



2025 ANNUAL REPORT

Form 10-K

A copy of our Annual Report on Form 10-K for the fiscal year ended December 31, 2025 (“**2025 10-K**”) is included as part of this 2025 Annual Report, has been furnished to all stockholders together with the proxy statement for the 2026 Annual Meeting of Stockholders, which is scheduled to be held on June 10, 2026, and is incorporated herein by reference. Additionally, copies of this 2025 Annual Report and the 2025 10-K are available without charge on the internet by visiting the EDGAR section of the Securities and Exchange Commission (SEC) website (www.sec.gov/edgar) or the Investors section of our website www.syndax.com. We will also provide a copy of the 2025 10-K without charge, upon written request from stockholders to Investor Relations at the address provided below.

The copy of the 2025 10-K that is included as part of this 2025 Annual Report is not accompanied by the exhibits that were filed with the SEC. These exhibits are accessible on the internet by visiting the EDGAR section of the SEC website (www.sec.gov/edgar) or the Investors section of our website www.syndax.com. We will furnish any printed copies of the exhibits to those stockholders who request copies upon payment to the Company of its reasonable expenses in furnishing the exhibits. Requests for copies of the exhibits should be made to Investor Relations at the address provided below.

Investor Relations

Syndax Pharmaceuticals, Inc.
730 Third Avenue, Floor 9
New York, New York 10017
Tel: 781-419-1400
Email: ir@syndax.com

Quarterly Reports and Proxy Statements

Our Quarterly Reports on Form 10-Q and proxy statements can be obtained on the internet by visiting the EDGAR section of the SEC website (www.sec.gov/edgar) or via the Investors section of our website www.syndax.com or by contacting Investor Relations.

2026 Annual Meeting of Stockholders

June 10, 2026, 12:00 p.m., Eastern time
Virtual meeting only via webcast at
www.meetnow.global/MUGAWRX

Common Stock Listing

NASDAQ Global Select Market
Trading Symbol: SNDX

Transfer Agent and Registrar

Fidelity Investments
Attn: Fidelity Stock Transfer Solutions
PO Box 770017
Cincinnati, OH 45277
Tel: 1-833-500-1036

Corporate Office

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730 Third Avenue, Floor 9
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References to websites are inactive textual references only and the contents of our website are not incorporated by reference into this 2025 Annual Report for any purpose.

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

FORM 10-K

(Mark One)

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

For the Fiscal Year Ended December 31, 2025

OR

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

For the Transition Period from _____ to _____

Commission File Number: 001-37708

Syndax Pharmaceuticals, Inc.

(Exact name of Registrant as specified in its charter)

Delaware
(State or Other Jurisdiction of
Incorporation or Organization)

2834
(Primary Standard Industrial
Classification Code Number)

32-0162505
(I.R.S. Employer
Identification Number)

730 Third Avenue, 9th Floor
New York, NY 10017

(781) 419-1400

(Address, Including Zip Code, and Telephone Number, Including Area Code, of Registrant's Principal Executive Offices)

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

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

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☒
No ☐

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Securities Act. Yes ☐
No ☒

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 the filing requirements for the past 90 days. Yes ☒ No ☐

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

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

Large Accelerated Filer ☒

Accelerated Filer ☐

Non-accelerated Filer ☐

Smaller Reporting
Company ☐

Emerging growth company ☐

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

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

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

The aggregate market value of the voting and non-voting of common equity held by non-affiliates of the registrant was \$799.2 million as of June 30, 2025 based on the closing price of \$9.36 as reported on the Nasdaq Global Select Market on such date. Shares of the registrant's common stock held by executive officers, directors, and their affiliates have been excluded from this calculation. The determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of February 23, 2026, there were 88,200,596 shares of common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for its 2025 Annual Meeting of Stockholders, which the registrant intends to file pursuant to Regulation 14A with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2025, are incorporated by reference into Part III of this Annual Report on Form 10-K.

Auditor Firm	#34	Auditor	Deloitte & Touche LLP	Auditor Location:	New York, New York
Id:		Name:			

TABLE OF CONTENTS

	<u>Page</u>
<u>SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS</u>	ii
<u>PART I</u>	
Item 1. <u>Business</u>	1
Item 1A. <u>Risk Factors</u>	21
Item 1B. <u>Unresolved Staff Comments</u>	57
Item 1C. <u>Cybersecurity</u>	57
Item 2. <u>Properties</u>	59
Item 3. <u>Legal Proceedings</u>	59
Item 4. <u>Mine Safety Disclosures</u>	59
<u>PART II</u>	
Item 5. <u>Market for Registrant’s Common Equity, Related Stockholder Matters and Issuers Purchases of Equity Securities</u>	60
Item 6. <u>[Reserved]</u>	62
Item 7. <u>Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>	63
Item 7A. <u>Quantitative and Qualitative Disclosures about Market Risk</u>	75
Item 8. <u>Financial Statements and Supplementary Data</u>	76
Item 9. <u>Changes in and Disagreements with Accountants on Accounting and Financial Disclosure</u>	76
Item 9A. <u>Controls and Procedures</u>	76
Item 9B. <u>Other Information</u>	78
Item 9C. <u>Disclosure Regarding Foreign Jurisdictions that Prevent Inspections</u>	78
<u>PART III</u>	
Item 10. <u>Directors, Executive Officers and Corporate Governance</u>	79
Item 11. <u>Executive Compensation</u>	79
Item 12. <u>Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters</u>	79
Item 13. <u>Certain Relationships and Related Transactions, and Director Independence</u>	79
Item 14. <u>Principal Accountant Fees and Services</u>	79
<u>PART IV</u>	
Item 15. <u>Exhibit and Financial Statement Schedules</u>	80
Item 16. <u>Form 10-K Summary</u>	84

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, or Annual Report, contains forward-looking statements 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, or the Exchange Act. All statements other than statements of historical fact are “forward-looking statements” for purposes of this Annual Report. 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 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, or UCB, and Vitae Pharmaceuticals LLC (formerly Vitae Pharmaceuticals, Inc.), or Vitae, a subsidiary of AbbVie Inc., or AbbVie;
- 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 REVUFORJ[®] (revumenib) and NIKTIMVO[™] (axatilimab-csfr) and our product candidates by physicians and patients;
- developments relating to our competitors and our industry; and
- the impact of geopolitical actions, including tariffs, war or the perception that hostilities may be imminent (such as the war between Russia and Ukraine and ongoing conflicts in the Middle East), 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.

Factors that may cause actual results to differ materially from current expectations include, among other things, those set forth in Part I, Item 1A, “Risk Factors,” below and for the reasons described elsewhere in this Annual Report. Any forward-looking statement in this Annual Report reflects our current view with respect to future events and is subject to these and other risks, uncertainties and assumptions. Given these uncertainties, you should not rely on these forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, our information may be incomplete or limited, and we cannot guarantee future results. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Annual Report also contains estimates, projections and other information concerning our industry, our business and the markets for certain drugs and consumer products, including data regarding the estimated size of those markets, their projected growth rates and the incidence of certain medical conditions. Information that is based on estimates, forecasts, projections or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained these industry, business, market and other data from reports, research surveys, studies and similar data prepared by third parties, industry, medical and general publications, government data and similar sources and we have not independently verified the data from third party sources. In some cases, we do not expressly refer to the sources from which these data are derived.

In this Annual Report, unless otherwise stated or as the context otherwise requires, references to “Syndax,” “the Company,” “we,” “us,” “our” and similar references refer to Syndax Pharmaceuticals, Inc. and its wholly owned subsidiaries. This Annual Report also contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to, including logos, artwork and other visual displays, may appear without the ® or TM symbols, but such references are not intended to indicate, in any way, that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto. We do not intend our use or display of other companies’ trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

PART I

Item 1. BUSINESS

Our Company

We are a commercial-stage biopharmaceutical company advancing innovative cancer therapies. We currently have two commercially approved medicines, Revuforj® (revumenib) and Niktimvo™ (axatilimab-csfr), and a robust slate of clinical development programs designed to unlock the full potential of our first two products.

Revuforj is our first-in-class menin inhibitor that was approved by the U.S. Food and Drug Administration, or FDA, in November 2024 for the treatment of relapsed or refractory, or R/R, acute leukemia with a lysine methyltransferase 2A gene, or KMT2A, translocation in adult and pediatric patients one year old and older. In October 2025, Revuforj received a second approval from the FDA for the treatment of R/R acute myeloid leukemia, or AML, with a susceptible nucleophosmin 1 mutation, or NPM1m, in adult and pediatric patients one year and older who have no satisfactory alternative treatment options. We are also studying revumenib in combination with standard-of-care agents in NPM1m AML and KMT2A-rearranged, or KMT2Ar, acute leukemia across the treatment landscape, including in newly diagnosed patients. Additionally, we are exploring the potential for menin inhibition in the treatment of myelofibrosis, or MF.

Niktimvo is our first-in-class colony stimulating factor-1 receptor, or CSF-1R, blocking antibody that was approved by the FDA in August 2024 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. Axatilimab is in development for the treatment of newly diagnosed cGVHD patients in combination with standard of care therapies, and for the treatment of idiopathic pulmonary fibrosis, or IPF.

We licensed the global rights to revumenib and axatilimab, the first two FDA approved medicines to emerge from our pipeline. We are leading the commercialization and further development of revumenib and working closely with our collaboration partner, Incyte, on the commercialization and further development of axatilimab. 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.

Our Strategy

- Successfully commercialize Revuforj in the U.S. for R/R acute leukemia patients with a KMT2A translocation and R/R AML patients with an NPM1 mutation; establish Revuforj as the preferred menin inhibitor, leveraging our first mover advantage and robust clinical data supporting the product's profile.
- Advance a robust pipeline of frontline trials of revumenib in combination with standard-of-care therapies to support potential additional listings in the NCCN Clinical Practice Guidelines in Oncology, or NCCN Guidelines®, and/or label expansion opportunities.
- Evaluate opportunities to commercialize Revuforj outside of the United States, potentially on our own or in collaboration with partners who have operations and expertise outside the United States.
- Successfully commercialize Niktimvo in the U.S. in partnership with Incyte, for the treatment of cGVHD patients who have failed at least two prior lines of systemic therapy.
- Continue to advance clinical development programs designed to unlock the opportunity for Niktimvo to address newly diagnosed patients with cGVHD and other diseases, starting with IPF.
- Leverage the technical, clinical, regulatory and business expertise of our management team, scientific collaborators, and organization and network of advisors to license, acquire and develop additional cancer therapies to expand our pipeline and development plans.

We believe that strong execution of our strategy will position us to realize our mission to extend and improve the lives of cancer patients.

Our Products & Ongoing Development Programs

We have a product portfolio consisting of two FDA-approved products, Revuforj (revumenib) and Niktimvo (axatilimab-csfr), and a robust slate of clinical development programs designed to expand the opportunities for both medicines.

Revuforj (revumenib)

Revuforj in R/R Acute Leukemia with a KMT2A translocation

Revuforj is our oral, first-in-class menin inhibitor that was approved by the FDA in November 2024 for the treatment of R/R acute leukemia with a KMT2A translocation in adult and pediatric patients one year and older. The approval was based on an FDA analysis of 104 patients with R/R acute leukemia with a KMT2A translocation who were treated with Revuforj in the Phase 1/2 AUGMENT-101 trial. In the efficacy population, the rate of complete remission, or CR, plus CR with partial hematological recovery, or CRh, was 21% (22/104 pts; 95% confidence interval [CI]: 13.8%, 30.3%). The median duration of CR+CRh was 6.4 months (95% CI: 2.7, not estimable) and the median time to CR or CRh was 1.9 months (range: 0.9, 5.6 months). 23% (24/104 pts) of patients underwent hematopoietic stem cell transplantation, or HSCT, following treatment with Revuforj. Results from the 104-patient efficacy analysis are consistent with the previously reported, protocol-defined Phase 2 interim analysis of patients with R/R KMT2Ar acute leukemia in the AUGMENT-101 trial (n=57) which were published in the Journal of Clinical Oncology in August 2024.

The original safety evaluation of Revuforj was based on an FDA analysis of 135 patients with R/R acute leukemia with a KMT2A translocation who were treated with Revuforj. The most common adverse reactions ($\geq 20\%$) including laboratory abnormalities were hemorrhage, nausea, phosphate increased, musculoskeletal pain, infection, aspartate aminotransferase increased, febrile neutropenia, alanine aminotransferase increased, parathyroid hormone intact increased, bacterial infection, diarrhea, differentiation syndrome, electrocardiogram QT prolonged, phosphate decreased, triglycerides increased, potassium decreased, decreased appetite, constipation, edema, viral infection, fatigue, and alkaline phosphatase increased. Adverse reactions leading to dose reduction or permanent discontinuation were low at 10% and 12% of patients, respectively.

In late November 2024, we launched Revuforj for commercial sale in the United States. In December 2024, revumenib was added to the NCCN Guidelines for AML and acute lymphoblastic leukemia, or ALL, as a category 2A recommendation for R/R acute leukemia with a KMT2A rearrangement. In October 2025, the indication for Revuforj in R/R acute leukemia with a KMT2A translocation was updated to include language referring to the use of an FDA-authorized test to detect KMT2A translocations.

Revuforj in R/R AML with an NPM1 mutation

In September 2025, revumenib was added to the NCCN Guidelines for R/R AML with an NPM1 mutation.

In October 2025, Revuforj received a second approval from the FDA for the treatment of R/R AML with a susceptible NPM1 mutation in adult and pediatric patients one year and older who have no satisfactory alternative treatment options.

The approval of Revuforj in R/R NPM1m AML was based on efficacy data from patients with R/R NPM1m AML in the Phase 2 portion of the pivotal AUGMENT-101 trial. The rate of CR plus CRh was 23% (15/65 pts; 95% CI: 14%, 35%). The median time to CR or CRh response was 2.8 months and the median duration of CR or CRh was 4.5 months. Results from the R/R NPM1m AML cohort in the Phase 2 portion of the AUGMENT-101 trial were published in the journal Blood and presented at the 2025 European Hematology Association, or EHA, Annual Congress.

In connection with the approval of Revuforj in R/R NPM1m AML, the Revuforj U.S. Prescribing Information was updated to include safety data from 241 patients (207 adult and 34 pediatric patients) with R/R acute leukemia with an NPM1 mutation or a KMT2A translocation who were treated with Revuforj in clinical trials. The most common adverse reactions in this combined population were consistent with the previously reported known safety profile of Revuforj. The updated Prescribing Information contains a boxed warning for differentiation syndrome, QTc prolongation, and Torsades de Pointes.

Revumenib Development Programs

We are studying the use of revumenib in multiple genetic subtypes of acute leukemia across the treatment continuum, including in combination with standard-of-care therapies in newly diagnosed patients with NPM1m and KMT2Ar AML.

Overview of revumenib trials and development pipeline

Revumenib (select trials)						Ph 1	Ph 2	Ph 3	FDA Approved
Setting	Study Name	Regimen	NPM1m	KMT2Ar	NUP98r				
R/R	AUGMENT-101	Rev mono	•	•					
	AUGMENT-102	Rev + IC	•	•	•				
	SAVE	Rev + ven/oral HMA	•	•	•				
Post-HSCT Maintenance	Ball study	Rev mono	•	•					
1L	Unfit for IC	BEAT AML	•	•					
		SAVE	•	•	•				
		EVOLVE-2	•	•					
	Fit for IC	708 and NCI	•	•	•				
		REVEAL-ND	•						
		RAVEN		•					

Multiple trials evaluating revumenib across the acute leukemia treatment continuum are ongoing, such as:

- **EVOLVE-2:** In the first quarter of 2025, we initiated a pivotal, Phase 3, randomized, double-blind, placebo-controlled trial of revumenib in combination with venetoclax and azacitidine in newly diagnosed NPM1m (primary efficacy analysis population) and KMT2Ar patients who are unfit for intensive chemotherapy. The trial is being conducted in collaboration with the HOVON network, a leading cooperative clinical trial group with extensive experience studying novel therapies for hematologic malignancies. The trial has dual primary endpoints of CR and overall survival, both in NPM1m patients only, to support the potential for accelerated and full approval, respectively.
- **REVEAL-ND:** In November 2025, we initiated a pivotal, Phase 3, randomized, double-blind, placebo-controlled trial of revumenib in combination with intensive chemotherapy in newly diagnosed NPM1m AML patients. The trial has dual primary endpoints of measurable residual disease, or MRD, negative CR and event free survival to support the potential for accelerated approval and full approval, respectively.
- **SAVE:** A Phase 1/2 trial evaluating an all-oral combination of revumenib with venetoclax and decitabine/cedazuridine in pediatric and adult patients with newly diagnosed and R/R AML or mixed-lineage acute leukemia harboring either NPM1m, KMT2Ar, or NUP98r alterations. The trial is being conducted by investigators from MD Anderson Cancer Center. New data presented at the 2025 American Society of Hematology, or ASH, Annual Meeting from the first cohort of newly diagnosed patients showed a CR rate of 76% (16/21), an overall response rate, or ORR, of 86% (18/21), and a MRD negativity rate among responders of 100% (18/18).
- **Intensive chemotherapy:** Two ongoing Phase 1 trials, referred to as the 708 and NCI trial, are evaluating the combination of revumenib with intensive chemotherapy (7+3) in newly diagnosed NPM1m or KMT2Ar acute leukemia patients. Preliminary data presented from both trials at the 2025 ASH Annual Meeting showed that the safety profile of the combination was consistent with the profile for intensive chemotherapy alone, along with high rates of response and MRD negativity.
- **BEAT AML:** A Phase 1 trial evaluating the combination of revumenib with venetoclax and azacitidine in newly diagnosed older adults (≥ 60 years) with NPM1m or KMT2Ar AML. The trial is being

conducted as part of the Leukemia & Lymphoma Society's Beat AML® Master Clinical Trial. Data presented at the 2025 EHA Annual Congress and simultaneously published in the *Journal of Clinical Oncology* showed a CR rate of 67% (29/43), an ORR of 88% (38/43), and a MRD negativity rate of 100% (37/37) among responders.

- Post-transplant maintenance: A Phase 1 trial, referred to as the Ball study, is evaluating the safety and preliminary efficacy of revumenib as post-transplant maintenance after HSCT in patients with KMT2Ar or NPM1m acute leukemia. The trial is being conducted by investigators from the City of Hope Medical Center.
- 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.
- 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. Data presented at the 2024 ASH Annual Meeting showed that 54% (6/11) of patients had MRD reduction at any time, including 36% (4/11) who achieved MRD negativity.

In addition to the ongoing trials described above, we expect to initiate the RAVEN trial in the second half of 2026. RAVEN is a Phase 2 collaborative trial of revumenib in combination with venetoclax and azacitidine in newly diagnosed KMT2Ar patients who would be considered eligible, or fit, for intensive chemotherapy. The RAVEN trial seeks to explore if the combination can provide high rates of clinical activity without the toxicity associated with intensive chemotherapy.

Revumenib in MF

We plan to initiate a program in 2026 designed to generate proof-of-principle clinical data with revumenib in MF. This program builds on preclinical data presented at the 2025 ASH Annual Meeting which showed that menin inhibition has the potential for disease-modifying activity by reversing key pathologic features of myeloproliferative neoplasms, or MPNs, including those seen in MF.

Revumenib in colorectal cancer (CRC)

In January 2026, we reported Phase 1 results from a Phase 1/2 proof-of-concept trial designed to assess the safety, tolerability, and anti-tumor activity of revumenib in patients with advanced CRC and other solid tumors at the ASCO Gastrointestinal Cancer Symposium. The study met the Phase 1 primary endpoint, demonstrating that revumenib was well tolerated. Limited anti-tumor efficacy was observed, with no patients achieving a clinical response and a 10.5% disease control rate. We have decided not to pursue further development of revumenib in CRC.

Niktimvo (axatilimab)

Niktimvo (axatilimab-csfr) is our first-in-class CSF-1R-blocking antibody that was approved by the FDA in August 2024 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.

The FDA approval was based on data from the global AGAVE-201 study evaluating the safety and efficacy of Niktimvo in 241 adult and pediatric patients with refractory cGVHD who received at least two prior lines of systemic therapy. The trial met the primary endpoint across all cohorts receiving Niktimvo. Results from the study showed durable responses across all organs studied and patient subgroups. Among patients who received Niktimvo at the approved dose of 0.3 mg/kg every two weeks (n=79), 75% achieved an overall response rate (ORR) within the first six months of treatment, with a median time to response of 1.5 months. Additionally, 60% maintained a response at 12 months (measured from first response to new systemic therapy or death, based on the Kaplan Meier estimate). The trial also met a key exploratory endpoint, with a majority (56%) of patients achieving a ≥ 7 -point improvement in the modified Lee Symptom Scale score. Organ-specific complete and partial responses were demonstrated across all organs studied that are affected by cGVHD, including lower gastrointestinal, or GI, upper

GI, esophagus, joints/fascia, mouth, lungs, liver, eyes and skin. The most common ($\geq 15\%$) adverse reactions, including laboratory abnormalities, were increased aspartate aminotransferase, infection (pathogen unspecified), increased alanine aminotransferase, decreased phosphate, decreased hemoglobin, viral infection, increased gamma glutamyl transferase, musculoskeletal pain, increased lipase, fatigue, increased amylase, increased calcium, increased creatine phosphokinase, increased alkaline phosphatase, nausea, headache, diarrhea, cough, bacterial infection, pyrexia and dyspnea.

