UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 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: 001-37708

Syndax Pharmaceuticals, Inc.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	**32-0162505**
(State or Other Jurisdiction of Incorporation or Organization)	**(IRS Employer Identification No.)**
35 Gatehouse Drive, Building D, Floor 3 Waltham, Massachusetts	**02451**
(Address of Principal Executive Offices)	**(Zip Code)**

(781) 419-1400
(Registrant's Telephone Number, Including Area Code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	SNDX	The Nasdaq Stock Market, LLC

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 (Section 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). **Yes** ☒ **No** ☐

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

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised 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 1, 2024, there were 85,357,675 shares of the registrant's Common Stock, par value $0.0001 per share, outstanding.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements and information within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, which are subject to the "safe harbor" created by those sections. All statements other than statements of historical fact are “forward-looking statements” for purposes of this Quarterly Report on Form 10-Q. In some cases, you can identify forward-looking statements by terminology such as “anticipate,” “believe,” “could,” “estimate,” “expects,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” or the negative or plural of those terms, and similar expressions.

Forward-looking statements include, but are not limited to, statements about:

- our estimates regarding our expenses, future revenues, anticipated capital requirements and our needs for additional financing;
- the commercialization of NIKTIMVO™ (axatilimab-csfr) for treatment of chronic graft versus host disease, or cGVHD, including the estimated timeline for launch in the United States;
- the initiation, cost, timing, progress and results of our research and development activities, clinical trials and preclinical studies;
- our ability to replicate results in future clinical trials;
- our expectations regarding the potential safety, efficacy or clinical utility of our product candidates as well as the potential use of our product candidates to treat various cancer indications and fibrotic diseases;
- our ability to obtain and maintain regulatory approval for our product candidates and the timing or likelihood of regulatory filings and approvals for such candidates;
- our ability to maintain our licenses with UCB Biopharma Sprl, and Vitae Pharmaceuticals, LLC, a subsidiary of AbbVie Inc.;
- the success of our collaboration with Incyte Corporation, or Incyte, to further develop and commercialize axatilimab;
- the potential milestone and royalty payments under certain of our license agreements;
- the implementation of our strategic plans for our business and development of our product candidates;
- the scope of protection we establish and maintain for intellectual property rights covering our product candidates and our technology;
- the market adoption of Niktimvo and our other product candidates by physicians and patients;
- developments relating to our competitors and our industry; and
- the impact of geo-political actions, including war or the perception that hostilities may be imminent (such as the ongoing wars between Russia and Ukraine and Hamas and Israel as well as the conflicts in the Middle East, including between Israel and Hezbollah), adverse global economic conditions, terrorism, public health crises or natural disasters on our operations, research and development and clinical trials and potential disruption in the operations and business of third-party manufacturers, contract research organizations, or CROs, other service providers, and collaborators with whom we conduct business.

These statements are only current predictions and are subject to known and unknown risks, uncertainties and other factors that may cause our or our industry’s actual results, levels of activity, performance, or achievements to be materially different from those anticipated by the forward-looking statements. We discuss many of these risks in this report in greater detail in the section titled “Risk Factors” and elsewhere in this report. You should not rely upon forward-looking statements as predictions of future events.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance, or achievements. Except as required by law, we are under no duty to update or revise any of the forward-looking statements, whether as a result of new information, future events or otherwise.

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Part I: FINANCIAL INFORMATION

Item 1: Financial Statements

SYNDAX PHARMACEUTICALS, INC.
(unaudited)
CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)

	September 30, 2024	December 31, 2023
ASSETS		
Current assets:		
Cash and cash equivalents	$ 133,019	$ 295,394
Short-term investments	256,588	275,304
Short-term deposits	16,788	6,885
Other receivable	3,507	—
Prepaid expenses and other current assets	4,907	3,293
Total current assets	414,809	580,876
Long-term investments	10,029	29,829
Property and equipment, net	—	8
Right-of-use asset, net	756	1,487
Restricted cash	217	217
Other assets	—	463
Total assets	$ 425,811	$ 612,880
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 4,898	$ 9,961
Collaboration payable, net	5,506	7,232
Accrued expenses and other current liabilities	48,121	39,856
Current portion of right-of-use liability	842	1,035
Current portion of capital lease	10	12
Total current liabilities	59,377	58,096
Long-term liabilities:		
Right-of-use liability, less current portion	—	578
Capital lease, less current portion	2	10
Total long-term liabilities	2	588
Total liabilities	59,379	58,684
Commitments and contingencies (Note 12)		
Stockholders' equity:		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; 0 shares outstanding at September 30, 2024 and December 31, 2023	—	—
Common stock, $0.0001 par value, 200,000,000 shares authorized; 85,571,205 and 84,826,632 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively	9	8
Additional paid-in capital	1,493,041	1,456,370
Accumulated other comprehensive gain	371	218
Accumulated deficit	(1,126,989)	(902,400)
Total stockholders' equity	366,432	554,196
Total liabilities and stockholders' equity	$ 425,811	$ 612,880

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

SYNDAX PHARMACEUTICALS, INC.
(unaudited)
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Revenue:				
Milestone and license revenue	$ 12,500	$ —	$ 16,000	$ —
Total revenues	12,500	—	16,000	—
Operating expenses:				
Research and development	$ 70,971	$ 39,087	$ 176,118	$ 107,906
Selling, general and administrative	31,106	17,268	83,189	44,143
Total operating expenses	102,077	56,355	259,307	152,049
Loss from operations	(89,577)	(56,355)	(243,307)	(152,049)
Other income (expense), net:				
Interest expense	(23)	(70)	(123)	(145)
Interest income	5,442	5,345	18,982	15,613
Other income (expense)	32	(66)	(141)	(306)
Total other income (expense), net	5,451	5,209	18,718	15,162
Net loss	$ (84,126)	$ (51,146)	$ (224,589)	$ (136,887)
Other comprehensive loss:				
Unrealized gain on marketable securities	1,188	119	371	351
Comprehensive loss	$ (82,938)	$ (51,027)	$ (224,218)	$ (136,536)
Net loss attributable to common stockholders	$ (84,126)	$ (51,146)	$ (224,589)	$ (136,887)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.98)	$ (0.73)	$ (2.63)	$ (1.97)
Weighted-average number of common shares used to compute net loss per share attributable to common stockholders —basic and diluted	85,433,569	69,855,766	85,307,660	69,645,888

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

SYNDAX PHARMACEUTICALS, INC.
(unaudited)
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Nine Months Ended September 30,	
	2024	**2023**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (224,589)	$ (136,887)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	8	9
Accretion of investments	(10,528)	(11,344)
Non-cash operating lease expense	781	496
Stock-based compensation	30,729	22,624
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(11,567)	(3,281)
Collaboration (payable) receivable, net	(1,726)	68
Other receivable	(3,507)	—
Other assets	463	—
Accounts payable	(5,063)	1,699
Accrued expenses and other liabilities	7,484	7,364
Net cash used in operating activities	(217,515)	(119,252)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of short and long-term investments	(180,738)	(225,316)
Proceeds from sales and maturities of short-term investments	229,936	359,215
Net cash provided by investing activities	49,198	133,899
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from Employee Stock Purchase Plan	1,081	633
Proceeds from stock option exercises	4,861	5,016
Net cash provided by financing activities	5,942	5,649
NET (DECREASE) INCREASE CASH, CASH EQUIVALENTS AND RESTRICTED CASH	(162,375)	20,296
CASH, CASH EQUIVALENTS AND RESTRICTED CASH—beginning of period	295,611	74,471
CASH, CASH EQUIVALENTS AND RESTRICTED CASH —end of period	$ 133,236	$ 94,767

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

SYNDAX PHARMACEUTICALS, INC.
(unaudited)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of Business

Syndax Pharmaceuticals, Inc. is a commercial-stage biopharmaceutical company with one commercially approved product and an innovative pipeline of cancer therapies under development. We were incorporated in Delaware in 2005. We have operations in New York, NY and Waltham, MA and we operate in one segment. References in these notes to condensed consolidated financial statements to "Syndax," "the Company," "we," "us" or "our" refer to Syndax Pharmaceuticals, Inc. and its wholly owned subsidiaries.

2. Basis of Presentation

The Company has prepared the accompanying condensed consolidated financial statements in conformity with accounting principles generally accepted in the United States of America, or U.S. GAAP. Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted. The interim unaudited condensed financial statements have been prepared on the same basis as the annual audited financial statements and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of September 30, 2024, and the results of operations and comprehensive loss 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. The results for the three and nine months ended September 30, 2024 are not necessarily indicative of the results to be expected for the year ending December 31, 2024, any other interim periods, or any future year or period. These interim financial statements should be read in conjunction with the audited financial statements as of and for the year ended December 31, 2023, and the notes thereto, which are included in the Company's Annual Report on Form 10-K as filed with the Securities and Exchange Commission, or the SEC, on February 27, 2024.

In 2011, the Company established a wholly owned subsidiary in the United Kingdom, which the Company dissolved in June 2024. In 2014, the Company established a wholly owned U.S. subsidiary, and in 2021, the Company established a wholly owned subsidiary in the Netherlands. To date, there have been no material activities for these entities. All intercompany balances and transactions have been eliminated in consolidation.

3. Summary of Significant Accounting Policies

The Company's significant accounting policies, which are disclosed in the audited consolidated financial statements for the year ended December 31, 2023, and the notes thereto are included in the Company's Annual Report on Form 10-K that was filed with the SEC on February 27, 2024. Since the date of filing, there have been no material changes to the Company's significant accounting policies except as noted below.

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of costs and expenses during the reporting period. The Company bases estimates and assumptions on historical experience when available and on various factors that it believes to be reasonable under the circumstances. The Company evaluates its estimates and assumptions on an ongoing basis.

Estimates and assumptions about future events and their effects cannot be determined with certainty and therefore require the exercise of judgment. As of the date of issuance of these financial statements, the Company is not aware of any specific event or circumstance that would require the Company to update its estimates, assumptions and judgments or revise the carrying value of its assets or liabilities. These estimates may change as new events occur and additional information is obtained and are recognized in the consolidated financial statements as soon as they become known. Actual results could differ from those estimates and any such differences may be material to the Company's condensed consolidated financial statements.

Significant Risks and Uncertainties

We are subject to challenges and risks specific to our business and our ability to execute on our strategy, as well as risks and uncertainties common to companies in the pharmaceutical industry with development and commercial operations, including, without limitation, risks and uncertainties associated with: obtaining regulatory approval of our late-stage product candidate; delays or problems in the supply of our products, loss of single source suppliers or failure to comply with manufacturing regulations; identifying, acquiring or in-licensing additional products or product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; the challenges of protecting and enhancing our intellectual property rights; and complying with applicable regulatory requirements.

Income taxes

In accordance with ASC 270, Interim Reporting, and ASC 740, Income Taxes, the Company is required at the end of each interim period to determine the best estimate of its annual effective tax rate and then apply that rate in providing for income taxes on a current year-to-date (interim period) basis. For the nine months ended September 30, 2024 and 2023, the Company recorded no tax expense or benefit due to the expected current year loss and its historical losses. As of September 30, 2024 and December 31, 2023, the Company concluded that a full valuation allowance would be necessary for all of its net deferred tax assets. The Company had no amounts recorded for uncertain tax positions, interest or penalties in the accompanying consolidated financial statements.

Recently Issued and Adopted Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board, or FASB, or other accounting standard setting bodies that we adopt as of the specified effective date. Unless otherwise discussed below, we do not believe that the adoption of recently issued standards have or may have a material impact on our consolidated statements or disclosures.

Segment Reporting

In December 2023, the FASB issued ASU 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, which requires all public entities, including public entities with a single reportable segment, to provide in interim and annual periods one or more measures of segment profit or loss used by the chief operating decision maker to allocate resources and assess performance. Additionally, the standard requires disclosures of significant segment expenses and other segment items as well as incremental qualitative disclosures. The guidance in this update is effective for fiscal years beginning after December 15, 2023, and interim periods after December 15, 2024. The Company is currently in the process of evaluating the effects of this pronouncement on our related disclosures.

4. Significant Collaborative Research and License Agreements

Incyte Collaboration

In September 2021, the Company entered into the Incyte License and Collaboration Agreement, or the Incyte License, with Incyte covering the worldwide development and commercialization of axatilimab. Also in September 2021, the Company entered into a share purchase agreement with Incyte, or the Incyte Share Purchase Agreement. These agreements are collectively referred to as the Incyte Agreements. Under the terms of the Incyte Agreements, Incyte received exclusive commercialization rights outside of the United States, subject to certain royalty payment obligations set forth below. In the United States, Incyte and the Company are co-commercializing and co-promoting axatilimab as Niktimvo™ (axatilimab-csfr). The Company and Incyte share equally the profits and losses from co-commercialization efforts in the United States.

The Company and Incyte have agreed to continue to co-develop axatilimab and to share development costs associated with global and additional U.S.-specific clinical trials, with Incyte responsible for 55% of such costs and the Company responsible for 45% of such costs. Each company will be responsible for funding any of its own independent development activities. Incyte is responsible for 100% of future development costs for trials that are specific to ex-U.S. countries. All development costs related to the collaboration will be subject to a joint development plan.

Under the terms of the Incyte Agreements, in December 2021, Incyte paid the Company a non-refundable cash payment of $117.0 million and the Company issued 1,421,523 shares of common stock with an aggregate purchase price of $35.0 million, or $24.62 per share. Additionally, under the terms of the Incyte Agreements, the Company was eligible, upon execution, to receive up to $220.0 million in future contingent development and regulatory milestones and up to $230.0 million in commercialization milestones as well as tiered royalties ranging in the mid-teens percentage on net sales of the licensed product comprising axatilimab in Europe and Japan and low double digit percentage in the rest of the world outside of the United States. The Company's right to receive royalties in any particular country will expire upon the last to occur of (a) the expiration of licensed patent rights covering the licensed product in that particular country, (b) a specified period of time after the first post - marketing authorization sale of a licensed product in that country, and (c) the expiration of any regulatory exclusivity for that licensed product in that country.

In August 2024, the U.S. Food and Drug Administration, or FDA, approved Niktimvo for the treatment of cGVHD after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg (88.2 lbs). As a result of the approval of Niktimvo, the Company earned a revenue milestone of $12.5 million, which was received in September 2024.

As of September 30, 2024, the Company has recorded approximately $1.7 million as a collaboration receivable due from Incyte related to the Company's development and pre-commercialization costs under the Incyte Agreements and has recorded approximately $7.2 million as a collaboration payable due to Incyte for development and pre-commercialization costs incurred by Incyte as of September 30, 2024. Both expense and cost offset are recorded as part of operating expenses.

Vitae Pharmaceuticals, Inc.

In October 2017, the Company entered into a license agreement, or the Vitae License Agreement, with Vitae Pharmaceuticals, LLC, or Vitae, a subsidiary of AbbVie, Inc., under which the Company was granted an exclusive, sublicensable, worldwide license to a portfolio of preclinical, orally available, small molecule inhibitors of the Menin–KMT2A binding interaction, or the Menin Assets. Upon execution of the agreement, the Company would potentially be required to pay Allergan up to $99.0 million in one-time development and regulatory milestone payments over the term of the Vitae License Agreement, subject to the achievement of certain milestone events. In the event that the Company or any of its affiliates or sublicensees commercializes the Menin Assets, the Company will also be obligated to pay Vitae low single to low double-digit royalties on sales, subject to reduction in certain circumstances, as well as up to an aggregate of $70.0 million in potential one-time, sales-based milestone payments based on achievement of certain annual sales thresholds. The Company is solely responsible for the development and commercialization of the Menin Assets. Each party may terminate the Vitae License Agreement for the other party's uncured material breach or insolvency, and the Company may terminate the Vitae License Agreement at any time upon advance written notice to Vitae. Vitae may terminate the Vitae License Agreement if the Company or any of its affiliates or sublicensees institutes a legal challenge to the validity, enforceability, or patentability of the licensed patent rights. Unless terminated earlier in accordance with its terms, the Vitae License Agreement will continue on a country-by-country and product-by-product basis until the later of: (i) the expiration of all of the licensed patent rights in such country; (ii) the expiration of all regulatory exclusivity applicable to the product in such country; and (iii) 10 years from the date of the first commercial sale of the product in such country.

As of the date of the Vitae License Agreement, the asset acquired had no alternative future use nor had it reached a stage of technological feasibility. As the processes or activities that were acquired along with the license do not constitute a "business," the transaction has been accounted for as an asset acquisition. Since the effective date of the Vitae License Agreement, the Company achieved certain development and regulatory milestones, resulting in $18.0 million in research and development expense, which includes an $8.0 million milestone paid during the first quarter of 2024 for the successful completion of the first pivotal trial in the first indication.

