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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, 2024

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: 001-38898

Applied Therapeutics, Inc.
(Exact Name of Registrant as Specified in its Charter)

Delaware	**81-3405262**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

545 Fifth Avenue, Suite 1400
New York, New York 10017
(212) 220-9226
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

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

Title of each class	**Trading Symbol**	**Name of each exchange on which registered**
Common Stock, par value $0.0001	APLT	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 6, 2024, the registrant had 116,356,474 shares of common stock, $0.0001 par value per share, outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q may contain "forward-looking statements" within the meaning of the federal securities laws made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 about us and our industry that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future financial condition, future operations, projected costs, prospects, plans, objectives of management and expected market growth, are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "design," "due," "estimate," "expect," "forecast," "goal," "intend," "may," "objective," "opportunity," "plan," "predict," "project", "positioned," "potential," "seek," "should," "target," "will," "would" and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology.

We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our financial condition, results of operations, business strategy and financial needs. These forward-looking statements are subject to a number of known and unknown risks, uncertainties and assumptions, including risks described in the section titled "Risk Factors" in this Quarterly Report on Form 10-Q:

- our plans to develop, market and commercialize our product candidates;
- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and our research and development programs;
- our ability to take advantage of expedited regulatory pathways for any of our product candidates;
- our estimates regarding expenses, future revenue, capital requirements, team, growth and needs for additional financing;
- our ability to successfully acquire or license additional product candidates on reasonable terms and advance product candidates into, and successfully complete, clinical studies;
- our ability to maintain and establish collaborations or obtain additional funding;
- our ability to obtain and timing of regulatory approval of our current and future product candidates;
- the anticipated indications for our product candidates, if approved;
- our expectations regarding the potential market size and the rate and degree of market acceptance of such product candidates;
- our ability to fund our working capital requirements and expectations regarding the sufficiency of our capital resources;
- the implementation of our business model and strategic plans for our business and product candidates;
- our intellectual property position and the duration of our patent rights;
- developments or disputes concerning our intellectual property or other proprietary rights;
- our expectations regarding government and third-party payor coverage and reimbursement;
- our ability to compete in the markets we serve;
- the impact of government laws and regulations and liabilities thereunder;
- developments relating to our competitors and our industry; and
- other factors that may impact our financial results.

The foregoing list of risks is not exhaustive. Other sections of this Quarterly Report on Form 10-Q may include additional factors that could harm our business and financial performance. Moreover, we operate in a very competitive and rapidly changing environment. New risk factors emerge from time to time, and it is not possible for our management to predict all risk factors nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in, or implied by, any forward-looking statements.

In light of the significant uncertainties in these forward-looking statements, you should not rely upon forward-looking statements as predictions of future events. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Quarterly Report on Form 10-Q, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur at all. You should refer to the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. Except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise.

Unless the context otherwise requires, the terms "Applied," "Applied Therapeutics," "the Company," "we," "us," "our", "the registrant" and similar references in this Quarterly Report on Form 10-Q refer to Applied Therapeutics, Inc.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

Applied Therapeutics, Inc.
Condensed Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	As of September 30, 2024	As of December 31, 2023
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 98,867	$ 49,898
Security deposits and leasehold improvements	253	254
Prepaid expenses and other current assets	5,483	4,234
Total current assets	104,603	54,386
Operating lease right-of-use asset	1,963	447
TOTAL ASSETS	$ 106,566	$ 54,833
LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)		
CURRENT LIABILITIES:		
Current portion of operating lease liabilities	$ 264	$ 429
Accounts payable	2,837	1,742
Accrued expenses and other current liabilities	13,489	15,286
Warrant liabilities	82,377	53,725
Total current liabilities	98,967	71,182
NONCURRENT LIABILITIES:		
Noncurrent portion of operating lease liabilities	1,707	38
Clinical holdback - long-term portion	—	759
Total noncurrent liabilities	1,707	797
Total liabilities	100,674	71,979
STOCKHOLDERS' EQUITY/(DEFICIT):		
Common stock, $0.0001 par value; 250,000,000 shares authorized as of September 30, 2024 and 200,000,000 shares authorized as of December 31, 2023; 116,356,474 shares issued and outstanding as of September 30, 2024 and 84,869,832 shares issued and outstanding as of December 31, 2023	11	8
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of September 30, 2024 and December 31, 2023; 0 shares issued and outstanding as of September 30, 2024 and December 31, 2023	—	—
Additional paid-in capital	624,098	451,432
Accumulated deficit	(618,217)	(468,586)
Total stockholders' equity/(deficit)	5,892	(17,146)
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY/(DEFICIT)	$ 106,566	$ 54,833

The Notes to Condensed Financial Statements are an integral part of these statements.

Applied Therapeutics, Inc.
Condensed Statements of Operations
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
REVENUE:				
License revenue	$ —	$ —	$ —	$ 10,660
Research and development services revenue	122	—	455	—
Total revenue	122	—	455	10,660
COSTS AND EXPENSES:				
Research and development	14,828	10,785	37,049	38,602
General and administrative	15,037	4,710	34,683	15,585
Total costs and expenses	29,865	15,495	71,732	54,187
LOSS FROM OPERATIONS	(29,743)	(15,495)	(71,277)	(43,527)
OTHER (EXPENSE) INCOME, NET:				
Interest income	1,357	392	2,572	1,020
Change in fair value of warrant liabilities	(40,184)	(27,277)	(80,845)	(39,611)
Other (expense) income, net	(21)	10	(81)	34
Total other expense, net	(38,848)	(26,875)	(78,354)	(38,557)
Net loss	$ (68,591)	$ (42,370)	$ (149,631)	$ (82,084)
Net loss per share attributable to common stockholders	$ (0.48)	$ (0.47)	$ (1.09)	$ (1.09)
Weighted-average common stock outstanding	144,345,781	90,669,969	137,893,249	75,482,234

The Notes to Condensed Financial Statements are an integral part of these statements.

Applied Therapeutics Inc.
Condensed Statements of Comprehensive Loss
(in thousands)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Net loss	$ (68,591)	$ (42,370)	$ (149,631)	$ (82,084)
Other comprehensive loss, net of tax				
Unrealized loss on marketable securities	—	—	—	(51)
Other comprehensive loss, net of tax	—	—	—	(51)
Comprehensive loss, net of tax	$ (68,591)	$ (42,370)	$ (149,631)	$ (82,135)

The Notes to Condensed Financial Statements are an integral part of these statements.

Applied Therapeutics Inc.
Condensed Statements of Stockholders' Equity/(Deficit)
(in thousands, except share and per share data)
(Unaudited)

	Common Stock $0.0001 Par Value		Additional Paid-in		Accumulated Other Comprehensive Income	Total Stockholders'
	Shares	Amount	Capital	Accumulated Deficit	(Loss)	Equity
BALANCE, January 1, 2023	48,063,358	$ 5	$ 352,828	$ (348,823)	$ 51	$ 4,061
Restricted Stock Units released for common stock issued under Equity Incentive Plan	50,203	—	—	—	—	—
Stock-based compensation expense	—	—	2,055	—	—	2,055
Net loss	—	—	—	(10,137)	—	(10,137)
Other comprehensive loss	—	—	—	—	(51)	(51)
BALANCE, March 31, 2023	48,113,561	5	354,883	(358,960)	—	(4,072)
Issuance of common stock and pre-funded warrants, net of issuance costs of $2.5 million	9,735,731	1	27,449	—	—	27,450
Exercise of pre-funded warrants for common stock	4,250,000	—	—	—	—	—
Exercise of stock options	20,174	—	22	—	—	22
Stock-based compensation expense	—	—	1,843	—	—	1,843
Net loss	—	—	—	(29,577)	—	(29,577)
BALANCE, June 30, 2023	62,119,466	6	384,197	(388,537)	—	(4,334)
Issuance of common stock, net of issuance costs of $0.4 million	4,284,344	—	7,004	—	—	7,004
Exercise of common warrants	10,250,000	1	26,754	—	—	26,755
Exercise of stock options	65,445	—	65	—	—	65
Exercise of stock options, not yet issued	(3,804)	—	—	—	—	—
Restricted Stock Units released for common stock issued under Equity Incentive Plan	418,065	—	—	—	—	—
Stock-based compensation expense	—	—	1,836	—	—	1,836
Net loss	—	—	—	(42,370)	—	(42,370)
BALANCE, September 30, 2023	77,133,516	7	419,856	(430,907)	—	(11,044)

Applied Therapeutics Inc.
Condensed Statements of Stockholders' (Deficit)/Equity
(in thousands, except share and per share data)
(Unaudited)

	Common Stock $0.0001 Par Value		Additional Paid-in	Accumulated	Total Stockholders' (Deficit)/Equity
	Shares	Amount	Capital	Deficit	
BALANCE, January 1, 2024	84,869,832	$ 8	$ 451,432	$ (468,586)	$ (17,146)
Issuance of common stock and pre-funded warrants, net of issuance costs of $8.2 million	15,285,714	2	104,746	—	104,748
Exercise of common warrants	9,025,000	1	61,217	—	61,218
Exercise of pre-funded warrants	4,206,285	—	—	—	—
Exercise of stock options	32,290	—	33	—	33
Restricted Stock Units released for common stock issued under Equity Incentive Plan	822,682	—	—	—	—
Stock-based compensation expense	—	—	2,379	—	2,379
Net loss	—	—	—	(83,938)	(83,938)
BALANCE, March 31, 2024	114,241,803	$ 11	$ 619,807	$ (552,524)	$ 67,294
Exercise of stock option	295,202	—	310	—	310
Restricted Stock Units released for common stock issued under Equity Incentive Plan	309,266	—	—	—	—
Stock-based compensation expense	—	—	1,890	—	1,890
Net income	—	—	—	2,898	2,898
BALANCE, June 30, 2024	114,846,271	11	622,007	(549,626)	72,392
Exercise of pre-funded warrants	1,385,525	—	—	—	—
Restricted Stock Units released for common stock issued under Equity Incentive Plan	124,678	—	—	—	—
Stock-based compensation expense	—	—	2,091	—	2,091
Net loss	—	—	—	(68,591)	(68,591)
BALANCE, September 30, 2024	116,356,474	$ 11	$ 624,098	$ (618,217)	$ 5,892

Applied Therapeutics, Inc.
Condensed Statements of Cash Flows
(in thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2024	**2023**
OPERATING ACTIVITIES:		
Net loss	$ (149,631)	$ (82,084)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	6,360	5,734
Amortization of insurance premium	566	1,723
Amortization of operating lease right-of-use assets	345	347
Amortization of leasehold improvements	1	1
Change in operating lease liability	(357)	(356)
Change in fair value of warrant liabilities	80,845	39,611
Changes in operating assets and liabilities:		
Financed insurance premium	—	(1,546)
Prepaid expenses	(1,815)	(480)
Accounts payable	1,095	1,471
Accrued expenses and other current liabilities	(1,486)	(2,816)
Other liabilities	(759)	227
Net cash used in operating activities	(64,836)	(38,168)
INVESTING ACTIVITIES:		
Proceeds from sale of available-for-sale securities	—	4,944
Proceeds from maturities of available-for-sale securities	—	8,928
Net cash provided by investing activities	—	13,872
FINANCING ACTIVITIES:		
Proceeds from issuance of common stock and pre-funded warrants, net of issuance costs	104,748	34,606
Proceeds from financed insurance premium	—	1,546
Repayments of short-term borrowings	(311)	(1,393)
Exercise of common warrants	9,025	10,250
Exercise of stock options	343	87
Net cash provided by financing activities	113,805	45,096
NET INCREASE IN CASH AND CASH EQUIVALENTS	48,969	20,800
Cash and cash equivalents at beginning of period	49,898	16,657
Cash and cash equivalents at end of period	$ 98,867	$ 37,457
SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION:		
Conversion of warrant liabilities to equity for warrant exercises	$ 52,193	$ 16,505
Operating lease right of use asset obtained in exchange for operating lease liability	$ 1,861	$ —
Unrealized loss on marketable securities	$ —	$ (51)
Offering costs still in accrued expense	$ —	$ 152

The Notes to Condensed Financial Statements are an integral part of these statements.

Applied Therapeutics, Inc.

Notes to Condensed Financial Statements (Unaudited)

1. ORGANIZATION AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Operations and Business

Applied Therapeutics, Inc. (the "Company") is a clinical-stage biopharmaceutical company developing a pipeline of novel product candidates against validated molecular targets in indications of high unmet medical need. In particular, the Company is currently targeting treatments for rare diseases such as Galactosemia and Sorbitol Dehydrogenase ("SORD") deficiency. The Company was incorporated in Delaware on January 20, 2016 and is headquartered in New York, New York.

The accompanying unaudited condensed financial statements have been prepared by the Company in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP") and, pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC") for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted pursuant to such rules and regulations. These condensed financial statements should be read in conjunction with the Company's audited financial statements and the notes thereto for the year ended December 31, 2023, included in the Annual Report, filed with the SEC on March 6, 2024 (the "Annual Report").

The unaudited condensed financial statements have been prepared on the same basis as the audited financial statements. In the opinion of management, the accompanying unaudited condensed financial statements contain all adjustments which are necessary for a fair presentation of the Company's financial position as of September 30, 2024, results of operations for the three and nine months ended September 30, 2024 and 2023 and cash flows for the nine months ended September 30, 2024 and 2023. Such adjustments are of a normal and recurring nature. The results of operations for the three and nine months ended September 30, 2024, are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2024. Certain reclassifications have been made to prior period financial statements to conform to the current period presentation.

Liquidity and Going Concern

Under ASC Topic 205-40, *Presentation of Financial Statements - Going Concern*, management is required at each reporting period to evaluate whether there are conditions and events, considered in the aggregate, that raise substantial doubt about an entity's ability to continue as a going concern within one year after the date that the financial statements are issued. As of September 30, 2024, financing through the Company's March 2024 Private Placement, Leerink ATM Agreement and warrant exercises has resulted in net proceeds of $113.8 million, after deducting placement agent commissions and other offering expenses (see Note 7 and Note 8). The Company continues to evaluate several potential long-term financing options, including equity capital, debt, convertible debt, and synthetic royalty financing. Additionally, the Company is in active dialogue with several potential partners regarding business development opportunities related to one or more of its programs. There can be no assurances that the Company's discussions with any of the current counterparties will be successful, and the Company expects to continue to pursue additional opportunities.

As reflected in the accompanying financial statements, the Company incurred a net loss of $149.6 million for the nine months ended September 30, 2024, and has an accumulated deficit of $618.2 million as of September 30, 2024. The exclusive licensing agreement with Advanz Pharma for commercialization rights to AT-007 in Europe provides a source of capital to the Company based on clinical and regulatory milestones. The Company received a $10.7 million upfront payment from Advanz Pharma in January 2023 in conjunction with signing the agreement. If actualization of these milestones aligns with the projected timelines, and product approvals are received in the timeframes expected, this source of capital may be sufficient to cover operating expenses through expected product approvals and potential revenues. However, there are no guarantees that this will materialize timely or at all, and delays or unexpected data could disrupt this potential liquidity. Broadly, the Company has not yet established an ongoing source of revenues sufficient to cover its operating costs and is dependent on debt and equity financing to fund its operations. The Company

currently expects that its existing cash and cash equivalents of $98.9 million as of September 30, 2024, will fund its operating expenses and capital requirements for at least twelve months from the date this Quarterly Report on Form 10-Q is issued.

Risks and Uncertainties

The Company is subject to risks common to companies in the biotechnology industry, including but not limited to, risks of failure of preclinical studies and clinical trials, the need to obtain marketing approval for any product candidate that it may identify and develop, the need to successfully commercialize and gain market acceptance of its product candidates, dependence on key personnel, protection of proprietary technology, compliance with government regulations, development by competitors of technological innovations and reliance on third-party manufacturers.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities and the Company's ability to continue as a going concern as of the date of the financial statements and the reported amounts of revenue and expenses during the reporting period. In preparing the financial statements, management used significant estimates in the following areas, among others: prepaid and accrued expenses; warrant liabilities valuation; license revenue; research and development services revenue; stock-based compensation expense; the likelihood of realization of deferred tax assets; and the evaluation of the existence of conditions and events that raise substantial doubt regarding the Company’s ability to continue as a going concern. Actual results could differ from those estimates.

Significant Accounting Policies

The significant accounting policies and estimates used in preparation of the condensed financial statements are described in the Company’s audited financial statements as of and for the year ended December 31, 2023, and the notes thereto, which are included in the Annual Report. There have been no material changes to the Company’s significant accounting policies during the three and nine months ended September 30, 2024.

Recent Accounting Pronouncements

Any recent pronouncements issued by the FASB or other authoritative standards groups with future effective dates are either not applicable or are not expected to be significant to the financial statements of the Company.

2. LICENSE AGREEMENT

Columbia University

In October 2016, the Company entered into a license agreement (the “2016 Columbia Agreement”) with the Trustees of Columbia University (“Columbia University”) to obtain an exclusive royalty-bearing sublicensable license in respect to certain patents. As part of the consideration for entering into the 2016 Columbia Agreement, the Company issued to Columbia University shares equal to 5% of its outstanding common stock on a fully diluted basis at the time of issue. The common stock had a fair value of $0.5 million at the time of issuance. The Company will be required to make further payments to Columbia University of up to an aggregate of $1.3 million for the achievement of specified development and regulatory milestones, and up to an aggregate of $1.0 million for the achievement of a specified level of aggregate annual net sales, in each case in connection with products covered by the 2016 Columbia Agreement. The Company will also be required to pay tiered royalties to Columbia University in the low- to mid-single digit percentages on the Company’s, its affiliates’ and its sublicensees’ net sales of licensed products, subject to specified offsets and reductions. In addition, the Company is required to make specified annual minimum royalty payments to Columbia University, which is contingent upon the approval of the licensed products, in the mid-six figures beginning on the 10th anniversary of the effective date of the 2016 Columbia Agreement. When we grant sublicenses under the 2016 Columbia Agreement we are required to pay Columbia University a portion of the net sublicensing revenue received from such third parties, at percentages between 10% and 20%, depending on the stage of development at the time such revenue is received from such third parties. The Advanz Agreement includes a sublicense under the 2016 Columbia Agreement.

The 2016 Columbia Agreement will terminate upon the expiration of all the Company's royalty payment obligations in all countries. The Company may terminate the 2016 Columbia Agreement for convenience upon 90 days' written notice to Columbia University. At its election, Columbia University may terminate the 2016 Columbia Agreement, or convert the licenses granted to the Company into non-exclusive, non-sublicensable licenses, in the case of (a) the Company's uncured material breach upon 30 days' written notice (which shall be extended to 90 days if the Company is diligently attempting to cure such material breach), (b) the Company's failure to achieve the specified development and funding milestone events, or (c) the Company's insolvency.

In January 2019, the Company entered into a second license agreement with Columbia University (the "2019 Columbia Agreement"). Pursuant to the 2019 Columbia Agreement, Columbia University granted the Company a royalty-bearing, sublicensable license that is exclusive with respect to certain patents, and non-exclusive with respect to certain know-how, in each case to develop, manufacture and commercialize PI3k inhibitor products. The license grant is worldwide. Under the 2019 Columbia Agreement, the Company is obligated to use commercially reasonable efforts to research, discover, develop and market licensed products for commercial sale in the licensed territory, and to comply with certain obligations to meet specified development and funding milestones within defined time periods. Columbia University retains the right to conduct, and grant third parties the right to conduct, non-clinical academic research using the licensed technology; provided that such research is not funded by a commercial entity or for-profit entity or results in rights granted to a commercial or for-profit entity. As consideration for entering into the 2019 Columbia Agreement, the Company made a nominal upfront payment to Columbia University. The Company will be required to make further payments to Columbia University of up to an aggregate of $1.3 million for the achievement of specified development and regulatory milestones, and up to an aggregate of $1.0 million for the achievement of a specified level of aggregate annual net sales, in each case in connection with products covered by the 2019 Columbia Agreement. The Company will also be required to pay tiered royalties to Columbia University in the low- to mid-single digit percentages on the Company's, its affiliates' and its sublicensees' net sales of licensed products, subject to specified offsets and reductions. In addition, the Company is required to make specified annual minimum royalty payments to Columbia University, which is contingent upon the approval of the licensed products, in the mid-six figures beginning on the tenth anniversary of the effective date of the 2019 Columbia Agreement.

In July 2022, following regulatory changes impacting development of the class of PI3k inhibitors and the Company's decision to discontinue its early stage preclinical PI3k program, the Company and Columbia entered into an agreement terminating the 2019 Columbia Agreement (the "2022 Columbia Termination Agreement") as of July 25, 2022. Under the terms of the 2022 Columbia Termination Agreement, the Company assigned certain regulatory documents regarding the preclinical PI3k inhibitor AT-104 to Columbia and granted Columbia a non-exclusive royalty free license (with rights to sublicense any future Columbia licensee) under certain know-how, technical information and data relating to AT-104 that was developed by the Company during the term of the 2019 Columbia Agreement.

In March 2019, and in connection with the 2016 Columbia Agreement, the Company entered into a research services agreement (the "2019 Columbia Research Agreement") with Columbia University with the purpose of analyzing structural and functional changes in brain tissue in an animal model of Galactosemia, and the effects of certain compounds whose intellectual property rights were licensed to the Company as part of the 2016 Columbia Agreement on any such structural and functional changes. The 2019 Columbia Research Agreement had a term of 12 months from its effective date and expired in accordance with its terms.