In September 2024, the pivotal data from the AGAVE-201 trial were published in the New England Journal of Medicine. In August 2024, axatilimab-csfr was added to the latest NCCN Guidelines as a category 2A recommendation for the treatment of chronic GVHD after the failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg.

In late January 2025, we launched Niktimvo for commercial sale in the U.S. in partnership with Incyte and in accordance with the collaboration agreement and license agreement with Incyte described below under the heading “Collaborations - Incyte Collaboration and License Agreement.” Under the agreement, we are co-commercializing Niktimvo along with Incyte.

Axatilimab Development Programs

Axatilimab is in development for the treatment of newly diagnosed chronic GVHD patients and for other fibrotic diseases, such as IPF.

Axatilimab is a monoclonal antibody targeting CSF-1R, a cell surface protein thought to control the survival and function of monocytes and macrophages. Axatilimab binds with high affinity to CSF-1R and blocks the binding of the two known CSF-1R ligands CSF-1 and IL-34. CSF-1R is expressed on the surface of specific immune cells known as macrophages and their precursor cells known as monocytes. CSF-1R signaling on these cells has been demonstrated in preclinical studies conducted in animal models of skin and lung cGVHD to be the key regulatory pathway involved in the expansion and infiltration of the macrophages that mediate fibrosis and the cGVHD disease process. Blocking CSF-1R activity with an experimental CSF-1R antibody in these studies was shown to prevent and treat the symptoms of cGVHD. We believe that by inhibiting CSF-1R activation on monocytes and macrophages, axatilimab also has the potential to be used to treat other fibrotic diseases where monocyte-derived macrophages have been shown to play a significant role.

Our near-term focus is to investigate the potential for axatilimab to provide meaningful clinical benefit earlier in the treatment of cGVHD and to establish proof-of-concept for the use of axatilimab to treat other fibrotic diseases where monocyte-derived macrophages have been shown to play a role.

Overview of axatilimab trials and development pipeline

Axatilimab (select trials)			Ph 1	Ph 2	Ph 3	FDA Approved
Setting	Study Name	Regimen				
R/R cGVHD	AGAVE-201	Axa mono				
1L cGVHD	AXemplify-357*	Axa + corticosteroids				
	NCT06388564*	Axa + ruxolitinib				
IPF	MAXPIRe	Axa on top of SOC				

*Trials led by Incyte. List is not inclusive of ongoing ex-U.S. trials.

Axatilimab for the treatment of newly diagnosed cGVHD patients

Our partner, Incyte, has initiated two clinical trials studying the use of axatilimab in combination with standard of care cGVHD therapies in the frontline setting.

One trial is a Phase 2, open-label, randomized, multicenter trial of axatilimab in combination with ruxolitinib in patients 12 years of age and older with newly diagnosed cGVHD. Interim safety data presented at the 2025 ASH Annual Meeting showed that the combination was well tolerated. Topline data from the trial is anticipated in early 2027.

The second trial, referred to as AXemplify-357, is a Phase 3, randomized, double-blind, placebo-controlled, multi-center trial that will investigate the use of axatilimab in combination with corticosteroids as initial treatment for cGVHD. Topline data from the trial is anticipated in early 2028.

Axatilimab for the treatment of IPF

In the first quarter of 2026, we completed enrollment in MAXPIRe, our randomized, double-blind, placebo-controlled, 26-week, Phase 2 trial of axatilimab on top of standard-of-care therapies in patients with IPF. The ongoing trial will assess the efficacy, safety and tolerability of axatilimab. We expect to report topline data from the trial in the fourth quarter of 2026.

Entinostat

Entinostat is our oral, small molecule product candidate that has direct effects on both cancer cells and immune regulatory cells, potentially enhancing the body's immune response to tumors. In March 2007, we 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. We have deprioritized the development of entinostat, but maintain a license, development and commercialization agreement, or the Eddingpharm License Agreement, with Eddingpharm Investment Company Limited, or Eddingpharm, under which we granted Eddingpharm an exclusive license under our intellectual property rights to develop and commercialize entinostat in China and certain other Asian countries. In April 2024, entinostat received marketing approval in China.

Disease and Market Overviews

KMT2A-Rearranged (KMT2Ar) Acute Leukemia

KMT2Ar, also known as mixed lineage leukemia rearranged, acute leukemias arise by rare, spontaneous translocations at the MLL1 locus (11q23). It is estimated that approximately 10% of AML and ALL harbor a KMT2A rearrangement with a worldwide incidence of approximately 5,000 to 7,000 cases per year. KMT2Ar acute leukemia is routinely diagnosed through currently available cytogenetic or molecular diagnostic techniques.

KMT2A-rearrangements give rise to an aggressive form of acute leukemia that is associated with a very poor prognosis and high relapse rates. It is estimated that more than 95% of patients with KMT2Ar acute leukemia have a KMT2A translocation, a type of rearrangement that occurs when part of one chromosome breaks and fuses to a different chromosome. More than half of patients with KMT2Ar acute leukemia will relapse after receiving conventional frontline therapies, with a median overall survival, or OS, of less than one year. With third line treatment or beyond, only 5% of patients achieve complete remission, and the median OS is less than three months.

Revuforj is the first and only FDA-approved treatment for R/R acute leukemia with a KMT2A translocation. Currently there are several other clinical-stage menin inhibitors in development for the treatment of KMT2Ar acute leukemias.

Mutant NPM1 (NPM1m) Acute Myeloid Leukemia (AML)

Mutations in the NPM1 gene are the most common genetic alteration in adult AML and are observed in approximately 30% of cases. Patients with R/R NPM1m AML have a poor prognosis and high unmet need.

Like KMT2Ar acute leukemia, NPM1m is readily diagnosed as part of the standard AML patient work-up today. Revuforj is the first and only treatment FDA approved in both adults and children with R/R NPM1m AML. There is one other menin inhibitor, Komzifti™ (ziftomenib), that is FDA approved in adults only with R/R NPM1m AML. There are several additional clinical stage agents currently advancing as potential treatments for NPM1m AML.

Myelofibrosis (MF)

MF is a subtype of a group of blood cancers called MPNs. MF affects the bone marrow and the production of blood cells. It is characterized by bone marrow fibrosis, extramedullary hematopoiesis, splenomegaly, cytopenias, and systemic symptoms. MF is estimated to affect approximately 16,000 to 20,000 patients in the United States.

There are currently four FDA approved therapies for MF, Jakafi® (ruxolitinib), Inrebic® (fedratinib), Vonjo® (pacritinib), and Ojjaara® (mometinib), all of which are JAK inhibitors. While these drugs reduce symptom burden and spleen volume, they do not substantially reduce the degree of bone marrow fibrosis, and most patients stop responding to JAK inhibitors within two to three years. Therefore, new therapeutic strategies are needed.

Preclinical data presented at the 2025 ASH Annual Meeting showed that menin inhibition has the potential for disease-modifying activity by reversing key pathologic features of MPNs, including those seen in MF. *In vitro* studies showed that revumenib caused a decrease in megakaryocyte progenitor cells and led to fewer mature megakaryocytes without affecting other blood cell lineages. *In vivo* data from MPN mouse models showed that revumenib lowered leukocyte and platelet counts, reduced megakaryocyte numbers in the bone marrow, ablated bone marrow fibrosis, normalized hemoglobin and hematocrit levels, and reduced spleen size. Certain effects were enhanced when revumenib was combined with ruxolitinib. These preclinical data provide rationale for the further investigation of menin inhibition in the treatment of MF.

Chronic Graft-Versus-Host Disease (cGVHD)

cGVHD, an immune response of the donor-derived hematopoietic cells against recipient tissues, is a serious, potentially life-threatening complication of allogeneic HSCT that can last for years. cGVHD is a leading cause of significant morbidity and mortality after HSCT and is estimated to develop in approximately 42% of transplant recipients, affecting approximately 17,000 patients in the U.S. Of those patients who develop chronic GVHD, nearly 50% require at least three lines of treatment, emphasizing the need for additional effective treatment options. cGVHD typically manifests across multiple organ systems, with the skin and mucosa being commonly involved, and is characterized by the development of fibrotic tissue.

cGVHD has historically been managed by off-label treatments. However, in the past several years, the FDA has approved three drugs, Imbruvica® (ibrutinib), Rezurock® (belomosalil) and Jakafi® (ruxolitinib), for use in patients with cGVHD after failure of one or more prior lines of systemic therapy. All three of these drugs may compete with Niktimvo in patients diagnosed with cGVHD. The first line of therapy for cGVHD is typically corticosteroids, though approximately 50% of patients may require treatment with additional systemic therapies, such as extracorporeal photopheresis, cytostatic agents such as mycophenolate mofetil, methotrexate, and immunomodulators such as rituximab, IL-2. Imbruvica® (ibrutinib), a BTK inhibitor, was the first FDA-approved therapy for cGVHD and is indicated for use after one or more lines of therapy. Imbruvica received approval based on Phase 1/2 clinical trial data that showed a 68% overall response rate, with 48% of responses lasting 20 weeks or longer and reduced dependence on steroids for most patients. Prior to the recent FDA approval of Niktimvo (axatilimab-csfr), the FDA had previously approved two other drugs, Rezurock® (belomosalil), for use in patients with cGVHD after failure of at least two prior lines of systemic therapy, and Jakafi® (ruxolitinib), for use in patients with cGVHD after failure of one or more prior lines of systemic therapy. While the other agents have shown a benefit in improving symptoms of this disease, none have demonstrated an improvement in long-term outcomes and a significant unmet medical need still remains for this patient population. Additionally, the other previously approved agents are believed to exert their effect through T- and B-cells, with minimal impact on macrophages. By inhibiting the work of monocyte-derived macrophages, axatilimab provides a differentiated way to treat cGVHD, which we expect to ultimately have a more pronounced impact on the fibrotic process. We also believe that shifting CSF-1R inhibition earlier in the treatment phase of cGVHD, to minimize formation of fibrotic tissue, could have a meaningful long-term impact on the disease process itself.

Idiopathic Pulmonary Fibrosis (IPF)

IPF is a specific form of chronic, fibrosing, interstitial pneumonia limited to the lungs with a median survival of approximately three to five years after diagnosis. IPF has an estimated prevalence of 281,000 people among the seven major market countries. IPF incidence and prevalence increase with age and are higher among males. Although relatively rare, the incidence of IPF is increasing, likely due to an increasing understanding of the disease and the recent development of uniform diagnostic criteria.

There are currently three FDA approved therapies for IPF, Ofev® (nintedanib), Esbriet® (pirfenidone), and Jascayd® (nerandomilast). Despite these recent advances, the unmet medical need in IPF remains high, with a five-year mortality rate of 50% to 70% with deaths occurring mainly due to respiratory failure. Lung transplantation remains the only curative treatment option, but less than five percent of IPF patients undergo lung transplantation. There is an urgent need for new and more effective treatments for IPF that can address the limitations of current treatment options, improve mortality, and quality of life.

Growing evidence suggests macrophages are critical regulators of lung fibrosis. Recent work has established a role for monocyte-derived macrophages as the pathogenic population of cells required for the development of fibrosis and that drive the fibrotic process. Reducing the circulating levels of pathogenic monocyte-derived macrophage precursors or inhibiting their activation in tissues provides an opportunity to therapeutically intervene and directly inhibit fibrosis. Recent experiments have demonstrated that the monocyte-derived, pro-fibrotic macrophages are CSF-1-dependent and CSF-1R inhibition through anti-CSF-1R antibody can block fibrosis in fibrotic disease models. We believe that using axatilimab to inhibit the work of monocyte-derived macrophages may provide a differentiated way to treat IPF, and could result in a more pronounced impact on the fibrotic process.

Collaborations

Incyte Collaboration and License Agreement

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 and milestone payment obligations set forth below. In the United States, Incyte and the Company are co-commercializing axatilimab as Niktimvo (axatilimab-csfr) and share equally the profits and losses from these co-commercialization efforts.

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 non-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.

License Agreements

Vitae Pharmaceuticals, Inc.

In October 2017, we entered into a license agreement, or the Vitae License Agreement, under which we were 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. Under the Vitae License Agreement, we are required to pay AbbVie 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 we or any of our affiliates or sublicensees commercializes the Menin Assets, we will also be obligated to pay Vitae low single to low double-digit percentage 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. We are 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 we may terminate the Vitae License Agreement at any time upon advance written notice to Vitae. Vitae may terminate the Vitae License Agreement if we or any of our 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.

UCB

In 2016, the Company entered into a license agreement, or the UCB License Agreement, as amended from time to time, with 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. Under the UCB License Agreement, the Company is 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. The Company will also be obligated to pay UCB low double-digit percentage 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.

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

In March 2007, the Company entered into a license agreement with 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 April 2013, the Company entered into the Eddingpharm License Agreement 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.

Royalty Financing with Royalty Pharma

On November 4, 2024, we entered into a Purchase and Sale Agreement, or Purchase and Sale Agreement, with Royalty Pharma Development Funding, LLC, or Royalty Pharma, pursuant to which Royalty Pharma purchased rights to certain revenue streams from net sales of products comprising or containing axatilimab (including Niktimvo) by Syndax, its affiliates and its licensees in the United States and its respective territories, districts, commonwealths and possessions (including Guam and Puerto Rico) in exchange for an upfront fee of \$350 million.

Pursuant to the Purchase and Sale Agreement, Royalty Pharma purchased the right to receive a percentage of net sales equal to a royalty rate of 13.8% on quarterly net sales of Niktimvo in the United States and its respective territories; provided that the royalty rate is subject to certain adjustments based on future aggregate net sales of the

product in the Territory, the Revenue Participation Right. Aggregate payments made to Royalty Pharma in respect of the Revenue Participation Right will be capped at \$822.5 million, or the Royalty Cap.

The Purchase and Sale Agreement contains customary representations, warranties and indemnities of the Company and Royalty Pharma and customary covenants relating to the royalty payments, including the grant of a back-up security interest in the purchased royalties and certain assets related to the product and restrictions on the incurrence of additional indebtedness and on the existence of liens on our assets related to the product. Upon a change of control, we will have the right, but not the obligation, to repurchase the Revenue Participation Right at a repurchase price set forth in the Purchase and Sale Agreement. In addition, the Purchase and Sale Agreement provides that if certain events of default occur, including certain bankruptcy events or certain termination events with respect to our license agreement with UCB Biopharma Srl, Royalty Pharma may require us to repurchase Royalty Pharma's interests in the Revenue Participation Right at a repurchase price equal to the Royalty Cap.

Sales and Marketing

To support the commercialization of Revuforj and Niktimvo in the United States, we have established a robust customer-facing team comprising highly experienced professionals with extensive experience in hematology and oncology and new product launches. Our targeted sales force focuses on a well-defined group of medical hematologists and oncologists and transplant physicians, primarily in academic and community settings, who are responsible for the care and treatment of cancer patients. For Revuforj, we manage sales, marketing and distribution through internal resources and third-party relationships. In accordance with our agreement, Incyte leads the commercialization of axatilimab globally and we are co-commercializing Niktimvo in the United States. Outside the United States, we plan to rely on our current partners and plan to seek additional pharmaceutical partners for development as well as sales and marketing activities.

Manufacturing

We do not own or operate manufacturing facilities for the production of revumenib, axatilimab, or entinostat, and we do not have plans to develop our own manufacturing operations in the foreseeable future. We currently rely on third-party contract manufacturers as well as Incyte for all of our required raw materials, active pharmaceutical ingredients and finished product for our preclinical research, clinical trials, and commercial supply. Development and commercial quantities of any products that we develop will need to be manufactured in facilities, and by processes, that comply with the requirements of the FDA and the regulatory agencies of other jurisdictions in which we are seeking approval.

Intellectual Property

Patents and Property Rights

Through licensed intellectual property and our owned intellectual property, we seek patent protection in the United States and internationally for our product candidates, their methods of use and processes for their manufacture, as well as for other technologies, where appropriate. Our policy is to actively seek to protect our proprietary position by, among other things, filing patent applications in the United States and abroad claiming our proprietary technologies that are important to the development of our business. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position.

We cannot be sure that patents will be granted with respect to any of our owned or licensed pending patent applications or with respect to any patent applications filed by us or our licensors in the future, nor can we be sure that any of our existing owned or licensed patents or any patents that may be granted to us or to our licensors in the future will protect our technology. Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for the technologies that we consider important to our business, defend our patents, preserve the confidentiality of our trade secrets, operate our business without infringing the patents and proprietary rights of third parties, and prevent third parties from infringing our proprietary rights.

Axatilimab Patent Portfolio

We in-licensed from UCB a patent portfolio directed to axatilimab. As of December 31, 2025, the in-licensed axatilimab composition-of-matter patent portfolio included two granted U.S. patents, 35 granted non-U.S. patents,

including a granted Eurasian patent which has been validated in 3 countries and two granted EP patents which have been validated in 37 countries each, and 6 non-U.S. pending patent applications. The in-licensed granted patents covering axatilimab, and any non-U.S. pending applications should they issue, will expire in August 2034 or later should patent term extension be granted.

Our in-licensed patent portfolio also includes patents and patent applications directed to methods for the treatment and/or prophylaxis of fibrotic disease by administration of an inhibitor of CSF-1R activity, methods for the treatment and/or prophylaxis of inflammatory bowel disease, or IBD, by administration of an inhibitor of CSF-1R activity, and liquid pharmaceutical compositions of anti-CSF-1R antibodies. As of December 31, 2025, the in-licensed method of use patent family included 11 granted non-U.S. patents, including a granted EP patent which has been validated in 7 countries. These in-licensed granted patents covering axatilimab, will expire in August 2034 or later should patent term extension be granted. As of December 31, 2025, the in-licensed liquid pharmaceutical compositions of anti-CSF-1R antibodies patent family included 1 granted U.S. patent, 17 granted non-U.S. patents including a granted EP patent which has been validated in 33 countries. These in-licensed granted patents covering axatilimab, and any non-U.S. pending applications should they issue, will expire in February 2036 or later should patent term extension be granted.

Our owned axatilimab patent portfolio includes one granted U.S. Patent, three granted non-U.S. patents, one pending U.S. patent application and five non-U.S. patent applications directed to combinations of entinostat and axatilimab. The granted U.S. patent will expire in January 2039 (including a patent term adjustment period of 237 days). Further, the non-U.S. granted patents and any pending applications should they issue, will expire in May 2038 or later should patent term extension be granted. Our owned axatilimab patent portfolio also consists of patent applications directed to the treatment regimens and methods of using axatilimab includes one allowed U.S. patent application, one allowed non-U.S. patent application, and 19 non-U.S. patent applications. Further, our owned axatilimab patent portfolio consists of patent applications directed to methods of treating chronic graft-versus-host disease-related bronchiolitis obliterans syndrome using an anti-colony stimulating factor 1 receptor antibody, which include one pending U.S. patent application, one pending non-U.S. patent application, and one pending Patent Cooperation Treaty, or PCT, application. If any one of these applications were to issue as one or more patents, these patents would expire between December 2040 and May 2044 or later should patent term extension be granted.

Menin Asset Patent Portfolio

We in-licensed from Vitae, a patent portfolio directed to a series of selective preclinical inhibitors targeting the binding interaction of menin with MLL-r. As of December 31, 2025, the in-licensed portfolio includes five granted U.S. patents, U.S. Patent Nos. 12,312,359; 11,479,557; 10,683,302; 11,739,085; and 10,899,758; 30 granted non-U.S. patents, including a granted European patent, which was validated in 30 member states and another granted European patent which was validated in 15 member states; two pending U.S. applications; and 20 non-U.S. pending patent applications covering composition of matter and methods of treating, e.g., MLL. The in-licensed granted patents, and any pending application should they issue, are expected to expire between June 2037 and September 2037 or later should patent term extension be granted.

Our owned menin patent portfolio consists of 14 pending non-U.S. patent applications and one allowed U.S. patent application, directed to combinations of a menin inhibitor and a CYP3A inhibitor for the treatment of various cancers. Our owned menin patent portfolio also consists of one pending U.S. non-provisional application, one pending PCT application, and one pending non-U.S. patent application directed to the salts and polymorphic forms of menin inhibitors and pharmaceutical combinations thereof. Furthermore, our owned menin portfolio includes 15 pending non-U.S. patent applications and one pending U.S. patent application directed to methods of treating colorectal cancer in a subject in need thereof with a menin-MLL inhibitor. We also own two pending non-U.S. patent applications and one pending PCT application, covering composition of matter and methods of treating cancer and other diseases mediated by the menin-MLL interaction. If any of these pending applications were to issue as one or more patents, these patents would expire between April 2041 and November 2044 or later should patent term extension be granted.

We co-own with Vitae one granted U.S. patent, eighteen pending non-U.S. patent applications, and one pending U.S. patent application, covering composition of matter and methods of treating cancer and other diseases

mediated by the menin-MLL interaction. The granted patent and any pending applications should they issue, are expected to expire in May 2042 or later should patent term extension be granted.

