prevalence may not be accurate, and the actual prevalence or addressable patient population for some or all of those indications, or any other indication that we elect to pursue, may be significantly smaller than our estimates. In estimating the potential prevalence of indications we are pursuing, or may in the future pursue, including our estimates as to the prevalence of FOS and bipolar disorder, we apply assumptions to available information that may not prove to be accurate. In each case, there is a range of estimates in the published literature and in marketing studies, which include estimates within the range that are lower than our estimates. The actual number of patients with these disease indications may, however, be significantly lower than we believe. Even if our prevalence estimates are correct, our product candidates may be developed for only a subset of patients with the relevant disease or disorder or our product candidates, if approved, may be indicated for or used by only a subset. In the event the number of patients with the diseases and disorders we are studying is significantly lower than we expect, we may have difficulties in enrolling patients in our clinical trials, which may delay or prevent development of our product candidates. If any of our product candidates are approved and our prevalence estimates with respect to any indication or our other market assumptions are not accurate, the markets for our product candidates for these indications may be smaller than we anticipate, which could limit our revenues and our ability to achieve profitability or to meet our expectations with respect to revenues or profits.

Competitive products may reduce or eliminate the commercial opportunity for our product candidates, if approved. If our competitors develop technologies or product candidates more rapidly than we do, or if their technologies or product candidates are more effective or safer than ours, our ability to develop and successfully commercialize our product candidates may be adversely affected.

The clinical and commercial landscapes for the treatment of neuroscience diseases are highly competitive and subject to rapid and significant technological change. We face competition with respect to our indications for our product candidates and will face competition with respect to any other drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell drugs or are pursuing the development of drug candidates for the treatment of the indications that we are pursuing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

We believe that a significant number of product candidates are currently under development for the same indications we are currently pursuing, and some or all may become commercially available in the future for the treatment of conditions for which we are trying or may try to develop product candidates. Our potential competitors include large pharmaceutical and biotechnology companies, specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions. See the section titled "*Business—Competition"* in our Annual Report for examples of the competition that our product candidates face.

In most cases, we do not currently plan to run head-to-head clinical trials evaluating our product candidates against the current standards of care, which may make it more challenging for our product candidates to compete against the current standards of care due to the lack of head-to-head clinical trial data.

Our competitors may have significantly greater financial resources, established presence in the market, expertise in research and development, manufacturing, nonclinical and clinical testing, obtaining regulatory approvals and reimbursement and marketing approved products than we do. Accordingly, our competitors may be more successful than we may be in obtaining regulatory approval for therapies and achieving widespread market acceptance. Our competitors' products may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render our therapies obsolete or non-competitive before we can recover development and commercialization expenses. If any of our product candidates are approved, it could compete with a range of therapeutic treatments that are in development. In addition, our competitors may succeed in developing, acquiring or licensing technologies and drug products that are more effective or less costly than our product candidates, which could render our product candidates obsolete and noncompetitive.

If we obtain approval for any of our product candidates, we may face competition based on many different factors, including the efficacy, safety and tolerability of our products, the ease with which our products can be administered, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Existing and future competing products could present superior treatment alternatives, including being more effective, safer, less expensive or marketed and sold more effectively than any products we may develop. Competitive products may make any products we develop obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. Such competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute our business plan.

In addition, our competitors may obtain patent protection, regulatory exclusivities or FDA approval and commercialize products more rapidly than we do, which may impact future approvals or sales of any of our product candidates that receive regulatory approval. If the FDA approves the commercial sale of any product candidate, we will also be competing with respect to marketing capabilities and manufacturing efficiency. We expect competition among products will be based on product efficacy and safety, the timing and scope of regulatory approvals, availability of supply, marketing and sales capabilities, product price, reimbursement coverage by government and private third-party payors, regulatory exclusivities and patent position. Our profitability and financial position will suffer if our product candidates receive regulatory approval but cannot compete effectively in the marketplace.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites, as well as in acquiring technologies complementary to, or necessary for, our programs.

If we are unable to develop our sales, marketing and distribution capability on our own or through collaborations with marketing partners, we will not be successful in commercializing our product candidates.

We currently have no marketing, sales or distribution capabilities. We intend to establish a sales and marketing organization, either on our own or in collaboration with third parties, with technical expertise and supporting distribution capabilities to commercialize one or more of our product candidates that may receive regulatory approval in key territories. These efforts will require substantial additional resources, some or all of which may be incurred in advance of any approval of a product candidate. Any failure or delay in the development of our or third parties' internal sales, marketing and distribution capabilities would adversely impact the commercialization of our product candidates.

Factors that may inhibit our or third parties' efforts to commercialize our product candidates include:

- ability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the ability of sales personnel to obtain access to or our failure to educate an adequate number of physicians on the benefits of any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

With respect to our existing and future product candidates, we may choose to collaborate with third parties that have direct sales forces and established distribution systems to serve as an alternative to our own sales force and distribution systems. Our future product revenue may be lower than if we directly marketed or sold our product candidates, if approved. In addition, any revenue we receive will depend in whole or in part upon the efforts of these third parties, which may not be successful and are generally not within our control. If we are not successful in commercializing any approved products, our future product revenue will suffer and we may incur significant additional losses.

If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

Risks Related to Employee Matters and Managing Growth

We expect to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of regulatory affairs and sales, marketing and distribution, as well as to continue to support our public company operations. To manage these growth activities, we must continue to implement and improve our managerial, operational, quality and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Our management may need to devote a significant amount of its attention to managing these growth activities. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion or relocation of our operations, retain key employees, or identify, recruit and train additional qualified personnel. Our inability to manage the expansion or relocation of our operations effectively 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 also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy, including the successful commercialization of our product candidates.

Our ability to develop product candidates, leverage our RAP technology platform and manage our future growth depends on attracting, hiring and retaining our key personnel and recruiting additional qualified personnel.

Our success depends upon the continued contributions of our key management and scientific personnel, many of whom have been instrumental for us and have substantial experience with developing therapies, identifying potential product candidates and building the technologies related to the clinical development of our product candidates. Given the specialized nature of neurological and psychiatric disorders and our approach, there is an inherent scarcity of experienced personnel in these fields. As we continue developing our product candidates in our pipeline, we will require personnel with medical, scientific, or technical qualifications specific to each program. The loss of key personnel would delay our research and development activities. We currently do not have "key person" insurance on any of our employees. Despite our efforts to retain valuable employees, members of our team may terminate their employment with us on short notice. The competition for qualified personnel in the biotechnology and biopharmaceutical industries is intense, and our future success depends upon our ability to attract, retain, and motivate highly skilled scientific, technical and managerial employees. We face competition for personnel from other companies, universities, public and private research institutions, and other organizations. If our recruitment and retention efforts are unsuccessful in the future, it may be difficult for us to implement our business strategy, which would have a material adverse effect on our business.

In addition, our clinical operations and research and development programs depend on our ability to attract and retain highly skilled scientists, data scientists, and engineers, particularly in Massachusetts and California. There is powerful competition for skilled personnel in these geographical markets, and we have from time to time experienced, and we expect to continue to experience, difficulty in hiring and retaining employees with appropriate qualifications on acceptable terms, or at all. Many of the companies with which we compete for experienced personnel have greater resources than we do, and any of our employees may terminate their employment with us at any time. If we hire employees from competitors or other companies, their former employers may attempt to assert that these employees or we have breached legal obligations, resulting in a diversion of our time and resources and, potentially, damages. In addition, job candidates and existing employees often consider the value of the stock awards they receive in connection with their employment. If the perceived benefits of our stock awards decline, it may harm our ability to recruit and retain highly skilled employees. If we fail to attract new personnel or fail to retain and motivate our current personnel, our business and future growth prospects would be harmed.

Risks Related to Our Dependence on Third Parties

We rely on third parties to assist in conducting our clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or commercialize our product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.

We have relied upon and plan to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials and expect to rely on these third parties to conduct clinical trials of any other product candidate that we develop. Our ability to complete clinical trials in a timely fashion depends on a number of key factors. These factors include protocol design, regulatory and IRB approval, patient enrollment rates and compliance with GCPs. We have opened clinical trial sites and are enrolling patients in a number of countries where our experience is limited. In most cases, we use the services of third parties, including CROs, to carry out our clinical trial-related activities and rely on such parties to accurately report their results. Our reliance on third parties for clinical development activities may impact or limit our control over the timing, conduct, expense and quality of our clinical trials. Moreover, the FDA requires us to comply with GCPs for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these GCPs through periodic inspections of clinical trial sponsors, principal investigators, clinical trial sites and IRBs. For certain commercial prescription drug products, manufacturers and other parties involved in the supply chain must also meet chain of distribution requirements and build electronic, interoperable systems for product tracking and tracing and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or other products that are otherwise unfit for distribution in the United States.

We remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. Our failure or the failure of third parties to comply with the applicable protocol, legal and regulatory requirements and scientific standards can result in rejection of our clinical trial data or other sanctions. If we or our third-party clinical trial providers or third-party CROs do not successfully carry out these clinical activities, our clinical trials or the potential regulatory approval of a product candidate may be delayed or be unsuccessful. Additionally, if we or our third-party contractors fail to comply with applicable GCPs for any reason, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the regulatory approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with GCPs. We are also required to register certain clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in public notification of noncompliance, fines, adverse publicity and civil and criminal sanctions, amongst other things. In addition, our ability to access and use clinical data from trials conducted in China, including trials conducted by or on behalf of our collaborators in China, may depend on acceptance and approvals from the Human Genetic Resources Administration of China ("HGRAC"). We rely on our collaborators in China to navigate and comply with HGRAC requirements in connection with clinical trials for our product candidates in China, and there is no guarantee that such collaborators will be able to obtain the necessary HGRAC approvals or that the HGRAC will not otherwise interfere with our ability to obtain and use clinical data from such trials in a timely manner or in a way that facilitates our use of such data for regulatory submissions or other purposes.

Furthermore, the third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. Moreover, many CROs, including some of those that we have engaged to conduct our clinical trials, are experiencing enrollment challenges as a result of, among other things, high employee turnover driven by the post-COVID macroeconomic environment and the inexperience of new employees. Furthermore, at clinical trial sites, the availability of staff and trial participants has been limited due to a decrease in the number of clinical investigative sites across the globe. These contractors may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could impede their ability to devote appropriate time to our clinical programs. If these third parties, including clinical investigators, do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed.

In addition, we may rely on other third parties to collect, report and analyze data for our clinical trials. For example, our Phase 2a proof-of-concept clinical trial for RAP-219 in adult patients with drug-resistant FOS relied on implantation of the RNS system from NeuroPace. NeuroPace has and continues to assist us with reporting and analysis of patient data collected from the implanted RNS systems in the Phase 2a clinical trial and in the open-label long-term safety study. If NeuroPace does not successfully carry out its contractual obligations for any reason, meet expected deadlines, conduct our Phase 2a clinical trial or the open-label long-term safety trial in accordance with applicable law, including regulatory and data privacy requirements, or encounters issues with its RNS system, including issues that raise questions of safety, effectiveness or data integrity, or we are otherwise unable to maintain our relationship with NeuroPace, we would have to redesign and conduct a new clinical trial to evaluate RAP-219 in patients with drug-resistant FOS and our business, financial condition and prospects would be harmed.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or regulatory approval of our product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue.