On October 3, 2019, and in connection with the 2019 Columbia Agreement, the Company entered into a research services agreement (the "PI3k Columbia Research Agreement" and collectively with the 2016 Columbia Agreement, 2019 Columbia Agreement and 2019 Columbia Research Agreement, the "Columbia Agreements") with Columbia University with the purpose of analyzing PI3k inhibitors for the treatment of lymphoid malignancies. The PI3k Columbia Research Agreement had a term of 18 months from its effective date and expired in accordance with its terms.

During the three and nine months ended September 30, 2024, the Company recorded $0.1 and $0.3 million, respectively, in expense related to the Columbia Agreements. During the three and nine months ended September 30, 2023, the Company recorded zero and $0.1 million in expense, respectively, related to the Columbia Agreements. In aggregate, the Company has incurred $3.2 million in expense from the execution of the Columbia Agreements through September 30, 2024.

As of September 30, 2024, the Company had $0.1 million due to Columbia University included in accrued expenses and no amounts in accounts payable. As of December 31, 2023, the Company had $29,000 due to Columbia University included in accrued expenses and no amounts in accounts payable.

University of Miami

2020 Miami License Agreement

On October 28, 2020, the Company entered into a license agreement with the University of Miami (the "2020 Miami License Agreement") relating to certain technology that is co-owned by the University of Miami (UM), the University of Rochester (UR) and University College London (UCL). UM was granted an exclusive agency from UR and UCL to license each of their rights in the technology. Pursuant to the 2020 Miami License Agreement, UM, on behalf of itself and UR and UCL, granted the Company a royalty-bearing, sublicensable license that is exclusive with respect to certain patent applications and patents that may grant from the applications, and non-exclusive with respect to certain know-how, in each case to research, develop, make, have made, use, sell and import products for use in treating and/or detecting certain inherited neuropathies, in particular those caused by mutation in the sorbitol dehydrogenase (SORD) gene. The license grant is worldwide. Under the 2020 Miami License Agreement, the Company is obligated to use commercially reasonable efforts to develop, manufacture, market and sell licensed products in the licensed territory, and to comply with certain obligations to meet specified development milestones within defined time periods. UM retains for itself, UR, and UCL the right to use the licensed patent rights and licensed technology for their internal non-commercial educational, research and clinical patient care purposes, including in sponsored research and collaboration with commercial entities.

Under the terms of the 2020 Miami License Agreement, the Company was obligated to pay UM an up-front non-refundable license fee of $1.1 million, and a second non-refundable license fee of $0.5 million due on the first anniversary of the date of the license. The Company will be required to make further payments to UM of up to an aggregate $2.2 million for the achievement of specified patenting and development milestones, and up to an aggregate of $4.1 million for achievement of late stage regulatory milestones. The Company will also be required to pay royalties ranging from 0.88% - 5% on the Company's, the Company's affiliates' and the Company's sublicensees' net sales of licensed products. When the Company sublicenses the rights granted under the 2020 Miami License Agreement to one or more third parties, the Company will be required to pay to UM a portion of the non-royalty sublicensing revenue received from such third parties ranging from 15% – 25%. The Advanz Agreement includes a sublicense under the 2020 Miami License Agreement.

The 2020 Miami License Agreement terminates upon the expiration of all issued patents and filed patent applications or 10 years after the first commercial sale of the last product or process for which a royalty is due, unless earlier terminated. In addition, the 2020 Miami License Agreement may be terminated by the Company at any time upon 60 days prior written notice to UM, and may be terminated by either the Company or UM upon material breach of an obligation if action to cure the breach is not initiated within 60 days of receipt of written notice.

The Company recorded $1.1 million and $1.3 million, respectively, in research and development expense related to the Miami License Agreement during the three and nine months ended September 30, 2024. The Company recorded $0.2 million, respectively, in research and development expense related to the Miami License Agreement during each of the three and nine months ended September 30, 2023.

As of September 30, 2024, the Company had $1.3 million due to UM included in accrued expenses relating to the 2020 Miami License Agreement. As of December 31, 2023, the Company had $0.4 million due to UM included in accrued expenses relating to the 2020 Miami License Agreement.

2020 Miami Option Agreement

On October 28, 2020, the Company entered into an option agreement with the University of Miami (the "2020 Miami Option Agreement") concerning certain research activities and technology relating to SORD neuropathy that may be pursued and developed by UM. Under the 2020 Miami Option Agreement, if UM conducts such research activities, then UM is obligated to grant us certain option rights to access and use the research results and to obtain licenses to any

associated patent rights upon us making specified payments to UM within specified time limits. If the Company elects to obtain option rights the Company will be required to make payments to UM in the low-six figures to the low-seven figures, depending upon the rights the Company elects to obtain, and the Company will be obligated to make certain milestone payments in the high-six figures to mid-seven figures if UM conducts and completes certain research activities within specified time periods and the Company elects to receive rights to use the results of that research.

2020 Miami Sponsored Research Agreement

On December 14, 2020, the Company entered into a research agreement with the University of Miami (the "2020 Miami Research Agreement"), under which the University of Miami will conduct a research study relating to SORD neuropathy and deliver a final report on the study to the Company. The term of the research agreement was from December 14, 2020 through December 30, 2021, and was extended through August 31, 2022, whereby the research study was completed. The total consideration for the 2020 Miami Research Agreement was $0.3 million.

During the three and nine months ended September 30, 2024 and 2023 , the Company recorded no research and development expense in relation to the 2020 Miami Research Agreement.

As of September 30, 2024, there are no amounts recorded as accrued expenses related to the 2020 Miami Research Agreement. As of December 31, 2023, the Company had $0.1 million in accrued expenses relating to the 2020 Miami Research Agreement.

Bayh-Dole Act

Some of the intellectual property rights the Company has licensed, including certain rights licensed in the agreements described above, may have been generated through the use of U.S. government funding. As a result, the U.S. government may have certain rights to intellectual property embodied in the Company's current or future product candidates under the Bayh-Dole Act of 1980, or Bayh-Dole Act, including the grant to the government of a non-exclusive, worldwide, freedom to operate license under any patents, and the requirement, absent a waiver, to manufacture products substantially in the United States. To the extent any of the Company's current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

3. FAIR VALUE MEASUREMENTS

The following tables summarize, as of September 30, 2024, the Company's financial assets and liabilities that are measured at fair value on a recurring basis, according to the fair value hierarchy described in the significant accounting policies in the Company's audited financial statements as of and for the year ended December 31, 2023, and the notes thereto, which are included in the Annual Report.

	As of September 30, 2024			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Cash	$ 13,757	$ —	$ —	$ 13,757
Money market funds	85,110	—	—	85,110
Total cash and cash equivalents	$ 98,867	$ —	$ —	$ 98,867
Total financial assets measured at fair value on a recurring basis	$ 98,867	$ —	$ —	$ 98,867
Warrant liabilities - common warrants	—	—	82,377	82,377
Total financial liabilities measured at fair value on a recurring basis	$ —	$ —	$ 82,377	$ 82,377

The following tables summarize, as of December 31, 2023, the Company's financial assets and liabilities that are measured at fair value on a recurring basis.

	As of December 31, 2023			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Cash	$ 24,887	$ —	$ —	$ 24,887
Money market funds	25,011	—	—	25,011
Total cash and cash equivalents	$ 49,898	$ —	$ —	$ 49,898
Total financial assets measured at fair value on a recurring basis	$ 49,898	$ —	$ —	$ 49,898
Warrant liabilities - common warrants	—	—	53,725	53,725
Total financial liabilities measured at fair value on a recurring basis	$ —	$ —	$ 53,725	$ 53,725

On June 27, 2022, the Company issued 30,000,000 warrants to purchase shares of common stock (the "Common Warrants") and 10,000,000 pre-funded warrants to purchase common stock (the "Pre-Funded Warrants") in connection with the June 2022 Offering (see Note 7 for more information on the June 2022 Offering). The Common Warrants were accounted for as liabilities under ASC 815-40, *Derivatives and Hedging, Contracts in Entity's Own Equity* ("ASC 815-40"), as these warrants provide for a settlement provision that does not meet the requirements of the indexation guidance under ASC 815-40. The Pre-Funded Warrants were initially recorded at fair value as a liability as the Company could be required to settle the Pre-Funded Warrants in cash under certain circumstances. In December 2022, the Company amended the Pre-Funded Warrants to remove the potential requirement that they could be settled in cash under certain circumstances. Upon the amendment to the Pre-Funded Warrants, the Pre-funded Warrants liability was reclassified to equity, using their fair value as of the amendment date.

The Common Warrant liabilities were measured at fair value at inception and are then subsequently measured on a recurring basis, with changes in fair value recognized in other income (expense) within the Company's statement of operations.

The Company uses a Black-Scholes option pricing model to estimate the fair value of the Common and Pre-Funded Warrants, which utilizes certain unobservable inputs and is therefore considered a Level 3 fair value measurement. Certain inputs used in this Black-Scholes pricing model may fluctuate in future periods based upon factors that are outside of the Company's control, including a potential change in control outside of the Company's control. A significant change in one or more of these inputs used in the calculation of the fair value may cause a significant change to the fair value of the Company's warrant liabilities, which could also result in material non-cash gains or losses being reported in the Company's condensed statement of operations.

The Common Warrants were remeasured using a Black-Scholes option pricing model with a range of assumptions included below as of September 30, 2024 and December 31, 2023.

	September 30, 2024	**December 31, 2023**
Expected term (in years)	2.1	2.4
Volatility	99.92%	109.46 %
Risk-free interest rate	3.77%	4.32%
Dividend yield	0.00%	0.00%

As of September 30, 2024, the Company utilized a probability-weighted approach that considered the probability of a change in control at the Company in the Black-Scholes option pricing model, whereby a 20% probability of change in control was used for years three and four and a 10% probability was used for year five in the term of the agreements.

As of December 31, 2023 the Company utilized a probability-weighted approach that considered the probability of a change in control at the Company in the Black-Scholes option pricing model, whereby a 20% probability of change

in control was used for years two and three and a 5% probability was used for years four and five in the term of the agreements.

The following table provides a roll forward of the aggregate fair values of the Company's warrant liability, for which fair value is determined using Level 3 inputs (in thousands):

	Warrant Liabilities
Balance as of January 1, 2024	$ 53,725
Warrants exercised	(52,193)
Change in fair value	80,845
Balance as of September 30, 2024	$ 82,377

The inputs utilized by management to value the warrant liabilities are highly subjective. The assumptions used in calculating the fair value of the warrant liabilities represent the Company's best estimates, but these estimates involve inherent uncertainties and the application of management judgment. As a result, if factors change and the Company uses different assumptions, the fair value of the warrant liability for Common Warrants may be materially different in the future.

4. PREPAID EXPENSES AND OTHER CURRENT ASSETS

Prepaid expenses and other current assets consisted of the following:

(in thousands)	September 30, 2024	December 31, 2023
Prepaid research and development expenses	$ 2,885	$ 2,880
Insurance premium asset	—	566
Prepaid manufacturing costs	327	—
Prepaid insurance	768	—
Prepaid commercial and patient advocacy	875	284
Research and development tax credit receivable	262	262
Other prepaid expenses and current assets	366	242
Total prepaid expenses & other current assets	$ 5,483	$ 4,234

5. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	September 30, 2024	December 31, 2023
Accrued pre-clinical and clinical expenses	$ 4,386	$ 10,142
Short-term insurance financing note	—	310
Deferred revenue	212	667
Accrued professional fees	1,166	1,307
Accrued compensation and benefits	2,013	2,066
Accrued commercial expenses	3,770	19
Accrued patent expenses	1,382	396
Other	560	379
Total accrued expenses & other current liabilities	$ 13,489	$ 15,286

6. STOCK-BASED COMPENSATION

Equity Incentive Plans

In May 2019, the Company's board of directors (the "Board") adopted its 2019 Equity Incentive Plan ("2019 Plan"), which was subsequently approved by its stockholders and became effective on May 13, 2019. As a result, no additional awards under the Company's 2016 Equity Incentive Plan, as amended (the "2016 Plan") will be granted and all outstanding stock awards granted under the 2016 Plan that are repurchased, forfeited, expired, or are cancelled will become available for grant under the 2019 Plan in accordance with its terms. The 2016 Plan will continue to govern outstanding equity awards granted thereunder.

The 2019 Plan provides for the issuance of incentive stock options ("ISOs") to employees, and for the grant of nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance stock awards, performance cash awards and other forms of stock awards to the Company's employees, officers, and directors, as well as non- employees, consultants, and affiliates to the Company. Under the terms of the 2019 Plan, stock options may not be granted at an exercise price less than fair market value of the Company's common stock on the date of the grant. The 2019 Plan is administered by the Compensation Committee of the Company's Board.

Initially, subject to adjustments as provided in the 2019 Plan, the maximum number of the Company's common stock that may be issued under the 2019 Plan was 4,530,000 shares, which is the sum of (i) 1,618,841 new shares, plus (ii) the number of shares (not to exceed 2,911,159 shares) that remained available for the issuance of awards under the 2016 Plan, at the time the 2019 Plan became effective, and (iii) any shares subject to outstanding stock options or other stock awards granted under the 2016 Plan that are forfeited, expired, or reacquired. The 2019 Plan provides that the number of shares reserved and available for issuance under the 2019 Plan will automatically increase each January 1, beginning on January 1, 2020, by 5% 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 Board. Subject to certain changes in capitalization of the Company, the aggregate maximum number of shares of common stock that may be issued pursuant to the exercise of ISOs is equal to 13,000,000 shares of common stock. Stock options awarded under the 2019 Plan expire 10 years after grant and typically vest over four years.

As of September 30, 2024, there were 473,973 shares of common stock available for issuance under the 2019 Plan.

Stock-Based Compensation Expense

Total stock-based compensation expense recorded for employees, directors and non-employees:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	2024	2023	2024	2023
Research and development	$ 951	$ 764	$ 2,907	$ 2,378
General and administrative	1,140	1,072	3,453	3,356
Total stock-based compensation expense	$ 2,091	$ 1,836	$ 6,360	$ 5,734

Stock Option Activity

During the nine months ended September 30, 2024, the Company did not grant any stock options. For the three months ended September 30, 2024 and 2023, amortization of stock compensation of options amounted to $0.4 million and $1.1 million, respectively, and for the nine months ended September 30, 2024 and 2023, amortization of stock compensation of options amounted to $1.6 million and $3.9 million, respectively. As of September 30, 2024 and 2023, the total unrecognized stock-based compensation expense for unvested options was $0.7 million and $3.7 million, respectively, which is expected to be recognized over 1.2 years and 1.8 years, respectively.

The following table summarizes the information about stock options outstanding at September 30, 2024:

(in thousands, except for share data)	Options Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding at December 31, 2023	4,695,619	$ 2.01	5.9	$ 7,875
Options granted	—	—		
Options exercised	(327,492)	1.05		
Forfeited	(58,123)	1.05		
Expired	—	—		
Outstanding at September 30, 2024	4,310,004	$ 2.09	5.0	$ 27,613
Exercisable at September 30, 2024	4,168,686	$ 2.13	5.0	$ 26,561
Nonvested at September 30, 2024	141,318	$ 1.05	7.2	$ 1,052

The aggregate intrinsic value is calculated as the difference between the exercise price of all outstanding and exercisable stock options and the fair value of the Company's common stock at September 30, 2024. The intrinsic value of stock options exercised during the three and nine months ended September 30, 2024, was zero and $1.1 million, respectively. The intrinsic value of stock options exercised during the three and nine months ended September 30, 2023, was $0.1 million. The total grant-date fair value of stock options vested during the three and nine months ended September 30, 2024 was $0.1 million and $0.2 million, respectively. The total grant-date fair value of stock options vested during the three and nine months ended September 30, 2023 was $0.1 million and $0.4 million, respectively.

Restricted Stock Unit Activity

During the nine months ended September 30, 2024, the Company granted 1,425,000 restricted stock units ("RSUs"). For the three months ended September 30, 2024 and 2023, amortization of stock compensation of RSUs amounted to $1.7 million and $0.7 million, respectively. For the nine months ended September 30, 2024 and 2023, amortization of stock compensation of RSUs amounted to $4.8 million and $1.8 million, respectively. As of September 30, 2024 and 2023, the unamortized compensation costs associated with non-vested restricted stock awards were $17.8 million and $4.5 million, respectively, with a weighted-average remaining amortization period of 2.6 years.

The following table summarizes the information about restricted stock units outstanding at September 30, 2024:

(in thousands, except for share data)	Shares	Weighted-Average Grant Date Fair Value
Outstanding at December 31, 2023	6,917,422	$ 2.35
Awarded	1,425,000	5.71
Released	(1,256,626)	1.86
Forfeited	(633,984)	2.92
Outstanding at September 30, 2024	6,451,812	$ 3.35
Nonvested at September 30, 2024	6,205,093	$ 3.38
Weighted Average Remaining Recognition Period (in years)	2.6	

2019 Employee Stock Purchase Plan

In May 2019, the Company's Board and its stockholders approved the 2019 Employee Stock Purchase Plan (the "ESPP"), which became effective as of May 13, 2019. The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the U.S. Internal Revenue Code of 1986, as amended. The number of shares

of common stock initially reserved for issuance under the ESPP was 180,000 shares. The ESPP provides for an annual increase on the first day of each year beginning in 2020 and ending in 2029, equal to the lesser of (i) 1% of the shares of common stock outstanding on the last day of the calendar month before the date of the automatic increase and (ii) 360,000 shares; provided that prior to the date of any such increase, the Board may determine that such increase will be less than the amount set forth in clauses (i) and (ii). As of September 30, 2024, no shares of common stock had been issued under the ESPP. The first offering period will commence on November 15, 2024.

7. STOCKHOLDERS' EQUITY

As of September 30, 2024, and December 31, 2023, the authorized capital stock of the Company consisted of 250,000,000 and 200,000,000 shares of common stock, respectively, par value $0.0001 per share and 10,000,000 shares of preferred stock, par value $0.0001 per share, respectively.

Common Stock

June 2022 Offering

On June 27, 2022, the Company completed the June 2022 Offering, an underwritten public offering of 20,000,000 shares of common stock, 10,000,000 Pre-Funded Warrants, and accompanying Common Warrants to purchase up to 30,000,000 shares of common stock. The shares and accompanying Common Warrants were offered at a price to the public of $1.00 per share and warrant, and the Pre-Funded Warrants and accompanying Common Warrants were offered at a price to the public of $0.9999, resulting in aggregate net proceeds of approximately $27.8 million, after deducting underwriting discounts and commissions and offering expenses. The Pre-Funded Warrants and the Common Warrants are immediately exercisable and will expire five years from the date of issuance. Holders may not exercise any Pre-Funded Warrants or Common Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of the Company's outstanding common stock immediately after exercise. Holders of the Pre-Funded Warrants and/or Common Warrants (together with affiliates) who immediately prior to June 27, 2022 beneficially owned more than 9.99% of the Company's outstanding common stock may not exercise any portion of their Pre-Funded Warrants or Common Warrants if the holder (together with affiliates) would beneficially own more than 19.99% of the Company's outstanding common stock after exercise. The Pre-Funded Warrants and Common Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to the Company's stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants and/or Common Warrants will be entitled to receive, upon exercise, the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants and/or Common Warrants immediately prior to such transaction. The Pre-Funded Warrants and Common Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which the Company's stockholders are entitled.

April 2023 Private Placement

On April 26, 2023, the Company completed its sale of a total of 9,735,731 shares of the Company's common stock, at a purchase price of $0.946 per share, and 22,000,000 Pre-Funded Warrants to purchase common stock, at a purchase price of $0.945 per Pre-Funded Warrant, in a private placement (the "April 2023 Private Placement") to a select group of accredited investors, pursuant to the Securities Purchase Agreement, dated as of April 23, 2023, by and between the Company and the 2023 Purchasers. The April 2023 Private Placement resulted in net proceeds to the Company of approximately $27.5 million, after deducting underwriting discounts, commissions and offering expenses. The Pre-Funded Warrants are immediately exercisable from the date of issuance and do not have an expiration date. They have an exercise price of $0.001. Holders may not exercise any Pre-Funded Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of the Company's outstanding common stock immediately after exercise. The Pre-Funded Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to the Company's stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants will be entitled to receive, upon exercise of the Pre-Funded Warrants, the kind and amount of securities, cash or other property that the holders would have

received had they exercised the Pre-Funded Warrants immediately prior to such transaction. The Pre-Funded Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which holders of common stock are entitled.

Leerink ATM Agreement

On August 11, 2023, the Company entered into (the "Leerink ATM Agreement") with Leerink Partners LLC, pursuant to which the Company may offer and sell, from time to time, shares of common stock having an aggregate offering price of up to $75.0 million through Leerink Partners LLC as sales agent. Under the Leerink ATM Agreement, the sales agents may sell shares of common stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415(a)(4) of the Securities Act of 1933, as amended. The Company will pay the sales agent a commission rate of up to 3% of the gross offering proceeds of any shares sold and has agreed to provide the sales agent with indemnification and contribution against certain liabilities. The Leerink ATM Agreement contains customary representations and warranties.

As of September 30, 2024, the Company has sold an aggregate of 20,615,976 shares of the company's common stock, pursuant to the Leerink ATM Agreement with an average sale price of $3.36 per share, resulting in net proceeds of $49.3 million, after deducting underwriting discounts, commissions and offering expenses. The Company intends to use the net proceeds from the Leerink ATM to fund research and development and registration of its pipeline candidates, and for working capital and general corporate purposes.

Venrock Warrant Exchange

On October 12, 2023, the Company entered into an exchange agreement (the "Exchange Agreement") with entities affiliated with Venrock Healthcare Capital Partners (the "Exchanging Stockholders"), pursuant to which the Company exchanged an aggregate of 5,658,034 shares of common stock, owned by the Exchanging Stockholders for pre-funded warrants to purchase an aggregate of 5,658,034 shares of common stock (subject to adjustment in the event of stock splits, recapitalizations and other similar events affecting common stock), with an exercise price of $0.001 per share. The common stock exchanged, and the pre-funded warrants issued were recorded at fair market value of $12.9 million.