We also own two pending PCT applications, two pending non-U.S. patent applications, one pending U.S. non-provisional patent application, and six U.S. provisional applications covering composition of matter and methods of treating cancer and other diseases mediated by the menin-MLL interaction. Should these pending applications issue as one or more patents, these patents would expire between December 2045 and February 2046 or later should patent term extension be granted.

We also co-own with St. Jude Children's Research Hospital, Inc. one pending PCT application, covering methods of using menin inhibitors or combinations of a menin inhibitor and a second therapeutic agent. Should this pending application issue as one or more patents, these patents would expire in 2045 or later should patent term extension be granted. We also co-own with Board of Regents, The University of Texas System six pending non-U.S. patent applications, one pending U.S. patent application, covering methods of treating cancer with combinations, including menin inhibitors and Bcl-2 inhibitors.

Entinostat Patent Portfolio

We have protected entinostat with multiple layers of patents. As of December 31, 2025, our portfolio included two owned pending U.S. non-provisional patent applications, five owned granted U.S. patents, U.S. Patent Nos. 10,226,472; 11,324,822; 11,397,184; 12,168,054; and 12,000,829; which expire in August 2032, March 2036, October 2036; January 2039; and September 2041, respectively, or later should patent term extension be granted, directed to methods of treating or selecting a patient for treatment with combinations comprising entinostat and another therapeutic agent, 28 granted non-U.S. patents (including one European patent validated in five countries), and 12 owned non-U.S. pending patent applications. Our owned entinostat patent portfolio includes pending U.S. and/or non-U.S. patent applications directed to methods of treating cancer patients by administration of entinostat and exemestane, treatments with entinostat combined with anti PD-1 or anti PD-L1 antibodies, entinostat and CSF-1 or CSF-1R combination therapies (also discussed above in the Axatilimab Patent Portfolio) and patient selection for combination therapy comprising entinostat and a second therapeutic agent. The granted patents would otherwise expire between August 2032 and May 2039 or later should patent term extension be granted.

The portfolio we licensed from Bayer includes U.S. Patent 7,973,166, or the '166- patent, which covers a crystalline polymorph of entinostat which is referred to as crystalline polymorph- B, the crystalline polymorph used in the clinical development of entinostat. Many compounds can exist in different crystalline forms. A compound which in the solid state may exhibit multiple different crystalline forms is called polymorphic, and each crystalline form of the same chemical compound is termed a polymorph. A new crystalline form of a compound may arise, for example, due to a change in the chemical process or the introduction of an impurity. Such new crystalline forms may be patented. By comparison, the U.S. Patent RE39,754, which expired in September 2017, covers the chemical entity of entinostat and any crystalline or non-crystalline form of entinostat. On March 7, 2014, our licensor Bayer applied for reissue of the '166 patent. The reissue application sought to add three additional inventors to the '166 patent. The reissue was granted as RE45,499 on April 28, 2015, at which time the original '166 patent was surrendered. The reissue patent has the same force and effect as the original '166 patent and the same August 2029 expiration date.

Of the unexpired foreign-granted patents we licensed from Bayer, there are at least 35 granted foreign counterparts of the '166 patent (now RE45,499) that cover crystalline polymorph B, including the European patent and Eurasian patent. The granted European patent comprises 37 national countries that have all been validated, and the granted Eurasian patent comprises nine countries that have all been validated. Likewise, there are 2 pending foreign counterparts of the '166 crystalline polymorph B patent. Other patents and patent applications in the licensed Bayer portfolio are expired and covered methods of treatment by administration of entinostat.

Patent Term

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the earliest non-provisional application or PCT application.

In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office, or USPTO in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. The term of a patent that covers an approved drug may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the development and regulatory review process. To obtain a patent extension in the United States, the term of the relevant patent must not have expired before the extension application, the patent cannot have been extended previously under this law, an application for extension must be submitted, the product must be subject to regulatory review prior to its commercialization, and the permission for the commercial marketing or use of the product after such regulatory review period is the first permitted commercial marketing or use of the product. If our future products contain active ingredients which have not been previously approved, we may be eligible for a patent term extension in the United States. In the United States, we are seeking for axatilimab and revumenib extension of patent terms under the Drug Price Competition and Patent Term Restoration Act of 1984, which permits a patent term extension of up to five years beyond the expiration of the patent for patent claims covering a new chemical entity. If patent extensions are available to us outside of the United States, we would expect to file for a patent term extension in applicable jurisdictions.

Confidential Information and Inventions Assignment Agreements

We require our employees and consultants to execute confidentiality agreements upon the commencement of employment, consulting or collaborative relationships with us. These agreements provide that all confidential information developed or made known during the course of the relationship with us be kept confidential and not disclosed to third parties except in specific circumstances.

In the case of employees, the agreements provide that all inventions resulting from work performed for us, utilizing our property or relating to our business and conceived or completed by the individual during employment shall be our exclusive property to the extent permitted by applicable law. Our consulting and service agreements also provide for assignment to us of any intellectual property resulting from services performed for us.

Government Regulation and Product Approval

United States Government Regulation

In the United States, the FDA regulates drugs and biologics under the Federal Food, Drug, and Cosmetic Act the Public Health Service Act, and related regulations. Drugs and biologics are also subject to other federal, state and local statutes and regulations. The FDA and comparable regulatory agencies in state and local jurisdictions impose substantial requirements upon, among other things, the testing, development, manufacture, quality control, safety, purity, potency, labeling, storage, distribution, record keeping and reporting, approval, import and export, advertising and promotion, and postmarket surveillance of drugs and biologics.

Biopharmaceutical Product Development Process

The process required by the FDA before biopharmaceutical products may be marketed in the United States generally involves the following:

- completion of extensive preclinical laboratory tests and animal studies in accordance with applicable regulations, including the FDA's good laboratory practice, or GLP regulations;
- submission of an Investigational New Drug, or IND, application which must become effective before clinical trials may begin;
- performance of adequate and well-controlled human clinical trials in accordance with applicable regulations, including the FDA's current good clinical practice, or cGCP, regulations to establish the safety and efficacy of the proposed drug for its intended use or uses;
- submission to the FDA of a New Drug Application, or NDA, for a new drug product or a Biologics License Application, or BLA, for biologics;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA to accept the application for filing and review;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the drug or biologic is produced to assess compliance with the FDA's current Good Manufacturing Practices, or cGMP, regulations to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- potential FDA audit of the preclinical and/or clinical trial sites that generated the data in support of an NDA or BLA; and
- FDA review and approval of an NDA or BLA prior to any commercial marketing or sale of the biopharmaceutical product in the United States.

Before testing any compounds with potential therapeutic value in humans, the product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry and formulation, as well as animal studies to assess the potential safety, toxicity profile and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs.

Prior to commencing the first clinical trial in humans, an IND must be submitted to the FDA, and the IND must become effective. A sponsor must submit preclinical testing results to the FDA as part of the IND and the FDA must evaluate whether there is an adequate basis for testing the drug in humans. The IND automatically becomes effective 30 days after receipt by the FDA unless the FDA within the 30-day time period raises concerns or questions about the submitted data or the conduct of the proposed clinical trial and places the IND on clinical hold. In such case, the IND application sponsor must resolve any outstanding concerns with the FDA before the clinical trial may begin. A separate submission to the existing IND application must be made for each successive clinical trial to be conducted during product development. Further, an independent Institutional Review Board, or IRB, for each site proposing to conduct the clinical trial must review and approve the protocol and informed consent for any clinical trial before it commences at that site. Informed consent must also be obtained from each study subject. Regulatory authorities, an IRB, a data safety monitoring board or the trial sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the participants are being exposed to an unacceptable health risk.

Human clinical trials are typically conducted in three sequential phases that may overlap:

- Phase 1—The drug is initially given to healthy human subjects or patients and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion, the side effects associated with increasing doses, and if possible, to gain early evidence on effectiveness.
- Phase 2—The drug is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases or conditions and to determine dosage tolerance, optimal dosage and dosing schedule.
- Phase 3—Clinical trials are undertaken to further evaluate dosage, clinical efficacy and safety at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall benefit-risk ratio of the product and to provide an adequate basis for product approval by the FDA.

Post-approval studies, or Phase 4 clinical trials, may be conducted after initial marketing approval. These studies may be required by the FDA as a condition of approval and are used to gain additional experience from the treatment of patients in the intended therapeutic indication. The FDA also has express statutory authority to require post-market clinical studies to address safety issues.

Some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data monitoring board or committee. This group provides recommendations for whether or not a trial may move forward at designated checkpoints based on access to certain data from the study. A sponsor may also suspend or terminate a clinical trial based on evolving business objectives and/or competitive climate.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, must include developed methods for testing the identity, strength, quality and purity of the finished product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

FDA Review and Approval Processes

In order to obtain approval to market a biopharmaceutical product in the United States, a marketing application must be submitted to the FDA that provides data establishing to the FDA's satisfaction the safety and effectiveness of the investigational drug for the proposed indication. Each NDA or BLA submission requires a substantial user fee payment unless a waiver or exemption applies. The application includes all relevant data available from pertinent nonclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators.

The FDA will initially review an NDA or BLA for completeness before it accepts it for filing. The FDA has 60 days from its receipt of an application to determine whether the application will be accepted for filing based on the agency's threshold determination that the application is sufficiently complete to permit substantive review. If it is not, the FDA may refuse to file the application and request additional information, in which case the application must be resubmitted with the supplemental information, and review of the application is delayed. After an NDA or BLA submission is accepted for filing, the FDA reviews the application to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA may refer applications for novel drug products or drug products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and, if so, under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Upon the filing of an NDA or BLA, the FDA may grant a priority review designation to a product, which sets the target date for FDA action on the application at 6 months, rather than the standard 10 months. Priority review is given for drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. Priority review designation does not change the scientific or medical standard for approval or the quality of evidence necessary to support approval.

Before approving an NDA or BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA or BLA, the FDA may inspect one or more clinical sites to assure compliance with GCP.

After the FDA completes its initial review of an NDA or BLA, it will communicate to the sponsor that the product is approved, or it will issue a complete response letter to communicate that the application will not be approved in its current form and inform the sponsor of changes that must be made or additional clinical, nonclinical or manufacturing data that must be received before the application can be approved.

Even if a product candidate receives regulatory approval, the approval may be limited to specific disease states, patient populations and dosages, or might contain significant limitations on use in the form of warnings, precautions or contraindications, or in the form of onerous risk management plans, restrictions on distribution, or post-marketing study requirements. For example, the FDA may require Phase 4 testing, which involves clinical trials designed to further assess a drug's safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized. The FDA may also determine that a risk evaluation and mitigation strategy, or REMS, is necessary to assure the safe use of the drug. If the FDA concludes a REMS is needed, the sponsor of an NDA must submit a proposed REMS, and the FDA will not approve an NDA without an approved REMS, if required.

Expedited Review Programs

Among other programs, the FDA may expedite the review of a product candidate designated as a breakthrough therapy, which is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A sponsor may request the FDA to designate a drug as a breakthrough therapy at the time of, or any time after, the submission of an IND application for the drug. If the FDA designates a drug as a breakthrough therapy, it may take actions appropriate to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the drug; providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the nonclinical and clinical data necessary for approval is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review; assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and taking steps to ensure that the design of the clinical trials is as efficient as practicable, when scientifically appropriate, such as by minimizing the number of patients exposed to a potentially less efficacious treatment. The FDA may rescind a breakthrough therapy designation in the future if further clinical development later shows that the criteria for designation are no longer met.

Breakthrough therapy designation does not change the standards for approval but may expedite the development or review process.

Post-Approval Requirements

If and when approved, any products manufactured or distributed by us or on our behalf will be subject to continuing regulation by the FDA, including requirements for record-keeping, reporting of adverse experiences and submitting annual reports.

Biopharmaceutical manufacturers are required to register their facilities with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs, which impose certain quality processes, manufacturing controls and documentation requirements upon us and our third-party manufacturers in order to ensure that the product is safe, has the identity and strength, and meets the quality and purity characteristics that it purports to have. The FDA and certain states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including technology capable of tracking and tracing product as it moves through the distribution chain. We cannot be certain that we or our present or future suppliers will be able to comply with the cGMP and other FDA regulatory requirements. If our present or future suppliers are not able to comply with these requirements, the FDA may halt our clinical trials, fail to approve any application, shut down manufacturing operations or withdraw approval of an application, or we may recall the product from distribution. Noncompliance with cGMP or other requirements can result in issuance of warning letters, civil and criminal penalties, seizures and injunctive action.

The FDA closely regulates the labeling, marketing and promotion of drugs and biologics. While doctors are free to prescribe any drug approved by the FDA for any use based on the doctor's independent medical judgment, a company can only make claims relating to safety and efficacy of a drug that are consistent with FDA approval, and a company is allowed to actively market a drug only for the particular use and treatment approved by the FDA. In addition, any claims we make for our products in advertising or promotion must be appropriately balanced with important safety information and otherwise be adequately substantiated. Failure to comply with these requirements can result in adverse publicity, warning letters, corrective advertising, injunctions and potential civil and criminal penalties. Government regulators recently have increased their scrutiny of the promotion and marketing of drugs.

Coverage and Reimbursement

In both domestic and foreign markets, sales of Revuforj, Niktimvo and any other products for which we may receive regulatory approval will depend in part upon the availability of formulary coverage and adequate reimbursement to healthcare providers from third-party payors. Such third-party payors include government health

programs, such as Medicare and Medicaid, as well as managed care organizations, private health insurers and other organizations. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are available. Assuming coverage is granted, the reimbursement rates paid for covered products might not be adequate. Even if favorable coverage status and adequate reimbursement rates are attained, less favorable coverage policies and reimbursement rates may be implemented in the future. The marketability of Revuforj, Niktimvo and any other products for which we may receive regulatory approval for commercial sale may suffer if the government and other third-party payors fail to provide coverage and adequate reimbursement to allow us to sell such products on a competitive and profitable basis. For example, under these circumstances, physicians may limit how much or under what circumstances they will prescribe or administer such products, and patients may decline to purchase them. This, in turn, could affect our ability to successfully commercialize our products and impact our profitability, results of operations, financial condition, and future success.

In the United States, the European Union and other potentially significant markets for our products and product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies. For example, the U.S. Department of Health and Human Services, or HHS, imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. HHS also has been empowered to negotiate the price of certain single-source drugs that have been on the market for at least seven years and single-course biologics that have been on the market for at least 11 years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to 20 products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit a company's revenue generated from the sale of any approved products. Such pressure, along with the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the European Union, will likely put additional downward pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions, governmental laws and regulations related to government healthcare programs, healthcare reform, and pharmaceutical coverage and reimbursement policies.

The market for Revuforj, Niktimvo and any other product candidates for which we may receive regulatory approval will depend significantly on the degree to which these products are listed on third-party payors' drug formularies or lists of medications for which third-party payors provide coverage and reimbursement to the extent products for which we may receive regulatory approval are covered under a pharmacy benefit or are otherwise subject to a formulary. The industry competition to be included on such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug on their formularies or otherwise restrict patient access to a branded drug when a less costly generic equivalent or other alternative is available. In addition, because each third-party payor individually approves coverage and reimbursement levels, obtaining coverage and adequate reimbursement is a time-consuming and costly process. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. We may be required to provide scientific and clinical support for the use of any product to each third-party payor separately with no assurance that approval would be obtained, and we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. We cannot be certain that Revuforj, Niktimvo and any other product candidates for which we may receive regulatory approval will be considered cost-effective. This process could delay the market acceptance of such products and could have a negative effect on our future revenue and operating results.

Federal and State Fraud and Abuse and Data Privacy and Security Laws and Regulations

In addition to FDA restrictions on marketing of pharmaceutical products, federal and state laws restrict business practices in the pharmaceutical industry. These laws include anti-kickback and false claims laws and regulations as well as data privacy and security laws and regulations. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This 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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively, 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;
- federal civil and criminal false claims laws, including, without limitation, the False Claims Act, or FCA, and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors 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 Affordable Care Act provides, and recent government cases against pharmaceutical manufacturers support, the view that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the FCA;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that, among other things, prohibit a person from knowingly and willfully executing a scheme or making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers, known as covered entities, and their respective business associates, individuals or entities that perform certain services on behalf of a covered entity that involves the use or disclosure of individually identifiable health information, and their covered subcontractors;
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to: (i) 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 (ii) ownership and investment interests held by physicians and their immediate family members; and
- state law equivalents of each of the above federal laws, state laws that require 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 pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or to adopt compliance programs as prescribed by state laws and regulations, or that otherwise restrict payments that may be made to healthcare providers, state laws that require manufactures to report pricing information regarding certain drugs, state and local laws that require the registration of pharmaceutical sales representatives, and state laws that govern the privacy and security of health information, which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

We may also be subject to federal and state laws that govern the privacy and security of other personal data, including federal and state consumer protection laws, state data security laws, and data breach notification laws. A data breach affecting sensitive personal data, including health information, could result in significant legal and financial exposure and reputational damages.

Because of the breadth of these laws and the narrowness of available statutory and regulatory exemptions, it is possible that some of our business activities could be subject to challenge, investigation or legal action under one or more of such laws. If our operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to us, we may be subject to significant civil, criminal, and administrative penalties, including, without limitation, damages, fines, imprisonment, disgorgement, exclusion from participation in government healthcare programs, 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.

To the extent that any of our product candidates receive approval and are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, international data protection laws (including the General Data Protection Directive ((EU) 2016/679) on the protection of individuals with regard to the processing of personal data and on the free movement of such data as well as EU member state implementing legislation), and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals.

Healthcare Reform

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding 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 Revuforj, Niktimvo, and any other product candidates for which we obtain marketing approval. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, in March 2010, the Affordable Care Act was passed, which has changed health care financing by both governmental and private insurers and significantly affected the U.S. pharmaceutical industry.

Since its enactment, there have been executive, judicial and Congressional challenges and amendments to certain aspects of the Affordable Care Act. For example, on July 4, 2025, the One Big Beautiful Bill Act, or the OBBBA, was signed into law, which narrowed access to Affordable Care Act marketplace exchange enrollment and declined to extend the Affordable Care Act's enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired Affordable Care Act subsidies. We expect that additional United States federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the federal government will pay for healthcare products and services, which could result in reduced demand for our products or additional pricing pressures.

There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Additionally, the current administration is pursuing policies to reduce regulations and expenditures across government including at HHS, the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with several pharmaceutical companies that require the drug manufacturers to offer, through a direct-to-consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency

workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the Make America Healthy Again Commission’s Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact “The Great Healthcare Plan,” to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager payment methodologies, among other things. These actions and policies may significantly reduce drug prices in the United States, potentially impacting manufacturers’ global pricing strategies and profitability, while increasing their operational costs and compliance risks. Additionally, in its June 2024 decision in *Loper Bright Enterprises v. Raimondo*, or *Loper Bright*, the U.S. Supreme Court greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

We expect that additional state, federal, and foreign healthcare reform measures will be adopted in the future. The full impact on our business of these health reform measures and other new laws is uncertain but may result in additional reductions in Medicare and other healthcare funding. Nor is it clear whether other legislative changes will be adopted, if any, or how such changes would affect our business, including the demand for our products.

Regulations Outside of the United States

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our product candidates to the extent we choose to sell any products outside of the United States. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country. As in the United States, post-approval regulatory requirements, such as those regarding product manufacture, marketing, or distribution would apply to any product that is approved outside the United States.

Other Regulations

We are also subject to numerous federal, state and local laws relating to such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control, and disposal of hazardous or potentially hazardous substances. We may incur significant costs to comply with such laws and regulations now or in the future.

Employees and Human Capital Resources

As of December 31, 2025, we had 298 full-time employees of which 144 were primarily engaged in research and development activities and 65 with M.D., Ph.D., or PharmD degree. As of February 16, 2026, we had 277 full-time employees. Of the full-time employees, 128 were primarily engaged in research and development activities and 56 have an M.D., Ph.D., or PharmD degree. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good. To allow us flexibility in meeting varying workflow demands, we also engage consultants and temporary workers when needed.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. All employees must adhere to a code of business conduct and ethics and our employee handbook, which combined, define standards for appropriate behavior and are annually trained to help prevent, identify, report, and stop any type of discrimination and harassment.

We consider a number of measures and objectives in managing our human capital assets, including, among others, employee engagement, development, and training, talent acquisition and retention, employee safety and wellness, diversity and inclusion, and compensation and pay equity. We provide our employees with salaries and

bonuses intended to be competitive for our industry, opportunities for equity ownership, development programs that enable continued learning and growth and a robust benefits package to promote well-being across all aspects of their lives, including health care, retirement planning and paid time off. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees and directors through the granting of equity-based compensation awards and cash-based compensation awards, in order to increase stockholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives.

In addition, we have conducted employee surveys to gauge employee engagement and identify areas of future focus for our human capital practices and benefits offerings.

Corporate and Other Information

We were incorporated in Delaware in 2005. In 2021, we established a wholly owned subsidiary in the Netherlands. There have been no material activities for these entities to date. We currently operate in one segment.