UCB Biopharma Sprl

In 2016, the Company entered into a license agreement, or the UCB License Agreement, as amended from time to time, with UCB Biopharma Sprl, or UCB, under which UCB granted to the Company a worldwide, sublicensable, exclusive license to UCB6352, which the Company refers to as axatilimab, an anti-CSF-1R monoclonal antibody. Upon execution of the agreement, the Company would potentially be required to pay UCB up to $119.5 million in one-time development and regulatory milestone payments over the term of the UCB License Agreement, subject to the achievement of certain milestone events. In the event that the Company or any of its affiliates or sublicensees commercializes axatilimab, the Company will also be obligated to pay UCB low double-digit royalties on sales, subject to reduction in certain circumstances, as well as up to an aggregate of $250.0 million in potential one-time, sales-based milestone payments based on achievement of certain annual sales thresholds. Under certain circumstances, the Company may be required to share a percentage of non-royalty income from sublicensees, subject to certain deductions, with UCB. The Company is solely responsible for the development and commercialization of axatilimab, except that UCB was responsible for performing a limited set of transitional chemistry, manufacturing and control tasks related to axatilimab. Each party may terminate the UCB License Agreement for the other party's uncured material breach or insolvency, and the Company may terminate the UCB License Agreement at any time upon advance written notice to UCB. UCB may terminate the UCB License Agreement if the Company or any of its affiliates or sublicensees institutes a legal challenge to the validity, enforceability, or patentability of the licensed patent rights. Unless terminated earlier in accordance with its terms, the UCB License Agreement will continue on a country-by-country and product-by-product basis until the later of: (i) the expiration of all of the licensed patent rights in such country; (ii) the expiration of all regulatory exclusivity applicable to the product in such country; and (iii) 10 years from the date of the first commercial sale of the product in such country.

As of the date of the UCB License Agreement, the asset acquired had no alternative future use nor had it reached a stage of technological feasibility. As the processes or activities that were acquired along with the license do not constitute a "business," the transaction has been accounted for as an asset acquisition. As a result, in 2016, the upfront payment of $5.0 million was recorded as research and development expense in the consolidated statements of operations. Additionally, in connection with its most recent amendment of the UCB License Agreement, in the second quarter of 2022 the Company paid UCB $5.8 million, which was recognized as a milestone expense. Since the effective date of the license agreement, the Company achieved certain development and regulatory milestones and has recorded $31.0 million as research and development expense, which includes a $15.0 million milestone paid during the three months ended September 30, 2024, upon the approval of Niktimvo.

Bayer Pharma AG (formerly known as Bayer Schering Pharma AG)

In March 2007, the Company entered into a license agreement with Bayer Schering Pharma AG, or Bayer, for a worldwide, exclusive license to develop and commercialize entinostat and any other products containing the same active ingredient. The Company will pay Bayer royalties on a sliding scale based on net sales, if any, and make future milestone payments to Bayer of up to $150.0 million in the event that certain specified development and regulatory goals and sales levels are achieved.

Eddingpharm Investment Company Limited

In August 2016, the Company entered into a license agreement with Eddingpharm Investment Company, or Eddingpharm, to develop and commercialize entinostat in China and certain other Asian countries. Eddingpharm will pay the Company royalties on a sliding scale based on net sales, if any, and make future milestone payments up to $10.0 million in the event that certain specified development and regulatory goals are achieved. In April 2024, a milestone was achieved under the Eddingpharm license agreement for the marketing approval of entinostat in China. As a result, the Company recognized $3.5 million of milestone and license revenue in the second quarter of 2024.

5. Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common shares outstanding for the period. Because the Company has reported a net loss for all periods presented, diluted net loss per common share is the same as basic net loss per common share for those periods. The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
	(In thousands, except share and per share data)		(In thousands, except share and per share data)	
Numerator—basic and diluted:				
Net loss	$ (84,126)	$ (51,146)	$ (224,589)	$ (136,887)
Net loss attributable to common stockholders—basic and diluted	$ (84,126)	$ (51,146)	$ (224,589)	$ (136,887)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.98)	$ (0.73)	$ (2.63)	$ (1.97)
Denominator—basic and diluted:				
Weighted-average number of common shares used to compute net loss per share attributable to common stockholders—basic and diluted	85,433,569	69,855,766	85,307,660	69,645,888

The following potentially dilutive securities have been excluded from the computation of diluted weighted-average shares outstanding because such securities have an antidilutive impact due to losses reported (in common stock equivalent shares):

	September 30,	
	2024	2023
Options to purchase common stock	12,205,960	10,530,750
Employee Stock Purchase Plan	73,200	35,479
Non-vested restricted stock units (RSUs)	1,461,005	524,236

For additional information related to the Company's common stock see Note 10.

6. Other Receivables

Contemporaneous with the Company's New Drug Application, or NDA, submission to the FDA for revumenib, it was required to pay a $6.1 million refundable fee under the Prescription Drug User Fee Act, or PDUFA. The $6.1 million fee was paid in January 2024 and was fully refunded in September 2024.

In April 2024, entinostat received marketing approval in China. As of September 30, 2024, the Company recorded a $3.5 million milestone receivable related to achieved milestones under the license agreement with Eddingpharm.

7. Fair Value Measurements

The carrying amounts of cash and cash equivalents, restricted cash, accounts payable, and accrued expenses approximated their estimated fair values due to the short-term nature of these financial instruments. Fair value is defined as the exchange price that would

be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value are performed in a manner to maximize the use of observable inputs and minimize the use of unobservable inputs. The accounting standard describes a fair value hierarchy based on three levels of inputs, of which the first two are considered observable and the last unobservable, that may be used to measure fair value, which are the following:

Level 1— Quoted prices (unadjusted) in active markets that are accessible at the market date for identical unrestricted assets or liabilities.

Level 2— Inputs other than Level 1 that are observable, either directly or indirectly, such as quoted prices for similar assets or liabilities; quoted prices in markets that are not active; or other inputs for which all significant inputs are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.

Level 3— Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

The table below presents information about the Company's assets and liabilities that are regularly measured and carried at fair value and indicate the level within the fair value hierarchy of valuation techniques the Company utilized to determine such fair values (in thousands):

		Fair Value Measurements Using		
	Total Carrying Value	Quoted Prices (unadjusted) in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
	(In thousands)			
September 30, 2024				
Assets:				
Cash and cash equivalents	$ 133,019	$ 133,019	$ —	$ —
Short-term investments	256,588	—	256,588	—
Long-term investments	10,029	—	10,029	—
Total assets	$ 399,636	$ 133,019	$ 266,617	$ —
December 31, 2023				
Assets:				
Cash and cash equivalents	$ 295,394	$ 295,394	$ —	$ —
Short-term investments	275,304	—	275,304	—
Long-term investments	29,829	—	29,829	—
Total assets	$ 600,527	$ 295,394	$ 305,133	$ —

There have been no material impairments of our assets measured and carried at fair value during the period ended September 30, 2024 and 2023. In addition, there have been no changes in valuation techniques during the periods ended September 30, 2024 and 2023. The fair value of Level 1 instruments classified as cash equivalents are valued using quoted market prices in active markets. The fair value of Level 2 instruments classified as short and long-term investments are determined based on quoted prices in active markets, which are either directly or indirectly observable as of the reporting date with fair value being determined using models or other valuation methodologies.

The Company's short and long-term investments are classified as available-for-sale securities. As of September 30, 2024, the remaining contractual maturities of the available-for-sale securities were 1 to 13 months, and the balance in the Company's accumulated other comprehensive gain was comprised solely of activity related to the Company's available-for-sale securities. There were no realized gains or losses recognized on the sale or maturity of available-for-sale securities, during the three and nine months ended September 30, 2024 and 2023. As a result, the Company did not reclassify any amounts out of accumulated other comprehensive gain for the same periods.

The following table summarizes the available-for-sale securities:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
	(In thousands)			
September 30, 2024				
Commercial paper	$ 81,096	$ 44	$ —	$ 81,140
Corporate bonds	40,456	17	—	40,473
US Treasury	134,716	259	—	134,975
Federal bonds	9,978	51	—	10,029
	$ 266,246	$ 371	$ —	$ 266,617
December 31, 2023				
Commercial paper	$ 160,657	$ 149	$ —	$ 160,806
Corporate bonds	47,150	62	—	47,212
US Treasury	68,111	46	—	68,157
Federal bonds	28,998	—	(40)	28,958
	$ 304,916	$ 257	$ (40)	$ 305,133

8. 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 insurance	1,488	807
Interest receivable on investments	1,183	1,227
Prepaid subscription	1,295	769
Prepaid state and local taxes	601	264
Prepaid rent	122	163
Other	218	63
Total prepaid expenses and other current assets	$ 4,907	$ 3,293

9. 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 clinical study and trial costs	$ 35,692	$ 16,346
Accrued compensation and related costs	11,602	11,172
Accrued professional fees	619	1,450
Accrued milestone costs	—	10,000
Other	208	888
Total accrued expenses and other current liabilities	$ 48,121	$ 39,856

10. Stock-Based Compensation

In January 2024, the number of shares of common stock available for issuance under the Company's 2015 Omnibus Incentive Plan, or the 2015 Plan, was increased by 3,393,065 shares of common stock due to the automatic annual provision to increase shares of common stock available under the 2015 Plan. Additionally in December 2023, the Company's board of directors approved an

increase of 1,100,000 shares of common stock available for issuance under the Company's 2023 Inducement Plan, or Inducement Plan.

As of September 30, 2024, there were 3,862,670 shares of common stock available for issuance under the 2015 Plan and 76,763 shares of common stock available for issuance under the Inducement Plan.

The Company recognized stock-based compensation expense related to the issuance of stock option awards and restricted stock units to employees and non-employees and related to the Company's 2015 Employee Stock Purchase Plan, or ESPP, in the condensed consolidated statements of comprehensive loss as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development	$ 5,886	$ 3,853	$ 14,347	$ 10,329
Selling, general and administrative	6,048	4,468	16,382	12,295
Total	$ 11,934	$ 8,321	$ 30,729	$ 22,624

Compensation expense by type of award in the three and nine months ended September 30, 2024 and 2023 was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Stock options	$ 9,300	$ 7,242	$ 24,285	$ 19,654
RSUs	2,455	990	6,071	2,749
ESPP	179	89	373	221
Total	$ 11,934	$ 8,321	$ 30,729	$ 22,624

As of September 30, 2024, there were $93.3 million of unrecognized compensation costs related to employee and non-employee unvested stock options and RSUs granted under the 2023 Inducement Plan, 2015 Plan and the Company's 2007 Stock Plan, which are expected to be recognized over a weighted-average remaining service period of 2.61 years.

11. Stockholders' Equity

The following table presents the changes in stockholders' equity for the three and nine months ended September 30, 2024:

(In thousands, except share data)	Common Stock $0.0001 Par Value		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss)/Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2023	84,826,632	$ 8	$ 1,456,370	$ 218	$ (902,400)	$ 554,196
Stock purchase under ESPP	35,463	—	—	—	—	—
Stock-based compensation expense	—	—	8,899	—	—	8,899
Unrealized loss on investments	—	—	—	(974)	—	(974)
Vesting of RSUs	3,750	—	—	—	—	—
Employee withholdings ESPP	—	—	309	—	—	309
Proceeds from exercise of stock options	113,841	—	1,859	—	—	1,859
Net loss	—	—	—	—	(72,400)	(72,400)
Balance as of March 31, 2024	84,979,686	$ 8	$ 1,467,437	$ (756)	$ (974,800)	$ 491,889
Stock-based compensation expense	—	—	9,896	—	—	9,896
Unrealized loss on investments	—	—	—	(61)	—	(61)
Vesting of RSUs	1,603	—	—	—	—	—
Employee withholdings ESPP	—	—	237	—	—	237
Proceeds from exercise of stock options	47,340	—	439	—	—	439
Par value adjustment	—	1	—	—	—	1
Net loss	—	—	—	—	(68,063)	(68,063)
Balance as of June 30, 2024	85,028,629	$ 9	$ 1,478,009	$ (817)	$ (1,042,863)	$ 434,338
Stock-based compensation expense	—	—	11,934	—	—	11,934
Unrealized gains on investments	—	—	—	1,188	—	1,188
Stock purchase under ESPP	29,440	—	—	—	—	—
Employee withholdings ESPP	—	—	535	—	—	535
Vesting of RSUs	19,057	—	—	—	—	—
Proceeds from exercise of stock options	208,362	—	2,563	—	—	2,563
Net loss	—	—	—	—	(84,126)	(84,126)
Balance as of September 30, 2024	85,285,488	$ 9	$ 1,493,041	$ 371	$ (1,126,989)	$ 366,432

The following table presents the changes in stockholders' equity for the three and nine months ended September 30, 2023:

(In thousands, except share data)	Common Stock $0.0001 Par Value		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss)/Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2022	68,111,385	$ 7	$ 1,161,288	$ (806)	$ (693,040)	$ 467,449
Stock purchase under ESPP	16,537	—	—	—	—	—
Stock-based compensation expense	—	—	6,238	—	—	6,238
Unrealized gain on short-term investments	—	—	—	470	—	470
Vesting of RSUs	5,000	—	—	—	—	—
Employee withholdings ESPP	—	—	196	—	—	196
Prefunded warrants, exercise	85,998	—	—	—	—	—
Proceeds from exercise of stock options	276,506	—	2,278	—	—	2,278
Net loss	—	—	—	—	(41,126)	(41,126)
Balance as of March 31, 2023	68,495,426	$ 7	$ 1,170,000	$ (336)	$ (734,166)	$ 435,505
Stock-based compensation expense	—	—	8,065	—	—	8,065
Unrealized loss on short-term investments	—	—	—	(238)	—	(238)
Vesting of RSUs	1,602	—	—	—	—	—
Employee withholdings ESPP	—	—	179	—	—	179
Prefunded warrants, exercise	771,133	—	(1)	—	—	(1)
Proceeds from exercise of stock options	163,037	—	1,146	—	—	1,146
Net loss	—	—	—	—	(44,615)	(44,615)
Balance as of June 30, 2023	69,431,198	$ 7	$ 1,179,389	$ (574)	$ (778,781)	$ 400,041
Stock-based compensation expense	—	—	8,321	—	—	8,321
Unrealized gains on short-term investments	—	—	—	119	—	119
Stock purchase under ESPP	18,260	—	—	—	—	—
Employee withholdings ESPP	—	—	259	—	—	259
Proceeds from exercise of stock options	188,710	—	1,592	—	—	1,592
Net loss					(51,146)	(51,146)
Balance as of September 30, 2023	69,638,168	$ 7	$ 1,189,561	$ (455)	$ (829,927)	$ 359,186

At-the Market Program

In May 2023, the Company entered into a sales agreement with Cowen and Company, or TD Cowen, under which the Company could, from time to time, issue and sell shares of its common stock having aggregate sales proceeds of up to $200.0 million, in a series of one or more ATM equity offerings, or the 2023 ATM Program. TD Cowen is not required to sell any specific share amounts but acts as the Company's sales agent, using commercially reasonable efforts consistent with its normal trading and sales practices. Pursuant to the sales agreement, shares will be sold pursuant to the previous shelf registration statement on Form S-3ASR (Registration No. 333-277424), which became automatically effective upon the filing on February 27, 2024. The Company's common stock will be sold at prevailing market prices at the time of the sale, and as a result, prices may vary. For the three and nine months ended September 30, 2024, the Company sold no shares of common stock under the 2023 ATM Program.

Pre-Funded Warrants

In December 2021, the Company sold pre-funded warrants to purchase 1,142,856 shares of common stock. As of September 30, 2024, 285,714 pre-funded warrants were issued and outstanding.

12. Commitments and Contingencies

From time to time, the Company may be subject to various claims and proceedings in the ordinary course of business. If the potential loss from any claim, asserted or unasserted, or proceeding is considered probable and the amount is reasonably estimable, the Company will accrue a liability for the estimated loss. There were no contingent liabilities recorded as of September 30, 2024.

13. Subsequent Events

Royalty Pharma Development Funding, LLC Royalty Monetization Agreement

In October 2024, the Company entered into a purchase and sale agreement, or Royalty Agreement, with Royalty Pharma Development Funding, LLC, or Royalty Pharma, pursuant to which Royalty Pharma purchased the right to receive 13.8% on quarterly net sales of Niktimvo in the United States of America and its respective territories, districts, commonwealths and possessions (including Guam and Puerto Rico) in exchange for an upfront payment of $350.0 million (gross) at closing, received in November 2024. Aggregate payments to Royalty Pharma pursuant to the Royalty Agreement will be capped at $822.5 million or 2.35 times the funded amount.

NYC Office Lease Extension

In October 2024, the Company entered into a three and a half year lease extension for its office at 730 Third Avenue, New York, NY, commencing on January 1, 2025. The term of the new lease expires on August 31, 2028.

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 our Annual Report on Form 10-K that was filed with the Securities and Exchange Commission, or SEC, on February 27, 2024.

Company Overview

We are a commercial-stage biopharmaceutical company with one commercially approved product and are developing an innovative pipeline of cancer therapies. NIKTIMVO™ (axatilimab-csfr) is our approved product and revumenib is our lead product candidate.