March 2024 Private Placement

On March 1, 2024, the Company completed its sale of a total of 12,285,714 shares of the Company's common stock, at a purchase price of $7.00 per share, and 2,000,000 Pre-Funded Warrants to purchase common stock, at a purchase price of $6.999 per Pre-Funded Warrant, in a private placement to a select group of accredited investors (the "March 2024 Private Placement"), pursuant to a Securities Purchase Agreement, dated as of February 27, 2024, by and between the Company and the purchasers named therein (the "2024 Purchasers"). The Private Placement resulted in net proceeds to the Company of approximately $92.3 million, before deducting placement agent commissions and other offering expenses.

The Pre-Funded Warrants are immediately exercisable from the date of issuance and do not have an expiration date. They have an exercise price of $0.001. Holders may not exercise any Pre-Funded Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of the Company's outstanding common stock immediately after exercise. The Pre-Funded Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to the Company's stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants will be entitled to receive, upon exercise of the Pre-Funded Warrants, the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such transaction. The Pre-Funded Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which holders of common stock are entitled.

8. WARRANTS

Warrants Issued with Series A Preferred Stock

On January 26, 2017, in connection with the sale and issuance of the Series A Preferred Stock, the Company issued equity-classified warrants to purchase 309,389 shares of common stock (the "2017 Warrants"), valued at $0.2 million, and included in the issuance costs of the Series A Preferred Stock. The warrants vested immediately and have an exercise price of $2.49 per share and expire on March 13, 2027.

The fair value of warrants issued was estimated using the Black-Scholes option pricing model with the following assumptions for the 2017 Warrants.

Contractual term (in years)	10.0
Volatility	74.48%
Risk-free interest rate	3.20%
Dividend yield	0.00%

Warrants Issued with the 2018 Notes

On January 18, 2018, the Company entered into a placement agent agreement through which it became obligated to issue common stock warrants in connection with the issuance of convertible promissory notes, issued on February 5, 2018 (the "2018 Notes"). The obligation to issue the 2018 Notes Warrants was recorded as a liability at its fair value, (see Note 3), which was initially $0.1 million, and was included in the issuance costs of the 2018 Notes. On November 5, 2018, in connection with the extinguishment of the 2018 Notes into shares of Series B Preferred Stock, the Company issued the 2018 Notes Warrants, which were equity-classified warrants upon issuance, to purchase 76,847 shares of common stock, valued at $0.3 million. The exercise price of the 2018 Notes Warrants resets each time the Company issues common stock with an issue price less than the exercise price of the Warrants. The 2018 Notes Warrants vested immediately upon issuance and expire on November 4, 2028. The exercise price of the 2018 Notes Warrants was $3.98 and $3.99 per share as of September 30, 2024 and December 31, 2023, respectively.

Warrants Issued with Series B Preferred Stock

In November and December 2018, in connection with the sale and issuance of the Series B Preferred Stock, the Company was obligated to issue equity-classified warrants to purchase 72,261 shares of common stock (collectively the "2018 Warrants"), valued in the aggregate at $0.2 million, which was included in the issuance costs for the Series B Preferred Stock. The exercise price of the warrants resets each time the Company issues common stock with an issue price less than the exercise price of the warrants. The warrants vested immediately upon issuance and expire 10 years from the date of issuance. The exercise price of the warrants was $4.84 and $4.86 per share as of September 30, 2024, and December 31, 2023, respectively.

The fair value of the 2018 Warrants was estimated using the Black-Scholes option pricing model with the following assumptions:

Contractual term (in years)	10.0
Volatility	73.22%
Risk-free interest rate	2.70%
Dividend yield	0.00%

In February 2019, in connection with the sale and issuance of the Series B Preferred Stock, the Company was obligated to issue warrants to purchase 23,867 shares of common stock (collectively the "2019 Warrants"), valued in the aggregate at $0.1 million, which was included in the issuance costs for the Series B Preferred Stock. The exercise price of the warrants resets each time the Company issues common stock with an issue price less than the exercise price of the warrants. The warrants vested immediately upon issuance and expire 10 years from the date of issuance. The exercise price of the warrants was $4.84 and $4.86 per share as of September 30, 2024, and December 31, 2023, respectively.

The fair value of the 2019 Warrants was estimated using the Black-Scholes option pricing model with the following assumptions:

Contractual term (in years)	10.0
Volatility	73.22%
Risk-free interest rate	2.70%
Dividend yield	0.00%

The inputs utilized by management to value the warrants are highly subjective. The assumptions used in calculating the fair value of the warrants represent the Company's best estimates, but these estimates involve inherent uncertainties and the application of management judgment. As a result, if factors change and the Company uses different assumptions, the fair value of the warrants may be materially different in the future.

Warrants Issued with June 2022 Offering

On June 27, 2022, in connection with the sale and issuance common stock as part of the June Offering, the Company issued 10,000,000 Pre-Funded Warrants at an exercise price of $0.0001 per share, and 30,000,000 accompanying Common Warrants at an exercise price of $1.00 per share. Each share of common stock and accompanying Common Warrant was sold at a public offering price of $1.00, less underwriting discounts and commissions, and each Pre-Funded Warrant and accompanying Common Warrant was sold at a public offering price of $0.9999, less underwriting discounts and commissions, as described in the prospectus supplement, dated June 22, 2022, filed with the Securities and Exchange Commission on June 24, 2022.

The Pre-Funded Warrants and the Common Warrants are immediately exercisable and will expire five years from the date of issuance. Holders may not exercise any Pre-Funded Warrants or Common Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of the Company's outstanding common stock immediately after exercise. Holders of the Pre-Funded Warrants and/or Common Warrants (together with affiliates) who immediately prior to June 27, 2022 beneficially owned more than 9.99% of the Company's outstanding common stock may not exercise any portion of their Pre-Funded Warrants or Common Warrants if the holder (together with affiliates) would beneficially own more than 19.99% of the Company's outstanding common stock after exercise. The Pre-Funded Warrants and Common Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to the Company's stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants and/or Common Warrants will be entitled to receive, upon exercise, the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants and/or Common Warrants immediately prior to such transaction. The Pre-Funded Warrants and Common Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which the Company's stockholders are entitled.

The June 2022 Pre-Funded Warrants were initially recorded at fair value as a liability as the Company could be required to settle the Pre-Funded Warrants in cash in the event of an acquisition of the Company under certain circumstances. In December 2022, the Company amended the Pre-Funded Warrants to remove the potential requirement that they could be settled in cash under certain circumstances. Beginning December 31, 2023, the Pre-funded Warrants are recorded as equity, using their fair value as of the amendment date.

As of September 30, 2024, warrant holders exercised 8,500,000 Pre-Funded Warrants on a cash basis and received 8,500,000 shares of common stock. The Company received $850 in cash proceeds for the exercise of these Pre-Funded Warrants.

As of September 30, 2024, the Company had 1,500,000 Pre-Funded Warrants from the June 2022 offering outstanding with a weighted average exercise price of $0.0001 per share and an average contractual life of 5 years.

The Common Warrants were accounted for as liabilities under ASC 815-40, as these warrants provide for a settlement provision that does not meet the requirements of the indexation guidance under ASC 815-40. These warrant liabilities are measured at fair value at inception and on a recurring basis, with changes in fair value presented within the statement of operations.

As of September 30, 2024, warrant holders exercised 19,275,000 Common Warrants on a cash basis and received 19,275,000 shares of common stock. The Company received $19.3 million in cash proceeds for the exercise of these Common Warrants.

As of September 30, 2024, the Company had 10,725,000 Common Warrants outstanding with a weighted average exercise price of $1.00 per share and an average contractual life of 5 years.

The Common Warrants were remeasured using a Black-Scholes option pricing model with a range of assumptions included below as of September 30, 2024 and December 31, 2023.

	September 30, 2024	December 31, 2023
Expected term (in years)	2.1	2.4
Volatility	99.92%	109.46%
Risk-free interest rate	3.77%	4.32%
Dividend yield	0.00%	0.00%

Warrants Issued with April 2023 Private Placement

On April 23, 2023, in connection with the sale and issuance of common stock as part of the April 2023 Private Placement, the Company issued 22,000,000 Pre-Funded Warrants at an exercise price of $0.001 per share. Each share of common stock was sold at a public offering price of $0.946, less underwriting discounts and commissions, and each Pre-Funded Warrant was sold at a public offering price of $0.945, less underwriting discounts and commissions, pursuant to a Securities Purchase Agreement, dated as of April 23, 2023, by and between the Company and the Purchasers.

The Pre-Funded Warrants are immediately exercisable from the date of issuance and do not have an expiration date. They have an exercise price of $0.001. Holders may not exercise any Pre-Funded Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of the Company's outstanding common stock immediately after exercise. The Pre-Funded Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to the Company's stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants will be entitled to receive, upon exercise of the Pre-Funded Warrants, the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such transaction. The Pre-Funded Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which holders of common stock are entitled. The Company intends to use the net proceeds to fund research and development and registration of its pipeline candidates, and for working capital and general corporate purposes. The April 2023 pre-funded warrants were classified as equity.

As of September 30, 2024, warrant holders exercised 3,092,680 Pre-Funded Warrants on a cashless basis and received 3,091,810 shares of common stock.

As of September 30, 2024, the Company had 18,907,320 Pre-Funded Warrants from the April 2023 private placement outstanding with a weighted average exercise price of $0.001 per share. The pre-funded warrants do not have an expiration date.

Warrants Issued with March 2024 Private Placement

On March 1, 2024, in connection with the sale and issuance of common stock as part of the March 2024 Private Placement, the Company issued 2,000,000 Pre-Funded Warrants at an exercise price of $0.001 per share. Each share of common stock was sold at a public offering price of $7.00, less underwriting discounts and commissions, and each Pre-Funded Warrant was sold at a public offering price of $6.999, less underwriting discounts and commissions, pursuant to a Securities Purchase Agreement, dated as of February 27, 2024, by and between the Company and the Purchasers.

The Pre-Funded Warrants are immediately exercisable from the date of issuance and do not have an expiration date. They have an exercise price of $0.001. Holders may not exercise any Pre-Funded Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of the Company's outstanding common stock immediately after exercise. The Pre-Funded Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to the Company's stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants will be entitled to receive, upon exercise of the Pre-Funded Warrants, the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such transaction. The Pre-Funded Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which holders of common stock are entitled. The March 2024 pre-funded warrants were classified as equity.

As of September 30, 2024, the Company had 2,000,000 Pre-Funded Warrants from the March 2024 private placement outstanding with a weighted average exercise price of $0.001 per share. The pre-funded warrants do not have an expiration date.

A summary of the Company's outstanding pre-funded and common stock warrants as of September 30, 2024 is as follows:

	Equity Classified Warrants	Liability Classified Warrants	Total Warrants
Outstanding as of January 1, 2024	31,783,652	19,750,000	51,533,652
Warrants granted and issued	2,000,000	—	2,000,000
Warrants exercised	(5,592,680)	(9,025,000)	(14,617,680)
Outstanding as of September 30, 2024	28,190,972	10,725,000	38,915,972

9. LEASES

The following table summarizes the Company's lease related costs for the three and nine months ended September 30, 2024 and 2023:

(in thousands)		Three Months Ended September 30,		Nine Months Ended September 30,	
Lease Cost	Statement of Operations Location	2024	2023	2024	2023
Operating Lease Cost	General and administrative	$ 132	$ 126	$ 386	$ 378
Total lease cost		$ 132	$ 126	$ 386	$ 378

Average lease terms and discount rates for the Company's operating leases were as follows:

	Nine Months Ended	
Other Information	September 30, 2024	September 30, 2023
Weighted-average remaining lease term		
Operating leases	5.0 years	1.1 years
Weighted-average discount rate		
Operating leases	15.45%	5.69%

The following table summarizes the maturities of lease liabilities as of September 30, 2024:

Year	Operating *(in thousands)*
2024	135
2025	546
2026	556
2027	550
2028	563
Thereafter	479
Total lease payments	2,829
Less: interest	858
Total lease liabilities	$ 1,971

On September 11, 2024, the Company entered into a lease renewal agreement for its New York office space located at 545 Fifth Avenue, suite 1400, New York, New York 10017 (the "New York Lease") for a five-year term with total lease payments totaling $2.7 million. In conjunction with the execution of the New York lease renewal, the Company leased additional space located in suite 1401 for a five-year term with lease payments totaling $1.3 million. The lease of suite 1401 is anticipated to commence on November 10, 2024, or such earlier date as the Company begins occupancy of the leased space for its business operations.

10. INCOME TAXES

During the nine months ended September 30, 2024 and the year ended December 31, 2023, the Company recorded a full valuation allowance on federal and state deferred tax assets since management does not forecast the Company to be in a profitable position in the near future.

11. BENEFIT PLANS

The Company established a defined contribution savings plan under Section 401(k) of the Internal Revenue Code in 2018. This plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Matching contributions to the plan may be made at the discretion of the Company’s board of directors. The Company made approximately $0.1 million and $0.3 million, respectively, in matching contributions to the plan during the three and nine months ended September 30, 2024. The Company made approximately $0.1 million and $0.3 million, respectively, in matching contributions to the plan during the three and nine months ended September 30, 2023.

12. NET LOSS PER COMMON SHARE

Basic net loss per common share is computed by dividing the net loss available to common stockholders by the weighted-average number of shares of common stock outstanding during the period, which includes shares issuable under pre-funded warrants agreements for little consideration.

Diluted net loss per common share is computed by giving the effect of all potential shares of common stock, including stock options, preferred shares, warrants and instruments convertible into common stock, to the extent dilutive. Basic and diluted net loss per common share was the same for the three and nine months ended September 30, 2024 and 2023, as the inclusion of all potential common shares outstanding would have been anti-dilutive.

The following tables set forth the computation of basic and diluted net loss per common share for the three and nine months ended September 30, 2024 and 2023:

(in thousands, except for share data)	Three Months Ended September 30, 2024	Three Months Ended September 30, 2023	Nine Months Ended September 30, 2024	Nine Months Ended September 30, 2023
Numerator:				
Net loss	$ (68,591)	$ (42,370)	$ (149,631)	$ (82,084)
Denominator:				
Weighted-average common stock outstanding	144,345,781	90,669,969	137,893,249	75,482,234
Net loss per share attributable to common stockholders - basic and diluted	$ (0.48)	$ (0.47)	$ (1.09)	$ (1.09)

The Company excluded the following potential common shares, presented based on amounts outstanding for the three and nine months ended September 30, 2024 and 2023, from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect:

	As of September 30, 2024	As of September 30, 2023
Options to purchase common stock	4,310,004	4,695,619
Restricted stock units	6,451,812	3,326,336
Warrants to purchase common stock	10,850,618	19,875,618

13. RELATED PARTIES

In December 2018, the Company entered into an agreement (the “LaunchLabs Agreement”) with ARE-LaunchLabs NYC LLC (“Alexandria LaunchLabs”), a subsidiary of Alexandria Real Estate Equities, Inc. for use of specified premises within the Alexandria LaunchLabs space on a month-to-month basis. A member of the Company’s board of directors is the founder and executive chairman of Alexandria Real Estate Equities, Inc. During the three and nine months ended September 30, 2024, the Company made payments to Alexandria LaunchLabs of approximately $25,000 and $0.1 million. During the three and nine months ended September 30, 2023, the Company made payments to Alexandria LaunchLabs of approximately $24,000 and $0.1 million, respectively, under the LaunchLabs Agreement, which was recognized in research and development expenses. As of September 30, 2024, there was no amounts due to Alexandria LaunchLabs under the LaunchLabs Agreement. As of December 31, 2023, there was $8,000 due to Alexandria LaunchLabs under the LaunchLabs Agreement.

14. REVENUE

License Agreement with Advanz Pharma

On January 3, 2023, the Company entered into an Exclusive License and Supply Agreement (the “Advanz Agreement”) with Mercury Pharma Group Limited (trading as Advanz Pharma Holdings) (“Advanz Pharma”). Pursuant to the Advanz Agreement, the Company granted Advanz Pharma the exclusive right and license to commercialize drug products containing AT-007 (also known as govorestat), our proprietary Aldose Reductase Inhibitor (ARI) (the “Licensed Product”), for use in treatment of Sorbitol Dehydrogenase Deficiency (“SORD”) and Galactosemia (each a “Licensed Indication”) in the European Economic Area, Switzerland and the United Kingdom (the “Territory”). The Agreement provides that the Company will perform research and development services prior to and subsequent to marketing authorization and manufacture and supply product for Advanz Pharma. The Company also granted Advanz Pharma a right of negotiation and “most-favored nation” rights with respect to acquiring the European commercialization rights for any additional indications for which the Licensed Product may be developed in the future (or any other products we may develop solely to the extent used for the Licensed Indications).

Advanz Pharma is required to use commercially reasonable efforts to launch and commercialize the Licensed Products in the major markets in the Territory in each Licensed Indication following, and subject to, receipt of marketing authorization therein. Under the Advanz Agreement, Advanz Pharma agreed to pay the Company (i) an upfront payment

of EUR 10.0 million (approximately USD $10.7 million), and certain development milestone payments upon clinical trial completion and receipt of marketing authorization in the territory, as well as certain commercial milestone payments, totaling EUR 134 million (approximately USD $142.2 million) in the aggregate, and (ii) royalties of 20% of net sales of the Licensed Product. Such royalty rate will be payable on a country-by-country basis until the later of (i) the expiration of the licensed patents covering the composition of matter of AT-007, or (ii) 10 years after the European Medicines Agency's grant of marketing authorization for the Licensed Product. The royalties are subject to certain deductions, including certain secondary finishing costs, certain step-in establishment costs and a portion of fees for any potential third-party patent licenses if applicable in the future. Following the initial term of the license, as described above, the royalty rate will be reduced to 10% and shall continue in perpetuity unless the Advanz Agreement is terminated in various circumstances in accordance with its terms. In addition, the Company is entitled to receive cost sharing consideration for post-marketing authorization studies, if applicable.

In accordance with the Company's ASC 606 assessment, Advanz Pharma is considered to be a customer. The Company identified three performance obligations, the exclusive license to commercialize the Licensed Product, the obligations to provide research and development for pre and post-marketing authorization and the obligation to manufacture and supply Advanz Pharma with the Product, at cost (a material right). The Company determined that the upfront payment of EUR 10 million (approximately USD $10.7 million) is the estimated transaction price at contract inception. The performance-based milestone payments, sales-based milestone payments, sales-based royalties, and post-marketing authorization study cost sharing are each determined to be variable consideration that are fully constrained due to the uncertainty of achievement.

At inception of the Advanz Agreement, the Company determined the estimate of standalone selling price for the license performance obligation by using the adjusted market assessment approach. Under this method, the Company forecasted and analyzed Galactosemia and SORD in the European market, the probability of marketing authorization approval as well as considered recent similar license arrangements within the same phase of clinical development, therapeutic area, type of agreement, forecasted sales for the contract period, probability of success and a market discount rate. To estimate the standalone selling price of the research and development services, the Company forecasted its expected costs of satisfying that performance obligation and added an appropriate margin for that service. To estimate the standalone selling price for the manufacturing supply agreement material right, the Company utilized an adjusted market assessment approach. The Company analyzed the discount Advance Pharma is expected to obtain by estimating the material right the customer is receiving for the future purchase of manufacturing and supply services. This estimation utilized the estimated number of patients to be treated per year in the Territory; the estimated volume of bottles per patient required per year; typical margin; probability of success; and discounted at market rate.

The Company allocated the total transaction price to each performance obligation on a relative standalone selling price basis and determined whether revenue should be recognized at a point in time or over time.

Revenue should be recognized when, or as, an entity satisfies a performance obligation by transferring a promised good or service to a customer, i.e., when the customer obtains control of the good or service. The license granted to Advanz Pharma is being accounted for as a distinct performance obligation. The Advanz Pharma license relates to functional IP for which revenue is recognized at a point in time – in the case of this license agreement, the point in time is at inception of the contract because the customer obtained control of the license and was able to use and benefit from its right to use the intellectual property at that point. The Company recognized the transaction price allocated to the license obligation of $10.7 million as license revenue on its statements of operations for the nine months ended September 30, 2023. There were no amounts recognized for the three months ended September 30, 2023.

As of December 31, 2023, the Company made certain immaterial adjustments, which resulted in the Company allocating $9.3 million to the license performance obligation; $1.3 million to the research and development performance obligation; and $0.1 million to the manufacturing and supply material right.

The research and development services performance obligation under the Advanz Agreement represents a separate performance obligation. The research and development services were provided to Advanz Pharma by the Company from inception of the agreement in January 2023 and will continue through completion of post marketing authorization approval studies. Revenue related to the research and development services performance obligation was recognized as services were performed based on the costs incurred through September 30, 2024, as a percentage of the estimated total costs to be incurred for this performance obligation. The Company recognized $0.1

million and $0.5 million of revenue during the three and nine months ended September 30, 2024, respectively. The Company had deferred revenue of $0.1 million and $0.5 million related to the research and development performance obligation at September 30, 2024 and December 31, 2023, respectively. The Company expects to recognize the deferred revenue as of September 30, 2024, over the next six months. Operating expenses for costs incurred pursuant to this arrangement are reported in their respective expense line items in the Statements of Operations, net of any payments due to or reimbursements due from Advanz, with such reimbursements being recognized at the time the party becomes obligated to pay.

The manufacturing supply agreement material right performance obligation provided to Advanz Pharma by the Company resulted in an allocation of the transaction price and resulting deferred revenue as of contract inception and September 30, 2024 and December 31, 2023, of approximately $0.1 million. Revenue associated with this performance obligation will be recognized when Advanz Pharma buys supply from the Company after regulatory approval or upon expiration of the material right.