Our principal office is located in New York, New York, where we lease approximately 12,000 square feet of office space pursuant to a lease that expires in August 2028. In February 2025, following the expiration of our lease, we closed our office in Waltham, Massachusetts and made New York our principal office. We believe our facilities are adequate to meet our current needs, although we may seek to negotiate new leases or evaluate additional or alternate space for our operations. We believe appropriate alternative space will be readily available on commercially reasonable terms.

We file electronically with the Securities and Exchange Commission, or SEC, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act. We make available on our website at www.syndax.com, under “Investors,” free of charge, copies of these reports as soon as reasonably practicable after filing or furnishing these reports with the SEC.

Item 1A. Risk Factors

This Annual Report contains forward-looking information based on our current expectations. Because our business is subject to many risks and our actual results may differ materially from any forward-looking statements made by or on behalf of us, this section includes a discussion of important factors that could affect our business, operating results, financial condition and the trading price of our common stock. You should carefully consider these risk factors, together with all of the other information included in this Annual Report as well as our other publicly available filings with the SEC.

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:

- Our business depends heavily on our ability to successfully commercialize Revuforj and Niktimvo in the United States and in other jurisdictions where we may obtain marketing approval. There is no assurance that our commercialization efforts with respect to Revuforj or Niktimvo will be successful or that we will be able to generate revenues at the levels or on the timing we expect.
- We have recently begun commercialization efforts and the sales, marketing, and distribution of Revuforj, Niktimvo, or any future approved products may be unsuccessful or less successful than anticipated.
- If the market opportunities for our products are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer.
- We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

- 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 may be significantly harmed.
- 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 Revuforj or Niktimvo may result in various adverse actions such as the suspension or withdrawal of Revuforj or 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.
- Revuforj, Niktimvo and our future products may not achieve adequate market acceptance among physicians, patients, healthcare payors and others in the medical community to be commercially successful.
- We currently rely, and expect to continue to rely, on third-party contract manufacturers as well as Incyte for all of our required raw materials, active pharmaceutical ingredients and finished product for our preclinical research, clinical trials, and commercial manufacturing and distribution.
- Our products and product candidates may face future development and regulatory difficulties.
- Our products 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 Revuforj or 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 near future.
- While we have product, collaboration and milestone revenue, we may never achieve or maintain profitability.
- We may 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.
- 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

Our business depends heavily on our ability to successfully commercialize Revuforj and Niktimvo in the United States and in other jurisdictions where we may obtain marketing approval. There is no assurance that our commercialization efforts with respect to Revuforj or Niktimvo will be successful or that we will be able to generate revenues at the levels or on the timing we expect.

In November 2024, Revuforj was approved by the FDA for the treatment of R/R acute leukemia with a KMT2A, translocation in adult and pediatric patients one year old and older. In October 2025, Revuforj received a second approval from the FDA for the treatment of R/R AML with a susceptible nucleophosmin 1 mutation, or NPM1m, in adult and pediatric patients one year and older who have no satisfactory alternative treatment options. The FDA approved Niktimvo in August 2024 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.

Our business currently depends heavily on our ability to successfully commercialize Revuforj in the United States and in other jurisdictions where we may obtain marketing approval as well as commercializing Niktimvo, with our collaboration partner Incyte, in the United States. We may never be able to successfully commercialize our products or meet our expectations with respect to revenues. We have never marketed, sold, or distributed for commercial use any other products, nor have we marketed, sold or distributed for commercial use any product in any other market outside of the United States. There is no guarantee that the infrastructure, systems, processes, policies, relationships, and materials we have built for the launch and commercialization of Revuforj or Niktimvo in the United States will be sufficient for us to achieve success at the levels we expect.

We may encounter issues and challenges in commercializing Revuforj and Niktimvo and generating substantial revenues. We may also encounter challenges related to reimbursement of Revuforj or Niktimvo, including potential limitations in the scope, breadth, availability, or amount of reimbursement covering Revuforj or Niktimvo, respectively. Similarly, healthcare settings or patients may determine that the financial burdens of treatment are not acceptable. We may face other limitations or issues related to the price of Revuforj or Niktimvo. Our results may also be negatively impacted if we have not adequately sized our field teams or our physician segmentation and targeting strategy is inadequate or if we encounter deficiencies or inefficiencies in our infrastructure or processes. Other factors that may hinder our ability to successfully commercialize Revuforj, Niktimvo, or any of our future approved drugs, and generate substantial revenues, include:

- the acceptance of Revuforj and Niktimvo by patients and the medical community;
- the ability of our third-party manufacturer(s) to manufacture commercial supplies of Revuforj or Niktimvo at acceptable costs, to remain in good standing with regulatory agencies, and to maintain commercially viable manufacturing processes that are, to the extent required, compliant with cGMP regulations;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;
- FDA-mandated package insert requirements and successful completion of any related FDA post-marketing requirements;
- the actual market size for Revuforj or Niktimvo, respectively, which may be different than expected;
- the length of time that patients who are prescribed our drug remain on treatment;
- the sufficiency of our drug supply to meet commercial and clinical demands which could be negatively impacted if our projections regarding the potential number of patients are inaccurate, we are subject to unanticipated regulatory requirements, or our current drug supply is destroyed, or negatively impacted at our manufacturing or storage sites, or in transit;
- our ability to effectively compete with other therapies that may emerge; and
- our ability to maintain, enforce, and defend third party challenges to our intellectual property rights in and to Revuforj and Niktimvo.

Any of these issues could impair our ability to successfully commercialize our products or to generate substantial revenues or profits or to meet our expectations with respect to the amount or timing of revenues or

profits. Any issues or hurdles related to our commercialization efforts may materially adversely affect our business, results of operations, financial condition, and prospects. There is no guarantee that we will be successful in our commercialization efforts with respect to Revuforj or Niktimvo. We may also experience significant fluctuations in sales of Revuforj or Niktimvo from period to period and, ultimately, we may never generate sufficient revenues from Revuforj and Niktimvo to reach or maintain profitability or sustain our anticipated levels of operations. Any inability on our part to successfully commercialize Revuforj or Niktimvo in the United States, and any other international markets where it may subsequently be approved, or any significant delay, could have a material adverse impact on our ability to execute upon our business strategy.

We have recently begun commercialization efforts and the sales, marketing, and distribution of Revuforj, Niktimvo, or any future approved products may be unsuccessful or less successful than anticipated.

We began commercialization of Revuforj in November 2024 and Niktimvo in February 2025. As a company, we had no prior experience commercializing a product. The success of our commercialization efforts for Revuforj, Niktimvo, and any future approved products is difficult to predict and subject to the effective execution of our business plan, including, among other things, the continued development of our internal sales, marketing, and distribution capabilities and our ability to navigate the significant expenses and risks involved with the development and management of such capabilities.

For example, we have expanded in areas to support commercialization, including in sales management, sales representatives, marketing, market access and reimbursement, sales support, and distribution. There are significant risks involved with establishing our own sales, marketing, and distribution capabilities, including risks related to our ability to hire, retain, and appropriately incentivize qualified individuals, provide adequate training to sales and marketing personnel, and effectively manage geographically dispersed sales teams to generate sufficient demand. Any failure or delay in the development of these capabilities could delay or negatively affect the success of our commercialization efforts and our business. In addition, the commercialization of Revuforj and Niktimvo may not develop as planned or anticipated, which may require us to adjust or amend our business plan and incur significant expenses.

Further, given our recent launches of Revuforj and Niktimvo, we do not have a track record of successfully executing on the commercialization of approved products. If we are unsuccessful in accomplishing our objectives and executing on our business plan, or if the commercialization of Revuforj and Niktimvo or any future approved products does not develop as planned, we may require significant additional capital and financial resources, we may not become profitable, and we may not be able to compete against more established companies in our industry.

If the market opportunities for our products 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 who have the potential to benefit from treatment with either Revuforj or Niktimvo are based on our 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 Revuforj or Niktimvo may be limited or may not be amenable to treatment with Revuforj or Niktimvo, and new patients may become increasingly difficult to identify or access, which would adversely affect our results of operations and our business. If the number of our addressable patients is not as significant as we estimate or the reasonably expected population for treatment is narrowed by competition, physician choice, or treatment guidelines, revenue from sales of products may be more limited than we expect. Even if we obtain significant market share for Revuforj or Niktimvo, we may never become or remain profitable nor generate sufficient revenue growth to sustain our business.

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

Revuforj, Niktimvo and any product candidate 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*®), belomosisidil (*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.

Revuforj is FDA approved for the treatment of patients one year and older with R/R acute leukemia with a KMT2A translocation or R/R AML with an NPM1 mutation. In November 2025, the FDA approved Komzifti™ (ziftomenib) for adult patients with R/R AML with an NPM1 mutation. At this time, other than Revuforj and Komzifti, there are no other drugs specifically approved for these genetic subtypes of acute leukemia, although other types of therapies can also be used in the treatment of these patient populations. While there are additional menin inhibitors in development for similar patient populations, Revuforj is the first and currently the only therapy FDA approved for both adults and pediatrics with R/R acute leukemia with a KMT2A translocation or R/R AML with an NPM1 mutation.

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 product candidates and the commercialization of those products. Our competitors may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours and may be more successful than us in obtaining FDA approval for drugs and achieving widespread market acceptance of their products. Our competitors' drugs may also 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.

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

- the market adoption of Revuforj, Niktimvo and any future products by physicians and patients;
- the efficacy and safety profile of our products and 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 Revuforj, Niktimvo and our product candidates if they receive regulatory approval;
- the price of Revuforj, Niktimvo and any future products, 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 Revuforj, Niktimvo and our product candidates, if they receive regulatory approval; and
- our ability to negotiate preferential formulary status for our product candidates.

We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. 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.

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.

Although we have begun generating revenue from product sales of Revuforj and Niktimvo, our financial success will also depend substantially on our ability to effectively and profitably commercialize our products and product candidates and expand the approved indications for Revuforj and Niktimvo. In order to commercialize our product candidates and expand the approved indications for Revuforj and Niktimvo, 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 tariffs, war or terrorism;
- 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 commercialize our product candidates, which will have a material adverse effect on our business and our prospects.

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

From time to time, we may publish interim top-line or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top-line data 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 revenue 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 additional regulatory development or sales milestones or that we will receive any future milestone or royalty payments under the Incyte 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 will 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;
- perception about the relative efficacy of our product candidates versus other compounds in clinical development or commercially available;
- evolving standard of care therapies 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 that:

- 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 revenue 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 Revuforj or Niktimvo may result in various adverse actions, such as the suspension or withdrawal of either product, 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, cGLP and cGCP, 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.

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 therapies 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. 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 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.

Disruptions at the FDA and other government agencies caused by layoffs, funding shortages or global health concerns could negatively impact our business.

The ability of the FDA to review proposed clinical trials or approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, including executive and congressional priorities, the impacts of which are inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may slow the time necessary for new product candidates to be reviewed and/or approved, which would adversely affect our business. For example, over the last several years, including in October 2025, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. In addition, the current administration has implemented substantial reductions in force at various government agencies including the FDA, and has implemented layoffs at the FDA, which could significantly reduce the FDA's capacity to perform its functions in a manner consistent with its past practices and could delay reviews and negatively impact our business.

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

Revuforj, Niktimvo and our future products 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 as a safe and effective treatment by physicians, clinics and patients;

- the potential and perceived advantages of our products 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 products.

If Revuforj, Niktimvo or future approved products 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 are in the process of building and maintaining our sales, marketing and distribution infrastructure.

To successfully market Revuforj and Niktimvo or any approved product candidate in the future, we must continue building and maintaining our sales, marketing, distribution, market access, 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 support the commercialization of Revuforj and Niktimvo in the United States, we have established a robust commercial field force comprising highly experienced professionals with extensive experience in hematology and oncology and new product launches. For Revuforj, we manage sales, marketing and distribution through internal resources and third-party relationships. In accordance with our agreement, Incyte leads the commercialization of axatilimab globally and we are co-commercializing Niktimvo in the United States.

We must invest significant amounts of financial and management resources to further develop our internal sales, distribution and marketing capabilities, we must invest significant amounts of financial and management resources in the future. For drug products 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, build and retain 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 revenue 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, 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 future 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.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for Revuforj, 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 Revuforj, 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 Revuforj and Niktimvo will depend substantially 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 may not be able to successfully commercialize either Revuforj or 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, CMS, an agency within HHS, 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 products such as ours.

In the United States, and certain other foreign jurisdictions, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies. For example, HHS imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. HHS also has been empowered to negotiate the price of certain single-source drugs that have been on the market for at least seven years and single-course biologics that have been on the market for at least 11 years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to 20 products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. 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 either Revuforj or Niktimvo. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our current and any future product candidates that we develop, which could have an adverse effect on our operating results and our overall financial condition. We expect to experience pricing pressures in connection with the sales of both Revuforj and 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.

We currently rely, and expect to continue to rely, on third-party contract manufacturers as well as Incyte for all of our required raw materials, active pharmaceutical ingredients and finished product for our preclinical research, clinical trials, and commercial manufacturing and distribution.

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 for our existing products and 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 products and product candidates may face future development and regulatory difficulties.

Our products 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, HHS's 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 FCA, 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 FCA 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 products 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 products 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. For example, the Revuforj U.S. Prescribing Information label contains a boxed warning for differentiation syndrome, which can be fatal. 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.

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.

Current and future legislation may adversely impact our business, operations, and profitability including, among other things, increasing the difficulty and cost for us to commercialize Revuforj, Niktimvo, and other product candidates, preventing or delaying marketing approval of our product candidates and affecting 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 adversely impact our business operations and profitability including, among other things, preventing or delaying marketing approval of our product candidates, restricting or regulating post-approval activities and affecting our ability to profitably sell Revuforj, Niktimvo, or any product candidate for which we obtain marketing approval.

For example, in March 2010, the Affordable Care Act was enacted and significantly affected the United States pharmaceutical industry. Since its enactment, there have been executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. For example, on July 4, 2025, the OBBBA was signed into law, which narrowed access to Affordable Care Act marketplace exchange enrollment and declined to extend the Affordable Care Act's enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired Affordable Care Act subsidies. We expect that additional United States federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the federal government will pay

for healthcare products and services, which could result in reduced demand for our products or additional pricing pressures.

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.

The current administration is also pursuing policies to reduce regulations and expenditures across government including at HHS, the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with several pharmaceutical companies that require the drug manufacturers to offer, through a direct-to-consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the Make America Healthy Again Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager payment methodologies, among other things. These actions and policies may significantly reduce drug prices in the United States, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. Additionally, in its June 2024 decision in *Loper Bright Enterprises v. Raimondo*, or *Loper Bright*, the United States Supreme Court greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

At the state level, legislatures are increasingly passing legislation and implementing 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. Any such state reform measures, may result in lower drug prices for impacted products. We cannot predict which additional measures may be adopted or the impact of current and additional measures on the marketing, pricing and demand for our products, which could have a material adverse effect on our business, financial condition and results of operations.

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 clinical testing of our product candidates in human trials and in commercially selling out products and any product candidates 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 products or product candidates 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 product liability insurance coverage consistent with industry standards covering the clinical testing and commercialization of our products, 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 significant penalties, including 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 Revuforj, Niktimvo, and 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 false claims statutes, including the FCA, 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;
- 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 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 data, including federal and state consumer protection laws, state data security laws, and data breach notification laws (a data breach affecting sensitive personal data, 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.

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

We have received regulatory approval to market Revuforj for the treatment of R/R acute leukemia with a KMT2A translocation as determined by an FDA-authorized test in adult and pediatric patients one year old and older and for the treatment of R/R AML with a susceptible NPM1 mutation in adult and pediatric patients one year and older who have no satisfactory alternative treatment options. We received regulatory approval to market 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. We may only promote or market Revuforj, Niktimvo, and our other product candidates, if approved by the FDA, for their specifically approved indications and in a manner consistent with the approved labeling. We train our marketing and sales force against promoting our product candidates for uses outside of the approved indications for use, known as “off-label uses.” We cannot, however, prevent a physician from using our products off-label, when in the physician’s independent professional medical judgment, he or she deems it appropriate. Furthermore, the use of our products for indications other than those approved by the FDA may not effectively treat such conditions. Any such off-label use of our product candidates could harm our reputation in the marketplace among physicians and patients. There may also be increased risk of injury to patients if physicians attempt to use our products for any off-label uses, which could lead to product liability suits that that might require significant financial and management resources and that could harm our reputation. Additionally, the

FDA imposes stringent restrictions on manufacturers' communications regarding off-label uses and if we, or our collaborators, do not promote our products in a manner consistent with the approved labeling, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the Federal Food, Drug, and Cosmetic Act and other statutes, including the FCA, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws and state consumer protection laws.

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

In the ordinary course of business, we 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, HIPAA as amended by 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.

Numerous U.S. states 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, or 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 and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA, and other comprehensive U.S. state privacy laws exempt some data processed in the context of clinical trials, these developments further complicate compliance efforts and increase legal risk and compliance costs for us and the third parties with whom we work. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future.

Outside the United States, an increasing number of laws, regulations, and industry standards may govern data privacy and security. For example, the EU's General Data Protection Regulation, or EU GDPR, and the United Kingdom's GDPR, or UK GDPR, collectively, GDPR, Australia's Privacy Act, and Canada's Personal Information Protection and Electronic Documents Act, impose strict requirements for processing personal data. For example, under the GDPR, companies may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR, 17.5 million pounds sterling under the UK GDPR or, in each case, 4% of 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 are subject to certain laws governing the privacy of consumer health data. 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 have proposed, enacted or are considering similar laws.

Our employees and personnel use generative artificial intelligence, or AI, technologies to perform their work, and the disclosure and use of personal data in AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating AI technologies. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If we are unable to use AI technologies, 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 or have already adopted similarly stringent 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.

Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restriction on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered persons (i.e., individuals and entities who are designated as such by the U.S. Attorney General or considered "foreign persons" and are majority owned by, organized under the laws of, a primary resident in, or a contractor of, a covered person or country of concern, as applicable) that may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to engage in transactions or agreements with certain third parties in the future.

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 concerning data privacy and security. Regulators are increasingly scrutinizing these statements 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.

Our business is reliant on revenue from behavioral, interest-based, or tailored advertising, collectively targeted advertising, but delivering targeted advertisements is becoming increasingly difficult due to changes to our ability to gather information about user behavior through third party platforms, new laws and regulations, and consumer resistance. Major technology platforms on which we rely to gather information about consumers have adopted or proposed measures to provide consumers with additional control over the collection, use, and sharing of their personal data for targeted advertising purposes. In addition, legislative proposals and present laws and

regulations regulate the use of cookies and other tracking technologies, electronic communications, and marketing. For example, in the EEA and the UK, regulators are increasingly focusing on compliance with requirements related to the targeted advertising ecosystem. European regulators have issued significant fines in certain circumstances where the regulators alleged that appropriate consent was not obtained in connection with targeted advertising activities. It is anticipated that the ePrivacy Regulation and national implementing laws will replace the current national laws implementing the ePrivacy Directive, which may require us to make significant operational changes. In the United States, the CCPA, for example, grants California residents the right to opt-out of a company's sharing of personal data for advertising purposes in exchange for money or other valuable consideration, and requires covered businesses to honor user-enabled browser signals from the Global Privacy Control. Partially as a result of these developments, individuals are becoming increasingly resistant to the collection, use, and sharing of personal data to deliver targeted advertising. Individuals are now more aware of options related to consent, "do not track" mechanisms (such as browser signals from the Global Privacy Control), and "ad-blocking" software to prevent the collection of their personal data for targeted advertising purposes. As a result, we may be required to change the way we market our products, and any of these developments or changes could materially impair our ability to reach new or existing customers or otherwise negatively affect our operations.

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 with whom we work. In addition, these obligations may require us to change our business model.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties with whom we work fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans 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; interruptions or stoppages in our business operations, including clinical trials inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations.

If our information technology systems or those of third parties with whom we work, or our data are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions; 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 and the third parties with whom we work collect, store, process and transmit large amounts of proprietary, confidential, and sensitive data, including personal data (such as health-related data), intellectual property, and trade secrets (collectively, sensitive information) and, as a result, we and the third parties with whom we work 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 information 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 the third-parties with whom we work experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if the third parties with whom we work fail to satisfy their privacy or security-related obligations 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 that of the third parties we work with have not been compromised.

In addition, we currently offer a hybrid-work environment, which increases risks to our information technology systems and data as more of our employees utilize network connections, computers, and devices outside our premises or network, including working at home, while in transit and in public locations. Additionally, 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 with whom we work). However, we have not and may not in the future detect and remediate all such vulnerabilities including on a timely basis. Further, we have (and may in the future) experienced 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 threaten the confidentiality, integrity and availability of our information systems and sensitive data and those of the third parties with whom we work. Such threats are prevalent and increasing in their frequency, levels of persistence, sophistication and intensity, are increasingly difficult to detect, 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, traditional computer "hackers," including organized criminal threat actors, "hacktivists," personnel (such as through theft or misuse), sophisticated nation states and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyber-attacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we, the third parties with whom we work, may be vulnerable to a heightened risk of these attacks, including retaliatory cyber-attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services. We and the third parties with whom we work are subject to a variety of evolving threats, including but not limited to malicious code (such as viruses or worms), malware (including as a result of advanced persistent threat intrusions), ransomware attacks, denial-of-service attacks, credential stuffing and/or harvesting, personnel misconduct or error, social engineering attacks (including through deep fakes, which are 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 similar threats. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

Significant disruptions of our, our third-party vendors' and/or business partners' information technology systems or other similar data security incidents caused by any of the previously identified or similar threats have in the past and could in the future adversely affect our business operations and/or result in the unauthorized, unlawful, or accidental acquisition, modification, destruction, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties with whom we work, which could result in financial, legal, regulatory, business and reputational harm to us. For example, we have been the target of unsuccessful phishing attempts in the past and we expect such attempts will continue in the future. 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 (and that of third parties with whom we work). 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 have in the past and may in the future expend significant resources or modify our business activities (including our clinical trial 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 information.