Any of the third-party organizations we utilize may terminate their engagements with us under certain circumstances. The replacement of an existing CRO or other third party may result in the delay of the affected trials or otherwise adversely affect our efforts to obtain regulatory approvals and commercialize our product candidates. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, even if there are suitable replacements for one or more of these service providers, there is a natural transition period when a new service provider begins work. As a result, delays may occur, which could negatively impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects.

Our use of third parties to manufacture our product candidates, including those located outside of the United States in jurisdictions such as China, may increase the risk that we will not have sufficient quantities of our product candidates, raw materials, active pharmaceutical ingredients ("APIs") or drug products when needed or at an acceptable cost.

We do not own or operate manufacturing facilities for the production of clinical or commercial quantities of our product candidates, and we lack the resources and the capabilities to do so. Our current strategy is to outsource all manufacturing of our product candidates to third parties, including in jurisdictions outside of the United States such as China.

We currently rely on and engage third-party manufacturers to provide all of the API and the final drug product formulation of all of our product candidates that are being used in our clinical trials and nonclinical studies. If we were to need an alternate manufacturer, we would incur added costs and delays in identifying and qualifying any such replacement. In addition, we typically order raw materials, API and drug product and services on a purchase order basis and do not enter into long-term dedicated capacity or minimum supply arrangements with any commercial manufacturer. We may not be able to timely secure needed supply arrangements on satisfactory terms, or at all. Our failure to secure these arrangements as needed could have a material adverse effect on our ability to complete the development of our product candidates or, to commercialize them, if approved. We may be unable to conclude agreements for commercial supply with third-party manufacturers or may be unable to do so on acceptable terms. There may be difficulties in scaling up to commercial quantities and formulation of our product candidates, and the costs of manufacturing could be prohibitive. Commitments that we make to supply drug product to collaboration partners, such as under our license agreement with Tenacia (the "Tenacia License Agreement"), will also be sourced from our third-party manufacturers and our failure to do so within agreed specifications and timelines could result in contractual liabilities and harm to the collaborations.

Many of the third-party manufacturers we rely on have only recently begun working with us and have limited or no experience manufacturing our API and final drug products. If our manufacturers have difficulty or suffer delays in successfully manufacturing material that meets our specifications, it may limit supply of our product candidates and could delay our clinical trials.

Even if we are able to establish and maintain arrangements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the failure of the third-party manufacturer to comply with applicable regulatory requirements and reliance on third parties for manufacturing process development, regulatory compliance and quality assurance;
- manufacturing delays if our third-party manufacturers give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreement between us;
- limitations on supply availability resulting from capacity and scheduling constraints of third parties;
- the failure of the third-party manufacturer to produce materials with acceptable quality on a larger scale;
- the possible breach of manufacturing agreements by third parties because of factors beyond our control;
- the possible termination, re-negotiation, or non-renewal of the manufacturing agreements by the third party, at a time or in a matter that is costly or inconvenient to us; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

If we do not maintain our key manufacturing relationships, we may fail to find replacement manufacturers or develop our own manufacturing capabilities, which could delay or impair our ability to obtain regulatory approval for our product candidates. If we do find replacement manufacturers, we may not be able to enter into agreements with them on terms and conditions favorable to us and there could be a substantial delay before new facilities could be qualified and registered with the FDA, EMA, and other comparable regulatory authorities.

Additionally, if any third-party manufacturer with whom we contract fails to perform its obligations, we may be forced to manufacture the materials ourselves, for which we may not have the capabilities or resources, or to enter into an agreement with a different manufacturer. In either scenario, our clinical trials supply could be delayed significantly as we establish alternative supply sources. In some cases, the technical skills required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. In addition, if we are required to change third-party manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA, EMA, MHRA or other comparable regulatory authorities. We may be unsuccessful in demonstrating the comparability of clinical supplies, which could require the conduct of additional clinical trials. The delays associated with the verification of a new third-party manufacturer could negatively affect our ability to develop product candidates or commercialize our products in a timely manner or within budget. Furthermore, a third-party manufacturer may possess technology related to the manufacture of our product candidate that such third party owns independently. This would increase our reliance on such third-party manufacturer or require us to obtain a license from such third-party manufacturer in order to have another third party manufacture our product candidates.

If any of our product candidates is approved by any regulatory agency, we intend to utilize arrangements with third-party contract manufacturers for the commercial production of those products. This process is difficult and time consuming and we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under cGMPs that are capable of manufacturing our product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization.

Some of our manufacturers are located outside of the United States, including in China. There is currently significant uncertainty about the future relationship between the United States and various other countries, including China, with respect to trade policies, treaties, government regulations and tariffs. In December 2025, the National Defense Authorization Act for Fiscal Year 2026 was enacted, which included Section 851, commonly referred to as the "BIOSECURE Act." The BIOSECURE Act restricts U.S. government agencies from procuring biotechnology equipment or services from, or entering into contracts with, entities that use biotechnology equipment or services from, designated "biotechnology companies of concern" ("BCCs"), and from expending federal loan or grant funds for such equipment or services. The Office of Management and Budget ("OMB") is required to publish an initial list of BCCs by December 2026, with implementing regulations expected to follow thereafter before the procurement prohibitions formally take effect. The BCC list has not yet been published and there can be no assurance that our existing or future Chinese manufacturing partners will not be designated in the future. If any of our current or future manufacturers or vendors is designated as a BCC, or if our research institutions, collaborators, or other partners become subject to BIOSECURE-related restrictions as a result of their relationships with such manufacturers, we could be required to terminate or restructure existing arrangements, transition manufacturing or other services to alternative suppliers, or delay or suspend development activities. In addition, even absent a formal BCC designation, U.S. research institutions and other recipients of federal grants and contracts may seek to limit relationships with manufacturers flagged under related national security frameworks, which could indirectly affect our clinical development programs. Any such transition or disruption could involve significant cost, operational complexity, regulatory risk, and delays, and alternative suppliers may not be available on acceptable terms or at all.

Increased tariffs or pending legislation that would impose federal contracting or federal funding limitations on parties directly using or connected to those using the services or equipment of certain foreign entities with known or alleged associations with foreign adversaries could potentially disrupt our existing supply chains and impose additional costs on our business. In particular, certain Chinese biotechnology companies and CMOs may become subject to trade restrictions, sanctions, and other regulatory requirements by the U.S. government, which could restrict or even prohibit our ability to work with such entities, thereby potentially disrupting our supplies and manufacturing. Additionally, it is possible further tariffs may be imposed that could affect imports of any APIs used in our product candidates in the future, or our business may be adversely impacted by retaliatory trade measures taken by China or other countries, including restricted access to such raw materials used in our product candidates. Given the unpredictable regulatory environment in China and the United States and uncertainty regarding how the U.S. or foreign governments will act with respect to tariffs, international trade agreements and policies, further governmental action related to tariffs, additional taxes, contracting matters, regulatory changes or other retaliatory trade measures in the future could occur with a corresponding detrimental impact on our business and financial condition.

Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, seizures or voluntary recalls of product candidates, operating restrictions and criminal prosecutions, any of which could significantly affect supplies of our product candidates. The facilities used by our contract manufacturers to manufacture our product candidates must be evaluated by the FDA. We do not control the manufacturing process of, and are largely dependent on, our contract manufacturing partners for compliance with cGMPs. If our contract manufacturers cannot successfully manufacture material that conforms to our

specifications and the strict regulatory requirements of the FDA, EMA, MHRA or other comparable regulatory authorities, we may not be able to secure and/or maintain regulatory approval for our product candidates manufactured at these facilities. In addition, we have limited control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA finds deficiencies or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which could significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Contract manufacturers may face manufacturing or quality control problems causing drug substance production and shipment delays or a situation where the contractor may not be able to maintain compliance with the applicable cGMP requirements. Any failure to comply with cGMP requirements or other FDA, EMA, MHRA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop our product candidates and market our products, if approved.

The FDA, EMA, MHRA or other comparable regulatory authorities require manufacturers to register manufacturing facilities, and also inspect these facilities to confirm compliance with cGMPs.

Furthermore, should we decide to use any APIs in any of our product candidates that are proprietary to one or more third parties, we would need to maintain licenses to those APIs from those third parties. If we are unable to gain or continue to access rights to such APIs prior to conducting nonclinical toxicology studies intended to support clinical trials, we may need to develop alternate product candidates from these programs by either accessing or developing alternate APIs, resulting in increased development costs and delays in commercialization of these product candidates. If we are unable to gain or maintain continued access rights to the desired APIs on commercially reasonable terms or develop suitable alternate APIs, we may not be able to commercialize product candidates from these programs.

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

We plan to opportunistically pursue strategic partnerships, as the advancement of our product candidates and development programs and the potential commercialization of our current and future product candidates will require substantial additional cash to fund expenses. If we believe that additional partnerships can accelerate the development or maximize the market potential of our product candidates, we will consider entering into product, target and/or geographic specific strategic partnerships on an opportunistic basis. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. In addition, if we are able to obtain regulatory approval for product candidates from foreign regulatory authorities, we may enter into additional partnerships or collaborations with international biotechnology or pharmaceutical companies for the commercialization of such product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a partnership or collaboration will depend upon, among other things, our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed partnerships or collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA, EMA, MHRA, or other comparable regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available for partnership or collaboration and whether such a partnership or collaboration could be more attractive than the one with us for our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

Partnerships and collaborations are each complex and time-consuming to negotiate and document. Further, 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. Any partnership or collaboration agreements that we enter into in the future may contain restrictions on our ability to enter into potential partnerships or collaborations or to otherwise develop specified product candidates. We may not be able to negotiate partnerships or 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 product 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 or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense.

Furthermore, if conflicts arise between our collaborators and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. Our collaborators could conduct multiple product development efforts and could develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop that are the subject of these partnerships or collaborations with us.

Competing products may preclude us from entering into partnerships or collaborations with their competitors, fail to obtain timely regulatory approvals, prevent us from obtaining timely regulatory approvals, terminate their agreements with us prematurely or fail to devote sufficient resources to the partnership or collaboration efforts, including development, delivery, manufacturing and commercialization of products. Any of these developments could harm our company and product development efforts.

We have entered into and may enter into additional collaborations with third parties for the development and commercialization of our product candidates, and our prospects with respect to those product candidates will depend in significant part on the success of those collaborations.