We are developing revumenib, a potent, selective, small molecule inhibitor of the menin-MLL binding interaction for the treatment of KMT2A rearranged, or KMT2Ar, also known as mixed lineage leukemia rearranged or MLLr, acute leukemias including acute lymphoblastic leukemia, or ALL, and acute myeloid leukemia, or AML, and nucleophosmin1, also known as NPM1, mutant AML. We are also exploring the use of revumenib as a treatment in solid tumors, specifically its activity in metastatic microsatellite stable, or MSS, colorectal cancer, or CRC. We continue to develop axatilimab, a monoclonal antibody that blocks the colony stimulating factor 1 receptor, or CSF-1R, for idiopathic pulmonary fibrosis, or IPF.

Our commercially approved product, Niktimvo, received approval on August 14, 2024 from the U.S. Food and Drug Administration, or FDA, in the United States, for the treatment of chronic graft versus host disease, or cGVHD, after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg (88.2 lbs), with a recommended dosage of 0.3 mg/kg, up to a maximum dose of 35 mg, as an intravenous infusion over 30 minutes every two weeks until progression or unacceptable toxicity. While Incyte leads U.S. commercialization efforts, we are co-promoting Niktimvo with Incyte in the U.S. To facilitate patient dosing and limit product waste, following the FDA's approval of Niktimvo, we, along with Incyte, have submitted to the FDA for the approval of two smaller vial sizes. We anticipate launching Niktimvo in the U.S. no later than early first quarter 2025. Incyte has exclusive commercialization rights for Niktimvo outside the U.S.

We plan to continue to leverage the technical and business expertise of our management team and scientific collaborators to license, acquire and develop additional therapeutics to expand our pipeline. We have not generated any product revenues from product sales to date. We continue to incur significant research and development and other expenses related to our ongoing operations. Except for 2021, we have not been profitable and have incurred losses in each period since our inception in 2005. For the nine months ended September 30, 2024 and 2023, we reported a net loss of $224.6 million and $136.9 million, respectively. As of September 30, 2024, we had an accumulated deficit of $1.1 billion. As of September 30, 2024, we had cash, cash equivalents and short- and long-term investments of $399.6 million.

Business Update

Revumenib

- The New Drug Application, or NDA, for revumenib, an oral menin inhibitor, for the treatment of adult and pediatric relapsed or refractory, or R/R, KMT2A-r acute leukemia was granted Priority Review and is being reviewed under the U.S. FDA's Real-Time Oncology Review, or RTOR, Program with a Prescription Drug User Fee Act, or PDUFA, target action date of December 26, 2024.
- We expect to report topline data from the AUGMENT-101 pivotal trial cohort of patients with R/R mutant nucleophosmin, or mNPM1, AML in the fourth quarter of 2024. Positive data could support a supplemental NDA, or sNDA, filing for revumenib in R/R mNPM1 AML in the first half of 2025.
- We announced that data from the pivotal Phase 2 portion of the AUGMENT-101 trial of revumenib in adult and pediatric patients with R/R KMT2Ar AML and ALL have been published in the Journal of Clinical Oncology.
- We announced that a larger data set and longer follow-up from the pivotal Phase 2 portion of the AUGMENT-101 trial of revumenib in R/R KMT2Ar acute leukemia will be presented at the upcoming 66th American Society of Hematology, or ASH, Annual Meeting. Analysis of the larger efficacy population (n=97), which includes the 57 patients from the previously reported interim efficacy analysis plus an additional 40 patients. Consistent with previously reported data, the updated analysis shows that revumenib provides durable responses and robust rates of overall response, minimal residual disease, or MRD, negativity, and hematopoietic stem cell transplantation, or HSTC. With seven months of additional follow-up, the median duration of CR/CRh extended to 13 months among the 13 CR/CRh responders included in the interim analysis presented at ASH 2023.
- Multiple trials evaluating the potential to expand revumenib use across the mNPM1 and KMT2Ar acute leukemia treatment landscape are ongoing. These trials include:

- BEAT AML: A Phase 1 trial evaluating the combination of revumenib with venetoclax and azacitidine in front-line AML patients. The trial is being conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial. We presented updated positive data from the trial at the European Hematology Association, or EHA, 2024 Congress, showing a 96% (23 of 24 pts) composite complete remission, or CRc, rate in patients with newly diagnosed mNPM1 or KMT2Ar AML. The BEAT AML trial is expanding to validate the recommended Phase 2 dose of the combination of revumenib with venetoclax and azacitidine. We anticipate that an update on the trial will be available in the fourth quarter of 2024.
- SAVE: A Phase 1 trial evaluating the all-oral combination of revumenib with venetoclax and decitabine/cedazuridine in R/R AML or mixed phenotype acute leukemias. The trial is being conducted by investigators from MD Anderson Cancer Center. We announced that updated data showing an 88% ORR (23 of 26 pts) in R/R patients with KMT2Ar, mNPM1, or NUP98r leukemias will be presented at the upcoming 66th ASH Annual Meeting. In addition to the R/R cohort, a frontline cohort is now enrolling patients.
- INTERCEPT: A Phase 1 trial evaluating the use of novel therapies, including revumenib, to target MRD and early relapse in AML. The trial is being conducted by the Australasian Leukaemia and Lymphoma Group as part of the INTERCEPT AML master clinical trial. Preliminary results from the first eight mNPM1 patients treated with revumenib will be presented at the upcoming 66th ASH Annual Meeting.
- Intensive chemotherapy: A Phase 1 trial evaluating the combination of revumenib with intensive chemotherapy (7+3) followed by revumenib maintenance treatment in newly diagnosed patients with mNPM1 or KMT2Ar acute leukemias. The trial is designed to identify the recommended Phase 2 dose for this combination to support further development.
- Break *Through* Cancer: A Phase 2 trial studying whether the combination of revumenib and venetoclax can eliminate MRD in patients with AML and extend progression-free survival. The trial is being conducted by Break *Through* Cancer, a collaboration between leading U.S. cancer research centers.

- We plan to initiate a pivotal trial of revumenib in combination with venetoclax and azacitidine in newly diagnosed mNPM1 or KMT2Ar acute leukemia patients unfit to receive intensive chemotherapy by year-end 2024.
- We are evaluating revumenib in patients with R/R metastatic MSS colorectal cancer. The trial is currently enrolling patients in the Phase 1b portion of its Phase 1/2 proof-of-concept trial.

Niktimvo™ (axatilimab-csfr)

- Niktimvo received FDA approval for the treatment of cGVHD after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg (88.2 lbs). We anticipate that Niktimvo will be launched in the U.S. no later than early first quarter 2025. In the U.S., Niktimvo will be co-commercialized by Incyte and us.
- We announced Niktimvo was added to the latest NCCN Clinical Practice Guidelines in Oncology, or NCCN Guidelines®, as a category 2A recommendation for the treatment of GVHD after the failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg. Treatments are classified as category 2A when there is uniform NCCN consensus that the intervention is appropriate, based on lower level evidence.
- We announced that results from the pivotal Phase 2 AGAVE-201 trial of Niktimvo in adult and pediatric patients with recurrent/refractory active cGVHD who had received at least two prior lines of systemic therapy were published in the New England Journal of Medicine.
- We announced a secondary analysis of overall and organ-specific responses from the pivotal Phase 2 AGAVE-201 trial of Niktimvo in adult and pediatric patients with recurrent/refractory active cGVHD who had received at least two prior lines of

systemic therapy will be presented at the 66th ASH Annual Meeting. The data demonstrated rapid responses and symptom improvement in inflammatory and fibrotic manifestations of cGVHD in heavily pretreated patients.

- Enrollment is ongoing in a 26-week randomized, double-blinded, placebo-controlled Phase 2 trial of axatilimab on top of standard of care in patients with IPF, now referred to as the MAXPIRe trial (NCT06132256). We expect to report topline data from the trial in 2026.
- Our partner, Incyte, is now recruiting patients for a Phase 2, open-label, randomized, multicenter trial of axatilimab in combination with ruxolitinib in patients ≥12 years of age with newly diagnosed chronic GVHD (NCT06388564). A Phase 3 trial of axatilimab in combination with steroids for the treatment for chronic GVHD is currently in preparation.

Corporate

- We announced a $350 million royalty funding agreement with Royalty Pharma Development Funding, LLC, or Royalty Pharma, based on U.S. net sales of Niktimvo. Under the agreement, we received $350 million in exchange for a 13.8% royalty on U.S. net sales of Niktimvo. Royalty payments to Royalty Pharma will cease upon reaching a multiple of 2.35x.

Significant Risks and Uncertainties

We are subject to challenges and risks specific to our business and our ability to execute on our strategy, as well as risks and uncertainties common to companies in the pharmaceutical industry with development and commercial operations, including, without limitation, risks and uncertainties associated with: obtaining regulatory approval of our late-stage product candidate; identifying, acquiring or in-licensing additional products or product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; the challenges of protecting and enhancing our intellectual property rights; and complying with applicable regulatory requirements.

Financial Overview

Revenue

Our ability to generate sales revenue depends upon our ability to obtain marketing approval of, and successfully commercialize our product candidates. Our first commercially approved product, Niktimvo, received approval from the FDA in August 2024. We anticipate launching Niktimvo in the U.S. no later than early first quarter 2025. Our revenue for the three and nine months ended September 30, 2024 was derived from the achievement of milestones in connection with our license agreements with Incyte and Eddingpharm Investment Company Limited, or Eddingpharm. We did not generated any product sales revenue during the three and nine months ended September 30, 2024.

Research and Development

Since our inception, we have primarily focused on our clinical development programs. Research and development expenses consist primarily of costs incurred for the development of our product candidates and include:

- expenses incurred under agreements related to our clinical trials, including the costs for investigative sites and contract research organizations that conduct our clinical trials;
- employee-related expenses associated with our research and development activities, including salaries, benefits, travel and non-cash stock-based compensation expenses;
- manufacturing process-development, clinical supplies and technology-transfer expenses;
- license fees and milestone payments under our license agreements;
- consulting fees paid to third parties;
- allocated facilities and overhead expenses; and
- costs associated with regulatory operations and regulatory compliance requirements.

Internal and external research and development costs are expensed as they are incurred. Cost-sharing amounts received by us are recorded as reductions to research and development expense. Costs for certain development activities, such as clinical trials, are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations or other information provided to us by our vendors.

Research and development activities are central to our business model. Drug candidates in late stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of late-stage clinical trials. We plan to continue to spend a significant amount of our resources on research and development activities for the foreseeable future as we continue to advance the development of our drug candidates. The amount of research and development expenses allocated to external spending will continue to grow, while we expect our internal spending to grow at a slower and more controlled pace.

It is difficult to determine, with certainty, the duration and completion costs of our current or future preclinical programs, research studies and clinical trials of our product candidates. The duration, costs and timing of research studies and clinical trials of our product candidates will depend on a variety of factors that include, but are not limited to, the following:

- per patient costs;
- the number of patients that participate;
- the number of clinical trial sites;
- the countries in which the clinical trials are conducted;
- the length of time required to enroll eligible patients;
- the potential additional safety monitoring or other studies requested by regulatory agencies;
- the duration of patient monitoring;
- the efficacy and safety profile of the product candidates; and
- timing and receipt of any regulatory approvals.

In addition, the probability of success for each drug candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability. The successful development of our drug candidates is highly uncertain. At this time, we cannot reasonably estimate the nature, timing or costs of the efforts that will be necessary to complete the remainder of the development of our product candidates for the period, if any, in which material net cash inflows from these potential drug candidates may commence. Clinical development timelines, the probability of success and development costs can differ materially from expectations.

Selling, General and Administrative

Selling, general and administrative expenses consist primarily of pre-commercialization costs as well as employee-related expenses, including salaries, benefits, non-cash stock-based compensation and travel expenses, for our employees in executive, finance, human resources, information technology, business development and support functions. Other selling, general and administrative expenses include facility-related costs not otherwise allocated to research and development expenses and accounting, tax, legal, information technology and consulting services. We anticipate that our selling, general and administrative expenses will further increase in the future as we continue to increase our headcount to support our continued research and development and anticipated commercialization of revumenib. Additionally, we expect an increase in employee-related and sales and marketing expenses and other commercial expenses as a result of Niktimvo's anticipated commercial launch and in anticipation of revumenib's expected regulatory approval.

Interest Expense

Interest expense consists primarily of interest expense on our operational and capital leases.

Interest Income

Interest income consists of income earned on our cash, cash equivalents and short- and long-term investment balances.

Other Income (Expense), net

Other income (expense), net includes income (expense), consisting of revaluation of foreign currency related to trade payables.

Recent Accounting Pronouncements

For a discussion of new accounting pronouncements please read *Note 3 "Summary of Significant Accounting Policies,"* to our unaudited condensed consolidated financial statements included in this report.

Critical Accounting Estimates

Our management's discussion and analysis of financial condition and results of operations are based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States, or U.S. GAAP. The preparation of these financial statements requires us to make estimates, judgments and assumptions that may affect the reported amounts of assets, liabilities, equity, revenue and expenses and related disclosures of contingent assets and liabilities. We base our estimates on historical experience, known trends and events and various other factors that are believed to be 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. Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies.

There have been no material changes to our critical accounting estimates described in the "Management's Discussion and Analysis of Financial Condition and Results of Operations" contained in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023.

Results of Operations

Comparison of the three and nine months ended September 30, 2024 and 2023:

	Three Months Ended September 30,		Change	Nine Months Ended September 30,		Change
	2024	2023	$	2024	2023	$
		(In thousands)			(In thousands)	
Revenue:						
Milestone and license revenue	$ 12,500	$ —	$ 12,500	$ 16,000	$ —	$ 16,000
Total revenues	12,500	—	12,500	16,000	—	16,000
Operating expenses:						
Research and development	$ 70,971	$ 39,087	$ 31,884	$ 176,118	$ 107,906	$ 68,212
Selling, general and administrative	31,106	17,268	13,838	83,189	44,143	39,046
Total operating expenses	102,077	56,355	45,722	259,307	152,049	107,258
Loss from operations	(89,577)	(56,355)	33,222	(243,307)	(152,049)	91,258
Other income (expense), net:						
Interest expense	(23)	(70)	47	(123)	(145)	22
Interest income	5,442	5,345	97	18,982	15,613	3,369
Other income (expense)	32	(66)	98	(141)	(306)	165
Total other income (expense), net	5,451	5,209	242	18,718	15,162	3,556
Net loss	$ (84,126)	$ (51,146)	$ 32,980	$ (224,589)	$ (136,887)	$ 87,702

Revenue

Revenue for the three months ended September 30, 2024, consisted of a $12.5 million milestone earned as a result of the approval of Niktimvo.

Revenue for the nine months ended September 30, 2024, consisted of a $12.5 million milestone earned in the third quarter of 2024 as a result of the approval of Niktimvo and $3.5 million of revenue recognized in the second quarter of 2024 under the license agreement with Eddingpharm.

Research and Development

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

	Three Months Ended September 30,		Change	Nine Months Ended September 30,		Change
	2024	2023	$	2024	2023	$
		(In thousands)			(In thousands)	
Revumenib-related costs	$ 24,454	$ 15,146	$ 9,308	$ 76,421	$ 44,215	$ 32,206
Axatilimab-related costs	24,204	4,580	19,624	38,309	15,161	23,148
Other research and development programs	669	2,765	(2,096)	2,169	4,656	(2,487)
Personnel cost and other expenses	15,807	12,763	3,044	44,922	33,545	11,377
Stock-based compensation	5,837	3,833	2,004	14,297	10,329	3,968
Total research and development expenses	$ 70,971	$ 39,087	$ 31,884	$ 176,118	$ 107,906	$ 68,212

For the three and nine months ended September 30, 2024, our total research and development expenses increased $31.9 million and $68.2 million respectively, from the comparable prior year period. The increase was primarily due to:

- The increase in costs related to revumenib for the three and nine month period ended September 30, 2024, compared to the prior year periods, was primarily driven by the continuation of the registrational trials, preparation for front-line / combination trials, companion diagnostic development costs, pre-commercial manufacturing activities, medical affairs activities in preparation for commercialization, and an $8.0 million milestone paid to Vitae in the first quarter of 2024, related to the successful completion of the first pivotal trial in the first indication.
- The increase in costs related to axatilimab for the three and nine month period ended September 30, 2024, compared to the prior year periods, was primarily driven by the continuation of the IPF trial, pre-commercial manufacturing activities and a $15.0 million milestone payment to UCB as a result of the approval of Niktimvo in the third quarter of 2024.
- The decrease in other research and development programs for the three and nine month periods ended September 30, 2024, compared to the prior year periods, was related to a decrease in clinical trial expense, not related to the revumenib and axatilimab programs.
- The increase in personnel costs and other expenses, and stock-based compensation, which includes salaries, overhead, and related expenses, for the three and nine month periods ended September 30, 2024, compared to the prior year periods, was related to support of the on-going clinical trials and NDA/sNDA activities.