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

If we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, including but not limited to a security incident involving personal data regarding employees or clinical trial patients, we may experience material adverse consequences, such as interruptions in our operations (including availability of data), harm to our reputation, government enforcement actions (for example, investigations, fines, penalties, audits, and inspections), additional reporting requirements and/or oversight litigation (including class claims), indemnification obligations, negative publicity, monetary fund diversions, diversion of management attention, financial loss, and other similar harms. 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 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 designed to protect against security incidents, there can be no assurance that such measures will be effective. Our contracts may not contain relevant 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. Relatedly, our contracts with third parties with whom we work may limit the types and/or amounts of damages that we can recover from those third parties, even where the third party is responsible for a privacy or cybersecurity incident or violation. 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. 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 third parties', including vendors', suppliers', and contractors' potential use of generative AI technologies which may negatively impact our company, including our ability to realize the benefit of our intellectual property.

Social media 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 data 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.

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 near 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 commercial-stage biopharmaceutical company with limited operating history. We launched Revuforj and Niktimvo for commercial sale in November 2024 and February 2025, respectively. We have no other products approved for commercial sale 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 year ended December 31, 2025, we reported a net loss of \$285.4 million. As of December 31, 2025, we had an accumulated deficit of \$1.5 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 near future, and we expect these losses to increase as we continue our commercialization activities for, and our continued development of, and seek additional regulatory approvals for, our products. 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 revenue, 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.

While we have product revenue, we may never achieve or maintain profitability.

We have begun generating product revenue from sales of Revuforj and Niktimvo. Our ability to generate additional product revenue and become profitable depends upon our ability to successfully commercialize our product candidates. Our ability to generate future product revenue also depends on a number of additional factors, including, but not limited to, our ability to:

- achieve market acceptance of Revuforj and Niktimvo;
- launch, commercialize and achieve market acceptance of our future product candidates, and if launched independently, successfully leverage our existing sales, marketing and distribution infrastructure;
- successfully complete the research and clinical development of, and receive regulatory approval for, our product candidates;
- 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 and maintain 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 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 revenue generated from the sale of Revuforj, Niktimvo or future product candidates continue increasing, 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 may 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 as well as for the commercial launch of Revuforj and Niktimvo. 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 may 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 as well as the ongoing conflicts in the Middle East, 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. 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 geopolitical actions, including war or the perception that hostilities may be imminent (such as the ongoing wars between Russia and Ukraine as well as the ongoing conflicts in the Middle East), 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 averse to us and adversely affect our business and financial condition. For example, the OBBBA, enacted in July 2025, introduced sweeping changes to the U.S. tax code, including the permanent extension of various provisions originally established under the Tax Cuts and Jobs Act. These changes, along with new OBBBA provisions such as the reinstatement of 100% bonus depreciation, the immediate expensing of domestic research and experimental expenditures, and modifications to the business interest expense limitations under Section 163(j), may significantly impact our effective tax rate and the timing of our tax payments.

While the OBBA provides federal clarity by making many temporary measures permanent, the long-term impact remains subject to future regulatory guidance. Furthermore, the Inflation Reduction Act, or IRA, continues to impact our operations through drug pricing reforms – including the Medicare drug price negotiation program and inflation-based rebate requirements – and the redesign of Part D benefits, which includes a \$2,000 cap on out-of-pocket spending beginning in 2025. Additionally, significant uncertainty exists regarding state-level taxation. Various states may not conform to all provisions of the OBBBA or other recent federal tax legislation. This patchwork of state conformity, where certain states may de-couple from federal incentives or expense treatments, could result in increased administrative complexity, higher effective state tax rates, and a divergence between our federal and state tax liabilities. The impact of changes under the OBBBA, the IRA, or other future legislative shifts remains difficult to predict and could have a material adverse effect on our financial results.

In addition, with the commercialization of Revuforj and Niktimvo, and as we continue to develop our product candidates, our tax profile may become more intricate. We may become subject to additional tax obligations, including state and local sales, use, and gross receipts taxes, as well as foreign tax obligations if we expand internationally. Our tax positions may be challenged, including the characterization of revenue, the deductibility of expenses, the allocation of income among jurisdictions, and the structure of intercompany arrangements. A tax audit or further examination could result in additional tax assessments, interest, and penalties, which could be material.

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. 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 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 2034 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, 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. Over the term of the UCB license agreement, and subject to the achievement of certain milestone events, we were required to pay UCB up to \$119.5 million in one-time development and regulatory milestone payments. After achievements already recognized and milestones paid to date of \$41.0 million, we may be required to pay UCB up to \$78.5 million in additional payments. If we or any of our affiliates or sublicensees commercializes axatilimab, we will also be obligated to pay UCB low double-digit percentage 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. After achievements already recognized and milestones realized to date, we may be subject to pay \$240 million of 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. Over the term of the Vitae license agreement, and subject to the achievement of certain milestone events, we were required to pay Vitae up to \$99.0 million in one-time development and regulatory milestone payments. After achievements already recognized and milestones paid to date of \$38.0 million, we may be required to pay Vitae up to \$61.0 million in additional payments. 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 percentage 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 Annual 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 war between Russia and Ukraine as well as the ongoing conflicts in the Middle East.

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 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. 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 December 31, 2025, our executive officers, directors, and holders of 5% or more of our capital stock and their respective affiliates beneficially owned approximately 39.1% 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 begins 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 1B. Unresolved Staff Comments

None.

Item 1C. Cyber Security

Risk Management and Strategy

We have implemented and maintain various information security processes designed to identify, assess and manage material risks from cybersecurity threats to our critical computer networks, third-party hosted services, communications systems, hardware and software, and our critical data, including intellectual property, confidential information that is proprietary, strategic or competitive in nature, and our clinical trial and related data, or Information Systems and Data.

Our information security function, which is led by our Vice President of Information Technology, or VP of IT, helps identify, assess and manage our cybersecurity threats and risks. The information security function identifies and assesses cybersecurity threats and risks by monitoring and evaluating our threat environment and our risk profile using various methods including, for example: manual and automated tools, subscribing to reports and services that identify cybersecurity threats, analyzing reports of threats, evaluating threats reported to us, internal and external audits, leveraging third party threat assessments, conducting vulnerability identification assessments, and leveraging external threat intelligence.

Depending on the environment or system, we implement and maintain various technical, physical, and organizational measures, processes, standards and policies designed to manage and mitigate material risks from cybersecurity threats to our Information Systems and Data, including, for example: incident detection and response, disaster recovery and business continuity plans, encryption of certain data, network security controls, data segregation for certain data, access controls, physical controls, systems monitoring, penetration testing, and cybersecurity insurance. Certain information about our assessment and management of material risks from cybersecurity threats is included in risk management reports as applicable to senior leadership and the audit committee.

We use third-party service providers to assist us from time to time to identify, assess, and manage material risks from cybersecurity threats, including, for example: cybersecurity consultants, managed cybersecurity service providers, and professional service firms, including legal counsel, forensic investigators, and penetration testing service providers.

We also use third-party service providers to perform a variety of functions throughout our business, such as software-as-a-service providers, hosting companies, supply chain resources, contract research organizations, and contract manufacturing organizations. Depending on the nature of the services provided, the sensitivity of the critical systems, information and assets at issue, and the identity of the provider, we may conduct a review of security assessments provided by the vendor and impose contractual obligations related to cybersecurity on the vendor.

For a description of the risks from cybersecurity threats that may materially affect the Company and how they may do so, see our risk factors under Part 1. Item 1A. “Risk Factors” in this Annual Report, including *“If our information technology systems or those of third parties with whom we work, or our data are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse consequences.”*

Governance

Our board of directors addresses the Company’s cybersecurity risk management as part of its general oversight function. Our board of directors’ audit committee is responsible for overseeing the Company’s cybersecurity risk management processes.

Our cybersecurity risk assessment and management processes are implemented and maintained by certain Company management, including our VP of IT, who has over 25 years of experience in information technology including IT leadership in Department of Defense Research. Our VP of IT who reports to our Chief Financial Officer, or CFO, who has 23 years of experience as a CFO, including supervisory responsibility over IT and cybersecurity functions.

Our VP of IT is responsible for hiring appropriate personnel, helping to integrate cybersecurity risk considerations into the Company’s overall risk management strategy, and communicating key priorities to relevant personnel. Our VP of IT is also responsible for approving budgets, helping prepare for cybersecurity incidents, approving cybersecurity processes, and reviewing security assessments and other security-related reports.

Our cybersecurity incident processes are designed to escalate certain cybersecurity incidents to members of management depending on the circumstances, including our CFO, General Counsel, or GC, and Chief Executive Officer, or CEO. Our GC, CFO, and other leadership personnel work with our incident response team to help assess impact and mitigate and remediate cybersecurity incidents of which they are notified, in addition to notifying the audit committee of our board of directors, as appropriate.

Our board of directors’ audit committee receives annual reports from our CFO concerning our significant cybersecurity threats and risk and the processes the Company has implemented to address them. Our audit committee also has access to various reports, summaries or presentations related to cybersecurity threats, risk and mitigation.

Item 2. Properties

Our principal office is located in New York, NY, where we lease approximately 12,000 square feet of office space pursuant to a lease that expires in August 2028. In February 2025, following the expiration of our lease, we closed our office in Waltham, MA and made New York our principal office location. In August 2025, following the expiration of our lease, we also closed approximately 4,000 square feet of additional office space in New York, NY. We believe that our existing facilities are sufficient for our foreseeable future needs. If we determine that additional or new facilities are needed in the future, we believe that appropriate alternative space would be available to us on commercially reasonable terms.

Item 3. Legal Proceedings

We are not currently a party to any material legal proceedings.

Item 4. Mine Safety Disclosures

Not applicable.

PART II

Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common stock began trading on the Nasdaq Global Select Market on March 2, 2016, under the symbol “SNDX.” Prior to that time, there was no public market for our common stock.

Holders of Record

As of February 2, 2025, we had approximately 11 holders of record of our common stock. Certain shares are held in “street” name and accordingly, the number of beneficial owners of such shares is not known or included in the foregoing number. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

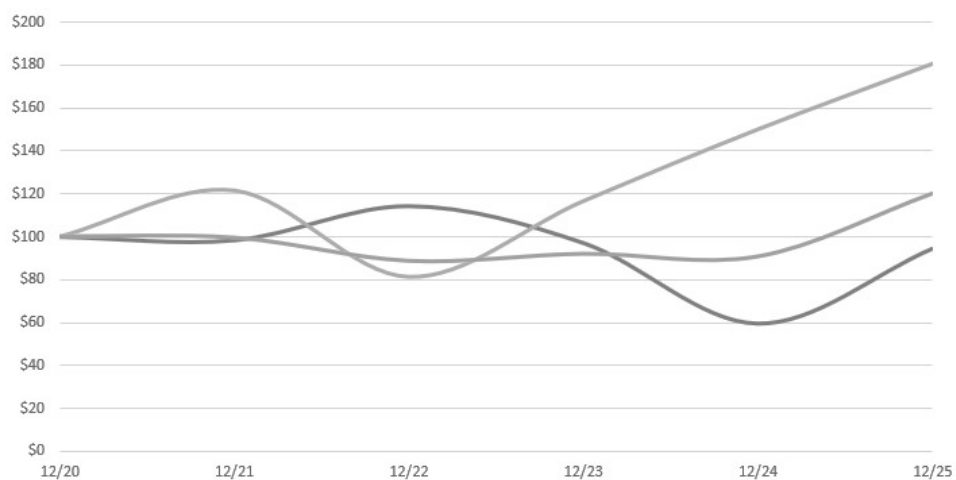
Dividend Policy

We have never declared or paid any cash dividends on our common stock. We currently intend to retain future earnings to fund the development and growth of our business. We do not expect to pay any cash dividends in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors and will depend on then-existing conditions, including our financial conditions, operating results, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

Performance Graph

The performance graph shown below compares the annual change in cumulative total stockholder return on our common shares with the Nasdaq Composite Index and the Nasdaq Biotechnology Index from December 31, 2020, through the year ended December 31, 2025. The graph assumes an investment of \$100 on December 31, 2020 in our common shares, the Nasdaq Composite Index and the Nasdaq Biotechnology Index and assumes that any dividends are reinvested. All index values are weighted by the capitalization of the companies included in the index. The comparisons shown in the graph below are based upon historical data. The stock price performance included in this graph is not necessarily indicative of future stock price performance. The following performance graph and related information shall not be deemed to be “soliciting material” or to be “filed” with the Securities and Exchange Commission, or SEC, for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, nor shall such information be incorporated by reference into any future filing under the Exchange Act or Securities Act, except to the extent that we specifically incorporate it by reference into such filing.

COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN*
Among Syndax Pharmaceuticals, Inc., the NASDAQ Composite Index
and the NASDAQ Biotechnology Index



*\$100 invested on 12/31/20 in stock or index, including reinvestment of dividends.
Fiscal year ending December 31.

— Syndax Pharmaceuticals, Inc. — NASDAQ Composite — NASDAQ Biotechnology

Item 6. [Reserved]

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis should be read in conjunction with our consolidated financial statements and related notes included elsewhere in this Annual Report. This discussion and analysis and other parts of this Annual Report contain forward-looking statements based upon current beliefs, plans and expectations that involve risks, uncertainties and assumptions, such as statements regarding our plans, objectives, expectations, intentions and projections. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under "Risk Factors" and elsewhere in this Annual Report. You should carefully read the "Risk Factors" section of this Annual Report to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section entitled "Special Note Regarding Forward-Looking Statements."

For the discussion of the financial condition and results of operations for the year ended December 31, 2024 compared to the year ended December 31, 2023, refer to "Management's Discussion and Analysis of Financial Condition and Results of Operations—Results of Operations" and "—Liquidity and Capital Resources" included in the Annual Report on Form 10-K filed with the SEC on March 3, 2025.

Overview

We are a commercial-stage biopharmaceutical company advancing innovative cancer therapies. We currently have two commercially approved medicines, Revuforj® (revumenib) and Niktimvo™ (axatilimab-csfr), and a robust slate of clinical development programs designed to unlock the full potential of our first two products.

Revuforj is our first-in-class menin inhibitor that was approved by the U.S. Food and Drug Administration, or FDA, in November 2024 for the treatment of relapsed or refractory, or R/R, acute leukemia with a lysine methyltransferase 2A gene, or KMT2A, translocation in adult and pediatric patients one year old and older. In October 2025, Revuforj received a second approval from the FDA for the treatment of R/R acute myeloid leukemia, or AML, with a susceptible nucleophosmin 1 mutation, or NPM1m, in adult and pediatric patients one year and older who have no satisfactory alternative treatment options. We are also studying revumenib in combination with standard-of-care agents in NPM1m AML and KMT2A-rearranged, or KMT2Ar, acute leukemia across the treatment landscape, including in newly diagnosed patients. Additionally, we are exploring the potential for menin inhibition in the treatment of myelofibrosis, or MF.

Niktimvo is our first-in-class colony stimulating factor-1 receptor, or CSF-1R, blocking antibody that was approved by the FDA in August 2024 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. Axatilimab is in development for the treatment of newly diagnosed cGVHD patients in combination with standard of care therapies, and for the treatment of idiopathic pulmonary fibrosis, or IPF.

We licensed the global rights to revumenib and axatilimab, the first two FDA approved medicines to emerge from our pipeline. We are leading the commercialization and further development of revumenib and working closely with our collaboration partner, Incyte, on the commercialization and further development of axatilimab. 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 incurred significant operating losses since our inception. While we generate product revenue from sales of Revuforj and collaboration as well as milestone revenue from sales of Niktimvo, 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 years ended December 31, 2025, 2024, and 2023, we reported a net loss of \$285.4 million and \$318.8 million, and \$209.4 million, respectively. As of December 31, 2025, we had an accumulated deficit of \$1.5 billion, which included non-cash charges for stock-based compensation, preferred stock accretion and extinguishment charges. As of December 31, 2025, we had cash, cash equivalents and short-term investments of \$394.1 million.

Significant Risks and Uncertainties

Ongoing high interest rates could make it more difficult for us to obtain traditional financing on acceptable terms, if at all. Additionally, the ongoing recession risk together with the foregoing, could result in further economic uncertainty and volatility in the capital markets in the near term and, as a result could negatively affect our operations. Furthermore, such economic conditions have produced downward pressure on share prices. Although we do not believe that inflation has had a material impact on our financial position or results of operations to date, the return of a high inflationary environment could increase our operating costs, including our labor costs and research and development costs. These costs may also be negatively impacted due to supply chain constraints, global geopolitical tensions, worsening macroeconomic conditions and employee availability and wage increases, which may result in additional stress on our working capital.

Additionally, we are subject to other 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

Net Product Revenue

Our second FDA-approved product, Revuforj, was approved by the FDA for commercial sale in the U.S. on November 15, 2024. Net product revenue from sales of Revuforj was \$124.8 million and \$7.7 million for the twelve months ended December 31, 2025 and 2024 respectively. In accordance with GAAP, we determine net product revenue for Revuforj, with specific assumptions for variable consideration components including, but not limited to, trade discounts and allowances, co-pay assistance programs and payor rebates. We record product revenue net of estimated discounts, chargebacks, rebates, product returns, and other gross-to-net revenue deductions.

We generated no net product revenue during the year ended December 31, 2023.

Collaboration revenue, net

In September 2021, we entered into the Incyte License and Collaboration Agreement, or the Incyte License, with Incyte covering the worldwide development and commercialization of axatilimab. In August 2024, the 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). Niktimvo sales began in February 2025.

In accordance with Topic ASC 808, *Collaboration Arrangements*, Incyte has been identified as the principal in product sales, therefore, we will recognize its 50% share of any profits or losses in the amount of net product sales less cost of goods sold and shared commercial and other expenses, including any royalties owed on license agreements, in the period in which the underlying sales and costs are recognized. Our share of net profits in connection with commercialization of Niktimvo will be presented as “Collaboration revenue, net” and our share of net losses will be presented as “Collaboration loss” within operating expenses. We will continue to recognize the costs associated with ongoing development services in the R&D operating expense line, including any cost-sharing components with Incyte.

Milestone, License, and Royalty Revenue

We enter into license agreements for the development and commercialization of our product candidates. License agreements may include non-refundable upfront payments, contingent milestone and/or royalty payments based on the occurrence of specified events under our license arrangements, partial or complete reimbursement of research and development expenses, license fees and royalties on sales if they are successfully approved and commercialized. Our performance obligations under the license agreements may include the transfer of intellectual property rights in the form of licenses, obligations to provide research and development services and related materials and participation on certain development and/or commercialization committees.

Revenue is recognized when, or as, performance obligations are satisfied, which occurs when control of the promised products or services is transferred to customers. Revenue is measured as the amount of consideration we expect to receive in exchange for transferring products or services to a customer, or the transaction price. To the extent that the transaction price includes variable consideration, we estimate the amount of variable consideration that should be included in the transaction price utilizing the most likely amount method. Variable consideration is included in the transaction price if, in our judgment, it is probable that a significant future reversal of cumulative revenue under the contract will not occur. Estimates of variable consideration and determination of whether to include estimated amounts in the transaction price are based largely on an assessment of our anticipated performance and all information (historical, current and forecasted) that is reasonably available.

We assessed the promises to determine if they are distinct performance obligations. Once the performance obligations are determined, the transaction price is allocated based on a relative standalone selling price basis. Milestone payments and royalties are typically considered variable consideration at the outset of the contract and are recognized in the transaction price either upon occurrence or when the constraint of a probable reversal is no longer applicable.

Cost of Product Sales

Our cost of product sales includes the cost of goods sold for Revuforj and license agreement royalties associated with its sales in the United States. Until we received regulatory approval for Revuforj in the United States in November 2024, we recorded expenses incurred for the manufacturing of pre-launch inventory that would support a U.S. launch as research and development expense. Cost of goods sold for a Revuforj may not include the full cost of manufacturing until the initial pre-launch, and previously expensed, inventory is depleted.

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, or CROs, 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. Product 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 product 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 products and 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 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 products and additional product 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 products and additional product candidates for the period, if any, in which material net cash inflows from these potential product 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 employee-related expenses, including salaries, benefits, non-cash stock-based compensation and travel expenses, for our employees in executive, finance, human resources, business development and support functions, as well as sales and marketing expenses to support the launch and commercialization of Revuforj and Niktimvo. 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.