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

The following information should be read in conjunction with the unaudited financial information and the notes thereto included in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in the Annual Report. In addition, this discussion and analysis contains forward-looking statements and involves numerous risks and uncertainties, including, but not limited to, those described under "Special Note Regarding Forward-Looking Statements" and under "Item 1A. Risk Factors" in this report, and in other reports we file with the SEC, that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biopharmaceutical company developing a pipeline of novel product candidates against validated molecular targets in indications of high unmet medical needs. We focus on molecules and pathways whose role in the disease process is well known based on prior research, but have previously failed to yield successful products due to poor efficacy and tolerability. Our unique approach to drug development leverages recent technological advances to design improved drugs, employs early use of biomarkers to confirm biological activity and focuses on abbreviated regulatory pathways. Our first molecular target is aldose reductase, or AR, an enzyme that converts glucose to sorbitol under oxidative stress conditions, and is implicated in multiple diseases. Prior attempts to inhibit this enzyme were hindered by nonselective, nonspecific inhibition, which resulted in limited efficacy and significant off-target safety effects. The detrimental consequences of AR activation have been well established by decades of prior research. Our AR program currently includes three small molecules, which are all potent and selective inhibitors of AR, but are engineered to have unique tissue permeability profiles to target different disease states, including diabetic complications, heart disease and rare metabolic diseases. The result of this unique multifaceted approach to drug development is a portfolio of highly specific and selective product candidates that we believe are significantly de-risked and can move quickly through the development process.

AT-007 (also called govorestat) is a novel central nervous system, or CNS, penetrant ARI that we are developing for the treatment of rare metabolic diseases, including Galactosemia and SORD Deficiency. Galactosemia is a devastating rare pediatric metabolic disease that affects how the body processes a simple sugar called galactose, and for which there is no known cure or approved treatment available. The U.S. Food and Drug Administration, or FDA, has granted both orphan drug designation and rare pediatric disease designation to AT-007 for the treatment of Galactosemia and in June 2021, the FDA granted Fast Track Designation to AT-007 for the treatment of Galactosemia. In clinical studies, AT-007 significantly reduced plasma galactitol levels in both adults and children with Galactosemia. Galactitol is a toxic metabolite of galactose, which is formed in Galactosemia patients by aberrant activity of AR when galactose is present at high levels. In a study in children with Galactosemia ages 2-17, we demonstrated that the galactitol reduction induced by AT-007 treatment resulted in clinical benefit on activities of daily living, behavioral symptoms, cognition, fine motor skills and tremor over 18 months of treatment. Change in clinical outcome measures correlated with galactitol level.

We submitted a New Drug Application (NDA) in December 2023 to the US FDA, and the FDA accepted the filing of the NDA for govorestat (AT-007) for the treatment of Classic Galactosemia in February of 2024. The NDA was granted Priority Review status, and the FDA initially assigned a Prescription Drug User Free Act (PDUFA) target action date of August 28, 2024. In March 2024 the FDA extended the review period for the New Drug Application (NDA) for govorestat (AT-007) for the treatment of Classic Galactosemia by three months, and set a new PDUFA target action date of November 28, 2024. We submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in the fourth quarter of 2023, which was subsequently validated and accepted for review in December 2023. The EMA recently granted a 3-month extension to the Day 120 clock stop in order to facilitate submission of additional information to the MAA to support potential approval. The NDA and MAA submission packages include clinical outcomes data from the Phase 3 registrational ACTION-Galactosemia Kids study in children ages 2-17 with Galactosemia, the Phase 1/2 ACTION-Galactosemia study in adult patients with Galactosemia, and preclinical data.

In the second quarter of 2024, in the process of preparing for FDA inspection, it was discovered that the vendor we engaged to compile NIH Toolbox data for the Company used an adult formula for calculation of about one third of composite cognition and motor skills scores. Adjusting the formula to the pediatric formula resulted in significantly improved data for cognition as compared to the prior data, demonstrating improvement in the govorestat treated group of approximately 8 points on a standard scale, which was statistically significant compared to placebo (p=0.032). This also resulted in a statistically significant effect on the primary endpoint sensitivity analysis which included cognition (p=0.034). The motor skills data did not change substantially. These updates were disclosed and discussed with the FDA and EMA and will be used in the ongoing evaluation of the NDA and MAA.

AT-007 is also being studied in a rare disease caused by deficiency in the enzyme Sorbitol Dehydrogenase. Aldose Reductase is the first enzyme in the polyol pathway, converting glucose to sorbitol. AR is then followed by Sorbitol Dehydrogenase, which converts sorbitol to fructose. Patients with SORD Deficiency accumulate very high levels of sorbitol in their cells and tissues as a result of the enzyme deficiency, which results in tissue toxicities such as peripheral neuropathy and motor neuron disease. Recent research in drosophila and cell models of SORD Deficiency demonstrated that treatment with an ARI that blocks sorbitol production may provide benefit in this disease. Preclinical studies on AT-007 have demonstrated significant reduction in sorbitol levels in fibroblasts from SORD deficient patients. Treatment with AT-007 in the drosophila model of SORD prevented the disease phenotype and protected from neuronal degeneration. On October 25, 2021, we reported data from a pilot open-label study in eight SORD Deficiency patients. AT-007 reduced blood sorbitol levels by approximately 66% from baseline through 30 days of treatment. AT-007 was safe and well tolerated in all treated patients. In December 2021, we initiated a Phase 2/3 registrational study in patients with SORD Deficiency, which is ongoing at multiple clinical sites in the US and Europe. On February 16, 2023, we announced that in a pre-specified interim analysis of the ongoing Phase 3 INSPIRE trial, AT-007 reduced sorbitol levels by a mean of approximately 52% (or approximately 16,000ng/ml) over 90 days of treatment ($p<0.001$ vs. placebo) in patients with SORD Deficiency. At baseline, the mean blood sorbitol level of SORD patients included in this interim analysis was approximately 29,000ng/ml, with a range of approximately 22,000ng/ml-38,000ng/ml. On February 15, 2024 we announced positive interim 12-month results from the ongoing Phase 3 INSPIRE trial, in which the primary endpoints and several key secondary endpoints were achieved. The interim 12-month analysis demonstrated statistically significant correlation between sorbitol level and the prespecified CMT-FOM composite clinical endpoint (10-meter walk-run test, 4 stair climb, sit to stand test,6-minute walk test and dorsiflexion) ($p=0.05$). AT-007 treatment also provided sustained reduction in sorbitol level in patients with SORD Deficiency over 12 months of treatment, which was statistically significant compared to placebo ($p<0.001$). Lastly, AT-007 treatment also resulted in a highly statistically significant effect ($p=0.01$) on the CMT Health Index (CMT-HI), an important patient-reported outcome measure of disease severity and well-being, which was a secondary endpoint in the study. Aspects of the CMT-HI that demonstrated a treatment effect included lower limb function, mobility, fatigue, pain, sensory function, and upper limb function. AT-007 was safe and well tolerated, with similar incidence of adverse events between active and placebo-treated groups.

In July 2024, the Company held a meeting with the FDA to align on the regulatory path forward for govorestat for the treatment of SORD Deficiency. The FDA's Neurology Division confirmed that the data generated to date was appropriate for a potential NDA submission under the FDA's Accelerated Approval Program, and discussed design of a new confirmatory study to be completed as a post-marketing requirement. The Company expects to submit an NDA early in the first quarter of 2025. If govorestat is approved for the treatment of Classic Galactosemia, the regulatory submission for the treatment of SORD will be submitted as a supplementary New Drug Application (sNDA). Patients in the INSPIRE study will be offered open-label govorestat treatment and will be followed for additional safety data generation. The review and potential approval of govorestat for SORD is independent of the ongoing review of govorestat for Classic Galactosemia.

We also plan to initiate a clinical development program on AT-007 in another pediatric rare disease, called PMM2-CDG. PMM2-CDG is a glycosylation disorder caused by deficiencies in the enzyme phosphomannomutase 2, which leads to CNS symptoms similar to Galactosemia, including low IQ, tremor, and speech and motor problems. Aldose Reductase is over-activated in this disease as a compensatory consequence of PMM2 deficiency, and a CNS penetrant ARI may be a compelling clinical option. Initial data in fibroblast cell lines derived from PMM2-CDG patients demonstrates that AT-007 treatment increases phosphomannomutase 2 activity. The FDA has granted pediatric rare disease designation and orphan designation for AT-007 in PMM2-CDG.

AT-001 (also called caficrestat) is a novel ARI with broad systemic exposure and peripheral nerve permeability that we are developing for the treatment of diabetic cardiomyopathy, or DbCM, a fatal fibrosis of the heart, for which no treatments are available. We completed a Phase 1/2 clinical trial evaluating AT-001 in approximately 120 patients with type 2 diabetes, in which no drug-related adverse effects or tolerability issues were observed. In September 2019, we announced the initiation of a Phase 3 registrational trial of AT-001 in DbCM. The study, called ARISE-HF, was designed to evaluate AT-001's ability to improve or prevent the decline of functional capacity in patients with DbCM at high risk of progression to overt heart failure. Although we did experience enrollment delays in 2020 associated with the Covid-19 pandemic, modifications were made to the trial to include additional sites and geographies to address Covid-19-related issues. On January 4, 2024, we reported topline results from the ARISE-HF study. AT-001 (caficrestat) demonstrated a strong trend in stabilizing cardiac functional capacity, while the placebo group declined over 15 months. The placebo-treated group declined by a mean of -0.31 ml/kg/min over 15 months of treatment, while the AT-001 1500mg twice daily treated group remained primarily stable, with a mean change of -0.01 ml/kg/min over 15 months. While a trend favored active treatment, the difference between active and placebo treated groups (0.30 ml/kg/min) was not statistically significant (p=0.210). Approximately 38% of study subjects were on SGLT2 or GLP-1 therapies for treatment of diabetes, while 62% were not. In a pre-specified subgroup analysis of the primary endpoint in patients not concomitantly treated with SGLT2 or GLP-1 therapies, the placebo group declined by a mean of -0.54 ml/kg/min, while the 1500mg BID AT-001 treated group improved by a mean of 0.08 ml/kg/min over 15 months of treatment, with a difference between groups of 0.62 ml/kg/min (p=0.040). Additionally, in this subgroup analysis, the number of patients who experienced a clinically significant worsening in cardiac functional capacity of 6% or more was substantially higher in the placebo group (46%) as compared to the 1500mg BID AT-001 treated group (32.7%), odds ratio 0.56 (p=0.035). A 6% change in cardiac functional capacity has been shown to predict long-term survival and hospitalization for heart failure. The effect of AT-001 was dose dependent, with the low dose (1000mg BID) demonstrating an intermediate effect between the high dose and placebo. AT-001 was generally safe and well tolerated, with no substantial differences in serious adverse events between AT-001 treated groups as compared to placebo.

As we advance our product candidates forward in additional indications, such as SORD deficiency, PMM2-CDG and retinopathy, we anticipate potential moderate growth in our clinical development and operations teams to support the additional clinical trials, as well as addition of a medical affairs team to support the late stage indications and preparations for commercialization.

AT-003 is a novel ARI designed to cross through the back of the eye when dosed orally, and has demonstrated strong retinal penetrance, for the treatment of diabetic retinopathy, or DR. DR is an ophthalmic disease that occurs in diabetic patients and for which treatments are currently limited to high-cost biologics requiring intravitreal administration. DR has been linked to AR activity, including elevations in sorbitol and subsequent changes in retinal blood vessels, which distorts vision and leads to permanent blindness.

AT-104 is a preclinical dual selective PI3K inhibitor. Due to regulatory changes impacting development of the PI3K inhibitor class of compounds, the Company discontinued its early stage preclinical PI3K program and further development of AT-104. The compound and all rights associated with the technology were returned to Columbia University.

Since inception in 2016, our operations have focused on developing our product candidates, organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio and conducting clinical trials. We do not have any product candidates approved for sale and have not generated any product revenue.

We have incurred significant operating losses since inception in 2016. Our ability to generate product revenue sufficient to achieve profitability will depend on the successful development and commercialization of one or more of our product candidates. Our net loss was $149.6 million for the nine months ended September 30, 2024. As of September 30, 2024, we had an accumulated deficit of $618.2 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future in connection with our ongoing activities, including the commercialization of AT 007. Furthermore, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses. As of September 30, 2024, we had cash and cash equivalents of $98.9 million.

April 2023 Private Placement

On April 26, 2023, we completed the April 2023 Private Placement, in which we sold a total of 9,735,731 shares of common stock, at a purchase price of $0.946 per share, and 22,000,000 Pre-Funded Warrants to purchase common stock, at a purchase price of $0.945 per Pre-Funded Warrant, in a private placement to a select group of accredited investors (the "2023 Purchasers"), pursuant to a Securities Purchase Agreement, dated as of April 23, 2023, by and between us and the 2023 Purchasers. The April 2023 Private Placement resulted in net proceeds to us of approximately $27.5 million, after deducting underwriting discounts, commissions and offering expenses. The Pre-Funded Warrants are immediately exercisable from the date of issuance and do not have an expiration date. They have an exercise price of $0.001. Holders may not exercise any Pre-Funded Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of our outstanding common stock immediately after exercise. The Pre-Funded Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to our stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants will be entitled to receive, upon exercise of the Pre-Funded Warrants, the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such transaction. The Pre-Funded Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which holders of common stock are entitled.

Leerink ATM Agreement

On August 11, 2023, we entered into the Leerink ATM Agreement with Leerink Partners LLC, pursuant to which we may offer and sell, from time to time, shares of common stock having an aggregate offering price of up to $75.0 million through Leerink Partners LLC as sales agent. Under the Leerink ATM Agreement, the sales agent may sell shares of common stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415(a)(4) of the Securities Act of 1933, as amended. We will pay the sales agent a commission rate of up to 3% of the gross offering proceeds of any shares sold and has agreed to provide the sales agent with indemnification and contribution against certain liabilities. The Leerink ATM Agreement contains customary representations and warranties.

As of September 30, 2024, we had sold an aggregate of 20,615,976 shares of our common stock, pursuant to the Leerink ATM Agreement with an average sale price of $3.36 per share, resulting in net proceeds of $49.3 million, after deducting underwriting discounts, commissions and offering expenses.

Venrock Warrant Exchange

On October 12, 2023, we entered into the Exchange Agreement with entities affiliated with Venrock Healthcare Capital Partners, pursuant to which we exchanged an aggregate of 5,658,034 shares of common stock, owned by the Exchanging Stockholders for pre-funded warrants to purchase an aggregate of 5,658,034 shares of common stock (subject to adjustment in the event of stock splits, recapitalizations and other similar events affecting common stock), with an exercise price of $0.001 per share.

March 2024 Private Placement

On March 1, 2024, we completed the March 2024 Private Placement, during which we sold a total of 12,285,714 common shares, at a purchase price of $7.00 per share, and 2,000,000 Pre-Funded Warrants at a purchase

price of $6.999 per Pre-Funded Warrant, in a private placement to a select group of purchasers (the "2024 Purchasers") pursuant to a Securities Purchase Agreement (the "Securities Purchase Agreement"). The March 2024 Private Placement resulted in gross proceeds to us of approximately $92.3 million, after deducting placement agent commissions and other offering expenses.

The Pre-Funded Warrants are immediately exercisable from the date of issuance and do not have an expiration date. They have an exercise price of $0.001. Holders may not exercise any Pre-Funded Warrants that would cause the aggregate number of shares of common stock beneficially owned by the holder to exceed 9.99% of our outstanding common stock immediately after exercise. The Pre-Funded Warrants are subject to adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock and also upon any distributions for no consideration of assets to our stockholders. In the event of certain corporate transactions, the holders of the Pre-Funded Warrants will be entitled to receive, upon exercise of the Pre-Funded Warrants, the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such transaction. The Pre-Funded Warrants do not entitle the holders thereof to any voting rights or any of the other rights or privileges to which holders of common stock are entitled.

The securities issued have the benefit of the Registration Rights Agreement (the "Registration Rights Agreement"), dated as of February 27, 2024, by and among us and the 2024 Purchasers, requiring us to prepare and file a registration statement with the SEC as soon as reasonably practicable, but in no event later March 28, 2024 (the "Filing Deadline"), and to use commercially reasonable efforts to have the registration statement declared effective within 30 days of the Filing Deadline, subject to extension under the terms of the Registration Rights Agreement. We satisfied our obligations under the Registration Rights Agreement by filing a registration statement on Form S-3 on May 12, 2023, which became effective on April 25, 2024. We intend to use the net proceeds to fund research and development and registration of its pipeline candidates, and for working capital and general corporate purposes.

Components of Our Results of Operations

Revenue

Since inception, we have not generated any product revenue and do not expect to generate any revenue from the sale of products until after we receive regulatory approval. We have generated revenue solely from licensing of intellectual property and sale of research and development services. If our development efforts for our product candidates are successful and result in regulatory approval, or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from a combination of product sales or payments from collaboration or license agreements.

Operating Expenses

Research and Development Expenses

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

- employee-related expenses, including salaries, related benefits and stock-based compensation expense for employees engaged in research and development functions;
- fees paid to consultants for services directly related to our product development and regulatory efforts;
- expenses incurred under agreements with contract research organizations, or CROs, as well as contract manufacturing organizations, or CMOs, and consultants that conduct and provide supplies for our preclinical studies and clinical trials;
- costs associated with preclinical activities and development activities;
- costs associated with our technology and our intellectual property portfolio; and
- costs related to compliance with regulatory requirements.

We expense research and development costs as incurred. Costs for external development activities are recognized based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our financial statements as prepaid or accrued research and development expenses.

Research and development costs also include costs incurred in connection with certain licensing arrangements. Before a compound receives regulatory approval, we record upfront and milestone payments made by us to third parties under licensing arrangements as expense. Upfront payments are recorded when incurred, and milestone payments are recorded when the specific milestone has been achieved. Once a compound receives regulatory approval, we will record any milestone payments in Identifiable intangible assets, commence amortization and, unless the asset is determined to have an indefinite life, we amortize the payments on a straight-line basis over the remaining agreement term or the expected product life cycle, whichever is shorter.

Research and development activities are central to our business model. We expect that our research and development expenses will continue to increase for the foreseeable future as we continue clinical development for our product candidates and continue to discover and develop additional product candidates. If any of our product candidates enter into later stages of clinical development, they will generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. Historically, we have incurred research and development expenses that primarily relate to the development of AT-007, AT-001 and our ARI program. As we advance our product candidates, we expect to allocate our direct external research and development costs across each of the indications or product candidates. As we progress towards commercialization, we expect to see our research and development costs decrease significantly for those trials.

The following table summarizes our research and development expenses for the three and nine months ended September 30, 2024 and 2023:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	**2024**	**2023**	**2024**	**2023**
Product pipeline research and development expenses				
AT-001	$ 404	$ 4,006	$ 4,429	$ 13,948
AT-007	11,187	4,108	23,327	16,581
Personnel-related expenses	1,845	1,426	5,437	4,535
Stock-based compensation	951	764	2,907	2,378
Other expenses	441	481	949	1,160
Total research and development expenses	$ 14,828	$ 10,785	$ 37,049	$ 38,602

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in our executive, finance, and commercial functions. General and administrative expenses also include professional fees for legal, accounting, auditing, tax and consulting services; travel expenses; and facility-related expenses, which include allocated expenses for rent and maintenance of facilities and other operating costs.

Commercial expenses consist of payroll expense for commercial personnel, as well as marketing, market research, market access, and other focused investments to support launch of drug candidates and generate evidence of commercial potential and value proposition. Commercial expenses are included in general and administrative expenses.

We expect that our general and administrative expenses will increase in the future as we increase our general and administrative headcount to support our continued research and development and potential commercialization of our product candidates. We also expect to incur increased expenses associated with being a public company, including costs of accounting, audit, legal, regulatory and tax compliance services; director and officer insurance costs; and investor and public relations costs.

Other (Expense) Income, Net

Other (expense) income, net consists of interest income (expense), net, other income (expense), net and change in fair value of warrant liabilities. Interest income (expense), net consists primarily of our interest income on our cash and cash equivalents and marketable securities. Other income (expense), net consists primarily of realized gains and losses on sales of marketable securities. Changes in fair value of warrant liabilities consists of mark to market changes on our common warrants that are classified as liabilities.

Results of Operations

Three months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the three months ended September 30, 2024 and 2023:

(in thousands)	Three Months September 30, 2024	2023
REVENUE:		
License revenue	$ —	$ —
Research and development services revenue	122	—
Total revenue	122	—
COSTS AND EXPENSES:		
Research and development	14,828	10,785
General and administrative	15,037	4,710
Total costs and expenses	29,865	15,495
LOSS FROM OPERATIONS	(29,743)	(15,495)
OTHER (EXPENSE) INCOME, NET:		
Interest income	1,357	392
Change in fair value of warrant liabilities	(40,184)	(27,277)
Other (expense) income, net	(21)	10
Total expense, net	(38,848)	(26,875)
Net loss	$ (68,591)	$ (42,370)

Revenue

Revenue for the three months ended September 30, 2024 was $0.1 million, compared to $0 for the three months ended September 30, 2023. For the three months ended September 30, 2024, the increase of $0.1 million was primarily related to:

- an increase in research and development services revenue of $0.1 million due to recognition of deferred revenue related to the Advanz agreement.