Selling, General and Administrative

The following tables summarizes the selling, general and administrative expenses for the three and nine months ended September 30, 2024 and 2023:

	Three Months Ended September 30,		Change	Nine Months Ended September 30,		Change
	2024	2023	$	2024	2023	$
		(In thousands)			(In thousands)	
Commercial-related expenses	$ 7,620	$ 3,390	$ 4,230	$ 21,012	$ 6,647	$ 14,365
Other selling, general and administrative expenses	3,854	3,802	52	12,111	10,040	2,071
Personnel cost and other expenses	13,535	5,608	7,927	33,635	15,161	18,474
Stock-based compensation	6,097	4,468	1,629	16,431	12,295	4,136
Total selling, general and administrative expenses	$ 31,106	$ 17,268	$ 13,838	$ 83,189	$ 44,143	$ 39,046

For the three and nine months ended September 30, 2024, our total selling, general and administrative expenses increased $13.8 million and $39.0 million, respectively, from the comparable prior year period. The increase primarily is due to:

- The increase in commercial-related expenses for the three and nine month periods ended September 30, 2024, compared to the prior year periods, was primarily driven by and increase in commercial readiness activities for Niktimvo and revumenib.
- The increase in other selling, general, and administrative expenses for the three and nine months ended September 30, 2024, compared to the prior year periods, was primarily due to an increase in IT, recruiting, and legal costs.
- The increase in personnel costs and other expenses and stock-based compensation, which includes salaries, overhead, and related expenses, for the three and nine months ended September 30, 2024, compared to the prior year periods, was primarily due to increased headcount in preparation for commercial readiness activities.

Interest Expense and Interest Income

For the three and nine months ended September 30, 2024, interest income, net of interest expense, increased from the comparable prior year period. The increase was primarily due to higher interest rates and an increased average balance on cash equivalents and short and long-term investments.

Other Income (Expense)

For the three and nine months ended September 30, 2024, other income (expense) decreased from the comparable prior year period primarily due to the decrease in foreign currency losses on short and long-term investments.

Liquidity and Capital Resources

Cash Flows

The following table sets forth the primary sources and uses of cash and cash equivalents for each period set forth below:

	Nine Months Ended September 30,	
	2024	2023
	(In thousands)	
Net cash used in operating activities	$ (217,515)	$ (119,252)
Net cash provided by investing activities	49,198	133,899
Net cash provided by financing activities	5,942	5,649
Net (decrease) increase cash, cash equivalents and restricted cash	$ (162,375)	$ 20,296

Net Cash Used in Operating Activities

Net cash used in operating activities increased from $119.3 million for the nine months ended September 30, 2023 to $217.5 million for the nine months ended September 30, 2024, primarily due to an increase in operating net loss, prepaid expenses, and collaboration receivables, and a decrease in accounts payable.

Net Cash Provided by Investing Activities

Net cash provided by investing activities for the nine months ended September 30, 2024, was $49.2 million and was related to $229.9 million from the maturities of available-for-sale securities, offset by the purchase of $180.7 million of available-for-sale securities.

Net cash provided by investing activities for the nine months ended September 30, 2023, was $133.9 million and was related to $359.2 million from the maturities of available-for-sale securities, offset by the purchase of $225.3 million of available-for-sale securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2024, increased by $0.3 million from the comparable prior year period primarily due to proceeds from the exercise of stock options and employee participation in our 2015 Employee Stock Purchase Plan, or ESPP.

Future Funding Requirements

Since our inception, our operations have been primarily financed by net proceeds from our public stock offerings, and revenue from our license agreements. We believe that our cash, cash equivalents and short- and long-term investments as of September 30, 2024, will fund our projected operating expenses and capital expenditure requirements for at least the next 12 months. In addition, we received a $350.0 million upfront payment in November 2024 pursuant to the purchase and sale agreement, or Royalty Agreement, with Royalty Pharma and are eligible to receive future aggregate payments up to $822.5 million. We are also and are eligible under our collaboration agreements to receive royalty payments, research and development funding, and milestone and other contingent payments for the achievement of defined collaboration objectives and certain development, regulatory and commercial milestones, which will continue to fund our future operations. Our ability to earn these milestone and contingent payments and the timing of achieving these milestones is primarily dependent upon the outcome of our collaborators' research and development activities and is uncertain at this time.

Additionally, the process of testing drug candidates in clinical trials is costly, and the timing of progress in these trials is uncertain. We cannot estimate the actual amounts necessary to successfully complete the development and commercialization of our product candidates or whether, or when, we may achieve profitability. Our future capital requirements will depend on many factors, including:

- our ability, along with Incyte, to successfully launch and generate sales of Niktimvo;
- the cost of establishing sales, marketing and distribution capabilities for our products and product candidates, including Niktimvo, which we are co-commercializing with Incyte, and revumenib, provided it receives regulatory approval and if we determine to commercialize it ourselves;
- the initiation, progress, timing, costs and results of clinical trials of our product candidates;
- the outcome, timing and cost of seeking and obtaining additional regulatory approvals from the FDA and comparable foreign regulatory authorities, including the potential for such authorities to require that we perform more trials than we currently expect;
- the cost to establish, maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with licensing, preparing, filing, prosecuting, defending and enforcing any patents or other intellectual property rights;
- market acceptance of our product candidates;
- the cost and timing of selecting, auditing and developing manufacturing capabilities, and potentially validating manufacturing sites for commercial-scale manufacturing;
- the cost and timing for obtaining pricing and reimbursement, which may require additional trials to address pharmacoeconomic benefit;
- the costs of acquiring, licensing or investing in additional businesses, products, product candidates and technologies;
- the interruption of key clinical trial activities, such as clinical trial site monitoring;
- the cost of disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of our products, which would adversely impact our ability to continue our clinical trial operations.
- the effect of competing technological and market developments; and
- our need to implement additional internal systems and infrastructure, including financial and reporting systems, as we grow our company.

We have one commercially approved product, Niktimvo, which we anticipate launching in the U.S. no later than early first quarter 2025. We otherwise have no products approved for commercial sale and have not generated any product revenues from product sales to date. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings and additional funding from license and collaboration arrangements. Except for payments received under the Royalty Agreement or any obligations of our collaborators to reimburse us for research and development expenses or to make milestone or royalty payments under our agreements with them, we will not have any committed external source of liquidity. Our material contractual obligations and commitments as of September 30, 2024, primarily relate to our maturities of operating leases for office space and equipment and capital leases for office equipment. As of September 30, 2024, we have $0.8 million payable within 12 months.

Except as disclosed above, we have no material non-cancelable purchase commitments with service providers, as we have generally contracted on a cancelable, purchase-order basis. We enter into contracts in the normal course of business with equipment and reagent vendors, CROs, CMOs and other third parties for clinical trials, preclinical research studies and testing and manufacturing services. These contracts are cancelable by us upon prior notice. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation. These payments are not determinable.

We have incurred losses and cumulative negative cash flows from operations since our inception, excluding the year ended December 31, 2021. As of September 30, 2024, we had an accumulated deficit of $1.1 billion. We anticipate that we will likely continue to incur significant losses for at least the next couple of years. We expect that our research and development and selling, general and administrative expenses will continue to increase. As a result, we will need additional capital to fund our operations, which we may raise through a combination of the sale of equity, debt financings, or other sources, including potential collaborations. To the extent that we raise additional capital through the future sale of equity or debt, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. If we raise additional funds through collaboration arrangements in the future, we may have to relinquish valuable rights to our technologies, future revenue streams or drug candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

At-the-Market Offering Program

In May 2023, we entered into a sales agreement with Cowen and Company, LLC, or TD Cowen, under which we could, from time to time, issue and sell shares of our common stock having aggregate sales proceeds of up to $200.0 million, in a series of one or more at-the-market equity offerings, or the 2023 ATM Program. TD Cowen is not required to sell any specific share amounts but acts as the Company's sales agent, using commercially reasonable efforts consistent with its normal trading and sales practices. Pursuant to the sales agreement, shares will be sold under the shelf registration statement on Form S-3ASR (Registration No. 333-277424), which became automatically effective upon filing on February 27, 2024. Our common stock will be sold at prevailing market prices at the time of the sale, and as a result, prices may vary. For the three and nine months ended September 30, 2024, we did not sell any shares of common stock under the 2023 ATM Program. As of September 30, 2024, we had $157.9 million available under the 2023 ATM Program.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of September 30, 2024, we had cash and cash equivalents of $133.0 million, consisting of overnight investments, interest-bearing money market funds and commercial paper, and short and long-term investments of $266.6 million, consisting of commercial paper, highly rated corporate bonds and treasuries. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. The primary objectives of our investment activities are to ensure liquidity and to preserve principal while at the same time maximizing the interest income, we receive from our marketable securities without significantly increasing risk. We have established guidelines regarding approved investments and maturities of investments, which are designed to maintain safety and liquidity. Due to the relative short-term maturities of our cash equivalents and the low risk profile of our short and long-term investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents and short and long-term investments. We have the ability to hold our investments until maturity, and therefore, we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investment portfolio.

We do not believe that inflation and changing prices had a significant impact on our results of operations for any periods presented herein.

Item 4. Controls and Procedures

Disclosure Controls and Procedures and Internal Control over Financial Reporting

Controls and Procedures

We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act), as of September 30, 2024. Based upon that evaluation, our principal executive officer and principal financial officer concluded that, as of the end of the period covered by this report, our disclosure controls and procedures are effective in ensuring that:

(a) the information required to be disclosed by us in the reports we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms; and

(b) the information is accumulated and communicated to our management, including our principal executive officer and principal financial officer as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting during the quarter 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

In the ordinary course of business, we are from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements and other matters. While the outcome of these proceedings and claims cannot be predicted with certainty, as of September 30, 2024, we were not party to any material legal or arbitration proceedings. No governmental proceedings are pending or, to our knowledge, contemplated against us.

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, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our unaudited condensed consolidated financial statements and related notes hereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. In these circumstances, the market price of our common stock could decline; and you may lose all or part of your investment. We cannot assure you that any of the events discussed below will not occur.

Summary of Selected Risks

Our business is subject to numerous risks and uncertainties, of which you should be aware before making a decision to invest in our securities. These risks and uncertainties include, among others, the following:

- We have limited experience in generating revenue from product sales.
- If the market opportunities for Niktimvo are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer.
- We are currently developing several product candidates. If we are unable to successfully complete clinical development of, obtain regulatory approval for, and commercialize our product candidates, our business prospects will be significantly harmed.
- Revumenib has undergone limited clinical testing and we may fail to show that it is well tolerated and provides sufficient clinical benefit for patients.
- 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 to the final data.
- We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of any of our product candidates.
- Incyte may fail to perform its obligations as expected under the collaboration or may deprioritize its investment to further develop and commercialize axatilimab.
- If we or our collaborators are unable to enroll patients in clinical trials, these clinical trials may not be completed on a timely basis or at all.
- Failure to comply with regulatory requirements or unanticipated problems with Niktimvo may result in various adverse actions such as the suspension or withdrawal of Niktimvo, closure of a facility or enforcement of substantial penalties or fines.
- The regulatory approval processes of the FDA and foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable. Our inability to obtain regulatory approval for our product candidates would harm our business.
- Niktimvo and our future product candidates may not achieve adequate market acceptance among physicians, patients, healthcare payors and others in the medical community to be commercially successful.
- We rely on third-party suppliers as well as Incyte to manufacture and distribute our clinical drug supplies for our product candidates, we intend to rely on third parties for commercial manufacturing and distribution of our product candidates and we expect to rely on third parties for manufacturing and distribution of preclinical, clinical and commercial supplies of any future product candidates.
- Our product and product candidates may face future development and regulatory difficulties.

- Our product and product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial scope of their approved use, or result in significant negative consequences following any marketing approval.
- The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for Niktimvo or any future products could limit our ability to market those products and decrease our ability to generate revenue.
- We have incurred net losses in each period since our inception, except in 2021, and anticipate that we will continue to incur net losses for the foreseeable future.
- We currently have no source of product revenue and may never achieve or maintain profitability.
- We will likely require additional capital to finance our planned operations, which may not be available to us on acceptable terms, or at all. As a result, we may not complete the development and commercialization of, or obtain regulatory approval for our existing product candidates or develop new product candidates.
- If we are unable to obtain or protect intellectual property rights, we may not be able to compete effectively in our market.
- We may not be able to protect our intellectual property rights throughout the world.
- The market price of our stock may be volatile and you could lose all or part of your investment.
- We may sell additional equity or debt securities or enter into other arrangements to fund our operations, which may result in dilution to our stockholders and impose restrictions or limitations on our business.

Risks Related to Our Business and Industry

We have limited experience in generating revenue from product sales.

Our ability to generate significant revenue from product sales depends on our ability to successfully commercialize Niktimvo and obtain the regulatory and marketing approvals necessary to commercialize revumenib as well as axatilimab for other indications. We currently have limited commercialization expertise, including sales, marketing, or distribution capabilities, and Niktimvo and future approved products may not remain in the market for a number of reasons, including ineffectiveness, harmful side effects, difficulty in scaling manufacturing, political and legislative changes, or competition from existing future alternatives. Our ability to generate substantial future revenue from product sales depends heavily on our success in many areas, including, but not limited to:

- developing a sustainable manufacturing process for approved products and establishing and maintaining supply and manufacturing relationships with third parties that can conduct the processes and provide adequate (in amount and quality) product supply to support market demand for approved products;
- launching and commercializing approved products;
- obtaining market acceptance of approved products;
- obtaining adequate market share, reimbursement and pricing for approved products;
- our ability to find patients so they can be diagnosed and begin receiving treatment;
- addressing any competing technological and market developments;
- negotiating favorable terms, including commercial rights, in any collaboration, licensing, or other arrangements into which we may enter, any amendments thereto or extensions thereof;
- maintaining, protecting, and expanding our portfolio of intellectual property rights, including patents, trade secrets, and know-how; and
- attracting, hiring, and retaining qualified personnel.

If the number of our addressable patients is not as significant as we estimate or the reasonably accepted population for treatment is narrowed by competition, physician choice, or treatment guidelines, we may not generate significant revenue from sales of approved products.

If the market opportunities for Niktimvo are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer.

Our projections of the number of adult and pediatric patients with cGVHD after failure of at least two prior lines of systemic therapy who have the potential to benefit from treatment with Niktimvo are based on our and Incyte's beliefs and estimates. These

estimates have been derived from a variety of sources and may prove to be incorrect or new studies may change the estimated incidence or prevalence, and the number of patients may turn out to be lower than expected. Additionally, the potentially addressable patient population for Niktimvo may be limited or may not be amenable to treatment with Niktimvo, and new patients may become increasingly difficult to identify or access, which would adversely affect our results of operations and our business. Even if we obtain significant market share for Niktimvo, we may never become or remain profitable nor generate sufficient revenue growth to sustain our business.

We are currently developing several product candidates. If we are unable to successfully complete clinical development of, obtain regulatory approval for, and commercialize our product candidates, our business prospects will be significantly harmed.

We have one approved product, Niktimvo. Our financial success will also depend substantially on our ability to effectively and profitably commercialize our other product candidates. In order to commercialize our product candidates, we will be required to obtain regulatory approvals by establishing that each of them is sufficiently safe and effective. The clinical and commercial success of our product candidates will depend on a number of factors, including the following:

- the initiation, cost, timing, progress and results of our research and development activities, clinical trials and preclinical studies;
- timely completion of any future clinical trials of revumenib and axatilimab;
- interruption of key clinical trial activities, in connection with public health threats or any future geopolitical tensions, such as the ongoing wars involving Russia and Israel;
- whether we are required by the FDA or foreign regulatory authorities to conduct additional clinical trials prior to receiving marketing approval;
- the prevalence and severity of adverse drug reactions in any of our clinical trials;
- the ability to demonstrate safety and efficacy of our product candidates for their proposed indications and the timely receipt of necessary marketing approvals from the FDA and foreign regulatory authorities;
- successfully meeting the endpoints in the clinical trials of our product candidates;
- achieving and maintaining compliance with all applicable regulatory requirements;
- the potential use of our product candidates to treat various cancer indications and fibrotic diseases;
- the availability, perceived advantages, relative cost, relative safety and relative efficacy of alternative and competing treatments;
- the effectiveness of our own or our potential strategic collaborators' marketing, sales and distribution strategy and operations in the United States and abroad;
- the ability of our collaboration partner and of third-party contract manufacturers to produce trial supplies and to develop, validate and maintain a commercially viable manufacturing process that is compliant with current Good Manufacturing Practices, or cGMP;
- our ability to successfully commercialize our product candidates in the United States and abroad, whether alone or in collaboration with others;
- our ability to prevent any significant disruptions of our information technology systems and protect the security of our data; and
- our ability to enforce our intellectual property rights in and to our product candidates.

If we fail to obtain regulatory approval for our product candidates, we will not be able to generate product sales, which will have a material adverse effect on our business and our prospects.

Revumenib has undergone limited clinical testing and we may fail to show that it is well tolerated and provides sufficient clinical benefit for patients.