Royalty Interest Expense

Royalty interest expense consists of the interest recorded related to the Royalty Pharma Purchase and Sale Agreement under the effective interest method.

Interest Expense

Interest expense consists primarily of expense related to the interest recognized for capital leases.

Interest Income

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

Other (Expense) Income, net

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

Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board, or FASB, or other standard setting bodies and adopted by us as of the specified effective date. Unless otherwise discussed in "Summary of Significant Accounting Policies" (Note 3) to our audited consolidated financial statements included in this Annual Report, we believe that the impact of recently issued standards that are not yet effective will not have a material impact on our financial position or results of operations upon adoption.

Critical Accounting Policies and Estimates

The preparation of our consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States, or U.S. GAAP, requires us to make estimates, judgments and assumptions that may affect the reported amounts of assets, liabilities, equity, revenue and expense and related disclosure of contingent assets and liabilities. On an ongoing basis we evaluate our estimates, judgments and assumptions. 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. Other significant accounting policies are outlined in "Summary of Significant Accounting Policies" (Note 3) to our consolidated financial statements included in this Annual Report.

We have listed below our critical accounting estimates that we believe to have the greatest potential impact on our consolidated financial statements. Historically, our assumptions, judgments and estimates relative to our critical accounting estimates have not differed materially from actual results.

Product Revenue Recognition

We recognize revenue when our customer obtains control of the promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. We recognize revenue following the five – step model prescribed under FASB Accounting Standards Codification (ASC 606), Revenue from Contracts with Customers: (i) identify contract(s) with customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) we satisfy the performance obligations. We only recognize revenue when it is probable that it will collect the consideration to which it is entitled in exchange for the goods or services that will be transferred to the customer.

We sell Revuforj to specialty distributors and specialty pharmacies, or collectively, Customers. These Customers subsequently resell Revuforj to healthcare providers, patients and other pharmacies. In addition to agreements with Customers, we enter into arrangements with healthcare providers and payors that provide for government-mandated and/or privately-negotiated rebates, chargebacks and discounts for the purchase of Revuforj.

We recognize revenue from product sales at the point Customers obtain control of the product, which generally occurs upon delivery. The transaction price that is recognized as product revenue includes an estimate of variable consideration which is described below. Payment terms with Customers do not exceed one year and, therefore, we do not account for a financing component in its arrangements. We expense incremental costs of obtaining a contract with a Customer (for example, sales commissions) when incurred as the period of benefit is less than one year. Shipping and handling costs for product shipments to Customers are recorded as selling, general and administrative expenses.

Revenue from products is recognized at the estimated net sales price (transaction price), which includes estimates of variable consideration. We include estimated amounts in the transaction price to the extent it is determined probable that a significant reversal of cumulative revenue recognized will not occur when the

uncertainty associated with the variable consideration is resolved. Our estimates of variable consideration and determination of whether to include estimated amounts in the transaction price are based largely on an assessment of its anticipated performance and all information (historical, current and forecasted) that is reasonably available. The components of our variable consideration around product revenue include provider chargebacks and discounts, trade discounts and allowances, product returns, government rebates, patient assistance, and distribution and other fees.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing contracts and vendor agreements, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. We make estimates of our accrued and prepaid clinical expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees paid to contract research organizations, or CROs, and investigative sites in connection with clinical studies and to vendors related to product manufacturing and development of clinical supplies.

We base our expenses related to clinical study and trial costs on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and CROs that conduct and manage clinical studies on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows and expense recognition. Payments under some of these contracts depend on factors out of our control, such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed, we may report amounts that are too high or too low in any particular period. To date, we have not experienced any significant adjustments to our estimates.

Results of Operations

Comparison of the years ended December 31, 2025 and 2024:

(in thousands)	Years Ended December 31,		2025 - 2024 Increase (Decrease)
	2025	2024	\$
Revenue:			
Net product revenue	\$ 124,844	\$ 7,680	\$ 117,164
Collaboration revenue, net	42,367	-	42,367
Milestone, license, and royalty revenue	5,141	16,000	(10,859)
Total revenue	172,352	23,680	148,672
Operating expenses:			
Cost of product sales	6,970	826	6,144
Research and development	258,784	241,647	17,137
Selling, general and administrative	179,682	120,879	58,803
Total operating expenses	445,436	363,352	82,084
Loss from operations	(273,084)	(339,672)	66,588
Other (expense) income, net:			
Royalty interest expense	(33,779)	(4,930)	(28,849)
Other interest (expense) income	(6)	1	(7)
Interest income	22,730	26,090	(3,360)
Other expense, net	(1,283)	(247)	(1,036)
Total other (expense) income, net	(12,338)	20,914	(33,252)
Net loss	<u>\$ (285,422)</u>	<u>\$ (318,758)</u>	<u>\$ 33,336</u>

Net product revenue

In November 2024, we began to generate product revenue from sales of Revuforj in the United States. Net product revenue from sales of Revuforj was \$124.8 million and \$7.7 million for the years ended December 31, 2025 and 2024, respectively.

Collaboration revenue, net

Collaboration revenue, net, representing our share of net profits in connection with commercialization of Niktimvo, for the year ended December 31, 2025 was \$42.4 million. We generated no collaboration revenue for the years ended December 31, 2024, as Niktimvo launched in January 2025.

Milestone, license, and royalty revenue

Milestone, license, and royalty revenue for the fiscal year ended December 31, 2025 included \$5.0 million milestone from Incyte, recognized for the achievement of \$150.0 million total net sales of Niktimvo under the Incyte Collaboration Agreement, and \$0.1 million of royalty revenue related to sales of entinostat.

In April 2024, a milestone was achieved under the Eddingpharm License Agreement for the marketing approval of entinostat in China. As a result, we recognized \$3.5 million of milestone revenue in the second quarter of 2024. As the receivable remains outstanding, we have assessed the receivable as non-recoverable and recorded a full reserve against the asset.

In August 2024, a milestone was achieved under the Incyte Collaboration Agreement for the approval of Niktimvo. As a result, we recognized \$12.5 million of milestone revenue in the third quarter of 2024.

Cost of Product Sales

Our cost of product sales were \$7.0 million and \$0.8 million for the twelve months ended December 31, 2025 and 2024, respectively. The increase is a result of increased sales of Revuforj. Included in cost of product sales are royalties owed to AbbVie on Revuforj sales as part of the Vitae License Agreement.

Research and Development

The following table summarizes the research and development expenses for the full years ended December 31, 2025 and 2024:

(in thousands)	Years Ended December 31,		2025 - 2024 Increase (Decrease)
	2025	2024	\$
Revumenib-related costs	\$ 112,422	\$ 107,909	\$ 4,513
Axatilimab-related costs	48,289	49,638	(1,349)
Other R&D programs	4,154	2,564	1,590
Personnel cost and other expenses	77,887	61,602	16,285
Stock-based compensation	16,032	19,934	(3,902)
Total research and development expenses	<u>\$ 258,784</u>	<u>\$ 241,647</u>	<u>\$ 17,137</u>

For the year ended December 31, 2025, our total research and development expenses increased by \$17.1 million from the prior year. The increase is primarily due to:

- An increase of \$4.5 million in revumenib-related costs due to the costs associated with initiation of trials evaluating revumenib in combination with standard-of-care agents in NPM1m AML and KMT2A acute leukemia in newly diagnosed patients, offset by the completion of an R/R NPM1m AML registrational trial that was ongoing in 2024 and a reduction in CMC expenses due to capitalization of inventory for commercial use. Higher development costs were also offset by a \$6.0 million decrease in revumenib-related milestone expense in 2025.
- A decrease of \$1.3 million in axatilimab related costs driven by a net reduction of \$5.0 million in milestone payments compared to prior year. This decrease was partially offset by higher expenses attributable to the ongoing Phase 2 IPF trial and our costs incurred in the execution of the frontline cGVHD combination trial with ruxolitinib.
- A increase in other research and development program related costs of \$1.6 million due to the de-prioritization of programs not pertaining to revumenib and axatilimab in the prior year period.
- An increase of \$16.3 million in personnel costs and other expenses, which includes salaries, overhead and related expenses to support on-going clinical trials, preparation of a supplemental New Drug Application, and medical affairs activities in support of commercialization of Revuforj and Niktimvo.
- A decrease of \$3.9 million in stock-based compensation expense related to the recognition of performance based awards in 2024 for certain regulatory achievements, as well as certain equity modifications.

The following table summarizes the selling, general and administrative expenses for the full years ended December 31, 2025 and 2024:

(in thousands)	Years Ended December 31,		2025 - 2024 Increase (Decrease)
	2025	2024	\$
Commercial related expenses	\$ 53,630	\$ 33,541	\$ 20,089
Other SG&A expenses	23,310	17,353	5,957
Personnel cost and other expenses	71,271	46,893	24,378
Stock-based compensation	31,471	23,092	8,379
Total selling, general and administrative expenses	<u>\$ 179,682</u>	<u>\$ 120,879</u>	<u>\$ 58,803</u>

For the year ended December 31, 2025, our total selling, general and administrative expenses increased by \$58.8 million from the prior year. The increase primarily is due to:

- An increase of \$20.1 million in commercial related expenses of which \$10.0 million of the increase is related to higher selling and marketing activities to support the launches of Revuforj and Niktimvo, and \$10.0 million of the increase was driven by a milestone expense triggered by the achievement of \$150.0 million in Niktimvo net sales in 2025.
- An increase of \$32.8 million in personnel costs and other expenses, including non-cash stock-based compensation, recognition of performance-based equity, based on certain commercial achievements. Also included are salaries, overhead and related expenses primarily due to increased headcount to support the commercialization of Revuforj and Niktimvo.
- An increase of \$6.0 million in other SG&A expenses related to increases in IT related expenses, corporate communications, legal and recruiting costs in support of commercialization and ongoing R&D activities.

Royalty Interest Expense

For the year ended December 31, 2025, royalty interest expense increased from the prior year due to 2025 being the first full year of accrued interest expense related to the Royalty Pharma Purchase and Sale Agreement.

Interest Income

For the year ended December 31, 2025, interest income, net of interest expense, decreased from the comparable prior year period. The decrease of interest income was primarily due to lower interest rates and a decreased average balance on cash equivalents and short- and long-term investments.

Other (Expense) Income, net

For the year ended December 31, 2025, the total other (expense) income, net increased from the comparable prior year period primarily due to the increase in foreign currency losses on short- and long-term investments.

Liquidity and Capital Resources

Overview

As of December 31, 2025, we had cash, cash equivalents and short-term investments totaling \$394.1 million. Since our inception, our operations have been primarily financed by net proceeds from our public stock offerings, revenue from our license agreements, and through our purchase and sale agreement with Royalty Pharma. We believe that our cash, cash equivalents and short-term investments as of December 31, 2025, will fund our projected operating expenses and capital expenditure requirements for at least the next 12 months. In addition to our existing cash, cash equivalents, short-term investments, we are eligible to receive research and development funding and to earn milestone and other contingent payments for the achievement of defined collaboration objectives and certain development, regulatory and commercial milestones, and royalty payments under our collaboration agreements. 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.

Purchase and Sale Agreement

In November 2024, we entered into a purchase and sale agreement with Royalty Pharma, pursuant to which Royalty Pharma purchased the right to receive a 13.8% royalty 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 are capped at \$822.5 million or 2.35 times the funded amount.

For additional details on our purchase and sale agreement with Royalty Pharma, see "Purchase and Sale Agreement" (Note 16) to our consolidated financial statements in this Annual Report.

Future Funding Requirements

We believe that the combination of our available cash, cash equivalents, short-term investments, as well as our expected product revenues from sales of Revuforj and Niktimvo collaboration revenue, is sufficient to fund existing and planned cash requirements. Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, clinical costs, commercialization costs, legal and other regulatory expenses and general overhead costs. We have based our estimates on assumptions that may prove to be incorrect, and we could use our capital resources sooner than we currently expect.

Additionally, the process of testing product candidates in clinical trials is costly, and the timing, progress and outcomes 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 growth rate;
- 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 approved products as well as 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 cost of maintaining and expanding sales, marketing and distribution capabilities for our products;
- 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 disruption 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 expect to continue to support our future cash needs through a combination of product sales, equity offerings, debt financings and additional funding from license and collaboration arrangements. Except for 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 cash requirements include the following contractual obligations as of December 31, 2025, and the effects that such obligations are expected to have on our liquidity and cash flows in future periods. For additional information, see our consolidated financial statements.

<i>(in thousands)</i>	Total	Less than 1 Year	1 to 3 Years	3 to 5 Years	More than 5 Years
Operating leases for office space ⁽¹⁾	\$ 1,735	\$ 656	\$ 1,079	\$ —	\$ —
Capital lease for office equipment ⁽²⁾	2	2	—	—	—
	<u>\$ 1,737</u>	<u>\$ 658</u>	<u>\$ 1,079</u>	<u>\$ —</u>	<u>\$ —</u>

- (1) In August 2022, we signed a 36-month extension of the lease for the office space in New York, NY. In May 2023, we signed a 27-month lease for an additional space in New York, NY. In October 2024, we signed a 36-month extension for the New York, NY office space. The minimum lease payments above do not include any related common area maintenance charges or real estate taxes.
- (2) In January 2022, we entered into two four-year non-cancelable leases for office equipment. In June 2023, we entered into one two-year non-cancelable lease for office equipment. All three leases are accounted for as a capital lease.

We have incurred losses and cumulative negative cash flows from operations since our inception, excluding year ending December 31, 2021. As of December 31, 2025, we had an accumulated deficit of \$1.5 billion. We anticipate that we will continue to incur significant losses for the near future. 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 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 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 under the shelf registration statement on Form S-3ASR (Registration No. 333-254661), which became automatically effective upon the filing on March 24, 2021. Our common stock will be sold at prevailing market prices at the time of the sale, and as a result, prices may vary. As of December 31, 2023, we sold 2,719,744 shares of common stock under the 2023 ATM Program for net proceeds of approximately \$42.1 million. There was no ATM equity activity during the twelve months ended December 31, 2025 and 2024.

Cash Flows

The following is a summary of cash flows:

(in thousands)	Years Ended December 31,		
	2025	2024	2023
Net cash used in operating activities	\$ (322,980)	\$ (274,903)	\$ (160,601)
Net cash provided by (used in) investing activities	290,036	(219,775)	117,609
Net cash provided by financing activities	13,676	353,367	264,132
Net (decrease) increase in cash and cash equivalents	<u>\$ (19,268)</u>	<u>\$ (141,311)</u>	<u>\$ 221,140</u>

Net Cash Used in Operating Activities

Net cash used in operating activities for the year ended December 31, 2025, was \$323.0 million and primarily consisted of our net loss of \$285.4 million adjusted for non-cash items including stock-based compensation of \$47.5 million, an investment increase of \$10.8 million, a net increase in operating assets and liabilities of \$79.3 million and non-cash operating lease expense of \$0.9 million. The significant items driving the increase in operating assets and liabilities include an increase in collaboration receivable, net, of \$43.0 million, an increase in accounts receivable of \$30.4 million, an increase in inventory of \$32.1 million, and an increase in prepaid expenses and other assets of \$14.5 million. This was partially offset by an increase in accrued expense and other current liabilities of \$42.2 million.

Net cash used in operating activities for the year ended December 31, 2024 was \$274.9 million and primarily consisted of our net loss of \$318.8 million adjusted for non-cash items including stock-based compensation of \$43.0 million, an investment increase of \$13.5 million, a net increase in operating assets and liabilities of \$13.3 million, and non-cash operating lease expense of \$1.0 million. The significant items driving the increase in operating assets and liabilities include an increase in accrued expenses and other liabilities of \$19.1 million, an increase in collaboration payable of \$12.0 million, an increase in accounts payable of \$1.7 million, and an increase in prepaid expenses and other assets of \$8.3 million.

Net Cash Provided by (Used in) Investing Activities

Net cash provided by investing activities for the year ended December 31, 2025 was \$290.0 million and was due to \$486.0 million in proceeds from the maturities of available-for-sale marketable securities, partially offset by the purchase of \$195.8 million of available-for-sale marketable securities.

Net cash used in investing activities for the year ended December 31, 2024 was \$219.8 million due to \$337.3 million in proceeds from the maturities of available-for-sale marketable securities, partially offset by the purchase of \$557.1 million of available-for-sale marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2025, was \$13.7 million and was primarily due to \$13.7 million of proceeds from the stock option exercises and ESPP purchases.

Net cash provided by financing activities for the year ended December 31, 2024, was \$353.4 million and was primarily due to proceeds of \$343.7 million from the Royalty Pharma Purchase and Sale agreement and \$9.7 million of proceeds from the stock option exercises and ESPP purchases.

Net Operating Loss and Research and Development Tax Credit Carryforwards

At December 31, 2025, we had federal and state tax net operating loss carryforwards, or NOLs, of approximately \$585.3 million and \$227.7 million, respectively. We have generated federal NOLs of \$563.2 million and state NOLs of \$8.7 million which have an indefinite carryforward period. The remaining \$22.1 million of federal NOLs and the \$219.0 million of state NOLs will begin to expire at various dates starting in 2026. At December 31, 2025, we had available research and development and orphan drug tax credits of approximately \$9.8 million and \$27.0 million, respectively. Income tax credits began to expire in 2024.

Effective January 1, 2024, we have reclassified as an active trade or business for tax purposes. This resulted in a reclass of capitalized start-up and other costs to net operating losses and valuation allowance in the 2025 tax statements.

Utilization of the net operating losses and credits may be subject to a substantial annual limitation due to ownership change limitations provided by the Internal Revenue Code of 1986, as amended. The annual limitation may result in the expiration of our net operating losses and credits before we can use them. We have recorded a valuation allowance on all of our deferred tax assets, including our deferred tax assets related to our net operating loss and research and development tax credit carryforwards.

Item 7A. 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 December 31, 2025, we had cash, cash equivalents and short-term investments of \$394.1 million, consisting of overnight investments, interest-bearing money market funds and highly rated federal bonds and short-term investments including 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-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-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 8. Financial Statements and Supplementary Data

Our consolidated financial statements, together with the report of our independent registered public accounting firm, appear in this Annual Report beginning on page F-1.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Evaluation of our Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2025. The term “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, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act are recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to our management, including our principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on an evaluation of our disclosure controls and procedures as of December 31, 2025, our principal executive officer and principal financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management’s Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the company’s principal executive and principal financial officers 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. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2025. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in *Internal Control – Integrated Framework (2013)*. Based on that assessment, our management concluded that, as of December 31, 2025, our internal control over financial reporting was effective.

Attestation Report of the Registered Public Accounting Firm

Deloitte & Touche LLP, the independent registered public accounting firm that audited the consolidated financial statements included in this Annual Report, has issued an attestation report on the effectiveness of internal control over financial reporting as of December 31, 2025, included herein.

Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the fiscal quarter ended December 31, 2025 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Syndax Pharmaceuticals, Inc.

Opinion on Internal Control over Financial Reporting

We have audited the internal control over financial reporting of Syndax Pharmaceuticals, Inc. and subsidiaries (the “Company”) as of December 31, 2025, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2025, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by COSO.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated financial statements as of and for the year ended December 31, 2025, of the Company and our report dated February 26, 2026, expressed an unqualified opinion on those financial statements.

Basis for Opinion

The Company’s management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management’s Annual Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company’s internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control over Financial Reporting

A company’s internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company’s internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company’s assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become

inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
February 26, 2026

Item 9B. Other Information

Trading Arrangements

During our last fiscal quarter, none of our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated a “Rule 10b5-1 trading arrangement” or a “non-Rule 10b5-1 trading arrangement,” as defined in Regulation S-K Item 408 for the purchase or sale of our securities.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not Applicable

PART III

Certain information required by Part III is omitted from this Annual Report because we will file with the SEC a definitive proxy statement pursuant to Regulation 14A, or the 2026 Proxy Statement, no later than 120 days after the end of our fiscal year ended December 31, 2025, and certain information included therein is incorporated herein by reference.

Item 10. Directors, Executive Officers, and Corporate Governance

The information required by this item is incorporated by reference to the information set forth in the sections titled “Information About Our Board of Directors,” “Executive Officers” and “The Board of Directors and Its Committees” and “Delinquent Section 16(a) Reports,” if applicable, in our 2026 Proxy Statement.

Item 11. Executive Compensation

The information required by this item is incorporated by reference to the information set forth in the sections titled “Compensation Discussion and Analysis,” “Executive Officer” and “Director Compensation,” “Pay Versus Performance” and “The Board of Directors and Its Committees - Compensation Committee Interlocks and Insider Participation” in our 2026 Proxy Statement.

We intend to promptly disclose on our website or in a Current Report on Form 8-K in the future (i) the date and nature of any amendment (other than technical, administrative or other non-substantive amendments) to the Code of Conduct that applies to our principal executive officer, principal financial officer, principal accounting officer or controller or persons performing similar functions and relates to any element of the code of ethics definition enumerated in Item 406(b) of Regulation S-K and (ii) the nature of any waiver, including an implicit waiver, from a provision of the Code of Conduct that is granted to one of these specified individuals that relates to one or more of the elements of the code of ethics definition enumerated in Item 406(b) of Regulation S-K, the name of such person who is granted the waiver and the date of the waiver. The full text of our Code of Conduct is available at the investors section of our website at www.syndax.com. The reference to our website address does not constitute incorporation by reference of the information contained at or available through our website, and you should not consider it to be a part of this Annual Report.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item is incorporated by reference to the information set forth in the section titled “Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters” and “Securities Authorized for Issuance Under Equity Compensation Plans” in our 2026 Proxy Statement.