We have entered into and may enter into additional collaborations for the development and commercialization of certain of our product candidates. Under such collaborations, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on any future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving our product candidates pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, which divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, including trade secrets and intellectual property rights, contract interpretation, or the preferred course of development might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours is involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

For example, we will depend on Tenacia for the development and commercialization of RAP-219 in China. Under the terms of the Tenacia License Agreement, Tenacia is responsible for the development and commercialization of RAP-219 in China. Tenacia will participate in the conduct of certain clinical trials for RAP-219 in Greater China and, as a result, we will depend on Tenacia for the enrollment of patients and the conduct of such clinical trials for RAP-219 in Greater China. We do not control the individual efforts of Tenacia, and any failure by Tenacia to devote sufficient time and effort to the development of RAP-219, including enrolling patients and conducting clinical trials for RAP-219 in Greater China, or to meet their respective obligations to us, could adversely impact our ability to complete our planned clinical studies on our anticipated timelines. We depend on Tenacia to comply with all applicable local laws relative to the development and commercialization of RAP-219. If Tenacia violates, or is alleged to have violated, any laws or regulations during the performance of its obligations for us, it is possible that we could suffer financial and reputational harm or other negative outcomes, including possible legal consequences. In the case of any termination of the Tenacia License Agreement, we may be required to devote additional efforts and to incur additional costs associated with pursuing the development and commercialization of RAP-219 in China, which could result in delays to our RAP-219 clinical program. Alternatively, we may attempt to identify and transact with a new sublicensee, but there can be no assurance that we would be able to identify a suitable sublicensee or transact on terms that are favorable to us.

If any third-party manufacturer of our product candidates is unable to increase the scale of its production of our product candidates or increase the product yield of its manufacturing, then our manufacturing costs may increase and commercialization may be delayed.

In order to produce sufficient quantities to meet the demand for clinical trials and, if approved, subsequent commercialization of our product candidates, our third-party manufacturers will be required to increase their production and optimize their manufacturing processes while maintaining the quality of our product candidates. The transition to larger scale production could prove difficult. In addition, if our third-party manufacturers are not able to optimize their manufacturing processes to increase the product yield for our product candidates, or if they are unable to produce increased amounts of our product candidates while maintaining the same quality then we may not be able to meet the demands of clinical trials or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operations.

Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.

As product candidates proceed through nonclinical studies to late-stage clinical trials towards potential approval and commercialization, it is common that various aspects of the manufacturing program, such as the vendors used to manufacture drug product or manufacturing methods and formulation, are altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives. Any such changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the materials manufactured using altered processes, and limit our ability to rely on data from clinical trials conducted with an earlier formulation of our drug candidate. Such changes may also require additional testing, FDA notification or FDA approval. This could delay or prevent completion of clinical trials, require conducting bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay or prevent approval of our product candidates and jeopardize our ability to commence sales and generate revenue.

Risks Related to Government Regulation

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval of a product candidate, the European Commission or comparable foreign regulatory authorities must also approve the manufacturing and marketing of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional nonclinical studies or clinical trials, as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the United States have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

Even if we receive regulatory approval of any product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

If any of our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies and submission of safety, efficacy and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities. In addition, we will be subject to continued compliance with cGMP and GCP requirements for any clinical trials that we conduct post-approval.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA, EMA, MHRA and other comparable regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations and applicable product tracking and tracing requirements. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA, other marketing application and previous responses to inspection observations. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production and quality control.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials and surveillance to monitor the safety and efficacy of the product candidate. Certain endpoint data we hope to include in any approved product labeling also may not make it into such labeling, including exploratory or secondary endpoint data such as patient-reported outcome measures. The FDA may also require a risk evaluation and mitigation strategies ("REMS") program as a condition of approval of our product candidates, which could entail requirements for long-term patient follow-up, 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, EMA, MHRA or other comparable regulatory authority approves our product candidates, we will have to comply with requirements including submissions of safety and other post-marketing information and reports and registration.

The FDA may impose consent decrees or withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with our product candidates, including AEs of unanticipated severity or frequency, or problems with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical trials to assess new safety risks or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of our products, withdrawal of the product from the market or voluntary product recalls;
- fines, FDA Form 483s, warning 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 withdrawal of approvals;
- product seizure or detention or refusal to permit the import or export of our product candidates; and
- injunctions or the imposition of civil or criminal penalties.

Additionally, sponsors of approved drugs and biologics must provide 6 months' notice to the FDA of any changes in marketing status or for discontinuing or interrupting the supply of certain drugs, such as the withdrawal of a drug, and failure to do so could result in a letter citing such failure to comply and public posting of such letter and redacted company response which could damage the company's reputation. The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Products may be promoted only for the approved indications and in accordance with the provisions of the approved label. The policies of the FDA, EMA, MHRA and other comparable regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. In addition, the U.S. Supreme Court's July 2024 decision to overturn established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which the FDA's regulations, policies and decisions may become subject to increasing legal challenges, delays and/or 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 lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

While we may in the future seek designations for our product candidates with the FDA, EMA, MHRA and other comparable regulatory authorities that are intended to confer benefits such as a faster development process, an accelerated regulatory pathway or regulatory exclusivity, there can be no assurance that we will successfully obtain such designations. In addition, even if one or more of our product candidates are granted such designations, we may not be able to realize the intended benefits of such designations.

The FDA, EMA, MHRA and other comparable regulatory authorities offer certain designations for product candidates that are designed to encourage the research and development of product candidates that are intended to address conditions with significant unmet medical need. These designations may confer benefits such as additional interaction with regulatory authorities, a potentially accelerated regulatory pathway and priority review. However, there can be no assurance that we will successfully obtain such designations for our product candidates. In addition, while such designations could expedite the development or approval process, they generally do not change the standards for approval. Even if we obtain such designations for our product candidates, there can be no assurance that we will realize their intended benefits.

For example, we may seek a Fast Track Designation for future product candidates we develop. If a product is intended for the treatment of a serious or life-threatening condition and nonclinical or clinical data demonstrate the potential to address an unmet medical need for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot be certain that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may rescind the Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development activities.

We may seek Breakthrough Therapy Designation for any product candidate that we develop. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for accelerated approval and priority review.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe a product candidate we develop meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if any product candidate we develop qualifies as a breakthrough therapy, the FDA may later decide that the drug no longer meets the conditions for qualification and rescind the designation.

Even in the absence of obtaining Fast Track and/or Breakthrough Therapy Designations, a sponsor can seek priority review at the time of submitting a marketing application. The FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting adverse reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, or evidence of safety and effectiveness in a new subpopulation. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from 10 months to 6 months. Priority review designation may be rescinded if a product no longer meets the qualifying criteria.

Where appropriate, we may secure approval from the FDA, EMA, MHRA or other comparable regulatory authorities through the use of expedited approval pathways, such as accelerated approval. If we are unable to obtain such approvals, we may be required to conduct additional nonclinical studies or clinical trials beyond those that we contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals. Even if we receive accelerated approval from the FDA, EMA, MHRA or other comparable regulatory authorities, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-marketing requirements, the FDA, EMA, MHRA or such other regulatory authorities may seek to withdraw the accelerated approval.

Where possible, we plan to pursue accelerated development strategies in areas of high unmet need. We may seek an accelerated approval pathway for one or more of our therapeutic candidates from the FDA, EMA, MHRA or other comparable regulatory authorities. Under the accelerated approval provisions in the Federal Food, Drug, and Cosmetic Act, and the FDA's implementing regulations, the FDA may grant accelerated approval to a therapeutic candidate designed to treat a serious or life-threatening condition that provides meaningful therapeutic benefit over available therapies upon a determination that the therapeutic candidate has an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as irreversible morbidity or mortality. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new drug over available therapy may not be a direct therapeutic advantage but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. Under FDORA, the FDA must specify the conditions for any post-approval trials by the date of accelerated approval and the agency has flexibility in setting forth such conditions, which may include enrollment targets, clinical trial protocol and milestones – including the target date of trial completion. Further, the FDA is permitted to require, as appropriate, that a post-approval confirmatory study or studies be underway prior to approval or within a specified time period after the date of approval for a product granted accelerated approval. FDORA also gives the FDA increased authority to withdraw approval of a drug or biologic granted accelerated approval on an expedited basis if the sponsor fails to conduct such studies in a timely manner, if the sponsor fails to send status updates on such studies to the FDA every 180 days to be publicly posted by the agency, or if such post-approval studies fail to verify the drug's predicted clinical benefit. The FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory study or submit timely reports to the agency on their progress.

Prior to seeking accelerated approval, we would seek feedback from the FDA, EMA, MHRA or other comparable regulatory authorities and would otherwise evaluate our ability to seek and receive such accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit an NDA or BLA for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that after subsequent feedback from the FDA, EMA, MHRA or other comparable regulatory authorities, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval, there can be no assurance that such application will be accepted or that any approval will be granted on a timely basis, or at all. The FDA, EMA, MHRA or other comparable regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type, including, for example, if other products are approved via the accelerated pathway and subsequently converted by FDA to full approval. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our therapeutic candidate would result in a longer time period to commercialization of such therapeutic candidate, could increase the cost of development of such therapeutic candidate and could harm our competitive position in the marketplace.

Changes in the FDA, other government agencies or comparable foreign regulatory authorities could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA or comparable foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key leadership and other personnel, and ability to accept the payment of user fees, and statutory, regulatory, leadership, and policy changes. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of other government agencies or comparable foreign regulatory authorities on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA, other government agencies or comparable foreign regulatory authorities may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, including as a result of reaching the debt ceiling, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain additional capital in the future.

Our relationships with healthcare providers and physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. These laws include anti-kickback statutes, false claims statutes, data privacy and security laws, as well as transparency laws regarding payments or other items of value provided to healthcare providers. Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we conduct research and would sell, market and distribute our products. As a pharmaceutical company, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations that may affect our ability to operate may apply. For more information on healthcare laws and regulations that may impact our company, see the section titled "*Business—Government Regulation—Other Healthcare Laws"* in our Annual Report.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Ensuring business arrangements comply with applicable healthcare and privacy laws, as well as responding to possible investigations by government authorities, can be time and resource-consuming and can divert a company's attention from the business.

It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance 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 laws and regulations that may apply to us, we may be subject to significant penalties, including administrative, civil and criminal penalties, damages, fines, disgorgement, the exclusion from participation in federal and state healthcare programs, individual imprisonment, reputational harm and the curtailment or restructuring of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Further, defending against any such actions can be costly and time consuming, and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other providers or entities with whom we expect to do business is found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment. If any of the above occur, our ability to operate our business and our results of operations could be adversely affected.

Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, if approved, which could make it difficult for us to sell any product candidates profitably.

The success of our product candidates, if approved, depends on the availability of coverage and adequate reimbursement from third-party payors. We cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, our product candidates or assure that coverage and reimbursement will be available for any product that we may develop. For more information on the laws and regulations that may impact coverage and reimbursement of our product candidates, see the section titled "*Business—Government Regulation—Coverage and Reimbursement*" in our Annual Report.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance.

Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. In the United States, the principal decisions about reimbursement for new medicines are typically made by the U.S. Centers for Medicare & Medicaid Services ("CMS"). CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of product candidates, once approved. Patients are unlikely to use our product candidates, once approved, unless coverage is provided and reimbursement is adequate to cover a significant portion of their cost. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates. 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.

Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. The FDA implemented regulations providing guidance for states to build and submit importation plans for drugs from Canada. In early 2024, the FDA issued its first approval for a state importation plan, and several other states now have pending applications with the FDA. The U.S. government has also directed the FDA to streamline and improve the drug importation process. If successfully implemented, importation of drugs from Canada may materially and adversely affect the price we receive for any of our product candidates. Legislation or regulations allowing the reimportation of drugs, if enacted, could decrease the price we receive for any products that we may develop and adversely affect our future revenues and prospects for profitability. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs. Payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives.

Moreover, increasing efforts by governmental and other 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 product candidates. There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological 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.

We expect that healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals or clearances of our product candidates, if any, may be.

Ongoing healthcare legislative and regulatory reform measures may have a material adverse effect on our business and results of operations.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example, (1) changes to our manufacturing arrangements, (2) additions or modifications to product labeling, (3) the recall or discontinuation of our products or (4) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business. See the section titled "*Business—Current and Future U.S. Healthcare Reform"* in our Annual Report.

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of products have been a focus in this effort. There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical products, limiting coverage and the amount of reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States. Governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their commercial products, which has resulted in several Congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for pharmaceutical products. Congress has indicated that it will continue to seek new legislative measures to control drug costs.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

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

We are exposed to the risk of employee fraud or other illegal activity by our current and any future employees, independent contractors, consultants, CMOs, and vendors. Misconduct by these parties could include intentional, reckless, and/or negligent conduct that fails to comply with FDA or other regulations, provide true, complete and accurate information to the FDA, EMA, MHRA and other comparable regulatory authorities, comply with manufacturing standards we may establish, comply with healthcare fraud and abuse laws and regulations, report financial information or data accurately, or disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under these laws will increase significantly, and our costs associated with compliance with these laws are likely to increase. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. Additionally, we are subject to the risk that a person could allege such fraud or other misconduct, even if none occurred. It is not always possible to identify and deter employee misconduct, 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 failure to comply with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a material and adverse effect on our business, financial condition, results of operations, and prospects.

Off-label use or misuse of our product candidates may harm our reputation in the marketplace or result in injuries that lead to costly product liability suits.

If our product candidates are approved by the FDA, we may only promote or market our product candidates in a manner consistent with their FDA-approved labeling. We will train our marketing and sales force against promoting our product candidates for uses outside of the approved indications for use, known as "off-label uses." We cannot, however, prevent a physician from using our product candidates off-label, when in the physician's independent professional medical judgment he or she deems it appropriate. Furthermore, the use of our product candidates for indications other than those approved by the FDA may not effectively treat such conditions. Further, FDA's Office of Prescription Drug Promotion ("OPDP") actively scrutinizes promotional communications, including digital and social media; any materials that are false, misleading or promote unapproved uses can lead to enforcement actions and could necessitate corrective communications. Any such off-label use of our product candidates could harm our reputation in the marketplace among physicians and patients. There may also be increased risk of injury to patients if physicians attempt to use our product candidates for these uses for which they are not approved, which could lead to product liability suits that might require significant financial and management resources and that could harm our reputation. In the current administration, the FDA has increased its enforcement scrutiny over prescription drug advertising, particularly direct-to-consumer product promotion and advertising. If the FDA finds any of our future promotional communications or advertising to be violative, we may receive an untitled or warning letter, requests for corrective advertising, or fines, amongst other enforcement tools available to the FDA.

Inadequate funding for the FDA or other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

Without appropriation of sufficient funding for federal agencies, our business operations related to our product development activities for the U.S. market could be impacted. The ability of the FDA to review and approve new products can be affected by a variety of factors, including staffing cuts, government budget and funding levels, ability to hire and retain key personnel and ability to accept the payment of user fees and statutory, regulatory, leadership and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other federal agencies, including substantial leadership departures, personnel cuts and policy changes, may also slow the time necessary for new drugs to be reviewed and/or approved, which would harm our business. Changes and cuts in FDA staffing have been reported as resulting in delays in the FDA's responsiveness or in its ability to review product submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all.

There is substantial uncertainty as to the extent and manner in which the U.S. government will seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates and any products for which we obtain approval. This uncertainty could present new challenges and/or opportunities as we navigate development and approval of our product candidates. Additionally, the current administration could issue or promulgate executive orders, regulations, policies or guidance that adversely affect us or create a more challenging or costly environment to pursue the development of new therapeutic candidates. Also, state governments may seek to address or react to changes at the federal level with changes to their regulatory frameworks in a manner that could impact our operations.

EU drug marketing and reimbursement regulations may materially affect our ability to market and receive coverage for our products in the EU Member States.

We intend to seek approval to market our product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the European Union, the pricing of products is subject to governmental control and other market regulations which could put pressure on the pricing and usage of our product candidates. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of a product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for our product candidates and may be affected by existing and future healthcare reform measures.

Much like the federal Anti-Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to reward improper performance generally is typically governed by the national anti-bribery laws of EU Member States and the Bribery Act 2010 in the UK. Infringement of these laws could result in substantial fines and imprisonment. EU Directive 2001/83/EC, which is the EU Directive governing medicinal products for human use, further provides that, where medicinal products are being promoted to persons qualified to prescribe or supply them, no gifts, pecuniary advantages or benefits in kind may be supplied, offered or promised to such persons unless they are inexpensive and relevant to the practice of medicine or pharmacy. This provision has been transposed into the Human Medicines Regulations 2012 and so remains applicable in the UK despite its departure from the European Union.

Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

In addition, in some foreign countries, including some countries in the European Union, the proposed pricing for a product must be approved before it may be lawfully marketed. The requirements governing product pricing and reimbursement vary widely from country to country. For example, some EU Member States have the option to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Reference pricing used by various EU Member States and parallel distribution, or arbitrage between low-priced and high-priced EU Member States, can further reduce prices. An EU Member State may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of any of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for biotechnology products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales and the potential profitability of any of our product candidates in those countries would be negatively affected.

We are subject to export controls, economic sanctions, imports and customs, and anti-corruption laws and regulations of the United States and other jurisdictions. We can face civil penalties, criminal liability and other serious consequences for violations of these laws and regulations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations and U.S. customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Control and the U.S. Department of State. Export controls and trade sanctions laws and regulations may restrict or prohibit altogether the provision, sale, or supply of our products to certain governments, persons, entities, countries, and territories, including those that are the target of comprehensive sanctions or an embargo. We are also subject to anti-corruption and anti-bribery laws, including the U.S. Foreign Corrupt Practices Act of 1977, as amended, 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 conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors, and other partners from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. 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 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.

If we or any third-party manufacturer we engage now or in the future fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs or liabilities that could have a material adverse effect on our business.

We and third-party manufacturers we engage now are, and any third-party manufacturer we may engage in the future will be, subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain general liability insurance as well as workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or commercialization efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Further, with respect to the operations of our current and any future third-party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products. In addition, our supply chain may be adversely impacted if any of our third-party contract manufacturers become subject to injunctions or other sanctions as a result of their non-compliance with environmental, health and safety laws and regulations.

Risks Related to Our Intellectual Property

We depend on in-licensed intellectual property. If we fail to comply with our obligations under our intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are a party to the Janssen License, which is a non-exclusive, fully paid-up, and royalty-free intellectual property license agreement. In connection with our efforts to expand our pipeline of product candidates, we expect to enter into additional license agreements in the future. We have certain obligations under the Janssen License and expect that future license agreements may impose various diligence, milestone payment, royalty, insurance, and other obligations on us. If we fail to comply with these obligations, our licensors may have the right to terminate the relevant agreement, in which event we would not be able to develop or market the products covered by such licensed intellectual property, or to pursue other remedies. Our current and future collaborations are, and may be, dependent upon the effectiveness of the Janssen License, and may be terminated if the Janssen License is terminated.

We may not be able to obtain licenses at a reasonable cost or on reasonable terms, or at all. Furthermore, if we lose intellectual property rights licensed under existing agreements or fail to obtain future licenses, we may be required to expend considerable time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected proprietary technologies and product candidates, which could harm our business significantly.

If we or our licensors are unable to obtain and maintain patent protection for our product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to our product candidates, and our ability to successfully commercialize our product candidates may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our product candidates, their respective components, formulations, combination therapies, methods used to manufacture them and methods of treatment that are important to our business. If we or our licensors do not adequately protect our or our licensors' intellectual property rights, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. We and our licensors seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates that are important to our business. We may in the future also license or purchase patent applications filed by others. If we or our licensors are unable to secure or maintain patent protection with respect to our product candidates and any proprietary product candidates and technology we develop, our business, financial condition, results of operations, and prospects could be materially harmed.

If the scope of the patent protection we or our licensors obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing products and technology similar or identical to our product candidates or otherwise maintain a competitive advantage. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our or our licensors' patents have, or that any of our or our licensors' pending patent applications that mature into issued patents will include, claims with a scope sufficient to protect our product candidates or otherwise provide any competitive advantage. In addition, to the extent that we license intellectual property, we cannot make assurances that those licenses will remain in force.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us, or otherwise provide us with any competitive advantage. The scope of the invention claimed in a patent application can be significantly reduced before the patent is issued, and this scope can be reinterpreted after issuance. Any patents that eventually issue may be challenged, narrowed or invalidated by third parties. Consequently, we do not know whether any of our product candidates will be protectable or remain protected by valid and enforceable patent rights. Our competitors or other third parties may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

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. In addition, we may not pursue or obtain patent protection in all relevant markets. 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. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering product candidates that we license from third parties and are reliant on our licensors. Therefore, we cannot be certain that these patents and applications will be prosecuted and enforced in a manner consistent with the best interests of our business. If such licensors fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated.

Furthermore, 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. 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, at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability, and commercial value of our and our licensors' patent rights are highly uncertain. Our and our licensors' pending and future patent applications may not result in patents being issued which protect our product candidates or which effectively prevent others from commercializing competitive products.

The issuance of a patent is not conclusive as to its inventorship, scope, validity, or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. We or our licensors may in the future, become subject to a third-party pre-issuance submission of prior art, opposition, derivation, revocation, re-examination, post-grant and *inter partes* review, or interference proceeding and other similar proceedings challenging our patent rights or the patent rights of others in the U.S. Patent and Trademark Office (the "USPTO") or other foreign patent office. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical products, or limit the duration of the patent protection of our product candidates.

Furthermore, given the amount of time required for the development, testing, and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to our product candidates.

In addition, we rely on certain of our licensors to prosecute patent applications and maintain patents and otherwise protect the intellectual property we license from them and may continue to do so in the future. We have limited control over these activities or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by these licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights or defend certain of the intellectual property that is licensed to us. It is possible that any licensors' infringement proceeding or defense activities may be less vigorous than had we conducted them ourselves.

Moreover, some of our owned and in-licensed patents and patent applications may in the future be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we or our licensors may need the cooperation of any such co-owners of our owned and in-licensed patents in order to enforce such patents against third parties, and such cooperation may not be provided to us or our licensors. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

If our efforts to protect the proprietary nature of the intellectual property related to our product candidates are not adequate, we may not be able to compete effectively in our market.