Research suggests that certain acute leukemias, such as lysine methyltransferase 2A rearranged, or KMT2Ar, acute myeloid or lymphoid leukemia, AML or ALL, and nucleophosmin 1, or NPM1, mutant AML, are driven by the interaction of menin, a nuclear protein involved in transcription, with the N-terminus of KMT2A protein. In NPM1 mutant AML the interaction with menin occurs via the wild type KMT2A protein, and in KMT2Ar acute leukemia, the interaction occurs via a mutant form of KMT2A, a fusion protein known as KMT2Ar. KMT2Ar results from a rare, spontaneous fusion between the N-terminus of the KMT2A gene and a host of signaling molecules and nuclear transcription factors. This fusion produces an aberrant transcription program that drives leukemic

transformation. In pre-clinical animal models, small molecule inhibitors of the menin-KMT2Ar interaction, such as revumenib, which bind to, and block the interaction of menin with either KMT2A rearranged or wildtype, have demonstrated deep and durable single agent treatment effects in multiple leukemic xenograft models harboring KMT2A fusions or NPM1 mutations. Our strategy for developing revumenib is to conduct a Phase 1/2 clinical trial in r/r patients with KMT2Ar and NPM1 mutant acute leukemias and determine if the observed clinical efficacy supports further development. The Phase 1 portion of the trial is assessing the safety, tolerability and pharmacokinetics of revumenib, and seeks to establish a recommended Phase 2 dose. It is open label, and we have released and may in the future release results from time to time that reflect small numbers of patients which may not be accurately predictive of safety or efficacy results later in the trial or in subsequent trials. The Phase 2 portion is evaluating the efficacy of revumenib across three expansion cohorts enrolling pediatric and adult R/R patients with KMT2Ar ALL, KMT2Ar AML, and NPM1 mutant AML. In October 2023, we announced positive topline data in patients with R/R KMT2Ar acute leukemia and in March 2024, we announced that the NDA had a Priority Review PDUFA date of September 26, 2024, which the FDA subsequently extended to December 26, 2024. Neither breakthrough therapy designation nor RTOR review change the standards for approval and may not ultimately expedite the approval process or lead to approval. While we believe that we have established sufficient efficacy to warrant NDA submission and continued development in these indications, we may not yet have sufficiently demonstrated a favorable risk-benefit of revumenib in patients.

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. For example, in each of November 2022 and 2023, we announced interim data from our Phase 1/2 clinical trial of revumenib. While we have presented final data from our pivotal AGAVE-201 trial and from two of three cohorts in our AUGMENT-101 trial, interim data from other 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. Preliminary or top-line data may include, for example, data regarding a small percentage of the patients enrolled in a clinical trial, and such preliminary data should not be viewed as an indication, belief or guarantee that other patients enrolled in such clinical trial will achieve similar results or that the preliminary results from such patients will be maintained. 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.

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

Before obtaining marketing approval from regulatory authorities for the sale of any of our product candidates, we or our collaborators must conduct extensive trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive and difficult to design and implement, can take many years to complete and is inherently uncertain as to the outcome. A failure of one or more trials can occur at any stage of testing. The outcome of preclinical studies and early clinical trials may not accurately predict the success of later trials, and interim results of a trial do not necessarily predict final results.

We are dependent upon our collaboration with Incyte to further develop and commercialize axatilimab. If we or Incyte fail to perform as expected the potential for us to generate future revenues under the collaboration could be significantly reduced, the development and/or commercialization of axatilimab may be terminated or substantially delayed, and our business could be adversely affected.

We are subject to numerous risks related to the Incyte Collaboration Agreement to collaborate on the development and commercialization of axatilimab.

For example, there is no assurance that the parties will achieve any of the regulatory development or sales milestones, that we will receive any future milestone or royalty payments under the collaboration agreement. Incyte's activities may be influenced by, among other things, the efforts and allocation of resources by Incyte, which we cannot control. If Incyte does not perform in the manner we expect or fulfill its responsibilities in a timely manner, or at all, the clinical development, manufacturing, regulatory approval, and commercialization efforts related to axatilimab could be delayed or terminated. In addition, our license with Incyte may be unsuccessful due to other factors, including, without limitation, the following:

- Incyte may terminate the agreement for convenience upon 90 or 180 days' notice depending on whether or not the parties have commercialized axatilimab in an indication in the respective territory;
- Incyte may change the focus of its development and commercialization efforts or prioritize other programs more highly and, accordingly, reduce the efforts and resources allocated to axatilimab

- Incyte may, within its commercially reasonable discretion, choose not to develop and commercialize axatilimab in all relevant markets or for one or more indications, if at all; and
- if Incyte is acquired during the term of our collaboration, the acquirer may have competing programs or different strategic priorities that could cause it to reduce its commitment to our collaboration or to terminate the collaboration.

We cannot ensure that the potential strategic benefits and opportunities expected from this collaboration with be realized on our anticipated timeline or at all.

If we or our collaborators are unable to enroll patients in clinical trials, these clinical trials may not be completed on a timely basis or at all.

The timely completion of clinical trials largely depends on patient enrollment. Many factors affect patient enrollment, including:

- the impact of public health crises, or geopolitical tensions, such as the ongoing wars involving Russia and Israel;
- perception about the relative efficacy of our product candidates versus other compounds in clinical development or commercially available;
- evolving standard of care in treating cancer patients;
- the size and nature of the patient population, especially in the case of an orphan indication, we are pursuing;
- the number and location of clinical trial sites enrolled;
- competition with other organizations or our own clinical trials for clinical trial sites or patients;
- the eligibility and exclusion criteria for the trial;
- the design of the trial;
- ability to obtain and maintain patient consent; and
- risk that enrolled subjects will drop out before completion.

As a result of the above factors, there is a risk that our or our collaborators' clinical trials may not be completed on a timely basis or at all.

We may be required to relinquish important rights to and control over the development and commercialization of our product candidates to our current or future collaborators.

Our collaborations, including any future strategic collaborations we enter into, 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 existing stockholders' percentage of ownership;
- 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 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, sales 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 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 also 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.

We may explore strategic collaborations that may never materialize or may fail.

We periodically explore a variety of possible strategic collaborations in an effort to gain access to additional product candidates or resources. At the current time, 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 enter into strategic collaborations that we subsequently no longer wish to pursue, and 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 additional strategic collaborations because of the numerous risks and uncertainties associated with establishing them.

Failure to comply with regulatory requirements or unanticipated problems with Niktimvo may result in various adverse actions, such as the suspension or withdrawal of Niktimvo, closure of a facility or enforcement of substantial penalties or fines.

Regulatory agencies will subject any marketed product(s), as well as the manufacturing facilities, to continual review and periodic inspection. If previously unknown problems with a product or with regulatory requirements are discovered, such as adverse events of unanticipated severity or frequency, serious or unexpected side effects or other safety risks, problems with a manufacturing process or laboratory facility, or failure to comply with applicable regulatory approval requirements, a regulatory agency may impose restrictions or penalties on that product or on us. Such restrictions or penalties may include, among other things:

- restrictions on the marketing or manufacturing of the product, the withdrawal of the product from the market or product recalls;
- warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of approvals;
- product seizure or detention, or refusal to permit the import or export of our product candidates; and
- closure of the facility, enforcement of substantial fines, injunctions, or the imposition of civil or criminal penalties.

The regulatory approval processes of the FDA and foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable. Our inability to obtain regulatory approval for our product candidates would harm our business.

The FDA and comparable foreign regulatory authorities extensively and rigorously regulate and evaluate the manufacture, testing, distribution, advertising and marketing of drug products prior to granting marketing approvals with respect to such products. This approval process generally requires, at minimum, testing of any product candidate in preclinical studies and clinical trials to establish its safety and effectiveness, and confirmation by the FDA and comparable foreign regulatory authorities that any such product candidate, and any parties involved in its manufacturing, testing and development, complied with cGMP, current Good Laboratory Practices and current Good Clinical Practices, regulations, standards and guidelines during such manufacturing, testing and development. The time required to obtain approval by the FDA and foreign regulatory authorities is unpredictable, but typically takes many years following the commencement of preclinical studies and 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. Other than the recent approval for marketing of entinostat tablets by the National Medical Products Administration, we have not obtained regulatory approval for any of our product candidates and it is possible that we will never obtain regulatory approval for any additional product candidates or any future product candidates.

In addition, our product candidates could fail to receive regulatory approval from the FDA or foreign regulatory authorities for other reasons, including but not limited to:

- failure to demonstrate that our product candidates are effective for their proposed indication and have an acceptable safety profile;
- failure of clinical trials to meet the primary endpoints or level of statistical significance required for approval;
- failure to demonstrate that the clinical and other benefits of a product candidate outweigh any of its safety risks;

- disagreement with our interpretation of data from preclinical studies or clinical trials;
- disagreement with the design, size, conduct or implementation of our or our collaborators' trials;
- the insufficiency of data collected from trials of our product candidates to support the submission and filing of an NDA, BLA or other submission or to obtain regulatory approval;
- failure to obtain approval of the manufacturing and testing processes or facilities of third-party manufacturers with whom we contract for clinical and commercial product supplies or preclinical or clinical testing;
- receipt of a negative opinion from an advisory committee due to a change in the standard of care regardless of the outcome of the clinical trials; or
- changes in the approval policies or regulations that render our preclinical and clinical data insufficient for approval.

The FDA or foreign regulatory authorities may require more information, including additional preclinical or clinical data, to support approval, which may delay or prevent approval and our commercialization plans, or may cause us to decide to abandon our development program. Even if we were to obtain approval, regulatory authorities may approve one or more of our product candidates for a more limited patient population than we request, may grant approval contingent on the performance of costly post-marketing trials, may impose a risk evaluation and mitigation strategy, or REMS, or foreign regulatory authorities may require the establishment or modification of a similar strategy that may, for instance, restrict distribution of one or more of our product candidates and impose burdensome implementation requirements on us, or may approve it with a label that does not include the labeling claims necessary or desirable for the successful commercialization of one or more of our product candidates, all of which could limit our ability to successfully commercialize our product candidates. Moreover, if adopted in the form proposed, the recent European Commission proposals to revise the existing European Union, or EU, laws governing authorization of medicinal products may result in a decrease in data and market exclusivity for our product candidates in the EU.

Niktimvo and our future product candidates may not achieve adequate market acceptance among physicians, patients, healthcare payors and others in the medical community to be commercially successful.

Niktimvo and our future product candidates may not gain sufficient market acceptance among physicians, patients, healthcare payors and others in the medical community. Our commercial success also depends on coverage and adequate reimbursement by third-party payors, including government payors, which may be difficult or time-consuming to obtain, may be limited in scope and may not be obtained in all jurisdictions in which we may seek to market our product candidates. The degree of market acceptance will depend on a number of factors, including:

- the efficacy and safety profile as demonstrated in trials and in post-marketing experience;
- the timing of market introduction as well as competitive products;
- the clinical indications for which the product candidate is approved;
- acceptance of the product candidate as a safe and effective treatment by physicians, clinics and patients;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the cost of treatment in relation to alternative treatments;
- pricing and the availability of coverage and adequate reimbursement by third-party payors, including government authorities;
- relative convenience and ease of administration;
- the frequency and severity of adverse events;
- the effectiveness of sales and marketing; and
- unfavorable publicity relating to our product candidates.

If Niktimvo or future approved product candidates do not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors and patients, we may not generate sufficient revenue to become or remain profitable.

We rely on third-party suppliers as well as Incyte to manufacture and distribute our clinical drug supplies for our product candidates, we intend to rely on third parties for commercial manufacturing and distribution of our product candidates and we

expect to rely on third parties for manufacturing and distribution of preclinical, clinical and commercial supplies of any future product candidates.

We do not currently have, nor do we plan to acquire, the infrastructure or capability to manufacture or distribute preclinical, clinical or commercial quantities of drug substance or drug product, including our existing product candidates. While we expect to continue to depend on third-party manufacturers and Incyte for the foreseeable future, we do not have direct control over the ability of these parties to maintain adequate manufacturing capacity and capabilities to serve our needs, including quality control, quality assurance and qualified personnel. In addition, public health crises, may impact the ability of our existing or future manufacturers to perform their obligations to us.

We are dependent on our third-party manufacturers and Incyte for compliance with cGMPs and for manufacture of both active drug substances and finished drug products. Facilities used by our third-party manufacturers and Incyte to manufacture drug substance and drug product for commercial sale must be approved by the FDA or other relevant foreign regulatory agencies pursuant to inspections that will be conducted after we submit our NDA or relevant foreign regulatory submission to the applicable regulatory agency. If our third-party manufacturers or Incyte cannot successfully manufacture materials that conform to our specifications and/or the strict regulatory requirements of the FDA or foreign regulatory agencies, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. Furthermore, these third-party manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which also exposes our third-party manufacturers to regulatory risks for the production of such materials and products. As a result, failure to meet the regulatory requirements for the production of those materials and products may also affect the regulatory clearance of a third-party manufacturers' facility. If the FDA or a foreign regulatory agency does not approve these facilities for the manufacture of our product candidates, or if it withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would impede or delay our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

Our product and our product candidates may face future development and regulatory difficulties.

Our product and product candidates are subject to ongoing requirements by the FDA and foreign regulatory authorities governing the manufacture, quality control, further development, labeling, packaging, storage, distribution, safety surveillance, import, export, advertising, promotion, recordkeeping and reporting of safety and other post-market information. The FDA and foreign regulatory authorities will continue to monitor closely the safety profile of any product even after approval. If the FDA or foreign regulatory authorities become aware of new safety information after approval of a product candidate, they may require labeling changes or establishment of a REMS or similar strategy, impose significant restrictions on its indicated uses or marketing, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance.

In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations and standards. If we or a regulatory agency discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, the manufacturing facility or us, including withdrawal of the product from the market or suspension of manufacturing, or we may recall the product from distribution. If we, or our third-party manufacturers, fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products or refuse to permit the import or export of products.

The occurrence of any event or penalty described above may inhibit our ability to commercialize and generate revenue from the sale of our product candidates.

Advertising and promotion of any product candidate that obtains approval in the United States is heavily scrutinized by the FDA's Office of Prescription Drug Promotion, the Department of Justice, the Department of Health and Human Services' Office of Inspector General, state attorneys general, members of Congress, other government agencies and the public. While physicians may

prescribe products for off-label uses as the FDA and other regulatory agencies do not regulate a physician's choice of drug treatment made in the physician's independent medical judgment, they do restrict promotional communications from companies or their sales force with respect to off-label uses of products for which marketing clearance has not been issued. Companies may only share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. Violations, including promotion of our products for unapproved (or off-label) uses, may be subject to enforcement letters, inquiries and investigations, and civil and criminal sanctions by the government. Additionally, foreign regulatory authorities will heavily scrutinize advertising and promotion of any product candidate that obtains approval in their respective jurisdictions.

In the United States, engaging in the impermissible promotion of our products for off-label uses can also subject us to false claims litigation under federal and state statutes, which can lead to administrative, civil and criminal penalties, damages, monetary fines, disgorgement, individual imprisonment, exclusion from participation in Medicare, Medicaid and other federal healthcare programs, curtailment or restructuring of our operations and agreements that materially restrict the manner in which a company promotes or distributes drug products. These false claims statutes include, but are not limited to, the federal civil False Claims Act, which allows any individual to bring a lawsuit against an individual or entity, including a pharmaceutical or biopharmaceutical company on behalf of the federal government alleging the knowing submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment or approval by a federal program such as Medicare or Medicaid. These False Claims Act lawsuits against pharmaceutical or biopharmaceutical companies have increased significantly in number and breadth, leading to several substantial civil and criminal settlements regarding certain sales practices, including promoting off-label drug uses involving fines in excess of $1.0 billion. This growth in litigation has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance obligations, and be excluded from participation in Medicare, Medicaid and other federal and state healthcare programs. If we, or any partner that we may engage, do not lawfully promote our approved products, we may become subject to such litigation, which may have a material adverse effect on our business, financial condition and results of operations.

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

Undesirable side effects caused by our product and product candidates could cause the interruption, delay or halting of the trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other foreign regulatory authorities. Results of the clinical trials may reveal a high and unacceptable severity and prevalence of side effects or other unexpected characteristics. In such event, the trials could be suspended or terminated, or the FDA or foreign regulatory authorities could deny approval of our product candidates for any or all targeted indications. Drug-related side effects could affect patient recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects.

Additionally, if our product candidates receive marketing approval, and we or others later identify undesirable side effects, a number of potentially significant negative consequences could result, including:

- we may suspend marketing of, or withdraw or recall, the product;
- regulatory authorities may withdraw approvals;
- regulatory authorities may require additional warnings on the product labels;
- the FDA or other regulatory authorities may issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings about the product;
- the FDA may require the establishment or modification of a REMS or foreign regulatory authorities may require the establishment or modification of a similar strategy that may, for instance, restrict distribution of the product and impose burdensome implementation requirements on us;
- regulatory authorities may require that we conduct post-marketing studies;
- we could be sued and held liable for harm caused to subjects or patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates for use in targeted indications or otherwise materially harm its commercial prospects, if approved, and could harm our business, results of operations and prospects.

Our failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our product candidates outside the United States.