Item 13. Certain Relationships and Related Transactions and Director Independence

The information required by this item is incorporated by reference to the information set forth in the sections titled “The Board of Directors and Its Committees – Board Independence” and “Certain Relationships and Related Party Transactions” in our 2026 Proxy Statement.

Item 14. Principal Accountant Fees and Services

The information required by this item is incorporated by reference to the information set forth in the sections titled “Independent Registered Public Accounting Firm Fees” and “Pre-Approval Policies and Procedures” contained in the proposal titled “Ratification of Selection of Independent Registered Public Accounting Firm” in our 2026 Proxy Statement.

PART IV

Item 15. Exhibits and Financial Statement Schedules

(a)(1) Financial Statements.

	Pages
<u>Report of Independent Registered Public Accounting Firm (PCAOB ID 34)</u>	F-2
<u>Consolidated Balance Sheets as of December 31, 2025 and 2024</u>	F-4
<u>Consolidated Statements of Operations for the Years Ended December 31, 2025, 2024 and 2023</u>	F-5
<u>Consolidated Statements of Comprehensive Loss for the Years Ended December 31, 2025, 2024 and 2023</u>	F-6
<u>Consolidated Statements of Stockholders' Equity for the Years Ended December 31, 2025, 2024 and 2023</u>	F-7
<u>Consolidated Statements of Cash Flows for the Years Ended December 31, 2025, 2024 and 2023</u>	F-8
<u>Notes to Consolidated Financial Statements</u>	F-10

(a)(2) Financial Statement Schedules.

All schedules have been omitted because they are not required or because the required information is given in the Consolidated Financial Statements or Notes thereto.

(a)(3) Exhibits.

Exhibit No.	Description
3.1	<u>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).</u>
3.2	<u>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).</u>
3.3	<u>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).</u>
4.1	<u>Specimen Common Stock Certificate of the Company (incorporated herein by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1/A (File No. 333-208861), as filed with the SEC on February 20, 2016).</u>
4.2	<u>Form of Pre-Funded Warrant issued pursuant to the securities purchase agreement between the Company and Certain Purchasers, dated December 16, 2021 (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on December 17, 2021).</u>
4.3	<u>Description of Capital Stock (incorporated herein by reference to Exhibit 4.3 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on March 3, 2025).</u>
10.1*	<u>2007 Stock Plan (incorporated herein by reference to Exhibit 10.3 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.2*	<u>2007 Stock Plan Amendment, dated as of March 8, 2013 (incorporated herein by reference to Exhibit 10.4 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.3*	<u>2007 Stock Plan Amendment, dated as of July 10, 2013 (incorporated herein by reference to Exhibit 10.5 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>

Exhibit No.	Description
10.4*	<u>2007 Stock Plan Amendment, dated as of January 23, 2014 (incorporated herein by reference to Exhibit 10.6 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.5*	<u>2007 Stock Plan Amendment, dated as of December 17, 2014 (incorporated herein by reference to Exhibit 10.7 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.6*	<u>2007 Stock Plan Amendment, dated as of May 28, 2015 (incorporated herein by reference to Exhibit 10.8 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.7*	<u>2007 Stock Plan Amendment, dated as of August 20, 2015 (incorporated herein by reference to Exhibit 10.9 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.8*	<u>Form of Incentive Stock Option Agreement under 2007 Stock Plan (incorporated herein by reference to Exhibit 10.10 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.9*	<u>Form of Non-Statutory Stock Option Agreement under 2007 Stock Plan (incorporated herein by reference to Exhibit 10.11 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.10*	<u>2015 Omnibus Incentive Plan (incorporated herein by reference to Exhibit 10.12 to the Company's Registration Statement on Form S-8 (File No. 333-210412), as filed with the SEC on March 25, 2016).</u>
10.11*	<u>Form of Incentive Stock Option Agreement under 2015 Omnibus Incentive Plan (incorporated herein by reference to Exhibit 4.13 to the Company's Registration Statement on Form S-8 (File No. 333-210412), as filed with the SEC on January 4, 2016).</u>
10.12*	<u>Form of Non-Qualified Option Agreement under 2015 Omnibus Incentive Plan (incorporated herein by reference to Exhibit 10.14 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on March 25, 2016).</u>
10.13*	<u>Form of Stock Unit Agreement under 2015 Omnibus Incentive Plan (incorporated herein by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on August 6, 2020).</u>
10.14*	<u>Form of Deferred Settlement Stock Unit Agreement under 2015 Omnibus Incentive Plan (incorporated herein by reference to Exhibit 10.14 to the Company's Annual Report on Form 10-K (file No. 001-37708), as filed with the SEC on March 12, 2021).</u>
10.15*	<u>2015 Employee Stock Purchase Plan (incorporated herein by reference to Exhibit 4.16 to the Company's Registration Statement on Form S-8 (File No. 333-210412), as filed with the SEC on March 25, 2016).</u>
10.16*	<u>Syndax Pharmaceuticals, Inc. 2023 Inducement Plan, as amended (incorporated herein by reference to Exhibit 10.16 to the Company's Annual Report on Form 10-K (filed No. 001-37708), as filed with the SEC on March 3, 2025).</u>
10.17*	<u>Form of Option Agreement pursuant to the Syndax Pharmaceuticals, Inc. 2023 Inducement Plan (incorporated herein by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on February 8, 2023).</u>
10.18*	<u>Form of Restricted Stock Unit Agreement pursuant to the Syndax Pharmaceuticals, Inc. 2023 Inducement Plan (incorporated herein by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on May 8, 2023).</u>

Exhibit No.	Description
10.19*	<u>Amended and Restated Executive Employment Agreement by and between the Company and Michael A. Metzger, dated as of February 2, 2022 (incorporated herein by reference to Exhibit 10.17 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on March 1, 2022).</u>
10.20*	<u>Amendment to Amended and Restated Executive Employment by and between the Company and Michael A. Metzger, dated as of February 26, 2024 (incorporated herein by reference to Exhibit 10.20 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on February 27, 2024).</u>
10.21*	<u>Amended and Restated Executive Employment Agreement by and between the Company and Luke J. Albrecht, dated as of April 27, 2020 (incorporated herein by reference to Exhibit 10.22 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on February 27, 2024).</u>
10.22*	<u>Amendment to Amended and Restated Executive Employment by and between the Company and Luke J. Albrecht, dated as of February 26, 2024 (incorporated herein by reference to Exhibit 10.23 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on February 27, 2024).</u>
10.23*	<u>Executive Employment Agreement by and between the Company and Keith A. Goldan, dated as of June 8, 2022 (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on June 13, 2022).</u>
10.24*	<u>Amendment to Executive Employment by and between the Company and Keith A. Goldan, dated as of February 26, 2024 (incorporated herein by reference to Exhibit 10.27 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on February 27, 2024).</u>
10.25*	<u>Executive Employment Agreement by and between the Company and Neil Gallagher, M.D., Ph.D., dated as of March 30, 2023 (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on March 30, 2023).</u>
10.26*	<u>Amendment to Executive Employment by and between the Company and Neil Gallagher, M.D., Ph.D., dated as of February 26, 2024 (incorporated herein by reference to Exhibit 10.30 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on February 27, 2024).</u>
10.27*	<u>Executive Employment Agreement by and between the Company and Steven Closter, dated as of March 18, 2024 (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on March 18, 2024).</u>
10.28*	<u>Executive Employment Agreement, dated May 12, 2025, by and between the Company and Dr. Nicholas Botwood (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 001-37708, as filed with the SEC on May 16, 2025).</u>
10.29*	<u>Non-employee Director Compensation Policy, as amended, dated as of February 5, 2025 (incorporated herein by reference to Exhibit 10.28 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on March 3, 2025).</u>
10.30*	<u>Non-employee Director Compensation Policy, as amended, dated as of February 4, 2026.</u>
10.31*	<u>Form of Indemnification Agreement by and between the company and each of its directors and officers (incorporated herein by reference to Exhibit 10.21 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.32†	<u>License, Development and Commercialization Agreement by and between the company and Bayer Schering Pharma AG, dated as of March 26, 2007 (incorporated herein by reference to Exhibit 10.22 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>

Exhibit No.	Description
10.33†	<u>First Amendment to the License, Development and Commercialization Agreement by and between the company and Bayer Pharma AG, dated as of October 13, 2012 (incorporated herein by reference to Exhibit 10.23 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.34	<u>Second Amendment to the License, Development and Commercialization Agreement by and between the company and Bayer Pharma AG, dated as of February 1, 2013 (incorporated herein by reference to Exhibit 10.24 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.35†	<u>Third Amendment to the License, Development and Commercialization Agreement by and between the company and Bayer Pharma AG, dated as of October 9, 2013 (incorporated herein by reference to Exhibit 10.25 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.36†	<u>Letter Agreement by and between the company and Bayer Pharma AG, dated as of September 18, 2014 (incorporated herein by reference to Exhibit 10.26 to the Company's Registration Statement on Form S-1 (File No. 333-208861), as filed with the SEC on January 4, 2016).</u>
10.37†	<u>License Agreement by and between the Company and UCB Biopharma Sprl, dated as of July 1, 2016 (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 001-37708), as filed with the SEC on October 7, 2016).</u>
10.38†	<u>Side Agreement by and between the Company and UCB Biopharma Sprl, dated March 8, 2017 (incorporated herein by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on May 9, 2017).</u>
10.39†	<u>Amendment No. 1 to License Agreement by and between the Company and UCB Biopharma Sprl, dated as of July 9, 2019 (incorporated herein by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on November 7, 2019).</u>
10.40†	<u>Mutual Release and Settlement Agreement by and between the Company and UCB Biopharma SRL, dated as of June 6, 2022 (incorporated herein by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on August 8, 2022).</u>
10.41†	<u>License Agreement by and between the Company and Vitae Pharmaceuticals, Inc., dated as of October 13, 2017 (incorporated herein by reference to Exhibit 10.47 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on March 8, 2018).</u>
10.42†	<u>Amendment No. 1 to License Agreement by and between the Company and Vitae Pharmaceuticals, Inc., dated as of January 25, 2019 (incorporated herein by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on May 8, 2019).</u>
10.43†	<u>Collaboration and License Agreement by and between the Company and Incyte Corporation, dated as of September 24, 2021 (incorporated herein by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on November 15, 2021).</u>
10.44	<u>Purchase Agreement by and between the Company and Incyte Corporation, dated as of September 24, 2021 (incorporated herein by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 001-37708), as filed with the SEC on November 15, 2021).</u>
10.45†	<u>Purchase and Sale Agreement by and between the Company and with Royalty Pharma Development Funding, LLC, dated November 4, 2024 (incorporated herein by reference to Exhibit 10.43 to the Company's Annual Report on Form 10-K (filed No. 001-37708), as filed with the SEC on March 3, 2025).</u>
19.1	<u>Insider Trading Policy.</u>

Exhibit No.	Description
21.1	<u>Subsidiaries of the Registrant.</u>
23.1	<u>Consent of Independent Registered Public Accounting Firm.</u>
24.1	<u>Power of Attorney (included on the signature page to this report).</u>
31.1	<u>Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.</u>
31.2	<u>Certification of the Principal Financial Officer and Principal Accounting Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.</u>
32.1+	<u>Certification of Principal Executive Officer and Principal Financial Officer pursuant to Rule 13a-14(b) or 15d-14(b) of the Exchange Act and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>
97	<u>Incentive Compensation Recoupment Policy (incorporated herein by reference to Exhibit 97 to the Company's Annual Report on Form 10-K (File No. 001-37708), as filed with the SEC on March 3, 2025).</u>
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Document.
104	Cover Page formatted as Inline XBRL and contained in Exhibit 101

* Indicates a management contract or compensatory plan.

+ Furnished herewith and not deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), 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.

† Confidential treatment has been granted for certain portions of this exhibit. These portions have been omitted and filed separately with the SEC.

Item 16. Form 10-K Summary

Not applicable.

SIGNATURES

Pursuant to the requirements of Section 13 of 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.

SYNDAX PHARMACEUTICALS, INC.

Date: February 26, 2026

By: /s/ Michael A. Metzger

Michael A. Metzger

Chief Executive Officer

POWER OF ATTORNEY

Each person whose individual signature appears below hereby authorizes and appoints Michael A. Metzger and Luke J. Albrecht, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file any and all amendments to this report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, ratifying and confirming all that said attorneys-in-fact and agents or any of them or their or his or her substitute or substitutes may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
<u>/s/ Michael A. Metzger</u> Michael A. Metzger	Chief Executive Officer and Director (Principal Executive Officer)	February 26, 2026
<u>/s/ Keith A. Goldan</u> Keith A. Goldan	Chief Financial Officer (Principal Financial and Accounting Officer)	February 26, 2026
<u>/s/ Dennis G. Podlesak</u> Dennis G. Podlesak	Chairman of the Board of Directors	February 26, 2026
<u>/s/ Martin H. Huber, M.D.</u> Martin H. Huber, M.D.	Director	February 26, 2026
<u>/s/ Jennifer Jarrett</u> Jennifer Jarrett	Director	February 26, 2026
<u>/s/ Keith A. Katkin</u> Keith A. Katkin	Director	February 26, 2026
<u>/s/ Pierre Legault</u> Pierre Legault	Director	February 26, 2026
<u>/s/ Aleksandra Rizo, M.D., Ph.D.</u> Aleksandra Rizo, M.D., Ph.D.	Director	February 26, 2026

Syndax Pharmaceuticals, Inc.
Index to Consolidated Financial Statements

	<u>Pages</u>
<u>Report of Independent Registered Public Accounting Firm (PCAOB ID 34)</u>	F-2
<u>Consolidated Balance Sheets as of December 31, 2025 and 2024</u>	F-4
<u>Consolidated Statements of Operations for the Years Ended December 31, 2025, 2024 and 2023</u>	F-5
<u>Consolidated Statements of Comprehensive Loss for the Years Ended December 31, 2025, 2024 and 2023</u>	F-6
<u>Consolidated Statements of Stockholders' Equity for the Years Ended December 31, 2025, 2024 and 2023</u>	F-7
<u>Consolidated Statements of Cash Flows for the Years Ended December 31, 2025, 2024 and 2023</u>	F-8
<u>Notes to Consolidated Financial Statements</u>	F-10

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and Board of Directors of Syndax Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Syndax Pharmaceuticals, Inc. and subsidiaries (the "Company") as of December 31, 2025 and 2024, the related consolidated statements of operations, comprehensive loss, stockholders' equity, and cash flows, for each of the three years in the period ended December 31, 2025, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2025 and 2024, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2025, in conformity with accounting principles generally accepted in the United States of America.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2025, based on criteria established in Internal Control — Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission and our report dated February 26, 2026, expressed an unqualified opinion on the Company's internal control over financial reporting.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current-period audit of the financial statements that was communicated or required to be communicated to the audit committee and that (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Prepaid and Accrued Clinical Costs— Refer to Notes 3 and 13 to the financial statements

Critical Audit Matter Description

The Company recognizes research and development expenses as incurred, which include costs relating to clinical trial and clinical study activities performed by the contract research organizations ("CROs"). Expenses related to clinical trials and studies are based on estimates of the services received and efforts expended pursuant to contracts with each of the CROs. Tracking the progress of the clinical study and trial activities, including payments made by the Company and by the CROs, allows the Company to record the appropriate expense, prepayments, and accruals under the terms of the agreements with the CROs.

We identified the estimates for research and development accrued and prepaid CRO expenses, including costs incurred by CROs as a critical audit matter due to the judgments necessary for management to estimate the level of services provided and the costs incurred for the service when the Company has not yet been invoiced or otherwise notified of actual costs. Prepaid CRO expenses are recorded within the balance of short-term deposits on the consolidated balance sheet, while accrued CRO expenses are recorded within the balance of accrued expenses and other current liabilities on the consolidated balance sheet. This required a high degree of auditor judgment and an increased extent of effort when performing audit procedures to audit management's estimates of such accrued and prepaid CRO expenses.

How the Critical Audit Matter Was Addressed in the Audit

Our audit procedures related to prepaid and accrued clinical costs included the following, among others:

- We tested the effectiveness of internal controls over the estimation of accrued CRO and prepaid CRO expense calculations, including management's controls over the assessment and estimation of the costs incurred for significant research and development activities performed by CROs.
- For a sample of contracts, we read the related contracts, purchase orders, statements of work and other contractual documentation. We tested the completeness and accuracy of the information used to develop the estimates and evaluated the significant assumptions used by management to estimate the recorded amounts by performing the following:
 - o Performed corroborating inquiries with the Company's research and development personnel to understand the nature and progress of the studies conducted by the CROs.
 - o Inspected information from third-party service provider CRO.
 - o Obtained corresponding invoices and evidence of payment to third-party service provider CROs.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
February 26, 2026

We have served as the Company's auditor since 2008.

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

	December 31,	
	2025	2024
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 134,930	\$ 154,083
Short-term investments	259,140	418,801
Accounts receivable, net	37,996	7,602
Inventory, net	32,754	366
Short-term deposits	19,616	10,029
Other receivables, net	6,404	3,635
Collaboration receivable, net	23,745	—
Prepaid expenses and other current assets	13,496	8,541
Total current assets	528,081	603,057
Long-term investments	—	119,520
Property and equipment, net	181	—
Right-of-use asset	1,342	2,022
Restricted cash	102	217
Total assets	<u>\$ 529,706</u>	<u>\$ 724,816</u>
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 16,580	\$ 11,626
Collaboration payable, net	—	19,231
Accrued expenses and other current liabilities	103,064	60,096
Current portion of royalty interest financing	-	12,116
Current portion of right-of-use liability	470	471
Current portion of capital lease	2	9
Total current liabilities	120,116	103,549
Long-term liabilities:		
Royalty interest financing, less current portion	343,909	331,565
Right-of-use liability, less current portion	1,051	1,578
Total long-term liabilities	344,960	333,143
Total liabilities	465,076	436,692
Commitments and contingencies (Note 18)		
Stockholders' equity:		
Preferred stock, \$0.001 par value, 10,000,000 shares authorized; 0 shares outstanding at December 31, 2025 and December 31, 2024	—	—
Common stock, \$0.0001 par value, 200,000,000 shares authorized at December 31, 2025 and December 31, 2024, respectively; 87,405,979 and 85,694,443 shares outstanding at December 31, 2025 and December 31, 2024, respectively	9	9
Additional paid-in capital	1,570,749	1,509,110
Accumulated other comprehensive income	452	163
Accumulated deficit	(1,506,580)	(1,221,158)
Total stockholders' equity	64,630	288,124
Total liabilities and stockholders' equity	<u>\$ 529,706</u>	<u>\$ 724,816</u>

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

SYNDAX PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except share and per share data)

	Years Ended December 31,		
	2025	2024	2023
Revenue:			
Net product revenue	\$ 124,844	\$ 7,680	\$ —
Collaboration revenue, net	42,367	—	—
Milestone, license, and royalty revenue	5,141	16,000	—
Total revenue	172,352	23,680	—
Operating expenses:			
Cost of product sales	6,970	826	—
Research and development	258,784	241,647	163,032
Selling, general and administrative	179,682	120,879	66,922
Total operating expenses	445,436	363,352	229,954
Loss from operations	(273,084)	(339,672)	(229,954)
Other (expense) income, net:			
Royalty interest expense	(33,779)	(4,930)	—
Other interest (expense) income	(6)	1	(208)
Interest income	22,730	26,090	21,163
Other expense, net	(1,283)	(247)	(361)
Total other (expense) income	(12,338)	20,914	20,594
Net loss	\$ (285,422)	\$ (318,758)	\$ (209,360)
Net loss attributable to common stockholders	\$ (285,422)	\$ (318,758)	\$ (209,360)
Net loss Per Share:			
Basic loss per share attributable to common stockholders	\$ (3.29)	\$ (3.72)	\$ (2.98)
Diluted loss per share attributable to common stockholders	\$ (3.29)	\$ (3.72)	\$ (2.98)
Weighted-average common shares used in calculating:			
Basic loss per share attributable to common stockholders	86,625,610	85,622,065	70,370,519
Diluted loss per share attributable to common stockholders	86,625,610	85,622,065	70,370,519

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

SYNDAX PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(In thousands)

	Years Ended December 31,		
	2025	2024	2023
Net loss	\$ (285,422)	\$ (318,758)	\$ (209,360)
Other comprehensive income (loss):			
Unrealized gain (loss) on marketable securities, net of tax	289	(55)	1,024
Comprehensive loss	<u>\$ (285,133)</u>	<u>\$ (318,813)</u>	<u>\$ (208,336)</u>

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

SYNDAX PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF
STOCKHOLDERS' EQUITY
(In thousands, except share and per share data)