We rely upon a combination of patents, confidentiality agreements, trade secret protection and license agreements to protect the intellectual property related to our product candidates. Any disclosure to or misappropriation by third parties of our confidential proprietary information could enable competitors to quickly duplicate or surpass our technological achievements, thus eroding our competitive position in our market. We, or any partners, collaborators, or licensors, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position.

It is possible that defects of form in the preparation or filing of our or our licensors' patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we or our partners, collaborators, or licensors fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, or licensors are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

We anticipate additional patent applications will be filed both in the United States and in other countries, as appropriate. However, we cannot predict:

- if additional patent applications covering new technologies related to our product candidates will be filed;
- if and when patents will issue;
- the degree and range of protection any issued patents will afford us against competitors, including whether third parties will find ways to invalidate or otherwise circumvent our patents;
- whether any of our intellectual property will provide any competitive advantage;
- whether any of our patents that may be issued may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- whether or not others will obtain patents claiming aspects similar to those covered by our or our licensors' patents and patent applications; or
- whether we or our licensors will need to initiate or defend litigation or administrative proceedings which may be costly regardless of whether we or our licensors win or lose.

Additionally, we cannot be certain that the claims in our pending patent applications covering our product candidates and their methods of use will be considered patentable by the USPTO, or by foreign patent offices, or that the claims in any of our issued patents will be considered valid or patentable by courts in the United States or foreign countries. Method of use patents protect the use of a product for the specified method. These types of patents do not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products "off-label." Although off-label prescriptions may, but not necessarily, contribute to a finding of infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

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.

We cannot be certain that an allowed patent application will become an issued patent because there may be events that cause withdrawal of the allowance of a patent application. For example, after a patent application has been allowed, but prior to being issued, material that could be relevant to patentability may be identified. In such circumstances, the applicant may pull the application from allowance in order for the USPTO to review the application in view of the new material. We cannot be certain that the USPTO will issue the application in view of the new material. Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign countries may require the payment of maintenance fees or patent annuities during the lifetime of a patent application and/or any subsequent patent that issues from the application. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process and following the issuance of a patent. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application. Such noncompliance can result in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. Such an event could have a material adverse effect on our business.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.

If we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering our product candidates, the defendant could counterclaim that the patent covering our product candidates, as applicable, is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are various grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, *inter partes* review, post grant review and equivalent proceedings in foreign jurisdictions (such as opposition proceedings). Such proceedings could result in revocation or amendment to our or our licensors' patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel, our licensors and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, or if we are otherwise unable to adequately protect our or our licensors' rights, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and product candidates.

Changes to patent law in the United States and in foreign jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biotechnology and biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and biopharmaceutical industry involves both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. Recent patent reform legislation in the U.S. and other countries, including the Leahy-Smith America Invents Act (the "Leahy-Smith Act"), signed into law on September 16, 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. patent system into a "first-to-file" system. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm our business, results of operations and financial condition.

Moreover, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our or our licensors' ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our or our licensors' ability to obtain new patents or to enforce our or our licensors' existing patents and patents that we or our licensors might obtain in the future. We cannot predict how future decisions by the courts, Congress or the USPTO may impact the value of our or our licensors' patents. Similarly, any adverse changes in the patent laws of other jurisdictions could have a material adverse effect on our business and financial condition. Changes in the laws and regulations governing patents in other jurisdictions could similarly have an adverse effect on our ability to obtain and effectively enforce our patent rights.

We may not be able to protect our intellectual property rights throughout the world.

We may not be able to pursue patent coverage of our product candidates in certain countries outside of the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States may be less extensive than those in the United States. 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. The breadth and strength of our or our licensors' patents issued in foreign jurisdictions or regions may not be the same as the corresponding patents issued in the United States. Consequently, we may not be able to prevent third parties from practicing our or our licensors' inventions in all countries outside the United States, or from selling or importing products made using our or our licensors' 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 further, may export otherwise infringing products to certain territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protections, particularly those relating to biotechnology and biopharmaceutical products. This difficulty with enforcing patents could make it difficult for us to stop the infringement of our or our licensors' patents or marketing of competing products otherwise generally in violation of our proprietary rights. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our or our licensors' patents at risk of being invalidated or interpreted narrowly, put our or our licensors' patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, 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 or license.

If we do not obtain patent term extension for any of our current product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our current product candidates, one or more of our or our licensors' U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984 (the "Hatch-Waxman Amendments"). The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during 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, only one patent may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply for a patent extension within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we believe we are entitled to, our competitors may obtain approval of competing products sooner than we would expect, and our business, financial condition, results of operations, and prospects could be materially harmed.

Third parties may initiate legal proceedings alleging that we are infringing or otherwise misappropriating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to commercialize, develop, manufacture, market, and sell our product candidates without infringing the proprietary rights of third parties. We have yet to conduct comprehensive freedom to operate searches to determine whether we would infringe patents issued to third parties. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates, including interference proceedings before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our product candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing product. In addition, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

If a third party alleges that we infringe its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property misappropriation which, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement or misappropriation, which we may have to pay if a court decides that the product or technology at issue infringes on or violates the third-party's rights, and, if the court finds we have willfully infringed intellectual property rights, we could be ordered to pay treble damages and the patent owner's attorneys' fees;
- an injunction prohibiting us from manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third party agrees to license its patent rights to us;
- even if a license is available from a third party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights protecting our product candidates; and

- we may be forced to try to redesign our product candidates or processes so they do not infringe third-party intellectual property rights, an undertaking which may not be possible or which may require substantial monetary expenditures and time.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

Third parties may assert that we are employing their proprietary technology without authorization. Patents issued in the United States by law enjoy a presumption of validity that can be rebutted only with evidence that is "clear and convincing," a heightened standard of proof. There may be issued third-party patents of which we are currently unaware with claims to compositions, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Patent applications can take many years to issue. There may be currently pending patent applications which may later result in issued patents that may be infringed by our product candidates. Moreover, we may fail to identify relevant patents or incorrectly conclude that a patent is invalid, not enforceable, exhausted, or not infringed by our activities. If any third-party patents, held now or obtained in the future by a third party, were found by a court of competent jurisdiction to cover the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product or methods of using the product, the holders of any such patents may be able to block our ability to commercialize the product unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover any aspect of our formulations, any combination therapies or patient selection methods, the holders of any such patent may be able to block our ability to develop and commercialize the product unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could in turn significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize our product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, could involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing product candidates, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need or may choose to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could harm our business significantly.

We may become involved in lawsuits to protect or enforce our patents, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming.

In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors' is invalid or unenforceable, or may 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. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. Defense against these assertions, non-infringement, invalidity or unenforceability regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

Post-grant proceedings provoked by third parties or brought by the USPTO may be brought to determine the validity or priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could result in a loss of our current patent rights and could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Litigation or post-grant proceedings may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as those within the United States.

Furthermore, 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. In addition, our licensors may have rights to file and prosecute such claims, and we are reliant on them.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

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, 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 securities. Such litigation or 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 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 greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

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

We generally enter into confidentiality and intellectual property assignment agreements with our employees, consultants, and contractors. These agreements generally provide that inventions conceived by the party in the course of rendering services to us will be our exclusive property. However, those agreements may not be honored and may not effectively assign intellectual property rights to us. Moreover, there may be some circumstances where we are unable to negotiate for such ownership rights. Disputes regarding ownership or inventorship of intellectual property can also arise in other contexts, such as collaborations and sponsored research. If we are subject to a dispute challenging our rights in or to patents or other intellectual property, such a dispute could be expensive and time-consuming. If we were unsuccessful, we could lose valuable rights in intellectual property that we regard as our own.

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

In addition to seeking patents for some of our product candidates, we also rely on trade secrets, including unpatented know-how, technology, and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors, and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and 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. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. These lawsuits may consume our time and other resources even if we are successful. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, 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 be subject to claims that we wrongfully hired an employee from a competitor or that our employees have misappropriated intellectual property, including trade secrets of their former employers.

Many of our employees were previously employed at, or may have previously provided or may be currently providing consulting services to, universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. We could in the future be subject to claims that we or our employees have inadvertently or otherwise used or disclosed alleged trade secrets or other confidential information of former employers or competitors. Although we try to ensure that our employees do not use the proprietary information or know how of others in their work for us, we may be subject to claims that we caused an employee to breach the terms of his or her non-competition or non-solicitation agreement, or that we or these employees have, inadvertently or otherwise, used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer or competitor. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

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.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We rely on both registration and common law protection for our trademarks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During the trademark registration process, we may receive Office Actions from the USPTO objecting to the registration of our trademark. Although we would be given an opportunity to respond to those objections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and/or to seek the cancellation of registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

European patents and patent applications could be challenged in the recently created Unified Patent Court for the European Union.

Our or our licensors' European patents and patent applications could be challenged in the recently created Unified Patent Court ("UPC") for the European Union. We may decide to opt out our European patents and patent applications from the UPC. However, if certain formalities and requirements are not met, our European patents and patent applications could be challenged for non-compliance and brought under the jurisdiction of the UPC. We cannot be certain that our or our licensors' European patents and patent applications will avoid falling under the jurisdiction of the UPC, if we decide to opt out of the UPC. Under the UPC, a granted European patent would be valid and enforceable in numerous European countries. A successful invalidity challenge to a European patent under the UPC would result in loss of patent protection in those European countries. Accordingly, a single proceeding under the UPC could result in the partial or complete loss of patent protection in numerous European countries, rather than in each validated European country separately as such patents always have been adjudicated. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize our technology and product candidates and, resultantly, on our business, financial condition, prospects and results of operations.

Risks Related to Ownership of Our Securities

An active trading market for our securities may not be sustained.

An active or liquid market in our securities may not be sustained. The lack of an active market may impair the value of our stockholders' shares, and our stockholders' ability to sell their shares at the desired time and price. An inactive market may also impair our ability to raise capital by selling our securities and our ability to enter into strategic collaborations or acquire other companies, products, or technologies by using our common stock as consideration.

The price of our securities may be volatile, which could result in substantial losses for our stockholders.

The trading price of our securities may be volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this section, these factors include:

- the commencement, enrollment, completion or results of our current or future nonclinical and clinical trials for our product candidates;
- any delay in identifying and advancing a clinical candidate for our other programs;
- any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results or delays, suspensions or terminations in future nonclinical studies or clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates or the failure of a regulatory authority to accept data from nonclinical studies or clinical trials conducted in other countries;
- changes in laws or regulations applicable to our product candidates, including but not limited to clinical trial requirements for approvals;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- our inability to establish collaborations, if needed;
- our failure to commercialize our product candidates, if approved;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to any of our current or future product candidates;
- introduction of new products or services offered by us or our competitors;

- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- actual or anticipated variations in quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry, or product candidates in particular, or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- changes in the structure of the healthcare payment systems;
- overall performance of the equity markets;
- sales of our securities by us or our stockholders in the future;
- trading volume of our securities;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the market for biotechnology and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our securities, regardless of our actual operating performance. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs, reputational harm and a diversion of management's attention and resources.