In order to market and sell our product candidates in other jurisdictions, we must obtain separate marketing approvals for those jurisdictions and comply with their numerous and varying regulatory requirements. We may not obtain foreign regulatory approvals on a timely basis, or at all. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, product reimbursement approvals must be secured before regulatory authorities will approve the product for sale in that country. 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 and regulatory approval in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. Our failure to obtain approval of our product candidates by foreign regulatory authorities may negatively impact the commercial prospects of such product candidates and our business prospects could decline. Also, if regulatory approval for our product candidates is granted, it may be later withdrawn. If we fail to comply with the regulatory requirements in international jurisdictions and receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential for our product candidates will be harmed and our business may be adversely affected.

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

Niktimvo and any product candidates that receives approval in the future would face significant competition from other therapies in the relevant indication. For example, cGVHD has historically been managed by off-label treatments. However, in the past several years, the FDA has approved three drugs, ibrutinib (*Imbruvica*®), belomosidil (*Rezurock*®) and ruxolitinib (*Jakafi*®), for use in patients with cGVHD after failure of one or more lines of systemic therapy. All three of these drugs may compete with Niktimvo in patients diagnosed with cGVHD.

Revumenib is being developed for the treatment of R/R adult and pediatric patients with KMT2Ar ALL, KMT2Ar AML and NPM1 mutant AML. At this time, there are no drugs approved for these defined populations and patients are managed using the standard of care treatment regimens developed for general AML and ALL populations. While there are other agents in early development for similar populations, revumenib has the potential to be the first defined therapy for patients with KMT2Ar ALL, KMT2Ar AML and/or NPM1 mutant AML.

Existing or potential competitors have or may have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Our competitors may be more successful than us in obtaining FDA approval for drugs and achieving widespread market acceptance. Our competitors' drugs may be more effective or more effectively marketed and sold than any drug we may commercialize and may render our product candidates obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our product candidates. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available.

We believe that our ability to successfully compete will depend on, among other things:

- the market adoption of Niktimvo and our other product candidates by physicians and patients;
- the efficacy and safety profile of our product candidates relative to marketed products and product candidates in development by third parties;
- the time it takes for our product candidates to complete clinical development and receive marketing approval;
- our ability to commercialize Niktimvo and our other product candidates if they receive regulatory approval;
- the price of Niktimvo and our product candidates, including in comparison to branded or generic competitors;
- whether coverage and adequate levels of reimbursement are available under private and governmental health insurance plans, including Medicare;
- our ability to manufacture commercial quantities of Niktimvo and our product candidates, if they receive regulatory approval; and
- our ability to negotiate preferential formulary status for our product candidates.

Even if we obtain regulatory approval of our other product candidates, the availability, commercial formulary placement, and price of our competitors' products could limit the demand and the price we are able to charge. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment, or if physicians switch to other new drug or biologic products or choose to reserve our drugs for use in limited circumstances.

Certain of our product candidates may require companion diagnostics in certain indications. Failure to successfully develop, validate and obtain regulatory clearance or approval for such tests could harm our product development strategy or prevent us from realizing the full commercial potential of our investigational products.

Companion diagnostics are subject to regulation by the FDA and comparable foreign regulatory authorities as a medical device and may require separate regulatory authorization prior to commercialization. Certain of our revumenib clinical trials include the use of an investigational or laboratory developed diagnostic test to help identify eligible patients. We currently do not have any plans to develop diagnostic tests internally. We are therefore dependent on the sustained cooperation and effort of third-party collaborators in developing and, if our investigational products are approved for use only with an approved companion diagnostic test, obtaining approval and commercializing these tests. If these parties are unable to successfully develop companion diagnostics for our investigational products, or experience delays in doing so, the development of our investigational products may be adversely affected and we may not be able to obtain marketing authorization for these investigational products. Furthermore, our ability to market and sell, as well as the commercial success, of any of our investigational products that require a companion diagnostic will be tied to, and dependent upon, the receipt of required regulatory authorization and the continued ability of such third parties to make the companion diagnostic commercially available on reasonable terms in the relevant geographies. Any failure to develop, validate, obtain and maintain marketing authorization and supply for a companion diagnostic we need may harm our business prospects.

We are dependent on UCB Biopharma Sprl, or UCB, to comply with the terms of our license agreement for axatilimab.

Our commercial success also depends upon our ability to develop, manufacture, market and sell axatilimab. We have a worldwide, sublicensable, exclusive license to axatilimab pursuant to a license agreement with UCB. Certain of the rights licensed to us under the UCB license agreement are in-licensed by UCB from third parties. We are dependent on UCB maintaining the applicable third-party license agreements in full force and effect, which may include activities and performance obligations that are not within our control. If any of these third-party license agreements terminate, certain of our rights to develop, manufacture, commercialize or sell axatilimab may be terminated as well. The occurrence of any of these events could adversely affect the development and commercialization of axatilimab, and materially harm our business.

Our employees, consultants and collaborators may engage in misconduct or other improper activities, including insider trading and non-compliance with regulatory standards and requirements.

We are exposed to the risk that our employees, consultants, distributors, and collaborators may engage in fraudulent or illegal activity. Misconduct by these parties could include intentional, reckless or negligent conduct or disclosure of unauthorized activities to us that violates the regulations of the FDA and non-U.S. regulators, including those laws requiring the reporting of true, complete and accurate information to such regulators, manufacturing standards, healthcare fraud and abuse laws and regulations in the United States and abroad or laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry, including the sale of pharmaceuticals, are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. We have adopted a code of conduct applicable to all of our employees, officers, directors, agents and representatives, including consultants, but it is not always possible to identify and deter misconduct by our employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a 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 the imposition of significant fines or other sanctions, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, disgorgement, individual imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings and curtailment of operations, any of which could adversely affect our ability to operate our business and our results of operations. Whether or not we are successful in defending against such actions or investigations, we could incur substantial costs, including legal fees, and divert the attention of management in defending ourselves against any of these claims or investigations.

We must attract and retain additional highly skilled employees in order to succeed.

We must recruit, retain, manage and motivate qualified clinical, scientific, technical, commercial and management personnel and we face significant competition for experienced personnel. If we do not succeed in attracting and retaining qualified personnel,

particularly at the management level, it could adversely affect our ability to execute our business plan and harm our operating results. In particular, the loss of one or more of our executive officers could be detrimental to us if we cannot recruit suitable replacements in a timely manner. The competition for qualified personnel in the pharmaceutical and biopharmaceutical industries is intense and as a result, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel.

Many of the other pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates than what we have to offer. If we are unable to continue to attract and retain high-quality personnel, the rate and success at which we can discover and develop product candidates and our business will be limited.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for Niktimvo or any future products could limit our ability to market those products and decrease our ability to generate revenue.

The pricing, coverage, and reimbursement of Niktimvo, and other product candidates, if approved, must be adequate to support our commercial infrastructure. Our per-patient prices must be sufficient to recover our development and manufacturing costs and potentially achieve profitability. Accordingly, the availability and adequacy of coverage and reimbursement by governmental and private payors are essential for most patients to afford expensive treatments such as ours. Sales of Niktimvo will depend substantially, both domestically and abroad, on the extent to which their costs will be paid for by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or reimbursed by government authorities, private health insurers, and other payors. If coverage and reimbursement are not available, are available only to limited levels, or are not available on a timely basis, we, along with Incyte, may not be able to successfully commercialize Niktimvo. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to sustain our overall enterprise.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, decides whether and to what extent a new drug will be covered and reimbursed under Medicare. Private payors tend to follow the coverage reimbursement policies established by CMS to a substantial degree. It is difficult to predict what CMS or private payors will decide with respect to reimbursement for a product such as ours.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products and, as a result, they may not cover or provide adequate payment for Niktimvo. We expect to experience pricing pressures in connection with the sale of Niktimvo due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, additional legislative changes, and statements by elected officials. The downward pressure on healthcare costs in general, and with respect to prescription drugs, surgical procedures, and other treatments in particular, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

Current and future legislation may increase the difficulty and cost for us to commercialize our product candidates and affect the prices we may obtain.

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidate for which we obtain marketing approval.

For example, then President Obama signed into law the Affordable Care Act. Among other cost containment measures, the Affordable Care Act established an annual, nondeductible fee on any entity that manufactures or imports branded prescription drugs and biologic agents, a Medicare Part D coverage gap discount program, and a formula that increased the rebates a manufacturer must pay under the Medicaid Drug Rebate Program.

There have been executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act.

While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the Affordable Care Act have been signed into law. The Tax Cuts and Jobs Act of 2017 includes a provision that repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the “individual mandate.” On June 17, 2021, the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the Affordable Care Act is unconstitutional in its entirety because the “individual mandate” was repealed by Congress. On August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 or IRA, into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in Affordable Care Act marketplaces through plan year 2025. The IRA also eliminates the “donut hole” under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket costs through a newly established manufacturer discount program. It is possible that the Affordable Care Act will be subject to judicial

or Congressional challenges in the future. It is unclear how any such challenges and the healthcare reform measures of the Biden administration will impact the Affordable Care Act and our business.

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which began in 2013, and due to subsequent legislative amendments to the statute, will remain in effect through 2032 unless additional Congressional action is taken.

Additional changes that may affect our business include the expansion of new programs such as Medicare payment for performance initiatives for physicians under the Medicare Access and CHIP Reauthorization Act of 2015, or MACRA, which ended the use of the statutory formula and established a quality payment program, also referred to as the Quality Payment Program. The Quality Payment Program consists of two payment tracks that eligible clinicians can participate in: Advanced Alternative Payment Models, APMs, and the Merit-Based Incentive Payment System, MIPS. Under both APMs and MIPS the Advanced Alternative Payment Models and the Merit-Based Incentive Payment System, performance data collected each performance year will affect Medicare payments in later years, including potentially reducing payments.

Also, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed products, which have resulted in several, Presidential executive orders, Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. At the federal level, in July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to President Biden's executive order, on September 9, 2021, the U.S. Department of Health and Human Services, or HHS, released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. In addition, the IRA, among other things, (i) directs the Secretary of HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare Part B and Medicare Part D, and subjects drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law, and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions took effect progressively starting in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon reimbursement prices of the first ten drugs that were subject to price negotiations, although the Medicare drug price negotiation program is currently subject to legal challenges. HHS will select up to fifteen additional drugs covered under Part D for price negotiation in 2025. In response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the CMS Innovation Center which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. Further, on December 7, 2023, the Biden administration announced an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act. March-in rights allow a federal agency to grant a compulsory license on a privately owned patent to third parties, if the invention was developed with federal funding and the agency finds that certain statutory criteria apply. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework. At the state level, legislatures have increasingly passed and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. For example, on January 5, 2024, the FDA approved Florida's Section 804 Importation Program, or SIP, proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this program will be implemented, including which drugs will be chosen, and whether it will be subject to legal challenges in the United States or Canada. Other states have also submitted SIP proposals that are pending review by the FDA. Any such approved importation plans, when implemented, may result in lower drug prices for products covered by those programs.

These and other healthcare reform measures may be adopted in the future, particularly in light of the upcoming U.S. Presidential and Congressional election, any of which may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. 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 products.

We are in the process of building our sales, marketing and distribution infrastructure.

In order to market any approved product candidate in the future, we must build our sales, marketing, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, as we do not presently have all of these capabilities. To develop our internal sales, distribution and marketing capabilities, we must invest significant amounts of financial and management resources in the future. For drugs where we decide to perform sales, marketing and distribution functions ourselves, we could face a number of challenges, including that:

- we may not be able to attract and build an effective marketing or sales organization;
- the cost of establishing, training and providing regulatory oversight for a marketing or sales force may not be justifiable in light of the revenues generated by any particular product;
- our direct or indirect sales and marketing efforts may not be successful; and
- there are significant legal and regulatory risks in drug marketing and sales that we have never faced, and any failure to comply with all legal and regulatory requirements for sales, marketing and distribution could result in enforcement action by the FDA or other authorities that could jeopardize our ability to market the product or could subject us to substantial liabilities.

Alternatively, we may rely on third parties to launch and market our product candidates, if approved. We may have limited or no control over the sales, marketing and distribution activities of these third parties and our future revenue may depend on the success of these third parties. Additionally, if these third parties fail to comply with all applicable legal or regulatory requirements, the FDA or another governmental agency could take enforcement action that could jeopardize their ability and our ability to market our product candidates.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of our product candidates.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human trials and will face an even greater risk if we commercially sell any products that we may develop. Product liability claims may be brought against us by subjects enrolled in our trials, patients, healthcare providers or others using, administering or selling our products. If we cannot successfully defend ourselves against claims that our product candidates or other products that we may develop caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for our product candidates;
- termination of clinical trial sites or entire trial programs;
- injury to our reputation and significant negative media attention;
- withdrawal of trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial subjects or patients;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize any products that we may develop.

While we currently hold trial liability insurance coverage consistent with industry standards, this may not adequately cover all liabilities that we may incur. We also may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise in the future. Our current insurance coverage includes commercial sales upon marketing approval for our product candidates. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business and financial condition.

Our relationships with healthcare providers, customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse, transparency and other healthcare laws and regulations as well as privacy and data security laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, fines, exclusion from participation in government healthcare programs, curtailments or restrictions of our operations, administrative burdens and diminished profits and future earnings.

Healthcare providers, including physicians and third-party payors 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 providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we conduct clinical research and market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include, but are not limited to, the following:

- the federal Anti-Kickback Statute prohibits persons from, among other things, knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, the referral of an individual for the furnishing or arranging for the furnishing, or the purchase, lease or order, or arranging for or recommending purchase, lease or order, or any good or service for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

- the federal Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other;
- the Affordable Care Act amended the intent requirement of the federal Anti-Kickback Statute so that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;
- the federal false claims, including the federal civil False Claims Act, impose criminal and civil penalties, including through civil whistleblower or qui tam actions, and civil monetary penalties laws, which prohibit knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which prohibits, among other things, knowingly and willfully executing, or attempting to execute, a scheme or artifice to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private), willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, also imposes obligations on covered entities, including certain health care providers, health plans and health care clearinghouses as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information for or on behalf of such covered entities, and their covered subcontractors, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to CMS information related to "payments or other transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians (as defined above) and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; state laws that require manufacturers to report pricing information regarding certain drugs; state and local laws that require the registration of pharmaceutical sales representatives; state and foreign laws that govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts; and federal, state, and foreign laws that govern the privacy and security of other personal information, including federal and state consumer protection laws, state data security laws, and data breach notification laws (a data breach affecting sensitive personal information, including health information, could result in significant legal and financial exposure and reputational damages).

Efforts to ensure that our business arrangements with third parties and our business generally, will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting 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 government funded healthcare programs, such as Medicare and Medicaid, contractual damages, reputational harm, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. Further, if any physician or other healthcare provider or entity with whom we expect to do business is found not to be in compliance with applicable laws, that person or entity may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

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

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share, or collectively, process, personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, sensitive third-party data, business plans, transactions, clinical trial data and financial information or collectively, sensitive data.

Our data processing activities subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). For example, the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, imposes specific requirements relating to the privacy, security, and transmission of individually identifiable protected health information. For more information regarding risks associated with HIPAA, please refer to the section above that discusses risks associated with healthcare laws and regulations.

In the past few years, numerous U.S. states—including California, Virginia, Colorado, Connecticut, and Utah—have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020, or CPRA and collectively, CCPA, applies to personal data of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. The CCPA provides for fines of up to $7,500 per intentional violation and allows private litigants affected by certain data breaches to recover significant statutory damages. Although there are limited exemptions for clinical trial data under the CCPA, the CCPA and other similar laws may impact (possibly significantly) our business activities depending on how it is interpreted, should we become subject to the CCPA in the future. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future. These developments may further complicate compliance efforts and increase legal risk and compliance costs for us and the third parties upon whom we rely.

Outside the United States, an increasing number of laws, regulations, and industry standards may govern data privacy and security. For example, the European Union's General Data Protection Regulation, or EU GDPR, and the United Kingdom's GDPR, or UK GDPR, impose strict requirements for processing personal data. For example, under GDPR, companies may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR, 17.5 million pounds sterling under the UK GDPR or, in each case, 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

We may be subject to new laws governing the privacy of consumer health data, including reproductive, sexual orientation, and gender identity privacy rights. For example, Washington's My Health My Data Act, or MHMD, broadly defines consumer health data, places restrictions on processing consumer health data (including imposing stringent requirements for consents), provides consumers certain rights with respect to their health data, and creates a private right of action to allow individuals to sue for violations of the law. Other states are considering and may adopt similar laws.

Our employees and personnel may occasionally use generative artificial intelligence, or AI, technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages.

In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the United States or other countries due to data localization requirements or limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic

Area, or EEA, and the United Kingdom, or UK, have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it generally believes are inadequate. Other jurisdictions may adopt similarly stringent interpretations of their data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA's standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activities groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data out of Europe for allegedly violating the GDPR's cross-border data transfer limitations.

In addition to data privacy and security laws, we are bound by other contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We also publish privacy policies, marketing materials, and other statements regarding data privacy and security and if these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources and may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties on which we rely fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans on processing personal data (including clinical trial data); and orders to destroy or not use personal data. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: loss of customers; inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations.