Research and Development Expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2024 and 2023:

(in thousands)	Three Months Ended September 30, 2024	2023	Increase/(Decrease)
Clinical and pre-clinical	$ 8,831	$ 8,215	$ 616
Drug manufacturing and formulation	174	(228)	402
Personnel expenses	1,845	1,426	419
Stock-based compensation	951	764	187
Regulatory and other expenses	3,027	608	2,419
Total research and development expenses	$ 14,828	$ 10,785	$ 4,043

Research and development expenses for the three months ended September 30, 2024 were $14.8 million, compared to $10.8 million for the three months ended September 30, 2023. For the three months ended September 30, 2024, the increase of $4.0 million was primarily related to:

- an increase in clinical and pre-clinical expense of $0.6 million, primarily due to increased costs related to AT-007 for the three months ended September 30, 2024, offset by decreased costs related to AT-001;
- an increase in drug manufacturing and formulation costs of $0.4 million primarily related to the release of legacy accruals for the three months ended September 30, 2023, which did not occur for the three months ended September 30, 2024;
- an increase in personnel expense of $0.4 million primarily due to salary increases;
- an increase in stock-based compensation of $0.2 million due amortization of additional RSU awards being granted in 2024; and
- an increase in regulatory and other expenses of $2.4 million primarily due to near completion of AT-007 study.

General and Administrative Expenses

The following table summarizes our general and administrative expenses for the three months ended September 30, 2024 and 2023:

(in thousands)	Three Months Ended September 30, 2024	2023	Increase/(Decrease)
Legal and professional fees	$ 3,783	$ 2,062	$ 1,721
Commercial expenses	6,312	(450)	6,762
Personnel expenses	2,084	736	1,348
Stock-based compensation	1,140	1,072	68
Insurance expenses	328	441	(113)
Other expenses	1,390	849	541
Total general and administrative expenses	$ 15,037	$ 4,710	$ 10,327

General and administrative expenses were $15.0 million for the three months ended September 30, 2024, compared to $4.7 million for the three months ended September 30, 2023. For the three months ended September 30, 2024, the increase of $10.3 million was primarily related to:

- an increase in legal and professional fees of $1.7 million related to higher external legal fees to support planned commercialization;
- an increase in commercial expenses of $6.8 million, due to planned commercialization;
- an increase in personnel expenses of $1.3 million primarily due to salary increases and hiring of employees during the three months ended September 30, 2024;

•an increase in stock-based compensation of $0.1 million primarily due to additional grants of RSUs offset by legacy options becoming fully vested;

•a decrease in insurance expenses of $0.1 million related to decreased insurance costs; and

•an increase in other expenses of $0.5 million due to an overall increase in data storage costs to support planned commercialization.

Interest Income, Net

Interest income was $1.4 million for the three months ended September 30, 2024 compared to $0.4 million for the three months ended September 30, 2023. Interest income increased due to higher interest rates and cash balances in the current period.

Change in Fair Value of Warrant Liabilities

Change in the fair value of warrant liabilities was a $40.2 million expense for the three months ended September 30, 2024, as compared to a $27.3 million expense for the three months ended September 30, 2023. The change in the fair value of warrant liabilities is primarily related to an overall increase in stock price,offset by a decrease in the number of liability-classified warrants outstanding as of September 30, 2024 (19,750,000 at September 30, 2023 compared to 10,725,000 at September 30, 2024).

Other (Expense) Income, Net

Other (expense) income, net expense was a $21,000 expense for the three months ended September 30, 2024, compared to a gain of $10,000 for the three months ended September 30, 2023, primarily due to foreign exchange gains/losses.

Nine months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the nine months ended September 30, 2024 and 2023:

	Nine Months Ended September 30,	
(in thousands)	**2024**	**2023**
REVENUE:		
License revenue	$ —	$ 10,660
Research and development services revenue	455	—
Total revenue	455	10,660
COSTS AND EXPENSES:		
Research and development services revenue	37,049	38,602
General and administrative	34,683	15,585
Total costs and expenses	71,732	54,187
LOSS FROM OPERATIONS	(71,277)	(43,527)
OTHER (EXPENSE) INCOME, NET:		
Interest income	2,572	1,020
Change in fair value of warrant liabilities	(80,845)	(39,611)
Other (expense) income, net	(81)	34
Total other expense	(78,354)	(38,557)
Net loss	$ (149,631)	$ (82,084)

Revenue

Revenue for the nine months ended September 30, 2024 was $0.5 million, compared to $10.7 million for the nine months ended September 30, 2023. For the nine months ended September 30, 2024 , the decrease of $10.2 million was primarily related to:

•a decrease in license revenue of $10.7 million, due to the Company not executing any new license arrangements for the nine months ended September 30, 2024;
•an increase in research and development services revenue of $0.5 million due to recognition of deferred revenue related to the Advanz agreement.

Research and Development Expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2024:

	Nine Months Ended September 30,		
(in thousands)	**2024**	**2023**	**Increase/(Decrease)**
Clinical and pre-clinical	$ 23,452	$ 28,776	$ (5,324)
Drug manufacturing and formulation	242	1,583	(1,341)
Personnel expenses	5,437	4,535	902
Stock-based compensation	2,907	2,378	529
Regulatory and other expenses	5,011	1,330	3,681
Total research and development expenses	$ 37,049	$ 38,602	$ (1,553)

Research and development expenses for the nine months ended September 30, 2024 were $37.0 million, compared to $38.6 million for the nine months ended September 30, 2023. For the nine months ended September 30, 2024, the decrease of $1.6 million was primarily related to:

•a decrease in clinical and pre-clinical expense of $5.3 million, primarily due to decreased expense related to AT-001, offset by increased expense related to AT-007 during the nine months ended September 30, 2024;
•a decrease in drug manufacturing and formulation costs of $1.3 million primarily due to near completion of AT-001 and AT-007 studies;
•an increase in personnel expense of $0.9 million primarily due to salary increases;
•an increase in stock-based compensation of $0.5 million due to additional RSU grants; and
•an increase in regulatory and other expenses of $3.7 million due to near completion of AT-001 and AT-007 studies.

General and Administrative Expenses

The following table summarizes our general and administrative expenses for the nine months ended September 30, 2024 and 2023:

	Nine Months Ended September 30,		
(in thousands)	**2024**	**2023**	**Increase/(Decrease)**
Legal and professional fees	$ 9,346	$ 5,154	$ 4,192
Commercial expenses	11,600	(79)	11,679
Personnel expenses	5,423	2,851	2,572
Stock-based compensation	3,453	3,356	97
Insurance expenses	1,176	1,846	(670)
Other expenses	3,685	2,457	1,228
Total general and administrative expenses	$ 34,683	$ 15,585	$ 19,098

General and administrative expenses were $34.7 million for the nine months ended September 30, 2024, compared to $15.6 million for the nine months ended September 30, 2023. For the nine months ended September 30, 2024, the increase of $19.1 million was primarily related to:

- an increase in legal and professional fees of $4.2 million related to higher external legal fees to support planned commercialization;
- an increase in commercial expenses of $11.7 million, due to planned commercialization.
- an increase in personnel expenses of $2.6 million primarily due to salary increases, severance packages and hiring of new employees (including new executives);
- an increase in stock-based compensation of $0.1 million is due to additional RSU awards being granted, offset by the termination of a key executive, which resulted in reversal of stock based compensation expense;
- a decrease in insurance expenses of $0.7 million related to decreased insurance costs; and
- an increase in other expenses of $1.2 million due to an overall increase in data storage costs to support planned commercialization.

Interest Income, Net

Interest income was $2.6 million for the nine months ended September 30, 2024, as compared to $1.0 million for the nine months ended September 30, 2023. Interest income increased due to higher interest rates and cash balances in the current period.

Change in Fair Value of Warrant Liabilities

Change in the fair value of warrant liabilities was a $80.8 million expense for the nine months ended September 30, 2024, as compared to a $39.6 million expense for the nine months ended September 30, 2023. The change in the fair value of warrant liabilities is primarily related to changes in our common share price, offset by a decrease in the number of liability-classified warrants outstanding as of September 30, 2024 (19,750,000 at September 30, 2023 compared to 10,725,000 at September 30, 2024).

Other (Expense) Income, Net

Other (expense) income, net expense was a $0.1 million loss for the nine months ended September 30, 2024, compared to a gain of $34,000 for the nine months ended September 30, 2023, primarily due to foreign exchange gains/losses.

Liquidity and Capital Resources

Since our inception and through September 30, 2024, we have not generated any product revenue and have incurred significant operating losses and negative cash flows from our operations. The exclusive licensing agreement with Advanz Pharma for commercialization rights to AT-007 in Europe is expected to provide a source of capital to the Company based on clinical and regulatory milestones. If actualization of these milestones aligns with the projected timelines, and product approvals are received in the timeframes expected, this source of capital may be sufficient to cover operating expenses through expected product approvals. However, there are no guarantees that this will materialize, and delays or unexpected data could disrupt this potential source of liquidity. Broadly, we have not yet established an ongoing source of product revenue sufficient to cover our operating costs and we are dependent on debt and equity financing to fund our operations. As of September 30, 2024, our cash and cash equivalents were $98.9 million. We believe that our cash and cash equivalents and the projected clinical and regulatory milestone payments expected in fiscal year 2025 from Advanz Pharma will be sufficient to fund our operations into 2026, and potentially beyond if clinical trial completion and marketing authorization in Europe as well as commercial sales milestones materialize in the expected timelines. Additionally, the Priority Review Voucher (PRV), which would be granted upon a potential Galactosemia NDA approval could substantially extend our cash runway.

Cash Flows

The following table summarizes our cash flows for each of the periods presented:

(in thousands)	Nine Months Ended September 30, 2024	2023
Net cash used in operating activities	$ (64,836)	$ (38,168)
Net cash provided by investing activities	—	13,872
Net cash provided by financing activities	113,805	45,096
Net increase in cash and cash equivalents	$ 48,969	$ 20,800

Operating Activities

Net cash used in operating activities for the nine months ended September 30, 2024, was $64.8 million primarily due to our net loss of $149.6 million, a decrease in accrued expenses and other current liabilities of $1.5 million, a decrease in other liabilities of $0.8 million, a decrease in prepaid expenses of $1.8 million, and a decrease in operating lease liability of $0.4 million. This is partially offset by an increase in fair value of warrant liabilities of $80.8 million, an increase of $6.4 million in non-cash stock-based compensation expense, amortization of insurance premium of $0.6 million, an increase in accounts payable of $1.1 million, amortization of operating lease right-of-use assets of $0.3 million and amortization of leasehold improvements of $1,000.

Net cash used in operating activities for the nine months ended September 30, 2023, was $38.2 million primarily due to our net loss of $82.1 million, a decrease in accrued expenses and other current liabilities of $2.8 million, a decrease in financed insurance premium of $1.5 million, a decrease in prepaid expenses of $0.5 million, and a decrease in operating lease liability of $0.4 million. This is partially offset by an increase in fair value of warrant liabilities of $39.6 million, an increase of $5.7 million in non-cash stock-based compensation expense, amortization of insurance premium of $1.7 million, an increase in accounts payable of $1.5 million, amortization of operating lease right-of-use assets of $0.3 million, an increase in other liabilities of $0.2 million, and amortization of leasehold improvements of $1,000.

Investing Activities

Net cash provided by investing activities for the nine months ended September 30, 2024, was $0 due to sale of all investments during 2023.

Net cash provided by investing activities for the nine months ended September 30, 2023 was $13.9 million relating to the proceeds from the sale of available-for-sale securities of $4.9 million and maturities of available-for-sale securities of $8.9 million.

Financing Activities

During the nine months ended September 30, 2024, net cash provided by financing activities was $113.8 million, primarily from proceeds from the issuance of shares and pre-funded warrants of $104.7 million as part of the March 2024 private placement and issuance of shares under the Leerink ATM Agreement, and $9.0 million from the exercise of common warrants, $0.3 million from exercise of stock options for common stock under the equity incentive plan, partially offset by repayment of short-term borrowings of $0.3 million.

During the nine months ended September 30, 2023, net cash provided by financing activities was $45.1 million, primarily from proceeds from the issuance of shares and pre-funded warrants of $27.5 million as part of the April 2023 private placement, $7.2 million of proceeds from the issuance of shares under the Leerink ATM Agreement, and $10.3 million from the exercise of common warrants, proceeds from financed insurance premium of $1.5 million, $0.1 million

from exercise of stock options for common stock under the equity incentive plan, partially offset by repayment of short-term borrowings of $1.4 million.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and clinical trials of our product candidates. We believe that our expenses may increase significantly if and as we:

- continue the ongoing and planned development of our product candidates;
- initiate, conduct and complete any ongoing, anticipated or future preclinical studies and clinical trials for our current and future product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- establish a sales, marketing, manufacturing and distribution infrastructure to commercialize any current or future product candidate for which we may obtain marketing approval;
- seek to discover and develop additional product candidates;
- continue to build a portfolio of product candidates through the acquisition or in-license of drugs, product candidates or technologies;
- maintain, protect and expand our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Furthermore, we have and expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses.

Due to the numerous risks and uncertainties associated with the development of our product candidates and programs, and because the extent to which we may enter into collaborations with third parties for development of our product candidates is unknown, we are unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future funding requirements, both near and long-term, will depend on many factors, including:

- the initiation, scope, progress, timing, costs and results of our ongoing and planned clinical trials for our product candidates;
- the outcome, timing, effort and cost of meeting regulatory requirements established by the FDA and other federal, state, local and foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions;
- the achievement of milestones or occurrence of other developments that trigger payments under the Columbia Agreements or other agreements we may enter into;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under future collaboration agreements, if any;
- the effect of competing technological and market developments;
- the cost and timing of completion of clinical or commercial-scale manufacturing activities;
- the costs of operating as a public company;
- the extent to which we in-license or acquire other products and technologies;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the cost of establishing sales, marketing and distribution capabilities for our product candidates in regions where we choose to commercialize our product candidates, if approved; and
- the initiation, progress, timing and results of the commercialization our product candidates, if approved, for commercial sale.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to finance our cash needs through offerings of securities, PIPE, debt financings, collaborations or other strategic transactions. The terms of financing may adversely affect the holdings or the rights of our stockholders. Funding may not be available to us on acceptable terms, or at all. If we are unable to obtain funding, we may be required to delay, limit, reduce or terminate some or all of our research and product development, product portfolio expansion or future commercialization efforts. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

Contractual Obligations and Commitments

We lease certain assets under noncancelable operating leases, which expire through 2029. The leases relate primarily to office space. As of September 30, 2024, aggregate future minimum commitments under these office leases are $2.8 million through 2029, excluding any related common area maintenance charges or real estate taxes.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or U.S. GAAP. The preparation of our financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities, costs and expenses and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and 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 that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

There have been no significant changes to our critical accounting policies from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations," included in the Annual Report.

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements and do not have any holdings in variable interest entities.

Recently Issued Accounting Pronouncements

Any recent accounting pronouncements issued by the FASB or other authoritative standards groups with future effective dates are either not applicable or are not expected to be significant to the financial statements of the Company.

Emerging Growth 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 irrevocably elected to "opt out" of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities and foreign currency sensitivities.

Interest Rate Sensitivity

Our exposure to market risk relates to our cash, and cash equivalents of $98.9 million. The primary objective of our investment activities is to preserve principal while at the same time maximizing the income we receive without significantly increasing risk. Some of the financial instruments in which we invest could be subject to market risk where the interest rates may cause the value of the instruments to fluctuate. To minimize this risk, we intend to maintain a portfolio which may include cash, cash equivalents and short-term investment securities available-for-sale in a variety of securities.

We do not believe that our cash has significant risk of default or illiquidity. While we believe our cash and cash equivalents does not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash at one or more financial institutions that are in excess of federally insured limits. Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation has had a material effect on our results of operations during the periods presented.

Foreign Currency Sensitivity

Our primary operations are transacted in U.S. Dollars, however, certain service agreements with third parties are denominated in currencies other than the U.S. Dollar, primarily the Euro. As such, we are subject to foreign exchange risk and therefore, fluctuations in the value of the U.S. Dollar against the Euro may impact the amounts reported for expenses and obligations incurred under such agreements. We do not participate in any foreign currency hedging activities and we do not have any other derivative financial instruments. We did not recognize any significant exchange rate gain or loss during the three and nine months ended September 30, 2024. A hypothetical 10% change in foreign exchange rates during any of the periods presented would not have a material impact on our financial condition or results of operations.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures.

As of September 30, 2024, our management, with the participation of our principal executive officer and our principal financial officer, evaluated the effectiveness of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to management, including our principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on this evaluation, our principal executive officer and principal financial officer concluded that, as of September 30, 2024, the design and operation of our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting.

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the three months ended September 30, 2024, that has materially affected, or is 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 legal proceedings arising in the ordinary course of our business. We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have an adverse effect on our business, operating results or financial condition.

Item 1A. Risk Factors.

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following information about these risks described below, together with other information appearing elsewhere in this Annual Report, including our financial statements and the related notes and the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations," before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline and you may lose all or part of your investment.

Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations and the market price of our common stock.

Risk Factor Summary

The following is a summary of the risk factors included in this Item 1A and is qualified entirely by the disclosure included in the rest of this Item 1A:

Risks Related to Our Financial Position and Capital Needs

- We have incurred and expect to continue to incur substantial operating losses and our ability to use our net operating losses to offset future taxable income may be subject to certain limitations.
- Our operating history makes evaluating our business and future viability more difficult.
- We will require substantial additional funding to finance our operations, and may suffer consequences to our development programs upon failure to do so.
- Raising additional capital may cause adverse effects.

Risks Related to the Development and Commercialization of Our Product Candidates

- Our success is substantially dependent on the successful clinical development, regulatory approval and commercialization of our product candidates and may be adversely affected upon failure to do so.
- We or Advanz Pharma may fail to perform under any of the agreements entered into in connection with the partnership for the commercialization of AT-007, which may subject us to liabilities, and we may fail to achieve anticipated benefits of the partnership.
- Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials and our results may not be sufficient for the necessary regulatory approvals.
- Clinical drug development involves a lengthy and expensive process and the development of additional product candidates is risky and uncertain.
- We may be unable to obtain/maintain or receive the benefits of regulatory approval, rare pediatric disease designation/exclusivity, accelerated registration pathways, breakthrough therapy designations or fast track designations for our product candidates, as applicable.
- Clinical trials are expensive, time consuming and subject to factors outside our control.
- Our product candidates may cause undesirable side effects, affecting regulatory approval, commercial potential or result in significant negative consequences following any potential marketing approval.
- Interim, "top line" and preliminary data from our clinical trials may be subject to change.

- The market opportunities for our product candidates may be smaller than we believe or approval we obtain may narrow our patient population.
- The costs and effort required to comply with regulatory requirements for marketing and commercializing our product candidates may be higher that we anticipated.
- We may face substantial competition.
- We may face risks related to strategic collaborations to develop our product candidates
- Our product candidates may fail to achieve market acceptance necessary for commercial success.
- We may face risks related to any potential international operations.
- We may be adversely affected by product liability lawsuits.
- Our insurance policies may be inadequate and potentially expose us to unrecoverable risks.

Risks Related to Regulatory Compliance

- We are subject to healthcare laws and regulations, which carry substantial penalties for noncompliance.
- Coverage and adequate reimbursement may not be available for our product candidates.
- Healthcare reform measures may have a negative impact on our business and results of operations.

Risks Related to Our Dependence on Third Parties

- Third-parties may conduct our preclinical studies and clinical trials in an unsatisfactory manner.
- We intend to rely on third parties to produce supplies of our product candidates.
- We and our third-party affiliates are subject to environmental, health and safety laws and regulations.

Risks Related to Our Intellectual Property

- Breaches of our license agreements may result in the adverse effects to our ability to continue the development and commercialization of our product candidates.
- Insufficient patent protection could allow our competitors to develop and commercialize products and technology similar or identical to ours.
- Obtaining and maintaining our patent rights is expensive, complicated and labor intensive and patent terms may be inadequate to protect our competitive position on our product candidates.
- We may be subject to claims alleging violations of intellectual property rights.
- Changes in patent law could impair our ability to protect our product candidates.
- We may be unable to protect our intellectual property rights.
- Intellectual property rights do not necessarily address all potential threats to our business.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

- We are highly dependent on the services of our current executive officers.
- We may experience difficulties resulting from the expansion of our organization.
- We may be subject to security breaches in our information technology systems.
- Our employees and the third-parties we deal with may engage in misconduct or improper activities.
- Our business may be adversely affected by the coronavirus outbreak.

Risks Related to Ownership of Our Common Stock

- The market price of our common stock is volatile and has fluctuated substantially.
- Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.
- We do not anticipate paying any cash dividends on our capital stock in the foreseeable future.

General Risk Factors

- We may be affected by unfavorable research or reports.
- We may use our cash and cash equivalents ineffectively or in ways with which you do not agree.
- We are subject to risks as an "emerging growth company" and a public company.
- We may avail ourselves of defensive and forum selection provisions in our governing documents and under Delaware law.

Risks Related to Our Financial Position and Capital Needs

We have incurred significant operating losses since inception and anticipate that we will continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

Since inception in January 2016, we have incurred significant operating losses. Our net loss was $119.8 million and $82.5 million for the years ended December 31, 2023, and 2022, respectively and $149.6 million and $82.1 million for the nine months ended September 30, 2024, and 2023, respectively. As of September 30, 2024, we had an accumulated deficit of $618.2 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. Since inception, we have devoted substantially all of our efforts to research and preclinical and clinical development of our product candidates, organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio and conducting clinical trials. To date, we have never obtained regulatory approval for, or commercialized, any drugs. It could be several years, if ever, before we have a commercialized drug. The net losses we incur may fluctuate significantly from quarter to quarter and year-to-year. We anticipate that our expenses will increase substantially if, and as, we:

- continue the ongoing and planned development of our product candidates;
- initiate, conduct and complete any ongoing, anticipated or future preclinical studies and clinical trials for our current and future product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- establish a sales, marketing, manufacturing and distribution infrastructure to commercialize any current or future product candidate for which we may obtain marketing approval;
- seek to discover and develop additional product candidates;
- continue to build a portfolio of product candidates through the acquisition or in-license of drugs, product candidates or technologies;
- maintain, protect and expand our intellectual property portfolio;
- meet the requirements and demands of being a public company;
- defend against any product liability claims or other lawsuits related to our products;
- hire additional clinical, regulatory and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Furthermore, we have incurred additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses.