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations.

Our quarterly and annual operating results may fluctuate significantly, due to a variety of factors, many of which are outside of our control and may be difficult to predict, including:

- the timing and cost of, and level of investment in, research, development and, if approved, commercialization activities relating to our current and future product candidates, which may change from time to time;
- the timing and status of enrollment for clinical trials;
- the cost of manufacturing our product candidates, as well as building out our supply chain, which may vary depending on the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we may incur to acquire, develop or commercialize additional product candidates and technologies;
- timing and amount of any milestone, royalty or other payments due under any collaboration or license agreement, including the Janssen License;
- future accounting pronouncements or changes in our accounting policies;

- the timing and success or failure of nonclinical studies and clinical trials for our product candidates or competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners;
- the timing of receipt of approvals for our product candidates from regulatory authorities in the United States and internationally;
- exchange rate fluctuations;
- coverage and reimbursement policies with respect to our product candidates, if approved, and potential future drugs that compete with our products; and
- the level of demand for our product candidates, if approved, which may vary significantly over time.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance.

This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our future revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if any forecasts we provide to the market are below the expectations of analysts or investors, the price of our securities could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

If securities or industry analysts do not continue to publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our securities depends in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who covers us downgrades our stock or Notes or publishes inaccurate or unfavorable research about our business, our stock price or trading price of the Notes may decline. Similarly, if one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock or Notes could decrease, which might cause our stock price or trading price of the Notes and trading volume to decline.

Our executive officers, directors, principal stockholders and their respective affiliates own a significant percentage of our common stock and have the ability to exert significant control over matters subject to stockholder approval.

Our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates collectively own a significant percentage of our outstanding common stock. As a result, these stockholders, if acting together, have significant influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, amendment of our organizational documents, any merger, consolidation or sale of all or substantially all of our assets and any other significant corporate transaction. In addition, certain of our principal stockholders, including Third Rock Ventures V, L.P., have designated certain members of our board of directors. The interests of these stockholders may not be the same as or may even conflict with the interests of our other stockholders. For example, these stockholders could delay or prevent a change of control of our company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

Sales of a substantial number of shares of our common stock in the public market could cause our common stock price to fall.

Our common stock price could decline as a result of sales of a large number of shares of common stock or the perception that these sales could occur, which might also make it more difficult for us to sell equity securities in the future at a time and price that we deem appropriate.

As of September 30, 2026, we had 48,088,413 shares of common stock outstanding. Shares of unvested restricted common stock will become available for sale immediately upon the vesting of such shares, as applicable. All shares of common stock sold in our IPO or otherwise outstanding as of our IPO are able to be sold in the public market. Shares issued upon the exercise of stock options pursuant to future awards that may be granted under our equity incentive plans or pursuant to future awards granted under those plans will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules, any applicable market stand-off and lock-up agreements, and Rule 144 and Rule 701 under the Securities Act of 1933, as amended (the "Securities Act").

Certain holders of our common stock have rights, subject to some conditions, to require us to file registration statements covering the sale of 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 stockholder agreements between us and such holders. We have registered the offer and sale of all shares of common stock that we may issue under our equity compensation plans, and those shares are available for sale in the open market, unless such shares are subject to vesting restrictions with us or lock-up restrictions. Once we register the offer and sale of shares for the holders of registration rights, they can be freely sold in the public market upon issuance, subject to the lock-up agreements.

In addition, in the future, we may issue additional shares of common stock, or other equity or debt securities convertible into common stock, in connection with a financing, acquisition, employee arrangement, or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and could cause the price of our common stock to decline.

Our issuance of additional capital stock in connection with financings, acquisitions, investments, our stock incentive plans or otherwise will dilute all other stockholders.

We expect to issue additional capital stock in the future that will result in dilution to all other stockholders. For example, in October 2026, we completed the October 2026 Offerings in which we issued and sold (i) 9,761,905 shares of common stock at the Offering Price and, in lieu of shares of common stock to certain investors, pre-funded warrants to purchase up to 1,190,476 shares of common stock at a public offering price of $20.999 per share, (ii) $172.5 million Notes and (iii) 1,428,571 PIPE Shares. Additionally, as of September 30, 2026, 8,019,681 shares of our common stock were reserved for issuance upon exercise of outstanding stock options, vested restricted stock units and performance based stock units. Subsequent to September 30, 2026, we have shares reserved for the issuance of 1,190,476 shares issued in connection with the 2026 Equity Offering, We expect to grant equity awards to employees, directors and consultants under our stock incentive plans. We may also raise capital through equity financings in the future. As part of our business strategy, we may acquire or make investments in complementary companies, products or technologies and issue equity securities to pay for any such acquisition or investment. Any such issuances of additional capital stock may cause stockholders to experience significant dilution of their ownership interests and the per share value of our common stock to decline.

We do not currently intend to pay dividends on our common stock and, consequently, our stockholders' ability to achieve a return on their investment will depend on appreciation of the value of our common stock.

We have never declared or paid cash dividends on our common stock. We currently intend to retain all available funds and any future earnings to support operations and to finance the growth and development of our business. We do not intend to declare or pay any cash dividends on our capital stock in the foreseeable future. As a result, any investment return on our common stock will depend upon increases in the value for our common stock, which is not certain.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current directors and members of management.

Our third amended and restated certificate of incorporation and our amended and restated bylaws contain provisions that may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our securities. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of directors is elected each year;

- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of not less than two-thirds of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our third amended and restated certificate of incorporation or amended and restated bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law (the "DGCL"), which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our amended and restated bylaws designate certain courts as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by 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 bylaws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of, or a claim based on, fiduciary duty owed by any of our current or former directors, officers, and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our third amended and restated certificate of incorporation or our amended and restated bylaws (including the interpretation, validity or enforceability thereof) or (iv) any action asserting a claim that is governed by the internal affairs doctrine (the "Delaware Forum Provision"). The Delaware Forum Provision does not apply to any causes of action arising under the Securities Act or the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Our amended and restated bylaws further provide that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the U.S. shall be the sole and exclusive forum for resolving any complaint asserting a cause or causes of action arising under the Securities Act or the Exchange Act (the "Federal Forum Provision"). In addition, our amended and restated bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing provisions; provided, however, that stockholders cannot and will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

The Delaware Forum Provision and the Federal Forum Provision in our amended and restated bylaws may impose additional litigation costs on stockholders in pursuing any such claims. Additionally, the forum selection clauses in our amended and restated bylaws may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. In addition, while the Delaware Supreme Court and other state courts have upheld the validity of federal forum selection provisions purporting to require claims under the Securities Act or Exchange Act be brought in federal court, there is uncertainty as to whether other courts will enforce our Federal Forum Provision. If the Federal Forum Provision is found to be unenforceable, we may incur additional costs associated with resolving such matters. The Federal Forum Provision may also impose additional litigation costs on stockholders who assert that the provision is not enforceable or invalid. The Court of Chancery of the State of Delaware and the federal district courts of the U.S. may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

We may not be able to continue to satisfy the listing requirements of The Nasdaq Stock Market.

We must meet certain financial and liquidity criteria to maintain our common stock's listing on The Stock Market ("Nasdaq"). If we fail to meet any of Nasdaq's listing standards, our common stock may be delisted. In addition, our board of directors may determine that the cost of maintaining our listing on a national securities exchange outweighs the benefits of such listing. A delisting of our common stock from Nasdaq may materially impair our stockholders' ability to buy and sell our common stock and could have an adverse effect on the market price of, and the efficiency of the trading market for, our securities. The delisting of our common stock could significantly impair our ability to raise capital and the value of our stockholders' investment.

Risks Related to Our Indebtedness

Our indebtedness and liabilities could limit the cash flow available for our operations, expose us to risks that could adversely affect our business, financial condition and results of operations and impair our ability to satisfy our obligations under the Notes.

As of September 30, 2026, we had no consolidated indebtedness for borrowed money and our subsidiary had no liabilities (after excluding intercompany obligations and liabilities of a type not required to be reflected on a balance sheet in accordance with GAAP). As a result of the 2026 Notes Offering, we incurred $172.5 million principal amount of indebtedness. We may also incur additional indebtedness in the future. Our indebtedness could have significant negative consequences for our security holders and our business, results of operations and financial condition by, among other things:

- increasing our vulnerability to adverse economic and industry conditions;
- limiting our ability to obtain additional financing;
- requiring the dedication of a substantial portion of our cash flow from operations to service our indebtedness, which will reduce the amount of cash available for other purposes;
- limiting our flexibility to plan for, or react to, changes in our business;
- diluting the interests of our existing stockholders as a result of issuing shares of our common stock upon conversion of the Notes; and
- placing us at a possible competitive disadvantage with competitors that are less leveraged than us or have better access to capital.

Our ability to make scheduled repayments of the principal of, to pay interest on or to refinance our indebtedness, including the Notes, and to pay any cash amounts due upon conversion of the Notes, depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate cash flow from operations in the future sufficient to service our debt and make necessary capital expenditures. If we are unable to generate such cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional debt or equity capital on terms that may be onerous or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

Regulatory actions, changes in market conditions and other events may adversely affect the trading price and liquidity of the Notes and the ability of investors to implement a convertible arbitrage trading strategy.

We expect that many investors in and purchasers of the Notes will employ, or seek to employ, a convertible arbitrage strategy with respect to the Notes. Investors would typically implement such a strategy by selling short the common stock underlying the Notes and dynamically adjusting their short position while continuing to hold the Notes. Investors may also implement this type of strategy by entering into swaps on our common stock in lieu of or in addition to short selling shares of common stock. We cannot assure investors that market conditions will permit investors to implement this type of strategy, whether on favorable pricing and other terms or at all. If market conditions do not permit investors to implement this type of strategy, whether on favorable pricing and other terms or at all, at any time while the Notes are outstanding, the trading price and liquidity of the Notes may be adversely affected.

The SEC and other regulatory and self-regulatory authorities have implemented various rules and taken certain actions, and may in the future adopt additional rules and take other actions, that may affect those engaging in short selling activity involving equity securities (including our common stock). Such rules and actions include Rule 201 of SEC Regulation SHO, the adoption by the Financial Industry Regulatory Authority, Inc. and the national securities exchanges of a "Limit Up-Limit Down" program, the imposition of market-wide circuit breakers that halt trading of securities for certain periods following specific market declines, and the implementation of certain regulatory reforms required by the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010 (the "Dodd-Frank Act"). Any governmental or regulatory action that restricts the ability of investors in, or potential purchasers of, the Notes to effect short sales of our common stock, borrow shares of our common stock or enter into swaps on our common stock could adversely affect the trading price and the liquidity of the Notes.