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

We are increasingly dependent on information technology systems and infrastructure, including mobile technologies, to operate our business. In the ordinary course of our business, we collect, store, process and transmit large amounts of sensitive data, and, as a result, we and the third parties upon which we rely face a variety of evolving threats that could cause security incidents. We have also outsourced elements of our operations (including elements of our information technology infrastructure) to third parties, and as a result, we manage a number of third-party vendors who may or could have access to our computer networks and our sensitive data. In addition, those third-party vendors may in turn subcontract or outsource some of their responsibilities to other parties. While all information technology operations are inherently vulnerable to inadvertent or intentional security breaches, incidents, attacks and exposures, the accessibility and distributed nature of our information technology systems, and the sensitive data stored on those systems, make such systems vulnerable to unintentional or malicious, internal and external attacks on our technology environment. Furthermore, our ability to monitor the aforementioned third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we

may be unable to recover such award. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been compromised.

In addition, we currently offer a hybrid-work environment, which may make us more vulnerable to cyberattacks as more of our employees utilize network connections, computers, and devices outside our premises or network, including working at home, while in transit and in public locations. Additionally, future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

Potential vulnerabilities can be exploited from inadvertent or intentional actions of our employees, third-party vendors, business partners, or by malicious third parties. We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties upon which we rely); however, we may not detect and remediate all such vulnerabilities including on a timely basis. Further, we may experience delays in deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident.

Cyberattacks, malicious internet-based activity, online and offline fraud, and other similar activities are increasing in their frequency, levels of persistence, sophistication and intensity, and are also being conducted by sophisticated and organized groups and individuals with a wide range of motives (including, but not limited to, industrial espionage) and expertise, including organized criminal groups, "hacktivists," nation states and others. Such attacks could include the deployment of harmful malware (including as a result of advanced persistent threat intrusions), ransomware attacks, denial-of-service attacks, credential stuffing and/or harvesting, social engineering (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of sensitive data or other information technology assets, adware, attacks enhanced or facilitated by AI, telecommunications failures, earthquakes, fires, floods and other means to affect service reliability and threaten the confidentiality, integrity and availability of our information systems and sensitive data. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

Significant disruptions of our, our third-party vendors' and/or business partners' information technology systems or other similar data security incidents could adversely affect our business operations and/or result in the loss, misappropriation and/or unauthorized access, use or disclosure of, or the prevention of access to, sensitive data, which could result in financial, legal, regulatory, business and reputational harm to us. In addition, information technology system disruptions, whether from attacks on our technology environment or from computer viruses, natural disasters, terrorism, war and telecommunication and electrical failures, could result in a material disruption of our development programs and our business operations. 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.

We may expend significant resources or modify our business activities to try to protect against security incidents. Additionally, certain data privacy and security obligations may require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive data.

Applicable data privacy and security obligations may require us to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences.

If we (or a third party upon whom we rely) experience a security incident or are perceived to have experienced a security incident, including but not limited to a security incident involving personal information regarding employees or clinical trial patients, we may experience adverse consequences, such as disruptions to our business, harm to our reputation, government enforcement actions (for example, investigations, fines, penalties, audits, and inspections), additional reporting requirements and/or oversight, or we may otherwise be subject to liability under laws, regulations, and contractual obligations, including those that protect the privacy and security of personal information. This could result in increased costs to us and result in significant legal and financial exposure and/or reputational harm. Any failure or perceived failure by us or our vendors or business partners to comply with our privacy, confidentiality or data security-related legal or other obligations to third parties, or any further security incidents or other inappropriate access events resulting in the unauthorized access, release or transfer of sensitive data, may result in governmental investigations, enforcement actions, regulatory fines, litigation, or public statements against us by advocacy groups or others, and could cause third parties, including clinical sites, regulators or current and potential partners, to lose trust in us or we could be subject to claims by third parties that we have breached our privacy or confidentiality-related obligations, which could materially and adversely affect our business and prospects. Moreover, data security incidents and other inappropriate access can be difficult to detect and any delay in identifying them may lead to increased harm of the type described above.

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

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

Social media platforms and AI-based platforms present new risks and challenges to our business.

As social media continues to expand, it also presents us with new risks and challenges. Social media is increasingly being used to communicate information about us, our programs and the diseases our product candidates are being developed to treat. Social media practices in the biopharmaceutical industry are evolving, creating uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media platforms to comment on the effectiveness of, or adverse experiences with, a product or a product candidate, which could result in reporting obligations or other consequences. Further, the accidental or intentional disclosure of non-public information by our workforce or others through media channels could lead to information loss. In addition, there is a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us, our products, or our product candidates on any social media platform. The nature of social media prevents us from having real-time control over postings about us on social media. We may not be able to reverse damage to our reputation from negative publicity or adverse information posted on social media platforms or similar mediums. If any of these events were to occur or we otherwise fail to comply with application regulations, we could incur liability, face restrictive regulatory actions or incur other harm to our business including quick and irreversible damage to our reputation, brand image and goodwill. Additionally, AI-based platforms are increasingly being used in the biopharmaceutical industry. The use of AI platforms by people, including our vendors, suppliers and contractors, with access to our proprietary and confidential information, including trade secrets, may continue to increase and may lead to the release of such information, which may negatively impact our company, including our ability to realize the benefit of our intellectual property.

Risks Related to Our Financial Position and Capital Needs

We have incurred net losses since our inception, except 2021, and anticipate that we will continue to incur net losses for the foreseeable future.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate efficacy or an acceptable safety profile, gain regulatory approval or be commercially viable. We are a clinical-stage biopharmaceutical company with limited operating history. The FDA approved Niktimvo in August 2024, and we, along with our partner Incyte, anticipate launching Niktimvo in the U.S. no later than early first quarter 2025. We have no other products approved for commercial sale and have not generated any product revenues to date, and we continue to incur significant research and development and other expenses related to our ongoing operations and clinical development of our product candidates. As a result, we are not and have never been profitable and have incurred losses in each period since our inception in 2005, except in 2021.

For the quarter ended September 30, 2024, we reported a net loss attributable to stockholders of $84.1 million. As of September 30, 2024, we had an accumulated deficit of $1.1 billion, which included non-cash charges for stock-based compensation, preferred stock accretion and historical extinguishment charges. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our pre-commercialization activities for, and our research and development of, and seek regulatory approvals for, our product candidates. We may also encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of growth of our expenses and our ability to generate revenues, if any. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We currently have no source of product revenue and may never achieve or maintain profitability.

Our ability to generate product revenue and become profitable depends upon our ability to successfully commercialize our product candidates. We do not anticipate generating revenue from the sale of our product candidates for the foreseeable future. Our ability to generate future product revenue also depends on a number of additional factors, including, but not limited to, our ability to:

- successfully complete the research and clinical development of, and receive regulatory approval for, our product candidates;

- launch, commercialize and achieve market acceptance of our product candidates, and if launched independently, successfully establish a sales, marketing and distribution infrastructure;
- continue to build a portfolio of product candidates through the acquisition or in-license of products, product candidates or technologies;
- initiate preclinical and clinical trials for any additional product candidates that we may pursue in the future;
- establish and maintain supplier and manufacturing relationships with third parties, and ensure adequate and legally compliant manufacturing of bulk drug substances and drug products to maintain that supply;
- obtain coverage and adequate product reimbursement from third-party payors, including government payors;
- establish, maintain, expand and protect our intellectual property rights; and
- attract, hire and retain additional qualified personnel.

In addition, because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of increased expenses, and if or when we will achieve or maintain profitability. In addition, our expenses could increase beyond expectations if we decide to or are required by the FDA or foreign regulatory authorities to perform studies or trials in addition to those that we currently anticipate. Even if we complete the development and regulatory processes described above, we anticipate incurring significant costs associated with launching and commercializing our current product candidates and any other product candidates we may develop.

Even if we generate revenues from the sale of our product candidates, we may not become profitable and may need to obtain additional funding to continue operations or acquire additional products that will require additional funding to develop them. If we fail to become profitable or do not sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations or even shut down.

We will likely require additional capital to finance our planned operations, which may not be available to us on acceptable terms, or at all. As a result, we may not complete the development and commercialization of, or obtain regulatory approval for our existing product candidates or develop new product candidates.

Our operations have consumed substantial amounts of cash since our inception, primarily due to our research and development efforts. We expect our research and development expenses to increase substantially in connection with our ongoing and planned activities. We believe that our existing cash, cash equivalents and short-term investments will fund our projected operating expenses and capital expenditure requirements for at least the next 12 months. Unexpected circumstances may cause us to consume capital more rapidly than we currently anticipate, including as a result of the global economic slowdown, including any recessions that have occurred or may occur in the future. In addition, we may discover that we need to conduct additional activities that exceed our current budget to achieve appropriate rates of patient enrollment, which would increase our development costs.

In any event, we will likely require additional capital to continue the development of, obtain regulatory approval for, and to commercialize our existing product candidates and any future product candidates. Any efforts to secure additional financing may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates.

While the long-term economic impacts associated with public health crises and global geopolitical tensions, like the ongoing wars between Russia and Ukraine and the Hamas-Israel wars as well as the conflicts in the Middle East, including between Israel and Hezbollah, are difficult to assess or predict, each of these events has caused significant disruptions to the global financial markets and contributed to a general global economic slowdown. Furthermore, inflation rates have increased recently to levels not seen in decades. Increased inflation may result in increased operating costs (including labor costs) and may affect our operating budgets. In addition, the U.S. Federal Reserve has raised and is expected to further raise, interest rates in response to concerns about inflation. Increases in interest rates, especially if coupled with reduced government spending and volatility in financial markets, may further increase economic uncertainty and heighten these risks. If the disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our financial condition and our ability to pursue our business strategy. We cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. If we do not raise additional capital when required or on acceptable terms, we may need to:

- delay, scale back or discontinue the development or commercialization of our product candidates or cease operations altogether;
- seek strategic alliances for our existing product candidates on terms less favorable than might otherwise be available; or
- relinquish, or license on unfavorable terms, our rights to technologies or any future product candidates that we otherwise would seek to develop or commercialize ourselves.

If we need to conduct additional fundraising activities and we do not raise additional capital in sufficient amounts or on terms acceptable to us, we may be unable to pursue development and commercialization efforts, which will harm our business, operating results and prospects.

Our future funding requirements, both short- and long-term, will depend on many factors, including:

- the initiation, progress, timing, costs and results of clinical trials of our product candidates;
- the outcome, timing and cost of seeking and obtaining regulatory approvals from the FDA and comparable foreign regulatory authorities, including the potential for such authorities to require that we perform more trials than we currently expect;
- the cost to establish, maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with licensing, preparing, filing, prosecuting, defending and enforcing any patents or other intellectual property rights;
- market acceptance of our product candidates;
- the cost and timing of selecting, auditing and developing manufacturing capabilities, and potentially validating manufacturing sites for commercial-scale manufacturing;
- the cost and timing for obtaining pricing, and coverage and reimbursement by third-party payors, which may require additional trials to address pharmacoeconomic benefit;
- the cost of establishing sales, marketing and distribution capabilities for our product candidates if any candidate receives regulatory approval and we determine to commercialize it ourselves;
- the costs of acquiring, licensing or investing in additional businesses, products, product candidates and technologies;
- the effect of competing technological and market developments;
- our need to acquire and implement additional internal systems and infrastructure, including compliance and financial and reporting systems, as we grow our company; and
- business interruptions resulting from geo-political actions, including war or the perception that hostilities may be imminent (such as the ongoing wars between Russia and Ukraine and the Hamas-Israel wars as well as the conflicts in the Middle East, including between Israel and Hezbollah), terrorism, natural disasters, including earthquakes, typhoons, floods and fires, or public health crises.

If we cannot expand our operations or otherwise capitalize on our business opportunities because we cannot secure sufficient capital, our business, financial condition and results of operations could be materially adversely affected.

Changes in tax laws or regulations could materially adversely affect our company.

New tax laws or regulations could be enacted at any time, and existing tax laws or regulations could be interpreted, modified or applied in a manner that is adverse to us, which could adversely affect our business and financial condition. For example, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act, enacted many significant changes to the U.S. tax laws, including changes in corporate tax rates, which collectively may impact the utilization of our NOLs and other deferred tax assets, the deductibility of expenses, and the taxation of foreign earnings. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the Coronavirus Aid, Relief and Economic Security Act, or the CARES Act, modified certain provisions of the Tax Act. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the CARES Act, or any newly enacted federal tax legislation. Most recently, the IRA included a number of significant drug pricing reforms, including the establishment of a drug price negotiation program within the U.S. Department of Health and Human Services that would require pharmaceutical manufacturers to charge a negotiated "maximum fair price" for certain selected drugs or pay an excise tax for noncompliance, the establishment of rebate payment requirements on manufacturers under Medicare Parts B and D to penalize price increases that outpace inflation, and a redesign of the Part D benefit, as part of which manufacturers are required to provide discounts on Part D drugs and Part D beneficiaries' annual out-of-pocket spending will be capped at $2,000 beginning in 2025. The impact of changes under the Tax Act, the CARES Act, the IRA, or future reform legislation could increase our future U.S. tax expense and could have a material adverse impact on our business and financial condition.

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

We have incurred substantial losses during our history. We do not expect to become profitable in the near future, and we may never achieve profitability. Unused losses generally are available to be carried forward to offset future taxable income, if any. Under Sections 382 and 383 of the Code if a corporation undergoes an "ownership change," generally defined as a greater than 50% change

(by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change taxable income or taxes may be limited. We last completed an analysis from January 1, 2021 through December 31, 2023 and determined that no ownership changes had occurred in that period. Prior analyses determined that on March 30, 2007, August 21, 2015, and May 4, 2020, ownership changes had occurred. We may also experience ownership changes in the future as a result of shifts in our stock ownership, some of which may be outside of our control. As a result, our ability to use our pre-change NOLs to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Risks Related to Intellectual Property

If we are unable to obtain or protect intellectual property rights, we may not be able to compete effectively in our market.

Our success depends in significant part on our and our licensors' and licensees' ability to establish, maintain and protect patents and other intellectual property rights and operate without infringing the intellectual property rights of others. We have filed patent applications both in the United States and in foreign jurisdictions to obtain patent rights to inventions we have discovered. We have also licensed from third parties rights to patent portfolios. Some of these licenses give us the right to prepare, file and prosecute patent applications and maintain and enforce patents we have licensed, and other licenses may not give us such rights.

The patent prosecution process is expensive and time-consuming, and we and our current or future licensors and licensees may not be able to prepare, file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we or our licensors or licensees will fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Moreover, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from or license to third parties and are reliant on our licensors or licensees. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. If our current or future licensors or licensees fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our licensors or licensees are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our current or future licensors' or licensees' patent rights are highly uncertain. Our and our licensors' or licensees' pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. The patent examination process may require us or our licensors or licensees to narrow the scope of the claims of our or our licensors' or licensees' pending and future patent applications, which may limit the scope of patent protection that may be obtained. It is possible that third parties with products that are very similar to ours will circumvent our or our licensors' or licensees' patents by means of alternate designs or processes. We cannot be certain that we are the first to invent the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware of that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by a court to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidate, but our competitors may achieve issued claims, including in patents we consider to be unrelated, which block our efforts or may potentially result in our product candidate or our activities infringing such claims. The possibility exists that others will develop products which have the same effect as our products on an independent basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our products. Our and our licensors' or licensees' patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications, and then only to the extent the issued claims cover the technology.

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

The portfolio that we licensed from UCB includes granted patents and patent applications with pending claims directed to the composition of matter of axatilimab (a humanized, full-length IgG4 (kappa light chain) antibody with high affinity for the CSF-1R) as

well as claims directed to methods of use of axatilimab. There is no guarantee that any further patents will be granted based on the pending applications we licensed from UCB or even if one or more patents are granted that the claims issued in those patents would cover axatilimab, methods of using axatilimab, or formulations of axatilimab. Based on the priority date and filing date of the applications in the portfolio we licensed from UCB, we expect that additional patents, if any, granted based on the currently pending applications would expire in 2036 or later should patent term extension be granted. The actual term of any patents granted based on the pending applications we licensed from UCB can only be determined after such patents are granted.

The portfolio that we licensed from Vitae Pharmaceuticals, or Vitae, which is now a subsidiary of AbbVie Inc., or AbbVie, includes granted patents and applications with pending claims directed to inhibitors of the interaction of menin with MLL and MLL fusion proteins, pharmaceutical compositions containing the same, and their use in the treatment of cancer and other diseases mediated by the menin-MLL interaction. There is no guarantee that any additional patents will be granted based on the pending applications that we licensed from Vitae or even if one or more patents are granted that the claims issued in those patents would cover the desired lead compounds, compositions, and methods of use thereof. Based on the priority date and filing date of the applications in the portfolio that we licensed from Vitae, we expect that a patent, if any, granted based on the currently pending applications would expire in 2037 or later should patent term extension be granted. The actual term of any patents granted based on the pending applications that we licensed from Vitae can only be determined after such patents are granted.