To become and remain profitable, we must succeed in developing and eventually commercializing drugs that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials of our current and future product candidates, obtaining regulatory approval, procuring commercial-scale manufacturing, marketing and selling any products for which we obtain regulatory approval (including through third parties), as well as discovering or acquiring and developing additional product candidates. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are sufficient to offset our expenses and achieve profitability.

Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will be able to achieve profitability. If we are required by regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in the initiation and completion of our clinical trials or the development of any of our product candidates, our expenses could increase.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our common stock could also cause you to lose all or part of your investment.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are a clinical-stage company with limited operational history, and our operations to date have been largely focused on raising capital, organizing and staffing our company, identifying and developing our product candidates, and undertaking preclinical and clinical development for our product candidates. As an organization, we have not yet demonstrated an ability to successfully complete clinical development, obtain regulatory approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization, or arrange for a third party to conduct these activities on our behalf. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history.

We have encountered, and may continue to encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We will need to transition at some point from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Additionally, we expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

We will require substantial additional funding to finance our operations. If we are unable to raise capital when needed, we could be forced to delay, reduce or terminate certain of our development programs or other operations.

As of September 30, 2024, our cash and cash equivalents was $98.9 million. We believe that our cash and cash equivalents as of September 30, 2024, and the projected clinical and regulatory milestone payments expected in fiscal year 2025 from Advanz Pharma will be sufficient to fund our operations into 2026, and potentially beyond if drug approvals materialize in the expected timelines. However, we will need to obtain substantial additional funding in connection with our continuing operations and planned research and clinical development activities. Our future capital requirements will depend on many factors, including:

- the timing, progress and results of our ongoing preclinical studies and clinical trials of our product candidates;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials of other product candidates that we may pursue;
- our ability to establish collaborations on favorable terms, if at all;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, regulatory compliance, 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;
- the cost of any milestone and royalty payments with respect to any approved 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;
- the costs of operating as a public company; and
- the extent to which we acquire or in-license other product candidates and technologies.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of drugs that we do not expect to be commercially available for several years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. We may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

In addition, our ability to access additional capital has been affected by overall macroeconomic trends, among other things, including rising interest rates, which have caused the price of our common stock to fluctuate significantly and/or decline. These macroeconomic trends have been exacerbated by recent hostilities between Russia and Ukraine and between Hamas and Israel, which have contributed to further economic instability in the global financial markets. As a result, adequate additional financing may not be available to us when needed or on attractive terms. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce, or altogether terminate our research and development programs or future commercialization efforts.

Raising additional capital may cause dilution to our stockholders and 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 cash needs through public or private equity or debt financings, including through third party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest in our company may be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. Debt and equity financings, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as redeeming our shares, making investments, incurring additional debt, making capital expenditures, declaring dividends or placing limitations on our ability to acquire, sell or license intellectual property rights.

If we raise additional capital through future collaborations, strategic alliances or third party licensing arrangements, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional capital when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise develop and market ourselves.

Our ability to use our net operating losses to offset future taxable income may be subject to certain limitations.

We have incurred substantial losses since inception and do not expect to become profitable in the near future, if ever. In general, under Section 382 of the United States Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an “ownership change” is subject to limitations on its ability to utilize its pre-change NOLs to offset future taxable income. We may have experienced ownership changes in the past and may experience ownership changes in the future as a result of subsequent changes in our stock ownership (some of which shifts are outside our control). As a result, if, and to the extent that we earn net taxable income, our ability to use our pre-change NOLs to offset such taxable income may be subject to limitations.

For NOLs arising in tax years beginning after December 31, 2017, the Code limits a taxpayer's ability to utilize NOL carryforwards to 80% of taxable income. In addition, NOLs arising in tax years ending after December 31, 2017, can be carried forward indefinitely, but carryback is generally prohibited. NOLs generated in tax years beginning before January 1, 2018, will not be subject to the taxable income limitation, and NOLs generated in tax years ending before January 1, 2018, will continue to have a two-year carryback and 20-year carryforward period. Deferred tax assets for NOLs will need to be measured at the applicable tax rate in effect when the NOL is expected to be utilized. The limitations in the carryforward/carryback periods, as well as the limitation on use of NOLs for NOLs arising in tax years beginning after December 31, 2017, may significantly impact our ability to utilize our NOLs to offset taxable income in the future.

In order to realize the future tax benefits of our NOL carryforwards, we must generate taxable income, of which there is no assurance. Accordingly, we have provided a full valuation allowance for deferred tax assets as of September 30, 2024 and December 31, 2023.

Risks Related to the Development and Commercialization of Our Product Candidates

Our future success is substantially dependent on the successful clinical development, regulatory approval and commercialization of our product candidates. If we are not able to obtain required regulatory approvals, we will not be able to commercialize our product candidates and our ability to generate product revenue will be adversely affected.

We have invested a significant portion of our time and financial resources in the development of AT-007, AT-001 and AT-003. Our business is dependent on our ability to successfully complete development of, obtain regulatory approval for, and, if approved, successfully commercialize our product candidates in a timely manner. We may face unforeseen challenges in our drug development strategy, and we can provide no assurances that our drug design will prove to be effective, that we will be able to take advantage of expedited regulatory pathways for any of our product candidates, or that we will ultimately be successful in our future clinical trials.

We have not obtained regulatory approval for any product candidate, and it is possible that any product candidates we may seek to develop in the future will not obtain regulatory approval. Neither we nor any future collaborator is permitted to market any product candidates in the United States or abroad until we receive regulatory approval from the FDA or applicable foreign regulatory agency. The time required to obtain approval or other marketing authorizations by the FDA and comparable foreign regulatory authorities is unpredictable and typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the 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.

Prior to obtaining approval to commercialize any product candidate in the United States or abroad, we must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidate is safe and effective for its intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe that the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or it may object to elements of our clinical development program, requiring their alteration.

Of the large number of products in development across the pharmaceutical industry, only a small percentage successfully complete the FDA or comparable foreign regulatory authorities approval processes and are commercialized. The lengthy approval or marketing authorization process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval or marketing authorization to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

Even if we eventually complete clinical testing and receive approval of a new drug application, or NDA, or foreign marketing for our product candidates, the FDA or the comparable foreign regulatory authorities may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-market clinical trials. The FDA or the comparable foreign regulatory authorities also may approve or authorize for marketing a product candidate for a more limited indication or patient population than we originally request, and the FDA or comparable foreign regulatory authorities may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval or other marketing authorization would delay or prevent commercialization of that product candidate and would adversely impact our business and prospects.

In addition, the FDA or comparable foreign regulatory authorities may change their policies, adopt additional regulations or revise existing regulations or take other actions, which may prevent or delay approval of our future product candidates under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may have obtained.

Furthermore, even if we obtain regulatory approval for our product candidates, we will still need to develop a commercial organization, establish a commercially viable pricing structure and obtain approval for coverage and adequate reimbursement from third party and government payors, including government health administration authorities. If we are unable to successfully commercialize our product candidates, we may not be able to generate sufficient revenue to continue our business.

We or Advanz Pharma may fail to perform under any of the agreements entered into in connection with the partnership for commercialization of AT-007, which may subject us to liabilities, and we may fail to achieve anticipated benefits of the partnership.

In January 2023, we announced a partnership with Advanz Pharma (“Advanz”) for commercialization of AT-007 (govorestat) in Europe, and entered into an Exclusive License and Supply Agreement (the “Advanz Agreement”) with Advanz. Under the terms of the Advanz Agreement, Advanz receives exclusive commercial rights in the European Economic Area, Switzerland, and the UK (the “Territory”) for AT-007 in Galactosemia and SORD Deficiency, with certain rights to future indications for AT-007 in the Territory. In return, we have the right to receive certain near-term development milestone payments upon clinical trial completion and marketing authorization in Europe as well as commercial sales milestones, which in the aggregate are expected to amount to over €130 million. We also have the right to receive royalties on any future net sales of AT-007 in the Territory of 20%. The royalty rate will be payable on a country-by-country basis until the later of (i) the expiration of the licensed patents covering the composition of matter of AT-007, or (ii) 10 years after the European Medicines Agency’s grant of marketing authorization for AT-007. The royalties are subject to certain deductions, including certain secondary finishing costs, certain step-in establishment costs and a portion of fees for any potential third party patent licenses if applicable in the future. Following the initial term of the license, as described above, the royalty rate shall be reduced to 10% and shall continue in perpetuity unless the Advanz Agreement is terminated in various circumstances in accordance with its terms. We will continue to be responsible for the development, manufacturing and supply of AT-007, and Advanz will be responsible for packaging, distribution and commercialization in the Territory. The Advanz Agreement includes a sublicense under the 2016 Columbia Agreement and the 2020 Miami License Agreement.

Our partnership with Advanz is subject to various risks, including but not limited to the following:

- If Advanz breaches the contractual obligations owed to us pursuant to the Advanz Agreement or any related agreements, we could be exposed to commercial, regulatory or other liabilities.
- We may be subject to liabilities in connection with the development, manufacturing and supply of AT-007
- under the Advanz Agreement, including with respect to the 2016 Columbia Agreement and the 2020 Miami License Agreement.
- We may not be able to adequately protect our intellectual property or may become involved in intellectual property enforcement actions, which may cause us to incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights, and such litigation may divert the

attention of our management and scientific personnel and adversely affect our development and commercialization efforts.

•We may have limited control over the commercialization efforts of Advanz, and Advanz may fail to successfully sell and market AT-007.

•Our ability to receive certain economic benefits from the partnership, including the milestone payments and royalties, depends on certain contingencies beyond our control.

•In certain circumstances, Advanz may exercise certain step-in rights, including the ability for Advanz to perform its own supply arrangements, and in some cases, specified development rights in the Territory and assignment of certain contract rights. In all such circumstances, Advanz must continue to pay royalties and milestone payments, but may recoup certain of its manufacturing and development establishment costs, and deduct such costs from royalties owed.

Any of these factors could cause us to incur higher costs, disrupt the supply of our product candidates or approved products, delay the approval of our product candidates or prevent or disrupt the commercialization of our approved products. As a result, we may not achieve some or all economic benefits expected from the partnership.

The development of additional product candidates is risky and uncertain, and we can provide no assurances that we will be able to replicate our approach to drug development for other disease indications.

Efforts to identify, acquire or in-license, and then develop, product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our efforts may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development, approved products or commercial revenues for many reasons, including the following:

•the methodology used may not be successful in identifying potential product candidates;

•competitors may develop alternatives that render any product candidates we develop obsolete;

•any product candidates we develop may be covered by third parties' patents or other exclusive rights;

•a product candidate may 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 physicians, patients, the medical community or third party payors.

We have limited financial and management resources and, as a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater market potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in circumstances under which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. In addition, we may not be successful in replicating our approach to drug development for other disease indications. If we are unsuccessful in identifying and developing additional product candidates or are unable to do so, our business may be harmed.

Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials and we cannot assure you that any ongoing, planned or future clinical trials will lead to results sufficient for the necessary regulatory approvals.

Success in preclinical testing and earlier clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Preclinical studies and Phase 1 clinical trials are primarily designed to test safety, to study pharmacokinetics and pharmacodynamics and to understand the side effects of product candidates at various doses and schedules. Success in preclinical studies and earlier clinical trials does not ensure that later efficacy trials will be successful, nor does it predict

final results. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through earlier clinical trials.

In addition, the design of a clinical trial can determine whether its results will support approval of a product, and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. As an organization, we have limited experience designing clinical trials and may be unable to design and execute a clinical trial to support regulatory approval. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects.

Clinical drug development involves a lengthy and expensive process. We may incur additional costs and encounter substantial delays or difficulties in our clinical trials.

We may not commercialize, market, promote or sell any product candidate without obtaining marketing approval from the FDA or other comparable regulatory authority, and we may never receive such approvals. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans and will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome.

A failure of one or more clinical trials can occur at any stage of testing. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

We may experience numerous unforeseen events prior to, during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including the following:

- delays in reaching a consensus with regulatory authorities on the design or implementation of our clinical trials;
- regulators or institutional review boards, or IRBs, may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites or investigators;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, participants may drop out of these clinical trials at a higher rate than we anticipate or fail to return for post-treatment follow-up or we may fail to recruit suitable patients to participate in a trial;
- clinical trials of our product candidates may produce negative or inconclusive results;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event, concerns with a class of product candidates or after an inspection of our clinical trial operations, trial sites or manufacturing facilities;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; or
- we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenue from future drug sales or other sources. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional testing to bridge our modified product candidate to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates, if approved, or allow our competitors to bring competing drugs to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval, or not obtain marketing approval at all;
- obtain approval for indications or patient populations that are different than or are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including in the form of a risk evaluation and mitigation strategy, or REMS;
- be subject to additional post-marketing testing requirements;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the drug or impose restrictions on its distribution in the form of a modified REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Our product development costs will also increase if we experience delays in testing or obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, need to be restructured or be completed on schedule, if at all.

Further, we, the FDA or an IRB may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's current Good Clinical Practice, or GCP, regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our investigational new drug applications, or INDs, or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be negatively impacted, and our ability to generate revenues from our product candidates may be delayed.

All of our current product candidates that have proceeded to clinical trials target inhibition of aldose reductase. There can be no assurance that aldose reductase inhibitors will ever receive regulatory approval.

All of our current product candidates that have proceeded to clinical trials target inhibition of the aldose reductase enzyme. There are no currently approved aldose reductase inhibitors on the market outside of Japan, India and China, and there can be no assurance that aldose reductase inhibitors will ever receive regulatory approval in all other countries, including the United States. Prior attempts to inhibit this enzyme were hindered by nonselective, nonspecific inhibition, which resulted in limited efficacy and significant off-target safety effects. Our current product candidates, including AT-007, AT-001 and AT-003, may face similar or different challenges that prevent their successful commercialization.

We may not be able to obtain or maintain rare pediatric disease designation or exclusivity for our product candidates, which could limit the potential profitability of our product candidates.

We have obtained orphan drug designation and rare pediatric disease designation, from the FDA for AT-007 for the treatment of Galactosemia and PMM2-CDG. The FDA may grant orphan drug designation to a drug intended to treat

a rare disease or condition, which is defined as a disease or condition that either affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals, there is no reasonable expectation that sales of the drug in the United States will be sufficient to offset the costs of developing and making the drug available in the United States. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

For the purposes of the rare pediatric disease program, a "rare pediatric disease" is a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years or a rare disease or conditions within the meaning of the Orphan Drug Act. Under the FDA's rare pediatric disease priority review voucher, or RPD-PRV, program, upon the approval of an NDA for the treatment of a rare pediatric disease, the sponsor of such application would be eligible for an RPD-PRV that can be used to obtain priority review for a subsequent NDA. The sponsor of the application may transfer (including by sale) the RPD-PRV to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. Congress has extended the RPD-PRV program until December 20, 2024, with potential for vouchers to be granted until 2026. This program has been subject to criticism, including by the FDA. As such it is possible that even though we have obtained qualification for a RPD-PRV, the program may no longer be in effect at the time of approval. Also, although priority review vouchers may be sold or transferred to third parties, there is no guaranty that we will be able to realize any value if we obtained, and subsequently were able to sell a priority review voucher. The RPD-PRV program is currently scheduled to sunset as of September 30, 2026.

Even if we obtain a breakthrough therapy designation by the FDA for a product candidate may not lead to a faster development or regulatory review or approval process, and it would not increase the likelihood that the product candidate will receive marketing approval.

We may seek a breakthrough therapy designation for one or more product candidates. A breakthrough therapy is defined as a product candidate 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 product candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates 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. Product candidates designated as breakthrough therapies by the FDA are also eligible for priority review if supported by clinical data at the time of the submission of the NDA.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates 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 a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to product candidates considered for approval under conventional FDA procedures and it would not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product candidate no longer meets the conditions for qualification or it may decide that the time period for FDA review or approval will not be shortened.

We have sought, and may in the future seek, fast track designation from the FDA for our product candidates. Even if granted, fast track designation may not actually lead to a faster development, regulatory review or approval process.

If a product candidate is intended for the treatment of a serious or life-threatening condition and demonstrates the potential to address unmet needs for this condition, the sponsor may apply for FDA fast track designation. If fast track designation is obtained, the FDA may prioritize interactions with the sponsor concerning the designated development program and initiate review of sections of an NDA before the application is complete, known as "rolling review." Fast track designation would not ensure that we would experience a faster development, regulatory review or approval process compared to conventional FDA procedures or that we would ultimately obtain regulatory approval. Additionally, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

We intend to seek approval from the FDA through the use of accelerated registration pathways. If we are unable to obtain approval under an accelerated pathway, we may be required to conduct additional preclinical studies or clinical trials, which could increase the expense of obtaining, reduce the likelihood of obtaining and/or delay the timing of obtaining, necessary marketing approvals. Even if we receive approval from the FDA to utilize an accelerated registration pathway, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-marketing requirements, the FDA may seek to withdraw accelerated approval.

We intend to seek an accelerated approval development pathway for our product candidates. Under the accelerated approval provisions of the Federal Food, Drug, and Cosmetic Act, or the FDCA, and the FDA's implementing regulations, the FDA may grant accelerated approval to a product designed to treat a serious or life-threatening condition that provides meaningful therapeutic advantage over available therapies and demonstrates 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. 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 development 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 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 profile or risks and benefits for accelerated approval. The FDA may require that any such confirmatory studies be initiated or substantially underway prior to the submission of an application for accelerated approval. If such post-approval studies fail to confirm the drug's clinical profile or risks and benefits, the FDA may withdraw its approval of the drug. The Food and Drug Omnibus Reform Act, or FDORA, which was signed into law on December 29, 2022, made amendments to the accelerated approval program, among them new requirements relating to post approval studies and enhanced enforcement and withdrawal authorities for the FDA, including in the event that a sponsor fails to comply with applicable requirements under the program. Because we are still in early stages of our clinical trials, we can provide no assurances that our biomarker-based approach will be successful in demonstrating a causal link to the relevant outcomes we are evaluating. If our approach is not successful, we may be required to conduct longer clinical trials.

If we choose to pursue accelerated approval, we intend to seek feedback from the FDA or will otherwise evaluate our ability to seek and receive such accelerated approval. There can be no assurance that, after our evaluation of the feedback from the FDA or other factors, we will decide to pursue or submit an NDA for accelerated approval or any other form of expedited development, review or approval. Furthermore, even if we submit an application for accelerated approval, there can be no assurance that the application will be accepted or that approval will be granted on a timely basis, or at all. The FDA also could require us to conduct further studies or trials prior to considering our application or granting approval of any type. We might not be able to fulfill the FDA's requirements in a timely manner, which would cause delays, or approval might not be granted because our submission is deemed incomplete by the FDA. A failure to obtain accelerated approval or any other form of expedited development, review or approval for a product candidate would result in a longer time period to commercialize such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

Even if we receive accelerated approval from the FDA, we will be subject to rigorous post-marketing requirements, including the completion of confirmatory post-market clinical trial(s) to verify the clinical benefit of the product, and submission to the FDA of all promotional materials prior to their dissemination. The FDA could seek to withdraw accelerated approval for multiple reasons, including if we fail to conduct any required post-market study with due diligence, a post-market study does not confirm the predicted clinical benefit, other evidence shows that the product is not safe or effective under the conditions of use, or we disseminate promotional materials that are found by the FDA to be false or misleading.

A failure to obtain accelerated approval or any other form of expedited development, review or approval for a product candidate that we may choose to develop would result in a longer time period prior to commercializing such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be delayed, made more difficult or rendered impossible by multiple factors outside our control.

Identifying and qualifying patients to participate in our clinical trials is critical to our success. We may encounter difficulties in enrolling patients in our clinical trials, thereby delaying or preventing development and approval of our product candidates. Even once enrolled, we may be unable to retain a sufficient number of patients to complete any of our trials. Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, the existing body of safety and efficacy data, the number and nature of competing treatments and ongoing clinical trials of competing therapies for the same indication, the proximity of patients to clinical sites and the eligibility criteria for the trial. Because our focus includes rare disorders, there are limited patient pools from which to draw in order to complete our clinical trials in a timely and cost-effective manner. Accordingly, enrollment of our clinical trials could take significantly longer than projected, which would delay any potential approval of our product candidates. Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining enrollment of such patients in our clinical trials.

Our efforts to build relationships with patient communities may not succeed, which could result in delays in patient enrollment in our clinical trials. Any negative results we may report in clinical trials of our product candidates may make it difficult or impossible to recruit and retain patients in other clinical trials of that same product candidate. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our product candidates or could render further development impossible. In addition, we may rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will be limited in our ability to ensure their actual performance.

For additional information regarding delays in enrollment and retention of patients, see “Risks Related to Our Business Operations, Employee Matters and Managing Growth—*Our business may be adversely affected by the coronavirus outbreak*.”

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries and discomforts, to their doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions. Regulatory authorities may draw different conclusions or require additional testing to confirm these determinations, if they occur.