In addition, the liquidity of the market for our common stock and other market conditions could deteriorate, which could reduce, or eliminate entirely, the number of shares of our common stock available for lending in connection with short sale transactions and the number of counterparties willing to enter into an equity swap on our common stock with a note investor may not be sufficient for the implementation of a convertible arbitrage strategy. These and other market events could make implementing a convertible arbitrage strategy prohibitively expensive or infeasible. We cannot assure investors that a sufficient number of shares of our common stock will be available to borrow on commercial terms, or at all, to purchasers in the 2026 Notes Offering or holders of the Notes. If investors in the 2026 Notes Offering that seek to employ a convertible arbitrage strategy are unable to do so on commercial terms, or at all, then the trading price of, and the liquidity of the market for, the Notes may significantly decline.

Volatility in the market price and trading volume of our common stock, the condition of the financial markets, prevailing interest rates and other factors could adversely affect the trading price of the Notes.

We expect that the trading price of the Notes will be significantly affected by the market price of our common stock, which could result in greater volatility in the trading price of the Notes than would be expected for non-convertible securities. The stock market in recent years has experienced significant price and volume fluctuations that have often been unrelated to the operating performance of companies. The market price of our common stock has been and may continue to be highly volatile and could fluctuate or decline significantly for many reasons, including in response to the risks described in this Quarterly Report or for reasons unrelated to our operations, many of which are beyond our control, such as reports by industry analysts, investor perceptions or negative announcements by our customers, competitors or suppliers regarding their own performance, as well as industry conditions and general financial, economic and political instability and other factors not currently known to us or that we do not believe are material.

A decrease in the market price of our common stock would likely adversely impact the trading price of the Notes. The market price of our common stock could also be affected by possible sales of our common stock by investors who view the Notes as a more attractive means of equity participation in us and by hedging or arbitrage trading activity that we expect to develop involving our common stock. This trading activity could, in turn, affect the trading price of the Notes. In addition, if investors and potential purchasers seeking to employ a convertible arbitrage strategy are unable to borrow or enter into swaps on our common stock, in each case on commercially reasonable terms, the trading price and liquidity of the Notes may be adversely affected.

In addition, the condition of the financial markets and changes in prevailing interest rates could have an adverse effect on the trading price of the Notes. For example, prevailing interest rates have fluctuated in the past and are likely to fluctuate in the future, and we would expect an increase in prevailing interest rates to depress the trading price of the Notes. An increase in short- or long-term interest rates, including as a result of a rise in actual or expected inflation, could cause the trading price of the Notes to fall significantly.

We may not have the ability to raise the funds necessary to settle conversions of the Notes in cash, to repay the Notes at maturity or to repurchase the Notes upon a fundamental change, and our future debt may contain limitations on our ability to pay cash upon conversion or repurchase of the Notes.

Holders of the Notes have the right, subject to certain conditions and limited exceptions, to require us to repurchase all or a portion of their Notes upon the occurrence of a fundamental change (as described in the indenture governing the Notes) at a fundamental change repurchase price equal to 100% of the principal amount of the Notes to be repurchased, *plus* accrued and unpaid interest to, but excluding, the fundamental change repurchase date. Upon maturity of the Notes, we must pay the principal amount of the Notes *plus* accrued and unpaid interest in cash, unless the Notes have been previously converted, redeemed or repurchased. In addition, upon any conversion of the Notes, unless we elect to deliver solely shares of our common stock to settle such conversion (other than paying cash in lieu of delivering any fractional share), we will be required to make cash payments in respect of the Notes being converted. We may not have enough available cash or be able to obtain financing at the time we are required to make repurchases of the Notes surrendered therefor or pay cash with respect to the Notes being converted or Notes being repaid at maturity. In addition, our ability to repurchase the Notes or to pay cash upon conversions or at maturity of the Notes may be limited by law, by regulatory authority or by agreements governing our future indebtedness. Our failure to repurchase the Notes at a time when the repurchase is required by the indenture or to pay any cash payable on future conversions of the Notes or at maturity of the Notes, as required by the indenture governing the Notes, would constitute a default under the indenture. A default under the indenture or the fundamental change itself could also lead to a default under agreements governing our then-existing indebtedness. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Notes or make cash payments upon conversions of the Notes.

Notwithstanding the foregoing, we will satisfy our conversion obligation by paying and/or delivering, as the case may be, cash, shares of our common stock or a combination of cash and shares of our common stock, at our election, we will elect to irrevocably fix the settlement method to physical settlement in the indenture governing the Notes.

Conversion of the Notes will dilute the ownership interest of our stockholders and may otherwise depress the price of our common stock.

The conversion of some or all of the Notes will dilute the ownership interests of our stockholders. Upon conversion of the Notes, we will satisfy our conversion obligation by delivering shares of our common stock, together with cash in lieu of fractional shares, if applicable. Any sales in the public market of our common stock issuable upon such conversion could adversely affect prevailing market prices of our common stock. In addition, the existence of the Notes may encourage short selling by market participants because the conversion of the Notes could be used to satisfy short positions, or anticipated conversion of the Notes into shares of our common stock could depress the price of our common stock.

The accounting method for the Notes could adversely affect our reported financial condition and results.

The accounting method for reflecting the Notes on our consolidated balance sheet, accruing interest expense for the Notes and reflecting the underlying shares of our common stock in our reported diluted earnings per share may adversely affect our reported earnings and financial condition.

Subject to our full accounting assessment with respect to the Notes, which is not complete as of the date of this Quarterly Report, we expect that the Notes will be reflected as a liability on our consolidated balance sheet, with the initial carrying amount equal to the principal amount of the Notes, net of issuance costs. Issuance costs are treated as a debt discount for accounting purposes, which are amortized into interest expense over the term of such Notes. As a result of this amortization, the interest expense that we expect to recognize for the Notes for accounting purposes will be greater than the cash interest payments we will pay on the Notes, which will result in lower reported net income or larger reported net loss.

In addition, we expect that the shares of common stock underlying the Notes will be reflected in our diluted earnings per share using the "if-converted" method, in accordance with GAAP, for fiscal periods in which we report net income. Under that method, diluted earnings per share would generally be calculated assuming that all the Notes were converted solely into shares of common stock at the beginning of the reporting period, unless the result would be anti-dilutive. The application of the if-converted method may reduce our reported diluted earnings per share to the extent we are profitable in the future.

We cannot be sure whether other changes may be made to the current accounting standards related to the Notes, or otherwise, that could have a material effect on our reported financial results. We have not reached a final determination regarding the accounting treatment for the Notes, and the description above is preliminary. Accordingly, we may account for the Notes in a manner that is significantly different than described above.

Any adverse rating of the Notes may cause their trading price to fall.

We do not intend to seek a rating on the Notes. However, if a rating service were to rate the Notes and if such rating service were to lower its rating on the Notes below the rating initially assigned to the Notes or otherwise announces its intention to put the Notes on credit watch, the trading price of the Notes could decline.

General Risk Factors

Unfavorable global economic conditions, political instability and geopolitical events could adversely affect our business, financial condition, stock price, and results of operations.

Our business could be adversely affected by unstable economic and political conditions within the United States and foreign jurisdictions, including as a result of an economic downturn and geopolitical events, such as changes in the U.S. federal policy that affect the geopolitical landscape. The global credit and financial markets have also generally experienced extreme volatility and disruptions (including as a result of actual or perceived changes in interest rates, inflation and macroeconomic uncertainties), which has included severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, high inflation, uncertainty about economic stability, global supply chain disruptions and increases in unemployment rates. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. A severe or prolonged economic downturn could result in a variety of risks to our business, including a decrease in the demand for our drug candidates and in our ability to raise additional capital when needed on acceptable terms, if at all.

The financial markets and the global economy may also be adversely affected by the current or anticipated impact of political uncertainty, including military conflicts, such as the ongoing conflicts between Russia and Ukraine and Israel and Hamas, the recent U.S. and Israeli military action in Iran and effects thereof, terrorism, or other geopolitical events. Sanctions imposed by the U.S. and other countries in response to such conflicts, including the one in Ukraine, may also continue to 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. Additionally, recent changes in U.S. policy have impacted and may in the future impact, among other things, the U.S. and global economy, international trade relations, unemployment, immigration, healthcare, taxation, the U.S. regulatory environment, inflation and other areas. For example, the implementation of tariffs by the U.S. government has led to increased trade disruptions and political tensions, not only between the U.S. and China, but also between the U.S. and other countries in the international community. In response to tariffs, other countries have implemented and may in the future implement additional retaliatory tariffs on U.S. goods. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets. For example, as described more fully above, the BIOSECURE Act may restrict the ability of U.S. biotechnology companies to purchase services or products from, or otherwise collaborate with, certain Chinese biotechnology companies of concern without losing the ability to contract with, or otherwise receive funding from, the U.S. government. We continue to assess this law to determine whether it could have an effect on our contractual relationships. Although we have taken steps to mitigate the impact of tariffs and trade restrictions on our operations, any changes in political, trade, regulatory, and economic conditions, including U.S. trade policies, could have a material adverse effect on our business, financial condition and results of operations. Until we know what policy changes are made, whether those policy changes are challenged and subsequently upheld by the court system and how those changes impact our business and the business of our competitors over the long term, we will not know if, overall, we will benefit from them or be negatively affected by them.

Furthermore, any disruptions to our supply chain as a result of unfavorable global economic conditions, including due to geopolitical conflicts or public health crises, could negatively impact the timely execution of our ongoing and future clinical trials. In addition, current inflationary trends in the global economy may impact salaries and wages, costs of goods and transportation expenses, among other things, and recent and potential future disruptions in access to bank deposits or lending commitments due to bank failures may create market and economic instability. We cannot anticipate all of the ways in which the foregoing, and the current economic climate and financial market conditions generally, could adversely impact our business.

We, or the third parties upon whom we depend, may be adversely affected by natural disasters, public health crises or other business interruptions and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters or public health crises could severely disrupt our operations, and have a material adverse impact on our business, results of operations, financial condition, and prospects. If a natural disaster, power outage, public health crisis or other event occurred that prevented us from conducting our clinical trials, releasing clinical trial results or delaying our ability to obtain regulatory

approval for our product candidates, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time.

We are eligible to be treated as an "emerging growth company" and a "smaller reporting company" and our election of reduced reporting requirements applicable to emerging growth companies and smaller reporting companies may make our securities less attractive to investors.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act ("JOBS Act"). For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements 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, as an emerging growth company, we are only required to provide two years of audited financial statements. We could be an emerging growth company until as late as December 31, 2029, although circumstances could cause us to lose that status earlier, including if we are deemed to be a "large accelerated filer," which occurs when the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30 unless we remain eligible to use the "smaller reporting company" requirements under the revenue test in Rule 12b-2 of the Exchange Act, or if we have total annual gross revenue of $1.235 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31, or if we issue more than $1.0 billion in non-convertible debt during any three-year period before that time, in which case we would no longer be an emerging growth company immediately.

Even after we no longer qualify as an emerging growth company, we could still qualify as a "smaller reporting company," which would allow us to take advantage of many of the same exemptions from disclosure requirements and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our securities less attractive because we may rely on these exemptions. If some investors find our securities less attractive as a result, there may be a less active trading market for our securities and our share or Notes price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can also take advantage of an extended transition period for complying with new or revised accounting standards until such time as those standards apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards, and therefore we are not subject to the same requirements to adopt new or revised accounting standards as other public companies that are not emerging growth companies.