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

Filing, prosecuting, enforcing and defending patents on product candidates in all countries throughout the world is prohibitively expensive, and our or our licensors' intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we and our licensors may not be able to prevent third parties from practicing our and our licensors' inventions in countries outside the United States, or from selling or importing products made using our and our licensors' inventions in and into the United States or other jurisdictions. Competitors may use our and our licensors' technologies in jurisdictions where we have not obtained patent protection to develop their own products and may export otherwise infringing products to territories where we and our licensors have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates and our and our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

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

The requirements for patentability may differ in certain countries, particularly developing countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the United States, there is no link between regulatory approval of a drug and its patent status. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our or our licensors' patents, requiring us or our licensors to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for, and launch generic versions of our products. In addition to India, certain countries in Europe and developing countries, including China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our and our licensors' efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license.

If we breach the UCB license agreement related to axatilimab or if the UCB license agreement is otherwise terminated, we could lose the ability to continue the development and commercialization of axatilimab.

Our commercial success depends upon our ability to develop, manufacture, market and sell axatilimab. Subject to the achievement of certain milestone events, we may be required to pay UCB up to $119.5 million in one-time development and regulatory milestone payments over the term of the UCB license agreement. If we or any of our affiliates or sublicensees commercializes axatilimab, we will also be obligated to pay UCB low double-digit royalties on sales, subject to reduction in certain circumstances, as well as up to an aggregate of $250.0 million in potential one-time sales-based milestone payments based on achievement of certain annual sales thresholds. Under certain circumstances, we may be required to share a percentage of non-royalty income from sublicensees, subject to certain deductions, with UCB.

Either party may terminate the UCB license agreement in its entirety or with respect to certain countries in the event of an uncured material breach by the other party. Either party may terminate the UCB license agreement if voluntary or involuntary bankruptcy proceedings are instituted against the other party, if the other party makes an assignment for the benefit of creditors, or upon the occurrence of other specific events relating to the insolvency or dissolution of the other party. UCB may terminate the UCB license agreement if we seek to revoke or challenge the validity of any patent licensed to us by UCB under the UCB license agreement or if we procure or assist a third party to take any such action.

Unless terminated earlier in accordance with its terms, the UCB license agreement will continue on a country-by-country and product-by-product basis until the later of: (i) the expiration of all of the licensed patent rights in such country; (ii) the expiration of all regulatory exclusivity applicable to the product in such country; and (iii) 10 years from the date of the first commercial sale of the product in such country. We cannot determine the date on which our royalty payment obligations to UCB would expire because no commercial sales of axatilimab have occurred and the last-to-expire relevant patent covering axatilimab in a given country may change in the future.

If the UCB license agreement is terminated, we would not be able to develop, manufacture, market or sell axatilimab and would need to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all. In addition, our collaboration with Incyte to further develop and commercialize axatilimab is dependent upon the effectiveness of the UCB license agreement. If the UCB license agreement is terminated, Incyte may terminate our collaboration and our business could be adversely affected.

If we breach the license agreement related to revumenib or if the license agreement is otherwise terminated, we could lose the ability to continue the development and commercialization of revumenib.

Our commercial success depends upon our ability to develop, manufacture, market and sell revumenib. Subject to the achievement of certain milestone events, we may be required to pay Vitae, which is now a subsidiary of AbbVie, up to $99.0 million in one-time development and regulatory milestone payments over the term of the Vitae license agreement. In the event that we or any of our affiliates or sublicensees commercializes revumenib, we will also be obligated to pay Vitae low single to low double-digit royalties on sales, subject to reduction in certain circumstances, as well as up to an aggregate of $70.0 million in potential one-time sales-based milestone payments based on achievement of certain annual sales thresholds. Under certain circumstances, we may be required to share a percentage of non-royalty income from sublicensees, subject to certain deductions, with Vitae.

Either party may terminate the license agreement in its entirety or with respect to certain countries in the event of an uncured material breach by the other party. Either party may terminate the license agreement if voluntary or involuntary bankruptcy proceedings are instituted against the other party, if the other party makes an assignment for the benefit of creditors, or upon the occurrence of other specific events relating to the insolvency or dissolution of the other party. Vitae may terminate the license agreement if we seek to revoke or challenge the validity of any patent licensed to us by Vitae under the license agreement or if we procure or assist a third party to take any such action.

Unless terminated earlier in accordance with its terms, the license agreement will continue on a country-by-country and product-by-product basis until the later of: (i) the expiration of all of the licensed patent rights in such country; (ii) the expiration of all regulatory exclusivity applicable to the product in such country; and (iii) 10 years from the date of the first commercial sale of the product in such country. We cannot determine the date on which our royalty payment obligations to Vitae would expire because no commercial sales of revumenib have occurred and the last-to-expire relevant patent covering revumenib in a given country may change in the future.

If the license agreement is terminated, we would not be able to develop, manufacture, market or sell revumenib and would need to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all.

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

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve technological and legal complexity, and obtaining and enforcing biopharmaceutical patents is costly, time-consuming, and inherently uncertain. The 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 and our licensors' ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts, and the United States Patent and Trademark Office, or the USPTO, the laws and regulations governing patents could change in unpredictable ways that may weaken our and our licensors' ability to obtain new patents or to enforce existing patents and patents we and our licensors or collaborators may obtain in the future. In view of recent developments in U.S. patent laws, in spite of our efforts and the efforts of our licensors, we may face difficulties in obtaining allowance of our biomarker based patient selection patent claims or if we are successful in obtaining allowance of our

biomarker based patient selection claims, we or our licensor may be unsuccessful in defending the validity of such claims if challenged before a competent court.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our and our licensors' patent applications and the enforcement or defense of our or our licensors' issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the America Invents Act, was signed into law. The America Invents Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The USPTO recently developed new regulations and procedures to govern administration of the America Invents Act, and many of the substantive changes to patent law associated with the America Invents Act and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the America Invents Act will have on the operation of our business. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our or our licensors' patent applications and the enforcement or defense of our or our licensors' issued patents, all of which could harm our business and financial condition.

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

Periodic maintenance and annuity fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. 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. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitors might be able to enter the market, which would harm our business.

We may become involved in lawsuits to protect or enforce our intellectual property, which could be expensive, time-consuming and unsuccessful and have an adverse effect on the success of our business and on our stock price.

Third parties may infringe our or our licensors' patents or misappropriate or otherwise violate our or our licensors' intellectual property rights. In the future, we or our licensors may initiate legal proceedings to enforce or defend our or our licensors' intellectual property rights, to protect our or our licensors' trade secrets or to determine the validity or scope of intellectual property rights we own or control. Also, third parties may initiate legal proceedings against us or our licensors to challenge the validity or scope of intellectual property rights we own or control. The proceedings can be expensive and time-consuming and many of our or our licensors' adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we or our licensors can. Accordingly, despite our or our licensors' efforts, we or our licensors may not be able to prevent third parties from infringing upon or misappropriating intellectual property rights we own or control, particularly in countries where the laws may not protect our rights as fully as in the United States. Litigation could result in substantial costs and diversion of management resources, which could harm our business and financial results. In addition, in an infringement proceeding, a court may decide that a patent owned by or licensed to us is invalid or unenforceable or may refuse to stop the other party from using the technology at issue on the grounds that our or our licensors' patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our or our licensors' patents at risk of being invalidated, held unenforceable or interpreted narrowly.

Third-party pre-issuance submission of prior art to the USPTO, or opposition, derivation, reexamination, inter partes review or interference proceedings, or other pre-issuance or post-grant proceedings in the United States or other jurisdictions provoked by third parties or brought by us or our licensors or collaborators may be necessary to determine the priority of inventions with respect to our or our licensors' patents or patent applications. An unfavorable outcome could require us or our licensors to cease using the related technology and commercializing our product candidates, or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us or our licensors a license on commercially reasonable terms or at all. Even if we or our licensors obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us or our licensors. In addition, if the breadth or strength of protection provided by our or our licensors' patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates. Even if we successfully defend such litigation or proceeding, we may incur substantial costs and it may distract our management and other employees. We could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent.

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 process. 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 a downward effect on the price of shares of our common stock.

Third parties may initiate legal proceedings against us alleging that we infringe their intellectual property rights or we may initiate legal proceedings against third parties to challenge the validity or scope of intellectual property rights controlled by third parties, the outcome of which would be uncertain and could have an adverse effect on the success of our business.

Third parties may initiate legal proceedings against us or our licensors or collaborators alleging that we or our licensors or collaborators infringe their intellectual property rights or we or our licensors or collaborators may initiate legal proceedings against third parties to challenge the validity or scope of intellectual property rights controlled by third parties, including in oppositions, interferences, reexaminations, *inter partes* reviews or derivation proceedings before the United States or other jurisdictions. These proceedings can be expensive and time-consuming and many of our or our licensors' adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we or our licensors or collaborators can.

An unfavorable outcome could require us or our licensors or collaborators to cease using the related technology or developing or commercializing our product candidates, or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us or our licensors or collaborators a license on commercially reasonable terms or at all. Even if we or our licensors or collaborators obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us or our licensors or collaborators. 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. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Some of these employees executed proprietary rights, non-disclosure and non-competition agreements in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed confidential information or intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, for some of our in-licensed patents and patent applications, we do not have access to every patent assignment or employee agreement demonstrating that all inventors have assigned their rights to the inventions or related patents. As a result, we may be subject to claims of ownership by such inventors.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we successfully prosecute or defend against such claims, litigation could result in substantial costs and distract management.

Our inability to protect our confidential information and trade secrets would harm our business and competitive position.

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, third-party manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts both within and outside the United States may be less willing or unwilling to protect trade secrets. If a competitor lawfully obtained or independently developed any of our trade secrets, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Risks Related to Ownership of Our Common Stock and Other General Matters

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

The trading price of our common stock is highly volatile and subject to wide fluctuations in response to various factors, some of which we cannot control. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this Quarterly Report on Form 10-Q report, these factors include:

- the success of competitive products or technologies;
- regulatory actions with respect to our products or our competitors' products;
- actual or anticipated changes in our growth rate relative to our competitors;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures, collaborations or capital commitments;
- results of trials of our product candidates or those of our competitors;
- regulatory or legal developments in the United States and other countries;
- 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;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors; and
- general economic, industry, political and market conditions, including, but not limited to new or ongoing public health crises and the wars between Russia and Ukraine and Hamas and Israel wars as well as the conflicts in the Middle East, including between Israel and Hezbollah.

In addition, the stock market in general, and the Nasdaq Global Select Market, or Nasdaq, and biopharmaceutical companies in particular, frequently experiences extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of such companies. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects or developments may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of the above risks or any of a broad range of other risks, including those described in this "Risk Factors" section, could have a dramatic and negative impact on the market price of our common stock.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and share price.

The global economy, including credit and financial markets, has experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, bank failures, declines in consumer confidence, declines in economic growth, increases in unemployment rates, increases in inflation rates and uncertainty about economic stability. For example, the COVID-19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the global capital markets. Similarly, the current Russia-Ukraine war exacerbated volatility in the global capital markets and continues to disrupt the global supply chain and energy markets. Any such volatility and disruptions may have adverse consequences for us or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of political unrest or war, it may make any necessary debt or equity financing more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. Inflation can adversely affect us by increasing our costs, including personnel costs (wages). Any significant increases in inflation and related increases in interest rates could have a material adverse effect on our business, results of operations and financial condition.

We may sell additional equity or debt securities or enter into other arrangements to fund our operations, which may result in dilution to our stockholders and impose restrictions or limitations on our business.

Until we can generate a sufficient amount of profit from our products, if ever, we expect to finance future cash needs through public or private equity or debt offerings. If we raise additional funds through the issuance of additional equity or debt securities, it may result in dilution to our existing stockholders and/or increased fixed payment obligations. For example, in December 2023, we sold a total of 12,432,431 shares of our common stock in a public offering. The issuance of these shares of our common stock resulted, and any future issuance pursuant to any future public or private equity financing or future sales under the 2023 ATM Program will result, in dilution to our stockholders.

In addition, we have a significant number of stock options and pre-funded warrants outstanding. To the extent that these have been or may be exercised, stockholders may experience further dilution.

We may also seek additional funding through government or other third-party funding and other collaborations, strategic alliances and licensing arrangements. These financing activities may have an adverse impact on our stockholders' rights as well as on our operations, and such additional funding may not be available on reasonable terms, if at all. Furthermore, these securities may have rights senior to those of our common stock and could contain covenants that would restrict our operations and potentially impair our competitiveness, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business.

Additionally, if we seek funds through arrangements with collaborative partners, these arrangements may require us to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us. Any of these events could significantly harm our business, financial condition and prospects.

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

The trading market for our common stock is influenced by the research and reports that industry or securities analysts publish about us or our business. If no or few securities or industry analysts continue coverage of us, the trading price for our stock could be negatively impacted. If any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our trials or operating results fail to meet the expectations of analysts, our stock price could decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant influence control over matters subject to stockholder approval.

As of September 30, 2024, our executive officers, directors, and holders of 5% or more of our capital stock and their respective affiliates beneficially owned approximately 33.5% of our outstanding voting stock and options. As a result, these stockholders will continue to have a significant influence over all matters requiring stockholder approval. For example, these stockholders may be able to influence elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile, and in the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

If we fail to maintain an effective system of internal control over financial reporting in the future, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. We are required, under Section 404 of the Sarbanes-Oxley Act, to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. This assessment needs to include disclosure of any material weaknesses identified by our management in our internal control over financial reporting. A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting that results in more than a reasonable possibility that a material misstatement of annual or interim financial statements will not be prevented or detected on a timely basis. Section 404

of the Sarbanes-Oxley Act also generally requires an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting.

We are required to get an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. Our compliance with Section 404 requires that we incur substantial expense and expend significant management efforts. We currently do not have an internal audit group, and we may need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge and compile the system and process documentation necessary to perform the evaluation needed to comply with Section 404. We may not be able to complete our evaluation, testing and any required remediation in a timely fashion. During the evaluation and testing process, if we identify one or more material weaknesses in our internal control over financial reporting, we will be unable to assert that our internal control over financial reporting is effective. We cannot assure you that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting once that firm begin its Section 404 reviews, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Some provisions of our charter documents and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would benefit our stockholders and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, or remove our current management. These provisions include a classified board of directors, a prohibition on actions by written consent of our stockholders and the ability of our board of directors to issue preferred stock without stockholder approval. 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, who are responsible for appointing the members of our management. Because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under the DGCL, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our amended and restated certificate of incorporation or amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change of control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock and could also affect the price that some investors are willing to pay for our common stock.

Item 5. Other Information

Trading Arrangements

During the quarter ended September 30, 2024, our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated contracts, instructions or written plans for the purchase or sale of our securities as set forth in the table below.

			Type of Trading Arrangement				
Name and Position	**Action**	**Adoption/Termination Date**	**Rule 10b5-1***	**Non- Rule 10b5-1****	**Total Shares of Common Stock to be Sold**	**Total Shares of Common Stock to be Purchased**	**Expiration Date**
Michael A. Metzger Chief Executive Officer, Director	Adoption***	8/2/2024	X		171,195	-	9/10/2025

* Contract, instructions, or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act.
** "Non-Rule 10b5-1 trading arrangement" as defined in Item 408(c) of Regulation S-K under the Exchange Act.

*** The selling start date of Mr. Metzger's plan is January 2025, after the Company's current NDA PDUFA date of December 26, 2024, and the plan solely includes equity grants with expiration dates prior to September 10, 2025.

Item 6. Exhibits

Exhibit No.	Description
3.1	Amended and Restated Certificate of Incorporation of the Company (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on March 8, 2016).
3.2	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of the Company (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on May 18, 2023).
3.3	Amended and Restated Bylaws of the Company (incorporated herein by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on March 8, 2016).
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) promulgated under the Securities Exchange Act of 1934, as amended.
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) promulgated under the Securities Exchange Act of 1934, as amended.
32.1*	Certifications of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the Inline XBRL Document.
101.SCH	Inline XBRL Taxonomy Extension Schema with Embedded Linkbase Document
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, or otherwise subject to the liability of that section, and shall not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, 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 Section 13 or 15(d) 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.

Date: November 5, 2024

By: /s/ Michael A. Metzger
Michael A. Metzger
Chief Executive Officer
(Principal Executive Officer)

By: /s/ Keith A. Goldan
Keith A. Goldan
Chief Financial Officer and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATIONS

I, Michael A. Metzger, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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 5, 2024

By: /s/ Michael A. Metzger

Michael A. Metzger
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Keith A. Goldan, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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 5, 2024

By: /s/ Keith A. Goldan

Keith A. Goldan
Chief Financial Officer and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of Syndax Pharmaceuticals, Inc. (the "Company") for the period ended September 30, 2024, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his or her knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

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

Date: November 5, 2024

By /s/ Michael A. Metzger
Michael A. Metzger
Chief Executive Officer

Date: November 5, 2024

By /s/ Keith A. Goldan
Keith A. Goldan
Chief Financial Officer and Treasurer