In addition, it is possible that as we test our product candidates in larger, longer and more extensive clinical trials, or as use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by subjects or patients. Many times, side effects are only detectable after investigational drugs are tested in large-scale pivotal trials or, in some cases, after they are made available to patients on a commercial scale after approval. If additional clinical experience indicates that any of our product candidates have side effects or cause serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked, which would harm our business, prospects, operating results and financial condition.

Interim, "top-line" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim, "top-line" 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 "top-line" data 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. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

The incidence and prevalence for target patient populations of our product candidates have not been established with precision. If the market opportunities for our product candidates are smaller than we believe they are or any approval we obtain is based on a narrower definition of the patient population, our business may suffer.

We currently focus our drug development on product candidates for the treatment of diseases with high unmet medical need. Our eligible patient population and pricing estimates may differ significantly from the actual market addressable by our product candidates. Our estimates of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on our beliefs and analyses. These estimates have been derived from a variety of sources, including the scientific literature, patient foundations or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of the diseases we are targeting. The number of patients may turn out to be lower than expected. Likewise, the potentially addressable patient population for each of our product candidates may be limited or may not be receptive to treatment with our product candidates, and new patients may become increasingly difficult to identify or access. If the market opportunities for our product candidates are smaller than we estimate, we may not be able to achieve our forecast revenue, which could hinder our business plan and adversely affect our business and results of operations.

We may face substantial competition, which may result in others developing or commercializing drugs before or more successfully than us.

The development and commercialization of new drugs is highly competitive. We may face potential competition with respect to our current product candidates and may face competition with respect to any other product candidates that we may seek to develop or commercialize in the future from pharmaceutical and biotechnology companies, academic institutions, government agencies and other public and private research institutions.

Our competitors may have an advantage over us due to their greater size, resources and institutional experience. In particular, these companies have greater experience and expertise in securing reimbursement, government contracts and relationships with key opinion leaders, conducting testing and clinical trials, obtaining and maintaining regulatory approvals and distribution relationships to market products and marketing approved drugs. These companies also have significantly greater research and marketing capabilities than we do. If we are not able to compete effectively against existing and potential competitors, our business and financial condition may be harmed.

As a result of these factors, our competitors may obtain regulatory approval of their drugs before we are able to, which may limit our ability to develop or commercialize our product candidates. Our competitors may also develop therapies that are safer, more effective, more widely accepted or less expensive than ours, and may also be more successful than we are in manufacturing and marketing their drugs. These advantages could render our product candidates obsolete or non-competitive before we can recover the costs of such product candidates' development and commercialization.

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 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, management and commercial personnel,

establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

We may explore strategic collaborations that may never materialize or we may be required to relinquish important rights to and control over the development and commercialization of our product candidates to any future collaborators.

Over time, our business strategy includes acquiring or in-licensing additional product candidates for treatments of diseases with high unmet medical need. As a result, we intend to periodically explore a variety of possible strategic collaborations in an effort to gain access to additional product candidates or resources. These strategic collaborations may include partnerships with large strategic partners, particularly for the development of DPN treatments using AT-001. At the current time however, we cannot predict what form such a strategic collaboration might take. We are likely to face significant competition in seeking appropriate strategic collaborators, and strategic collaborations can be complicated and time consuming to negotiate and document. We may not be able to negotiate strategic collaborations on acceptable terms, or at all. We are unable to predict when, if ever, we will enter into any strategic collaborations because of the numerous risks and uncertainties associated with establishing them.

Future collaborations could subject us to a number of risks, including:

- we may be required to undertake the expenditure of substantial operational, financial and management resources;
- we may be required to issue equity securities that would dilute our stockholders' percentage ownership of our company;
- we may be required to assume substantial actual or contingent liabilities;
- we may not be able to control the amount and timing of resources that our strategic collaborators devote to the development or commercialization of our product candidates;
- strategic collaborators may select indications or design clinical trials in a way that may be less successful than if we were doing so;
- strategic collaborators may delay clinical trials, provide insufficient funding, terminate a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new version of a product candidate for clinical testing;
- strategic collaborators may not pursue further development and commercialization of products resulting from the strategic collaboration arrangement or may elect to discontinue research and development programs;
- strategic collaborators may not commit adequate resources to the marketing and distribution of our product candidates, limiting our potential revenues from these products;
- disputes may arise between us and our strategic collaborators that result in the delay or termination of the research, development or commercialization of our product candidates or that result in costly litigation or arbitration that diverts management's attention and consumes resources;
- strategic collaborators may experience financial difficulties;
- strategic collaborators may not properly maintain, enforce or defend our intellectual property rights or may use our proprietary information in a manner that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- business combinations or significant changes in a strategic collaborator's business strategy may adversely affect a strategic collaborator's willingness or ability to complete its obligations under any arrangement;
- strategic collaborators could decide to move forward with a competing product candidate developed either independently or in collaboration with others, including our competitors; and
- strategic collaborators could terminate the arrangement or allow it to expire, which would delay the development and may increase the cost of developing our product candidates.

Even if any product candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third party payors or others in the medical community necessary for commercial success.

Even if any product candidates receive marketing approval, they may fail to gain market acceptance by physicians, patients, third party payors and others in the medical community. If such product candidates do not achieve an adequate level of acceptance, we may not generate significant drug revenue and may not become profitable. The degree of market acceptance of any product candidate, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the convenience and ease of administration compared to alternative treatments and therapies;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the efficacy and potential advantages compared to alternative treatments and therapies;
- the effectiveness of sales and marketing efforts;
- the strength of our relationships with patient communities;
- the cost of treatment in relation to alternative treatments and therapies, including any similar generic treatments;
- our ability to offer such drug for sale at competitive prices;
- the strength of marketing and distribution support;
- the availability of third party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of the drug together with other medications.

Our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our product candidates. Because we expect sales of our product candidates, if approved, to generate substantially all of our revenues for the foreseeable future, the failure of our product candidates to find market acceptance would harm our business.

Even if we obtain regulatory approvals for our product candidates, they will remain subject to ongoing regulatory oversight.

Even if we obtain regulatory approvals for our product candidates, such approvals will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 trials, and surveillance to monitor the quality, safety and efficacy of the drug. Such regulatory requirements may differ from country to country depending on where we have received regulatory approval.

In addition, drug manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practices, or cGMP, requirements and adherence to commitments made in the NDA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a drug, such as adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured or if a regulatory authority disagrees with the promotion, marketing or labeling of that drug, a regulatory authority may impose restrictions relative to that drug, the manufacturing facility or us, including requesting a recall or requiring withdrawal of the drug from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of our product candidates, a regulatory authority may, among other actions:

- issue an untitled letter or warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;

- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending NDA or comparable foreign marketing application or any supplements thereto submitted by us or our partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

Moreover, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant civil, criminal and administrative penalties.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and harm our business.

Further, if we are not able to maintain regulatory compliance with the FDCA, Cures Act, or other applicable requirements, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our vaccine candidates. For example, on June 28, 2024, the U.S. Supreme Court, in *Loper Bright Enterprises v. Raimondo,* overturned long-standing precedent regarding the deference courts owe to agencies' interpretation of ambiguous statutes in their rulemaking. While the impact of the *Loper Bright* decision on our business and regulatory strategy is unknown, the decision generally may, among other things, increase the frequency of challenges to decisions and rulemaking of health regulators, including FDA determinations of drug approval and market exclusivity and the Centers for Medicare & Medicaid Services' rules regarding reimbursement, and also impact the speed at which such health regulators make decisions and issue regulations.

In addition, we cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the U.S. administration may impact our business and industry. It is difficult to predict how executive actions, including executive orders, may be implemented and the extent to which they will affect the FDA's ability to exercise its regulatory authority. If legislative, administrative or executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be successful in commercializing them, if and when they are approved.

To successfully commercialize any product candidate that may result from our development programs, we will need to build out our sales and marketing capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any product candidate we may develop will be expensive and time-consuming and could delay any drug launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may seek to enter into collaborations with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current or future collaborators do not commit sufficient resources to commercialize our product candidates, or we are unable to develop the necessary capabilities on our own, we may be unable to generate sufficient revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We will likely also face competition if we seek third parties to assist us with the sales and marketing

efforts of our product candidates. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval outside the United States, which would limit our market opportunities.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable foreign regulatory authorities also must 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 more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any product candidates, if approved, is also subject to approval. Obtaining approval for our product candidates in the European Union from the European Commission following the opinion of the European Medicines Agency, or the EMA, is a lengthy and expensive process. Even if a product candidate is approved, the EMA may limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. 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 product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for our product candidates may be withdrawn. If we fail to comply with the applicable regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects could be harmed.

If we commercialize our product candidates outside the United States, a variety of risks associated with international operations could harm our business.

We intend to seek approval to market our product candidates outside the United States, and may do so for future product candidates. If we market approved products outside the United States, we expect that we will be subject to additional risks in commercialization, including:

- different regulatory requirements for approval of therapies in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, international hostilities, or natural disasters including earthquakes, typhoons, floods and fires.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by many of the individual countries in which we may operate, with which we will need to comply.

Many biopharmaceutical companies have found the process of marketing their products in foreign countries to be challenging.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidate that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and may face an even greater risk if we commercialize any product candidate that we may develop. If we cannot successfully defend ourselves against claims that any such product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidate that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- increased insurance costs;
- the inability to commercialize any product candidate that we may develop; and
- injury to our reputation and significant negative media attention.

Any such outcomes could negatively impact our business, financial condition, results of operations and prospects.

Our insurance policies may be inadequate and potentially expose us to unrecoverable risks.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidate. Insurance availability, coverage terms and pricing continue to vary with market conditions. We endeavor to obtain appropriate insurance coverage for insurable risks that we identify; however, we may fail to correctly anticipate or quantify insurable risks, we may not be able to obtain appropriate insurance coverage and insurers may not respond as we intend to cover insurable events that may occur. We have observed rapidly changing conditions in the insurance markets relating to nearly all areas of traditional corporate insurance. Such conditions have resulted in higher premium costs, higher policy deductibles, and lower coverage limits. For some risks, we may not have or maintain insurance coverage because of cost or availability.

Risks Related to Regulatory Compliance

Our relationships with customers, physicians, and third party payors are subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Healthcare providers, physicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors subject us to various federal and state fraud and abuse laws and other healthcare laws, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims laws and the law commonly referred to as the Physician Payments Sunshine Act and regulations.

Ensuring that our business arrangements with third-parties comply with applicable healthcare laws and regulations will likely be costly. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative

penalties, damages, fines, disgorgement, imprisonment, exclusion from participating in government-funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of noncompliance with these laws, contractual damages, reputational harm and the curtailment or restructuring of our operations.

If the physicians or other providers or entities with whom we expect to do business are found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs. Even if resolved in our favor, litigation or other legal proceedings relating to healthcare laws and regulations 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. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common shares. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development, manufacturing, sales, marketing or distribution activities. Uncertainties resulting from the initiation and continuation of litigation or other proceedings relating to applicable healthcare laws and regulations could have an adverse effect on our ability to compete in the marketplace.

Coverage and adequate reimbursement may not be available for our product candidates, which could make it difficult for us to sell profitably, if approved.

Market acceptance and sales of any product candidates that we commercialize, if approved, will depend in part on the extent to which reimbursement for these drugs and related treatments will be available from third-party payors, including government health administration authorities, managed care organizations and other private health insurers. Third-party payors decide which therapies they will pay for and establish reimbursement levels. While no uniform policy for coverage and reimbursement exists in the United States, third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any product candidates that we develop will be made on a payor-by-payor basis. Therefore, one payor's determination to provide coverage for a drug does not assure that other payors will also provide coverage, and adequate reimbursement, for the drug. Additionally, a third-party payor's decision to provide coverage for a therapy does not imply that an adequate reimbursement rate will be approved. Each payor determines whether or not it will provide coverage for a therapy, what amount it will pay the manufacturer for the therapy, and on what tier of its formulary it will be placed. The position on a payor's list of covered drugs, or formulary, generally determines the co-payment that a patient will need to make to obtain the therapy and can strongly influence the adoption of such therapy by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our product candidates, if approved, unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products.

Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for any drug that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for, or the price of, any drug for which we obtain marketing approval. If coverage and adequate reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize any product candidates that we develop.

Healthcare reform measures may have a negative impact on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes, as well as judicial challenges, regarding the healthcare system that could prevent or delay marketing approval of product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, since 2003, Medicare coverage for drug purchases by the elderly have been subject to a reimbursement methodology based on average sales prices for physician-administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

Further, in March 2010, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or, collectively, the PPACA, was passed, which substantially changed the way healthcare is financed by both governmental and private payors in the United States. Some of the provisions of the PPACA have yet to be fully implemented, while certain provisions have been subject to executive, judicial and Congressional challenges. For example, the Tax Cuts and Jobs Act of 2017, or Tax Act includes a provision that repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year, which is commonly referred to as the “individual mandate.”. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or Texas District Court Judge, ruled that the individual mandate was a critical and inseverable feature of the PPACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the PPACA were invalid as well. The U.S. Supreme Court heard arguments in the case in November 2020 and issued its decision in June 2021, ruling that the plaintiffs lacked the standing to challenge the individual mandate provision. In so holding, the Supreme Court did not consider larger constitutional questions about the validity of this provision or the validity of the PPACA in its entirety. Another case challenging the PPACA’s requirement that insurers cover certain preventative services is currently pending before the same U.S. District Court Judge in the Northern District of Texas who ruled against the individual mandate in 2018. In September 2022, the judge held that the PPACA requirement to cover certain preventative services violated the U.S. Constitution and set a schedule for additional briefing, which was completed in January 2023. In March 2023, the judge struck down the requirement with immediate nationwide effect by ruling, in part, that members of a panel charged under the PPACA with recommending preventative services coverage had been appointed in an unconstitutional manner. Parties on both sides of the lawsuit appealed this ruling, and in June 2024 the U.S. Court of Appeals for the Fifth Circuit (Fifth Circuit) held, among other things, that the PPACA’s requirement that group health plans and health insurance issuers cover certain preventative services without cost-sharing is unconstitutional. The Fifth Circuit also reversed the district court’s nationwide injunction on procedural grounds and remanded the case to the district court for additional consideration. The parties have petitioned to appeal the case to the U.S. Supreme Court. It is unclear how this decision, subsequent decisions and appeals, or any potential future litigation and other efforts to repeal and replace the PPACA will impact the PPACA. Congress may consider additional legislation to repeal or repeal and replace other elements of the PPACA. We continue to evaluate the effect that the PPACA and its possible repeal and replacement may have on our business and the potential profitability of our product candidates.

Other legislative changes have been proposed and adopted since the PPACA was enacted. These changes include aggregate reductions to Medicare payments to providers of 2% per fiscal year pursuant to the Budget Control Act of 2011, which began in 2013, and due to subsequent legislative amendments to the statute, including the BBA, which will remain in effect through 2027 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Additional changes that may affect our business include the expansion of programs such as Medicare payment for performance initiatives for physicians under the Medicare Access and CHIP Reauthorization Act of 2015. At this time, the full impact of the introduction of the Medicare quality payment program on overall physician reimbursement is unclear.

Further, in the United States there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several 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 government payor programs, and review the relationship between pricing and manufacturer patient programs. While some of the proposed measures will require authorization through additional legislation to become effective, the U.S. Congress have indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. For example, the Inflation Reduction Act of 2022 also seeks to reduce prescription drug costs by, among other provisions, allowing Medicare to negotiate prices for certain high-cost prescription drugs in Medicare Parts B and D, imposing an excise tax on pharmaceutical manufacturers that refuse to negotiate pricing with Medicare, requiring inflation rebates to limit annual drug price increases in Medicare, redesigning the Medicare Part D formula, and limiting cost-sharing for insulin products. It is unclear whether additional drug pricing and other healthcare reform measures will be enacted, and if enacted, what effect they would have on our business. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our current or any future product candidates or additional pricing pressures. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing or new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our current or any future product candidates we may develop may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Further, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, or the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

We expect that these and other 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 drug, which could have an adverse effect on demand for our product candidates if they are approved. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our product candidates.

Risks Related to Our Dependence on Third Parties

We intend to rely on third parties to produce clinical and commercial supplies of our product candidates.

We do not own or operate facilities for drug manufacturing, storage and distribution, or testing. We are dependent on third parties to manufacture the clinical supplies of our current and any future product candidates. The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the FDA pursuant to inspections that will be conducted after we submit our NDA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the cGMP requirements, for manufacture of both active drug substance and finished drug product. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, we will not be able to secure and/or maintain regulatory approval for our product candidates. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA 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 would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Any significant delay in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates.

We also intend to rely on third-party manufacturers to supply us with sufficient quantities of our product candidates to be used, if approved, for commercialization. We do not yet have a commercial supply agreement for commercial quantities of drug substance or drug product. If we are not able to meet market demand for any approved product, it would negatively impact our ability to generate revenue, harm our reputation, and could have an adverse effect on our business and financial condition.

Further, our reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured product candidates ourselves, including:

- inability to meet our product specifications and quality requirements consistently;
- delay or inability to procure or expand sufficient manufacturing capacity;
- issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- our third party manufacturers may not be able to execute our manufacturing procedures and other logistical support requirements appropriately;
- our third party manufacturers may fail to comply with cGMP or other requirements or inspections by the FDA or other comparable regulatory authorities;
- our inability to negotiate manufacturing agreements with third parties under commercially reasonable terms, if at all;
- breach, termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- reliance on single sources for drug components;
- lack of qualified backup suppliers for those components that are currently purchased from a sole or single-source supplier;
- our third party manufacturers may not devote sufficient resources to our product candidates;
- we may not own, or may have to share, the intellectual property rights to any improvements made by our third party manufacturers in the manufacturing process for our product candidates;
- operations of our third party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier; and
- carrier disruptions or increased costs that are beyond our control.

In addition, if we enter into a strategic collaboration with a third party for the commercialization of our current or any future product candidates, we will not be able to control the amount of time or resources that they devote to such efforts. If any strategic collaborator does not commit adequate resources to the marketing and distribution of our product candidates, it could limit our potential revenues.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize our current or any future product candidates once approved. Some of these events could be the basis for FDA action, including injunction, request for recall, seizure, or total or partial suspension of production.

Our business involves the use of hazardous materials and we and our third-party manufacturers and suppliers must comply with environmental, health and safety laws and regulations, which can be expensive and restrict how we do, or interrupt our, business.

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the generation, storage, use and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds and wastes. We and our manufacturers and suppliers are subject to environmental, health and safety laws and regulations governing, among other matters, the use, manufacture, generation, storage, handling, transportation, discharge and disposal of these hazardous materials and wastes and worker health and safety. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination or injury, which could result in an interruption of our commercialization efforts, research and development efforts and business operations, damages and significant cleanup costs and liabilities under applicable environmental, health and safety laws and regulations. We also cannot guarantee that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials and wastes generally comply with the standards prescribed by these laws and regulations. We may be held liable for any resulting damages, costs or liabilities, which could exceed our resources, and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental, health and safety laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. Failure to comply with these environmental, health and safety laws and regulations may result in substantial fines, penalties or other sanctions. We do not currently carry environmental insurance coverage.

We rely on third-parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We do not currently have the ability to independently conduct any clinical trials. We intend to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our preclinical studies and clinical trials, and we expect to have limited influence over their actual performance. We rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future preclinical studies. We expect to control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs are required to comply with the good laboratory practices, or GLPs, and GCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities in the form of International Conference on Harmonization guidelines for any of our product candidates that are in preclinical and clinical development. Regulatory authorities enforce GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. Although we rely on CROs to conduct GCP-compliant clinical trials, we remain responsible for ensuring that each of our GLP preclinical studies and clinical trials is conducted in accordance with its investigational plan and protocol and applicable laws and regulations. If we or our CROs fail to comply with GCPs, the clinical data generated in our clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the regulatory approval process.

Our reliance on third parties to conduct clinical trials will result in less direct control over the management of data developed through clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with CROs and other third parties can be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Such parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience legal or regulatory compliance issues; or
- undergo changes in priorities or become financially distressed.

These factors may adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or fail to comply with regulatory requirements, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize, any product candidate that we develop. As a result, our financial results and the commercial prospects for any product candidate that we develop would be harmed, our costs could increase, and our ability to generate revenue could be delayed. While we will have agreements governing their activities, our CROs will not be our employees, and we will not control whether or not they devote sufficient time and resources to our future clinical and preclinical programs. These CROs 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 harm our business. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology.

If our relationship with any of these CROs terminates, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can negatively impact our ability to meet our desired clinical development timelines. While we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a negative impact on our business, financial condition and prospects.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of our product candidates.

Risks Related to Our Intellectual Property

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. If we breach our license agreements with Columbia University or any of the other agreements under which we acquired, or will acquire, the intellectual property rights to our product candidates, we could lose the ability to continue the development and commercialization of the related product.

The licensing of intellectual property is of critical importance to our business and to our current and future product candidates, and we expect to enter into additional such agreements in the future. In particular, our current product candidates AT-007, AT-001 and AT-003 are dependent on our license agreement with The Trustees of Columbia University in the City of New York, or Columbia University. Pursuant to that license agreement with Columbia University, or the 2016 Columbia Agreement, Columbia University granted us an exclusive license under two important patent families, and a nonexclusive license to certain know-how, owned by Columbia University to develop, manufacture or commercialize certain compounds, including AT-001, AT-003 and AT-007, for the diagnosis and treatment of human and animal diseases and conditions. The license grant is worldwide, with the exception of the patent family that covers AT-001 and AT-003. The license grant for the patent family that covers AT-001 and AT-003 excludes patent rights in China, Taiwan, Hong Kong and Macao, which Columbia University has exclusively licensed to a third party. We cannot prevent Columbia University's third party licensee from developing, manufacturing or commercializing certain compounds, including AT-001 and AT-003, but not including AT-007, in China, Taiwan, Hong Kong and Macao, and we cannot develop, manufacture or commercialize AT-001 or AT-003 in these territories, which could have a negative effect on our business.