We are also a "smaller reporting company" as defined in the Exchange Act, and have elected to take advantage of certain of the scaled disclosures available to smaller reporting companies. As a smaller reporting company, we have availed ourselves of the exemption from the requirement that our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting under Section 404 of the Sarbanes-Oxley Act of 2002.

On the last business day of our second quarter in 2026, the aggregate market value of our shares of common stock held by non-affiliates exceeded $700 million. As a result, beginning with our Quarterly Report on Form 10-Q for the quarter ended March 31, 2027, we will no longer be eligible to use the scaled disclosure accommodations available to smaller reporting companies and will become subject to additional disclosure requirements.

Upon losing our smaller reporting company status, we will no longer be able to rely on the scaled disclosure requirements and other accommodations available to smaller reporting companies. This may require us to provide additional disclosures in our periodic reports, including more detailed executive compensation disclosures and additional financial statement information. Due to this upcoming transition, we expect to devote significant time and effort to implement and comply with the additional standards, rules and regulations that may apply to us upon becoming a large accelerated filer. The transition from smaller reporting company status will require additional resources, including personnel, enhanced systems and processes, and increased professional fees for accounting, legal, and compliance services. We will need to implement more comprehensive internal controls and procedures to meet the heightened requirements applicable to larger public companies. Due to the complexity and logistical difficulty of implementing the standards, rules and regulations that apply to non-smaller reporting companies, there is an increased risk that we may be found to be in non-compliance with such standards, rules and regulations or to have significant deficiencies or material weaknesses in our internal controls over financial reporting. Any failure to maintain effective disclosure controls and internal control over financial reporting could materially and adversely affect our business, results of operations and financial condition, and could cause a decline in the trading price of our securities.

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

As a public company, we incur significant legal, accounting and other expenses. We are subject to the reporting requirements of the Exchange Act, which require, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act"), as well as rules subsequently adopted by the SEC and The Nasdaq Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements, including requiring establishment and maintenance of effective disclosure and financial reporting controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years after completion of a company's IPO. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

The rules and regulations applicable to public companies have substantially increased, and will continue to increase, our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have an adverse effect on our business. These increased costs will continue to decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, these rules and regulations make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

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 securities.

Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be reevaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. We currently maintain and review our internal controls and procedures for compliance with Section 404 of the Sarbanes-Oxley Act, which requires an annual management assessment of the effectiveness of our internal control over financial reporting. Any failure to implement required new or improved controls, or any 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 financial statements or identify other areas for further attention or improvement. Inferior 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 stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management is required to assess the effectiveness of these controls annually. However, for as long as we are an emerging growth company or a non-accelerated filer, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal controls over financial reporting could lead to restatements of our financial statements and require us to incur the expense of remediation.

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 must design 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, 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 a required related party transaction disclosure. 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.

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

As of December 31, 2025, we had approximately $46.1 million of federal net operating losses ("NOLs"). Federal NOLs generated in taxable years beginning after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOL carryforwards in a taxable year is limited to 80% of our taxable income in such year. As of December 31, 2025, we had approximately $15.1 million of state NOLs. Of the state NOLs, some are of indefinite life, but most expire at various dates, beginning in 2043. As of December 31, 2025, we had approximately $8.0 million of federal research and development tax credit carryforwards. Federal tax credit carryforwards expire at various dates, beginning in 2042. As of December 31, 2025, we had approximately $3.2 million of state research and development tax credit carryforwards. The state tax credits, which have various carryforward rules, begin to expire in 2037. Our ability to use these NOLs and tax credit carryforwards to offset future tax liabilities depends on the successful development of our product candidates and future financial performance.

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the "Code"), if a corporation undergoes an "ownership change," 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 NOLs and other pre-change tax attributes (such as research and development tax credits) to offset its post-change income or taxes may be limited. A corporation that experiences an ownership change will generally be subject to an annual limitation on the use of its pre-ownership change NOLs equal to the value of the corporation immediately before the ownership change, multiplied by the long-term tax-exempt rate (subject to certain adjustments). We may have experienced ownership changes in the past and may experience ownership changes in the future. There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs by federal or state taxing authorities or other unforeseen reasons, portions of our existing NOLs could expire or otherwise be unavailable to reduce future income tax liabilities. As a result, our ability to use our pre-change NOLs and tax credits to offset future taxable income, if any, or taxes could be subject to limitations. Similar provisions of state tax law may also apply. As a result, even if we attain profitability, we may be unable to use a material portion of our NOLs and tax credits.

Changes in tax law could adversely affect our business and financial condition.

U.S. federal, state, local and foreign tax laws, regulations and administrative guidance are subject to change as a result of the legislative process and review and interpretation by the U.S. Internal Revenue Service, the U.S. Treasury Department and other taxing authorities. For example, the One Big Beautiful Bill Act (the "OBBBA") was signed into law on July 4, 2025 and made significant changes to U.S. federal tax law. Changes to tax laws (which changes may have retroactive application), including with respect to net operating losses and research and development tax credits, could adversely affect us or holders of our common stock. For example, under Section 174 of the Code, in taxable years beginning after December 31, 2021, expenses that are incurred for research and development performed outside the U.S. will be capitalized and amortized, which may have an adverse effect on our cash flow by deferring the timing of tax deductions and potentially increasing taxable income and cash taxes payable in periods in which we generate taxable income. The OBBBA provides that for taxable years beginning after December 31, 2024, expenses that are incurred for research and development performed in the U.S. may, at the taxpayer's election, be immediately deducted or capitalized and amortized. In addition, the OBBBA provides that for taxable years beginning after December 31, 2021 and before January 1, 2025, certain eligible taxpayers generally may elect to retroactively deduct expenses for research and development performed in the U.S. in such taxable years by filing amended tax returns for such taxable years, and all other taxpayers that are not eligible to make such an election and that amortized expenses for research and development performed in the U.S. in such taxable years generally may elect to accelerate and deduct the remaining unamortized amounts of such research and development expenses (i) in the first taxable year beginning after December 31, 2024, or (ii) ratably over the two-taxable year period beginning with the first taxable year beginning after December 31, 2024. In recent years, many changes to tax laws have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our securities.

Clinical trial and product liability lawsuits against us could divert our resources and could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of clinical trial and product liability exposure related to the testing of our product candidates in clinical trials, and we will face an even greater risk if we commercially sell any products that we develop. While we currently have no products that have been approved for commercial sale, the ongoing, planned and future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- termination of clinical trials;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we currently hold clinical trial liability insurance, we will need to obtain and maintain additional insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. We may not be able to obtain and maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

We may become involved in litigation that could divert management's attention and harm our business, and insurance coverage may not be sufficient to cover all costs and damages.

From time to time we may be subject to litigation claims through the ordinary course of our business operations regarding, but not limited to, securities litigation, employment matters, security of patient and employee personal data, contractual relations with collaborators and licensors and intellectual property rights. In the past, securities class action litigation has often followed certain significant business transactions, such as the sale of a company or announcement of any other strategic transaction, the announcement of negative events, such as negative results from clinical trials, or periods of volatility in the market price of a company's securities. These events may also result in or be concurrent with investigations by the SEC. We may be exposed to such litigation or investigation even if no wrongdoing occurred. Litigation and investigations are usually expensive and divert management's attention and resources, which could adversely affect our business and cash resources and our ability to consummate a potential strategic transaction or the ultimate value our stockholders receive in any such transaction.

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

(a) Recent Sales of Unregistered Equity Securities

None.

(b) Use of Proceeds from Initial Public Offering and Concurrent Private Placement

On June 6, 2024, our Registration Statement on Form S-1 (No. 333-279486) relating to our IPO was declared effective by the U.S. Securities and Exchange Commission. Concurrently with the IPO, we also completed a private placement in which we issued and sold an aggregate of 1,058,824 shares of our common stock at the IPO price. We intend to use the remaining proceeds from our IPO and concurrent private placement primarily to fund research and development and clinical development to support the advancement of current or future product candidates and the expansion of our research and development programs, and the remainder for working capital, capital expenditures and other general corporate purposes.

(c) Issuer Repurchases of Securities

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

(a) None.

(b) Not applicable.

(c) Trading Plan.

During the quarter ended September 30, 2026, the following officer (as defined in Rule 16a-1(f) under the Exchange Act) adopted a certain trading plan intended to satisfy the requirements of Rule 10b5-1(c):

Name *(Title)*	Action Taken *(Date of Action)*	Type of Trading Arrangement	Nature of Trading Arrangement	Duration of Trading Arrangement	Aggregate Number of Securities
Troy Ignelzi (*Chief Financial Officer*)	Adoption (*September 4, 2026*)	Trading plan intended to satisfy the affirmative defense conditions of Securities Exchange Act Rule 10b5-1(c)	Sale of the Company's common stock pursuant to the terms of the plan	Approximately 12 months	72,000

During the quarter ended September 30, 2026, no other directors or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted, modified, and/or terminated any "Rule 10b5-1 trading arrangement" or any "non-Rule 10b5-1 trading arrangement," as such terms are defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits.

Exhibit Number	Description
3.1	Third Amended and Restated Certificate of Incorporation of Rapport Therapeutics, Inc. (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on June 10, 2024).
3.2	Amended and Restated Bylaws of Rapport Therapeutics, Inc. (incorporated by reference to Exhibit 3.2 to The Company's Current Report on Form 8-K filed on June 10, 2024).
4.1	Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1 filed on May 17, 2024).
4.2	Amended and Restated Investors' Rights Agreement, by and among the Company and certain of its stockholders, dated as of August 7, 2023 (incorporated by reference to Exhibit 4.2 to the Company's Registration Statement on Form S-1 filed on May 17, 2024).
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.
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.
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.
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.
101.SCH*	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents.
104*	Cover Page Interactive Data File (embedded within the Inline XBRL document and included in Exhibit 101).

* Filed herewith.
** Furnished herewith.
# Indicates a management contract or any compensatory plan, contract or arrangement.

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.

Rapport Therapeutics, Inc.

Date: November 4, 2026

By: /s/ Abraham N. Ceesay
Abraham N. Ceesay, M.B.A.
Chief Executive Officer and Director
(Principal Executive Officer)

Date: November 4, 2026

By: /s/ Troy Ignelzi
Troy Ignelzi
Chief Financial Officer
(Principal Financial and Accounting Officer)

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, Abraham N. Ceesay, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Rapport Therapeutics, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2026

By: /s/ Abraham N. Ceesay

Abraham N. Ceesay, MBA
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, Troy Ignelzi, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Rapport Therapeutics, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2026

By: /s/ Troy Ignelzi

Troy Ignelzi

Chief Financial 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 Rapport Therapeutics, Inc. (the "Company") on Form 10-Q for the period ending September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officers of the Company hereby 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 result of operations of the Company.

Date: November 4, 2026

By: /s/ Abraham N. Ceesay

Abraham N. Ceesay, MBA
Chief Executive Officer
(Principal Executive Officer)

Date: November 4, 2026

By: /s/ Troy Ignelzi

Troy Ignelzi
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)