We do not have the right to control the preparation, filing, prosecution and maintenance of patents and patent applications covering the technology that we license under either of the Columbia Agreements. Therefore, we cannot always be certain that these patents and patent applications will be prepared, filed, prosecuted and maintained in a manner consistent with the best interests of our business. Although we have a right to have our comments considered in connection with the prosecution process, if Columbia University fails to prosecute and maintain such patents, or loses rights to those patents or patent applications as a result of its control of the prosecution activities, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected.

If we fail to meet our obligations under the Columbia Agreement in any material respect and fail to cure such breach in a timely fashion, then Columbia University may terminate the Columbia Agreement. If the Columbia Agreement is terminated, and we lose our intellectual property rights under the Columbia Agreement, this may result in complete termination of our product development and any commercialization efforts for the product candidates that are subject to the agreement, including AT-007, AT-001, and AT-003. While we would expect to exercise all rights and remedies available to us, including seeking to cure any breach by us, and otherwise seek to preserve our rights under the Columbia Agreement, we may not be able to do so in a timely manner, at an acceptable cost or at all.

Furthermore, license agreements we enter into in the future may not provide exclusive rights to use intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses.

If we are unable to obtain and maintain patent protection for our product candidates and technology, or if the scope of the patent protection obtained is not sufficiently broad or robust, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our product candidates and technology 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 and our technology. We and our licensors have sought, and intend to seek, to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates and our technology that are important to our business.

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 patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we or our licensors were the first to file a patent application relating to any particular aspect of a product candidate. Furthermore, if third parties have filed such patent applications, an interference proceeding in the United States can be initiated by such third party, or by the U.S. Patent and Trademark Office, or USPTO, itself, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. 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.

We or our licensors have not pursued or maintained, and may not pursue or maintain in the future, patent protection for our product candidates in every country or territory in which we may sell our products, if approved. 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. Consequently, we may not be able to prevent third parties from infringing our patents in all countries outside the United States, or from selling or importing products that infringe our patents in and into the United States or other jurisdictions.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if the patent applications we license or own do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. 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 intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Furthermore, our owned and in-licensed patents may be subject to a reservation of rights by one or more third parties. For example, the research resulting in certain of our owned and in-licensed patent rights and technology was funded in part by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a nonexclusive license authorizing the government to use the invention for noncommercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

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

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. In addition, periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or patent applications will have to be paid to the USPTO and various government patent agencies outside the United States over the lifetime of our owned and licensed patents and/or applications and any patent rights we may own or license in the future. We rely on our service providers or our licensors to pay these fees. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. 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, nonpayment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our products or technologies, we may not be able to stop a competitor from marketing products that are the same as or similar to our product candidates, which would have an adverse effect on our business. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could harm our business.

In addition, if we fail to apply for applicable patent term extensions or adjustments, we will have a more limited time during which we can enforce our granted patent rights. In addition, if we are responsible for patent prosecution and maintenance of patent rights in-licensed to us, any of the foregoing could expose us to liability to the applicable patent owner.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Given the amount of time required for the development, testing and regulatory review of product candidates such as AT-007, AT-001, and AT-003, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we have or will obtain patent rights. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent; provided that the patent is not enforceable for more than 14 years from the date of drug approval, which is limited to the approved indication (or any additional indications approved during the period of extension). Furthermore, only one patent per approved product can be extended and only those claims covering the approved product, a method for using it or a method for manufacturing it may be extended. However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their drug earlier than might otherwise be the case.

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

Our commercial success depends, in part, upon our ability and the ability of others with whom we may collaborate to develop, manufacture, market and sell our current and any future product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and any future product candidates and technology, including interference proceedings, post grant review and inter partes review before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could have a negative impact on our ability to commercialize our current and any future product candidates. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this is a high burden and requires us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Moreover, given the vast number of patents in our field of technology, we cannot be certain that we do not infringe existing patents or that we will not infringe patents that may be granted in the future. Other companies and research institutions have filed, and may file in the future, patent applications related to AR inhibitors and their therapeutic use. Some of these patent applications have already been allowed or issued, and others may issue in the future. While we may decide to initiate proceedings to challenge the validity of these or other patents in the future, we may be unsuccessful, and courts or patent offices in the United States and abroad could uphold the validity of any such patent. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our product candidates. Regardless of when filed, we may fail to identify relevant third-party patents or patent applications, or we may incorrectly conclude that a third-party patent is invalid or not infringed by our product candidates or activities. If a patent holder believes that our product candidate infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from

nonpracticing entities that have no relevant drug revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the drug or product candidate that is the subject of the actual or threatened suit.

If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing our product candidate(s) and technology. Under any such license, we would most likely be required to pay various types of fees, milestones, royalties or other amounts. Moreover, we may not be able to obtain any required license on commercially reasonable terms or at all.

The licensing or acquisition of third-party intellectual property rights is a competitive area, and more established companies may also pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have an adverse effect on our business, financial condition, results of operations and prospects. Furthermore, even if we were able to obtain a license, it could be nonexclusive, thereby giving our competitors and other third-parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidate. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. We may be required to indemnify collaborators or contractors against such claims. A finding of infringement could prevent us from manufacturing and commercializing our current or any future product candidates or force us to cease some or all of our business operations, which could harm our business. Even if we are successful in defending against such claims, litigation can be expensive and time-consuming and would divert management's attention from our core business. 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. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common stock.

Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. 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.

In addition, we may in the future be subject to claims by our former employees or consultants asserting an ownership right in our patents or patent applications, as a result of the work they performed on our behalf. Although it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in

executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own, and we cannot be certain that our agreements with such parties will be upheld in the face of a potential challenge or that they will not be breached, for which we may not have an adequate remedy. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

We may be involved in lawsuits to protect or enforce our patents, the patents of our licensors or our other intellectual property rights, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe, misappropriate or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming and are likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is 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 or defense proceedings could put one or more of our owned or licensed patents at risk of being invalidated or interpreted narrowly and could put our owned or licensed patent applications at risk of not issuing. The initiation of a claim against a third party might also cause the third-party to bring counterclaims against us, such as claims asserting that our patent rights are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is or will be no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future product candidates. Such a loss of patent protection could harm our business.

We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license, or if the license offered as a result is not on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

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. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have an adverse effect on the price of our common stock.

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 and more mature and developed intellectual property portfolios. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating or from successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have an adverse effect on our ability to compete in the marketplace.

Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our current and any future product candidates.

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, or the America Invents Act, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third-party was the first to invent the claimed invention. The America Invents Act also includes a number of significant changes that affect the way patent applications are prosecuted and also may affect patent litigation. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO-administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have an adverse effect on our business, financial condition, results of operations, and prospects.

In addition, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce patents that we own, have licensed or might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions, changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we own or have licensed or that we may obtain in the future.

We may not be able to protect our intellectual property rights throughout the world, which could negatively impact our business.

Filing, prosecuting and defending patents covering our current and any future product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we or our licensors have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain patent protection but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents, and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so 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 protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our 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 and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Since we rely on third parties to help us discover, develop and manufacture our current and any future product candidates, or if we collaborate with third parties for the development, manufacturing or commercialization of our current or any future product candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Moreover, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary technology and processes. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators, or others is inadvertently disclosed or subject to a breach or violation, we may be exposed to liability to the owner of that confidential information. Enforcing a claim that a third-party illegally or unlawfully obtained and is using our trade secrets, like patent litigation, is expensive and time-consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets.

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

In addition to seeking patent and trademark protection for 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 our trade secrets, in part, by entering into nondisclosure 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, advisors and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets. Monitoring unauthorized uses and disclosures of our intellectual property is difficult, and we do not know whether the steps we have taken to protect our intellectual property will be effective. In addition, we may not be able to obtain adequate remedies for any such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

Moreover, our competitors may independently develop knowledge, methods and know-how equivalent to our trade secrets. Competitors could purchase our products and replicate some or all of the competitive advantages we derive from our development efforts for technologies on which we do not have patent protection. 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, or those to whom they communicate it, 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 also seek to preserve the integrity and confidentiality of our data and other confidential information by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and detecting the disclosure or misappropriation of confidential information and enforcing a claim that a party illegally disclosed or misappropriated confidential information is difficult, expensive and time-consuming, and the outcome is unpredictable. Further, we may not be able to obtain adequate remedies for any breach. In addition, our confidential information may otherwise become known or be independently discovered by competitors, in which case we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us.

Any trademarks we may obtain may be infringed or successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish any of our product candidates that are approved for marketing from the products of our competitors. We have selected trademarks for some of our later stage product candidates and filed applications to register those trademarks for our current or any future product candidates. For other earlier stage product candidates, we have not yet selected trademarks or begun the process of applying to register trademarks. Our pending trademark applications, and any future trademark applications may not be approved. Third parties may oppose our trademark applications or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our products, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks, and we may not have adequate resources to enforce our trademarks.

In addition, any proprietary name we propose to use with our current or any other product candidate in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of the potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable proprietary product name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

Intellectual property rights do not necessarily address all potential threats to our business.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business. The following examples are illustrative:

- others may be able to make compounds or formulations that are similar to our product candidates but that are not covered by the claims of any patents, should they issue, that we own or license;
- we or our licensors might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or license;
- we or our licensors might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or license may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities,

as well as in countries where we do not have patent rights, and then use the information learned from such activities to develop competitive drugs for sale in our major commercial markets;

•we may not develop additional proprietary technologies that are patentable; and

•the patents of others may have an adverse effect on our business.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

We are highly dependent on the services of our Chief Executive Officer and Chairman, Dr. Shoshana Shendelman, and our Chief Medical Officer, Dr. Riccardo Perfetti, and if we are not able to retain these members of our management team or recruit and retain additional management, clinical and scientific personnel, our business will be harmed.

We are highly dependent on our Chief Executive Officer and Chairman, Dr. Shoshana Shendelman, and our Chief Medical Officer, Dr. Riccardo Perfetti. Each of them may currently terminate their employment with us at any time. The loss of the services of either of these persons could impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining other senior executives, qualified scientific and clinical personnel and, if we progress the development of any of our product candidates, commercialization, manufacturing and sales and marketing personnel, will be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize our product candidates. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high-quality personnel, our ability to pursue our growth strategy will be limited.

Our future performance will also depend, in part, on our ability to successfully integrate newly hired executive officers into our management team and our ability to develop an effective working relationship among senior management. Our failure to integrate these individuals and create effective working relationships among them and other members of management could result in inefficiencies in the development and commercialization of our product candidates, harming future regulatory approvals, sales of our product candidates and our results of operations. Additionally, we do not currently maintain “key person” life insurance on the lives of our executives or any of our employees.

We expect to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

As of September 30, 2024, we had 37 full-time employees. As the clinical development of our product candidates progresses, we also expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of research, drug development, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities, and continue to recruit and train additional qualified personnel. 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 of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a significant disruption of our product development programs and our ability to operate our business effectively, and adversely affect our business and operating results.

Our internal computer systems, cloud-based computing services and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage or interruption from computer viruses, data corruption, cyber-based attacks (including phishing attempts, denial of service attacks, and malware or ransomware incidents, attacks enhanced or facilitated by artificial intelligence), unauthorized access, natural disasters, terrorism, war, international hostilities and telecommunication and electrical failures. While we have not experienced any significant system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Furthermore, federal, state and international laws and regulations, such as the European Union's General Data Protection Regulation, which took effect in May 2018, can expose us to enforcement actions and investigations by regulatory authorities, and potentially result in regulatory penalties and significant legal liability, if our information technology security efforts fail. In addition, our software systems include cloud-based applications that are hosted by third-party service providers with security and information technology systems subject to similar risks. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed.

Risks of unauthorized access and cyber-attacks have increased as most of our personnel, and the personnel of many third-parties with which we do business, have adopted remote working arrangements as a result of the Covid-19 pandemic, and may increase as a result of recent hostilities between Russia and Ukraine. Improper or inadvertent employee behavior, including data privacy breaches by employees, contractors and others with permitted access to our systems, may also pose a risk that sensitive data may be exposed to unauthorized persons or to the public. If a system failure or security breach occurs and interrupts our operations or the operations at one of our third-party vendors, it could result in intellectual property and other proprietary or confidential information being lost or stolen or a material disruption of our drug development programs. For example, the loss of clinical trial data from ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, loss of trade secrets or inappropriate disclosure of confidential or proprietary information, including protected health information or personal data of employees or former employees, access to our clinical data, or disruption of the manufacturing process, we could incur liability and the further development of our drug candidates could be delayed. We may also be vulnerable to cyber-attacks by hackers or other malfeasance. This type of breach of our cybersecurity may compromise our confidential information and/or our financial information and adversely affect our business or reputation or result in legal or regulatory proceedings. In addition, if a ransomware attack or other cyber-attack occurs, either internally or at our third-party vendors, we could be prevented from accessing our systems or data, which may cause interruptions or delays in our business, cause us to incur remediation costs or subject us to demands to pay ransom, or adversely affect our business reputation.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in other jurisdictions, provide accurate information to the FDA and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs, and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of

clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. 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 government investigations or other actions or lawsuits stemming from a failure to comply with these 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 result in significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participating in government-funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of noncompliance with these laws, contractual damages, reputational harm and the curtailment or restructuring of our operations, any of which could have a negative impact on our business, financial condition, results of operations and prospects.

Any future acquisitions or strategic collaborations may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and/or subject us to other risks.

From time to time, we may evaluate various acquisitions and strategic collaborations, including licensing or acquiring complementary drugs, intellectual property rights, technologies or businesses, as deemed appropriate to carry out our business plan. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent or unknown liabilities;
- assimilation of operations, intellectual property and drugs of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing drug programs and initiatives in pursuing such a strategic partnership, merger or acquisition;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing drugs or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired technology and/or drugs sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In addition, if we engage in future acquisitions or strategic partnerships, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses, and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities, and this inability could impair our ability to grow or obtain access to technology or drugs that may be important to the development of our business.

Risks Related to Ownership of Our Common Stock

The market price of our common stock is volatile and has fluctuated substantially, which could result in substantial losses for purchasers of our common stock.

The stock market in general and the market for biopharmaceutical and pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the price at which you purchased it. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this Annual Report, the market price for our common stock has been, and is likely to continue to be influenced by the following:

- the commencement, enrollment, results of , or any delays in our planned or future clinical trials of our product candidates or those of our competitors;
- the success of competitive drugs or therapies;
- regulatory or legal developments in the United States and other countries;
- the success of competitive products or technologies;

- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- our inability to obtain or delays in obtaining adequate drug supply for any approved drug or inability to do so at acceptable prices;
- 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;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems, including coverage and adequate reimbursement for any approved drug;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, political, and market conditions and overall fluctuations in the financial markets in the United States and abroad; and
- investors' general perception of us and our business.

These and other market and industry factors have caused the market price and demand for our common stock to fluctuate substantially, regardless of our actual operating performance, which may limit or prevent investors from selling their shares at or above the price paid for the shares and may otherwise negatively affect the liquidity of our common stock. Furthermore, we believe our stock has been, and may in the future be, adversely affected as a result of actions by third-parties. Short sellers and others, some of whom post anonymously on social media, may be positioned to profit if our stock declines and their activities can negatively affect our stock price.

Some companies that have experienced volatility in the trading price of their shares have been the subject of securities class action litigation. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our business practices. Defending against litigation is costly and time-consuming, and could divert our management's attention and our resources. Furthermore, during the course of litigation, there could be negative public announcements of the results of hearings, motions or other interim proceedings or developments, which could have a negative effect on the market price of our common stock.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Based upon shares of our common stock outstanding as of September 30, 2024, our executive officers, directors and stockholders who owned more than 10% of our outstanding common stock, in the aggregate, beneficially own shares representing approximately 18.2% of our outstanding common stock. If our executive officers, directors and stockholders who owned more than 10% of our outstanding common stock acted together, they may be able to significantly influence all matters requiring stockholder approval, including the election and removal of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. The concentration of voting power and transfer restrictions could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in the management of our company in ways with which other stockholders disagree.

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

You should not rely on an investment in our common stock to provide dividend income. We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude

us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock.

Future sales of common stock by holders of our common stock, or the perception that such sales may occur, could depress the market price of our common stock.

Sales of a substantial number of shares of our common stock in the public market could occur at any time, subject to certain restrictions described below. These sales, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of September 30, 2024, we had outstanding 116,356,474 shares of common stock. A substantial number of such shares are currently restricted as a result of securities laws or lock-up agreements but will be able to be sold in the future.

We further have registered all shares of common stock that we may issue in the future or have issued to date under our equity compensation plans. These shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and certain lock-up agreements. Sales of a large number of the shares issued under these plans in the public market could have an adverse effect on the market price of our common stock.

General Risk Factors

If research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or financial analysts publish about us or our business. Equity research analysts may discontinue research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. We do not have any control over the analysts, or the content and opinions included in their reports. The price of our shares could decline if one or more equity research analysts downgrade our shares or issue other unfavorable commentary or research about us. If one or more equity research analysts cease coverage of us or fail to publish reports on us regularly, demand for our shares could decrease, which in turn could cause the trading price or trading volume of our common stock to decline.

We have broad discretion in the use of our cash and cash equivalents and may use them ineffectively, in ways with which you do not agree or in ways that do not increase the value of your investment.

Our management has broad discretion in the application of our cash and cash equivalents and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in additional operating losses that could have a negative impact on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest our cash and cash equivalents in a manner that does not produce income or that loses value.

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

We are an "emerging growth company," or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not EGCs, including:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;

- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We cannot predict whether investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our share price may be more volatile. We currently take advantage of some or all of these reporting exemptions until we are no longer an EGC. We will remain an EGC until the earlier of (i) December 31, 2024, (ii) the last day of the fiscal year in which we have total annual gross revenue of at least $1.235 billion, (iii) the last day of the first fiscal year in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, and (iv) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period. Even after we no longer qualify as an EGC, we may still qualify as a "smaller reporting company" or "non-accelerated filer," which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

In addition, under Section 107(b) of the JOBS Act, EGCs can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same requirements to adopt new or revised accounting standards as other public companies that are not EGCs.

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

As a public company, and particularly after we are no longer an EGC, we incur and will continue to incur significant legal, accounting and other expenses. In addition, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and rules subsequently implemented by the SEC and The Nasdaq Stock Market LLC have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to comply with these requirements. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. However, while we remain an EGC, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions also could 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 common stock. 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 not all members of the board are elected at one time;
- 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 the board;
- 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 stockholder rights plan, or so-called "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 the holders of at least $66^{2}/_{3}\%$ of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or 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. These provisions could discourage potential acquisition proposals and could delay or prevent a change in control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Our governing documents 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 certificate of incorporation provides that, with respect to any state actions or proceedings under Delaware statutory or common law, the Court of Chancery of the State of Delaware is the exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us or any of our directors, officers, employees or agents arising under the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws;
- any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws; and
- any action asserting a claim against us or any of our directors, officers, employees or agents that is governed by the internal-affairs doctrine.

In addition, our amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States are, to the fullest extent permitted by law, the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act.

These exclusive-forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees. If a court were to find an exclusive-forum provision in our amended and restated certificate of incorporation or our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could harm our business.

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

None.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

Exhibit Number	Description
31.1	Rule 13a-14(a)/15d-14(a) Certification under the Exchange Act by Shoshana Shendelman, President and Chief Executive Officer (Principal Executive Officer).
31.2	Rule 13a-14(a)/15d-14(a) Certification under the Exchange Act by Les Funtleyder, Chief Financial Officer (Principal Accounting Officer and Principal Financial Officer).
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 Section 1350 Certifications.
32.2*	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 Section 1350 Certifications.
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document With Embedded Linkbase Documents.
104	Cover Page formatted as Inline XBRL and contained in Exhibit 101.

* Furnished herewith and not deemed to be “filed” for purposes of Section 18 of the Exchange Act, and shall not be deemed to be incorporated by reference into any filing under the Securities Act, or the Exchange Act (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.

SIGNATURES

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

APPLIED THERAPEUTICS, INC.

Date: November 7, 2024

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

Date: November 7, 2024

By: /s/ Les Funtleyder
Les Funtleyder
Chief Financial Officer
(Principal Accounting Officer and Principal Financial Officer)

Exhibit 31.1

CERTIFICATIONS

The undersigned certifies that:

1. I have reviewed this Form 10-Q of Applied 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 7, 2024

By: */s/ Shoshana Shendelman*
Name: Shoshana Shendelman, Ph.D.
Title: Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

The undersigned certifies that:

1.I have reviewed this Form 10-Q of Applied 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 7, 2024

By: */s/ Les Funtleyder*
Name: Les Funtleyder
Title: Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), the undersigned hereby certifies that, to the best of their knowledge:

1.The Quarterly Report on Form 10-Q of Applied Therapeutics, Inc. (the "Company") for the period ended September 30, 2024, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

2.The information contained in the Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 7, 2024

By: */s/ Shoshana Shendelman*
Name: Shoshana Shendelman, Ph.D.
Title: Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), the undersigned hereby certifies that, to the best of their knowledge:

1.The Quarterly Report on Form 10-Q of Applied Therapeutics, Inc. (the "Company") for the period ended September 30, 2024, to which this Certification is attached as Exhibit 32.2 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

2.The information contained in the Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 7, 2024

By: */s/ Les Funtleyder*
Name: Les Funtleyder
Title: Chief Financial Officer
(Principal Financial Officer)