	Common Stock \$0.0001 Par Value		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount				
Balance—January 1, 2023	68,111,385	\$ 7	\$ 1,161,288	\$ (806)	\$ (693,040)	\$ 467,449
Proceeds from direct offering, net of \$14,044 offering expenses	12,432,431	1	215,954	—	—	215,955
Proceeds from at-the-market offerings, net of \$1,086 offering expense	2,719,744	—	42,139	—	—	42,139
Stock-based compensation expense	—	—	30,951	—	—	30,951
Unrealized gain on investments	—	—	—	1,024	—	1,024
Stock purchase under ESPP	34,797	—	—	—	—	—
Employee withholdings ESPP	—	—	910	—	—	910
Pre-funded warrant cashless exercise	857,131	—	—	—	—	—
Vesting of RSU	9,102	—	—	—	—	—
Proceeds from exercise of stock options	662,042	—	5,128	—	—	5,128
Net loss	—	—	—	—	(209,360)	(209,360)
Balance—December 31, 2023	84,826,632	\$ 8	\$ 1,456,370	\$ 218	\$ (902,400)	\$ 554,196
Stock-based compensation expense	—	—	43,026	—	—	43,026
Unrealized loss on investments	—	—	—	(55)	—	(55)
Stock purchase under ESPP	64,903	—	—	—	—	—
Employee withholdings ESPP	—	—	1,715	—	—	1,715
Vesting of RSU	85,495	—	—	—	—	—
Proceeds from exercise of stock options	717,413	1	7,999	—	—	8,000
Net loss	—	—	—	—	(318,758)	(318,758)
Balance—December 31, 2024	85,694,443	\$ 9	\$ 1,509,110	\$ 163	\$ (1,221,158)	\$ 288,124
Stock-based compensation expense	—	—	47,963	—	—	47,963
Unrealized gain on investments	—	—	—	289	—	289
Stock purchase under ESPP	164,852	—	—	—	—	—
Employee withholdings ESPP	—	—	1,538	—	—	1,538
Vesting of RSU	410,707	—	—	—	—	—
Proceeds from exercise of stock options	1,135,977	—	12,138	—	—	12,138
Net loss	—	—	—	—	(285,422)	(285,422)
Balance—December 31, 2025	<u>87,405,979</u>	<u>\$ 9</u>	<u>\$ 1,570,749</u>	<u>\$ 452</u>	<u>\$ (1,506,580)</u>	<u>\$ 64,630</u>

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

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

	Years Ended December 31,		
	2025	2024	2023
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net loss	\$ (285,422)	\$ (318,758)	\$ (209,360)
Adjustments to reconcile net loss to net cash (used in) provided by operating activities:			
Depreciation	6	8	12
Accretion of investments	(10,753)	(13,468)	(14,803)
Non-cash operating lease expense	946	989	739
Stock-based compensation	47,503	43,026	30,951
Amortization of debt issuance costs	228	30	—
Provision for credit losses	3,667	—	—
Inventory reserves	163	—	—
Changes in operating assets and liabilities:			
Accounts receivable	(30,394)	(7,602)	—
Inventory	(32,091)	(366)	—
Prepaid expenses and other current assets	(14,542)	(8,393)	676
Collaboration (payable) receivable, net	(42,976)	11,999	10,706
Other receivable	(6,436)	(3,635)	—
Other assets	—	463	—
Accounts payable	4,954	1,665	5,611
Accrued expenses and other current liabilities	42,167	19,139	14,867
Net cash used in operating activities	<u>(322,980)</u>	<u>(274,903)</u>	<u>(160,601)</u>
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchases of property and equipment	(187)	—	—
Purchases of short and long-term investments	(195,807)	(557,052)	(354,606)
Proceeds from maturities of short-term investments	486,030	337,277	472,215
Net cash provided by (used in) investing activities	<u>290,036</u>	<u>(219,775)</u>	<u>117,609</u>
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from issuance of common stock in follow on public offerings, net	—	—	215,955
Proceeds from issuance of common stock in at-the-market offering, net	—	—	42,139
Proceeds from Purchase and Sale agreement, net	—	343,652	—
Proceeds from ESPP	1,538	1,715	910
Proceeds from stock option exercises	12,138	8,000	5,128
Net cash provided by financing activities	<u>13,676</u>	<u>353,367</u>	<u>264,132</u>
NET (DECREASE) INCREASE IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH	(19,268)	(141,311)	221,140
CASH, CASH EQUIVALENTS AND RESTRICTED CASH—beginning of year	154,300	295,611	74,471
CASH, CASH EQUIVALENTS AND RESTRICTED CASH—end of year	<u>\$ 135,032</u>	<u>\$ 154,300</u>	<u>\$ 295,611</u>
Supplemental Schedule of Cash Flow Information:			
Cash paid for royalty interest financing	\$ 13,191	\$ —	\$ —
Issuance costs in accounts payable and accrued expenses	—	—	250
Lease assets recognized upon lease remeasurement	—	1,524	—

The following table provides a reconciliation of cash, cash equivalents, and restricted cash equivalents reported within the consolidated balance sheets that sum to the total of the amounts shown in the consolidated statements of cash flows:

	Years Ended December 31,		
	2025	2024	2023
		(In thousands)	
Cash and cash equivalents	\$ 134,930	\$ 154,083	\$ 295,394
Restricted cash	102	217	217
Cash, cash equivalents and restricted cash	<u>\$ 135,032</u>	<u>\$ 154,300</u>	<u>\$ 295,611</u>

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

SYNDAX PHARMACEUTICALS, INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of Business

Syndax Pharmaceuticals, Inc., or the Company or Syndax, is a commercial-stage biopharmaceutical company developing an innovative pipeline of cancer therapies. The Company currently has two commercially approved medicines and a robust slate of clinical development programs.

Revuforj® (revumenib) is a first-in-class menin inhibitor that was FDA-approved in November 2024 for the treatment of relapsed or refractory, or R/R, acute leukemia with a lysine methyltransferase 2A gene, or KMT2A, translocation in adult and pediatric patients one year old and older. In October 2025, Revuforj received a second approval from the FDA for the treatment of R/R acute myeloid leukemia, or AML, with a susceptible nucleophosmin 1 mutation, or NPM1m, in adult and pediatric patients one year and older who have no satisfactory alternative treatment options. We are also studying revumenib in combination with standard-of-care agents in NPM1m AML or KMT2A acute leukemia across the treatment landscape, including in newly diagnosed patients. Additionally, we are exploring the potential for menin inhibition in myelofibrosis.

Niktimvo™ (axatilimab-csfr) is a first-in-class colony stimulating factor-1 receptor, or CSF-1R, blocking antibody that was FDA-approved in August 2024 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. Axatilimab is in development for the treatment of newly diagnosed cGVHD patients in combination with standard of care therapies, as well for the treatment of idiopathic pulmonary fibrosis. The Company plans to continue to leverage the technical and business expertise of its management team and scientific collaborators to license, acquire and develop additional therapeutics to expand its pipeline.

The Company is subject to risks common to companies with newly approved products and robust ongoing clinical programs, including, but not limited to, successful commercialization of its medicines, successful development of additional therapeutics, obtaining additional funding, protection of proprietary therapeutics, compliance with government regulations, fluctuations in operating results, dependence on key personnel and collaborative partners, and risks associated with industry changes. The Company's long-term success is dependent upon its ability to successfully market its current products, develop new product candidates, expand its oncology drug pipeline, earn revenue, obtain additional capital when needed, and ultimately, achieve profitable operations. Management expects to incur substantial losses on the ongoing development of its product candidates and the Company may not achieve positive cash flow from operations in the near future, if ever. As a result, the Company may continue to require additional capital to move forward with its business plan. While certain amounts of this additional capital were raised in the past, there can be no assurance that funds necessary beyond these amounts will be available in amounts or on terms sufficient to ensure ongoing operations.

The Company's management believes that the cash, cash equivalents and short-term investments balances as of December 31, 2025, should enable the Company to maintain its planned operations for at least 12 months from the issuance date of these financial statements. The Company's ability to fund all of its planned operations internally beyond that date, including the successful commercialization of its products and the completion of its ongoing and planned clinical trial activities, may be substantially dependent upon whether the Company can obtain sufficient funding on terms acceptable to the Company. Proceeds from additional capital transactions may allow the Company to accelerate and/or expand its planned research and development activities. In the event that sufficient funds were not available, the Company may be required to delay or reduce expenditures to conserve cash, which could involve scaling back or curtailing development and selling, general and administrative activities.

The Company is subject to challenges and risks specific to its business and ability to execute on the 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: successfully launching the Company's products; obtaining regulatory approval for additional indications for the Company's products and product candidates; delays or problems in the supply of the Company's 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; and the challenges of protecting and enhancing the Company's intellectual property rights; complying with applicable regulatory requirements.

2. Basis of Presentation

The Company has prepared the accompanying consolidated financial statements in conformity with accounting principles generally accepted in the United States of America or U.S. GAAP.

Certain prior amounts reported in the accompanying consolidated financial statements and notes thereto have been reclassified to conform to the current year presentation. Specifically, the change in royalty payable presented within the consolidated balance sheet for the period ended December 31, 2024, of \$0.3 million was originally reported within the caption royalty payable and had been separately presented in the comparative presentation. For the period ended December 31, 2025, the royalties payable to AbbVie and UCB, based on net sales of Revuforj and Niktimvo, respectively, is included in accrued expenses and other current liabilities. Additionally, the interest expense recognized as part of the Royalty Pharma Purchase and Sale Agreement is presented separately on the consolidated statement of operations. For the period ended December 31, 2024, this expense was reported within interest expense. Such reclassification did not affect loss from operations, total assets, total liabilities, cash used in operations, or net loss.

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, which the Company dissolved in July 2025. 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

Estimates

The preparation of 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 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. The Company's actual results may differ from these estimates under different assumptions or conditions.

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 consolidated financial statements.

Cash Equivalents

Cash equivalents include all highly liquid investments maturing within 90 days or less from the date of purchase. Cash equivalents include money market funds, corporate debt securities, U.S. government agency notes, and overnight deposits.

Restricted Cash

The Company classifies as restricted cash all cash pledged as collateral to secure long-term obligations and all cash whose use is otherwise limited by contractual provisions. Amounts are reported as non-current unless restrictions are expected to be released in the next 12 months.

Marketable Securities

All investments in marketable securities are classified as available-for-sale and are reported at fair value with unrealized gains and losses excluded from earnings and reported net of tax in accumulated other comprehensive income, which is a component of stockholders' equity. Unrealized losses that are determined to be other-than-temporary, based on current and expected market conditions, are recognized in earnings. Declines in fair value determined to be credit related are charged to earnings. The cost of marketable securities sold is determined by the specific identification method.

Segment Reporting

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker, or CODM, or decision-making group, in making decisions regarding resource allocation and assessing performance. The CODM is the Company's Chief Executive Officer. The Company has one operating and reportable segment.

Concentrations of Credit Risk

Cash and cash equivalents, restricted cash, and short- and long-term investments are financial instruments that potentially subject the Company to concentrations of credit risk. Substantially all of the Company's cash, cash equivalents, and short- and long-term investments were deposited in accounts at two financial institutions, and at times, such deposits may exceed federally insured limits. The Company has not experienced any losses in such accounts, and management believes that the Company is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held. The Company's available-for-sale investments primarily consist of government money market funds, corporate debt securities, commercial paper, credit card asset-backed securities and overnight deposits and potentially subject the Company to concentrations of credit risk.

Customer and Supplier Concentration

The Company has exposure to credit risk in accounts receivable from sales of Revuforj. Revuforj is sold to Specialty Distributors and Specialty Pharmacies, who subsequently sell Revuforj to pharmacies, hospitals and other customers. Four customers represented 92% of the Company's accounts receivable and net product sales as of and for the year ended December 31, 2025.

Five customers represented 100% of the Company's accounts receivable and net product sales as of and for the year ended December 31, 2024.

The Company outsources the manufacturing of its products to contract manufacturers. In addition, the Company currently relies on a limited number of suppliers for the active pharmaceutical ingredients in its products. Accordingly, the Company has concentration risk associated with its commercial manufacturing.

Accounts Receivable

Accounts receivable primarily relates to amounts due from customers, which are typically due within 45 to 65 days. The Company analyzes accounts that are past due for collectability. To date, the Company has not experienced any credit losses with respect to the collection of its accounts receivable and has not recorded an allowance for credit losses as of December 31, 2025 and 2024. The Company has no financial instruments with off balance sheet risk of loss.

Inventory

Inventories are stated at the lower of cost or net realizable value. Costs of inventories, which include amounts related to materials and manufacturing overhead, are determined on a first-in, first-out basis. An assessment of the

recoverability of capitalized inventory is performed during each reporting period and any excess and obsolete inventories are reserved for or written down to their estimated net realizable value in the period in which the impairment is first identified. The Company has recognized prepaid inventory within prepaid expenses and other current assets for the year ended December 31, 2025, representing the cost of goods purchased in advance from vendors, which will be recognized as inventory upon delivery and acceptance to the Company's designated location.

Property and Equipment

Property and equipment are recorded at cost. Depreciation is recorded using the straight-line method over the estimated useful lives of the assets (three to five years). Assets under capital leases are amortized over the shorter of their useful lives or lease term using the straight-line method. Major replacements and improvements are capitalized, while general repairs and maintenance are expensed as incurred.

Impairment of Long-Lived Assets

Long-lived assets are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be recoverable. When such events occur, the Company compares the carrying amounts of the assets to their undiscounted expected future cash flows. If this comparison indicates that there is impairment, the amount of impairment is calculated as the difference between the carrying value and fair value. To date, no such impairments have been recognized.

Revenue Recognition

The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. The Company recognizes revenue following the five – step model prescribed under FASB Accounting Standards Codification (ASC 606), Revenue from Contracts with Customers: (i) identify contract(s) with customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) we satisfy the performance obligations. The Company only recognizes revenue when it is probable that it will collect the consideration to which it is entitled in exchange for the goods or services that will be transferred to the customer.

Product Revenue:

The Company sells Revuforj to specialty distributors and specialty pharmacies, or collectively, Customers. These Customers subsequently resell Revuforj to healthcare providers, patients and other pharmacies. In addition to agreements with Customers, the Company enters into arrangements with healthcare providers and payors that provide for government-mandated and/or privately-negotiated rebates, chargebacks and discounts for the purchase of Revuforj.

The Company recognizes revenue from product sales at the point Customers obtain control of the product, which generally occurs upon delivery. The transaction price that is recognized as product revenue includes an estimate of variable consideration which is described below. Payment terms with Customers do not exceed one year and, therefore, the Company does not account for a financing component in its arrangements. The Company expenses incremental costs of obtaining a contract with a Customer (for example, sales commissions) when incurred as the period of benefit is less than one year. Shipping and handling costs for product shipments to Customers are recorded as selling, general and administrative expenses.

Revenue from products is recognized at the estimated net sales price (transaction price), which includes estimates of variable consideration. The Company includes estimated amounts in the transaction price to the extent it is determined probable that a significant reversal of cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is resolved. The Company's estimates of variable consideration and determination of whether to include estimated amounts in the transaction price are based largely on an assessment of its anticipated performance and all information (historical, current and forecasted) that is reasonably available. The components of the Company's variable consideration include the following:

Provider Chargebacks and Discounts

Chargebacks for fees and discounts to providers represent the estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to

Customers who directly purchase the product from the Company. Customers charge the Company for the difference between what they pay for the product and the ultimate selling price to the qualified healthcare providers. These components of variable consideration are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable. These reserves are recorded as a reduction of revenue and accounts receivable in the period in which the related product revenue is recognized.

Trade Discounts and Allowances

The Company generally provides Customers with discounts that include incentive fees which are explicitly stated in the Company's contracts. These discounts are recorded as a reduction of revenue and accounts receivable in the period in which the related product revenue is recognized. These reserves are recorded as a reduction of revenue and accounts receivable in the period in which the related product revenue is recognized.

Product Returns

Consistent with industry practice, the Company has a product returns policy that provides Customers a right of return for product purchased within a specified period prior to and subsequent to the product's expiration date. The Company estimates the amount of its products that may be returned and presents this amount as a reduction of revenue in the period the related product revenue is recognized, in addition to establishing a current liability. The Company considers several factors in the estimation process, including expiration dates of product shipped to Customers, inventory levels within the distribution channel, product shelf life, prescription trends and other relevant factors. These reserves are recorded as a reduction of revenue in the period in which the related product revenue is recognized.

Government Rebates

The Company is subject to discount obligations under state Medicaid programs and Medicare. Reserves related to these discount obligations are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability. The Company's liability for these rebates consists of estimates of claims for the current reporting period and estimated future claims that will be made for product that has been recognized as revenue but remains in the distribution channel inventories at the end of the reporting period. These reserves are recorded as a reduction of revenue in the period in which the related product revenue is recognized.

Patient Assistance

Other programs that the Company offers include voluntary co-pay patient assistance programs intended to provide financial assistance to eligible patients with prescription drug co-payments required by payors and coupon programs for cash payors. The calculation of the current liability for this assistance is based on an estimate of claims and the cost per claim that the Company expects to receive associated with product that has been recognized as revenue but remain as in the distribution channel inventories at the end of each reporting period. These reserves are recorded as a reduction of revenue in the period in which the related product revenue is recognized.

Distribution and Other Fees

We pay distribution and other fees to certain customers in connection with the sales of our products. We record distribution and other fees paid to our customers as a reduction of revenue, unless the payment is for a distinct good or service from the customer and we can reasonably estimate the fair value of the goods or services received. If both conditions are met, we record the consideration paid to the customer as an operating expense. These costs are typically known at the time of sale, resulting in minimal adjustments subsequent to the period of sale. These reserves are recorded as a reduction of revenue in the period in which the related product revenue is recognized.

Development Milestone Payments: The Company evaluates whether milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are generally not considered probable of being achieved until those approvals are received. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of such development milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect license fees and earnings in the period of adjustment.

Commercial Milestone Payments and Royalties: For arrangements that include sales-based royalties, including milestone payments based on the level of commercial sales, and the license is deemed to be the predominant item to which the royalties or commercial milestones relate, the Company will recognize revenue at the later of when the related sales occur or when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). A \$10.0 million commercial milestone expense was recognized as part of selling, general and administrative expenses in the fourth quarter of 2025 upon the achievement of \$150.0 million of net sales of Niktimvo.

When no performance obligations are required of the Company, or following the completion of the performance obligation period, such amounts are recognized as revenue upon transfer of control of the goods or services to the customer. Generally, all amounts received or due other than sales-based milestones and royalties are classified as license fees.

Deferred revenue arises from amounts received in advance of the culmination of the earnings process and is recognized as revenue in future periods as performance obligations are satisfied. Deferred revenue expected to be recognized within the next twelve months is classified as a current liability. Upfront payment contract liabilities resulting from the Company's license agreements do not represent a financing component as the payment is not financing the transfer of goods or services, and the technology underlying the licenses granted reflects research and development expenses already incurred by the Company.

For additional information on our collaboration and license arrangements, please read *Note 4, Collaborative Research and License Agreements*, to these consolidated financial statements.

Advertising expenses

The Company expenses the costs of advertising, including promotional expenses, as incurred. Advertising expenses were \$14.4 million, \$7.3 million, and \$6.1 million for the years ended December 31, 2025, 2024, and 2023, respectively.

Research and Development

Research and development costs are expensed as incurred. Research and development expenses include payroll and personnel expenses, consulting costs, external contract research and development expenses, and allocated overhead, including rent, equipment depreciation, and utilities. Research and development costs that are paid in advance of performance are capitalized as a prepaid expense and amortized over the service period as the services are provided. The Company expenses upfront license payments related to acquired technologies that have not yet reached technological feasibility and have no alternative future use.

In instances where the Company enters into cost-sharing arrangements, all research and development costs reimbursed by the collaborators are accounted for as reductions to research and development expense. During the years ended December 31, 2025, 2024 and 2023 the Company has incurred external costs related to the Incyte cost-sharing collaboration.

Clinical Costs

Clinical study and trial costs are a component of research and development expenses. The Company expenses clinical trial activities performed by third parties based on an evaluation of the progress to completion of specific contract tasks using data such as patient enrollment, clinical site activations, or other information provided to us by our vendors. Depending on the progression of the contract and timing of invoicing and payment the research and development expense can be an accrued expense or a prepaid expense. Prepaid clinical study and trial costs are included in short-term deposits on the accompanying consolidated balance sheet.

Income Taxes

The Company records deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the Company's financial statement carrying amounts and the tax bases of assets and liabilities and for loss and credit carryforwards using enacted tax rates expected to be in effect in the years in which the differences reverse. A valuation allowance is provided to reduce the net deferred tax assets to the amount that will more likely than not be realized. The Company determines whether it is more likely than not that a tax position will be sustained upon examination. If it is not more likely than not that a position will be sustained, none of the benefit attributable to the position is recognized. The tax benefit to be recognized for any tax position that meets the more-likely-than-not recognition threshold is calculated as the largest amount that is more than 50% likely of being realized upon resolution of the contingency. The Company accounts for interest and penalties related to uncertain tax positions as part of its provision for income taxes.

Guarantees and Indemnifications

As permitted under Delaware law, the Company indemnifies its officers, directors, and employees for certain events or occurrences that happen by reason of the relationship with, or position held at, the Company. The Company has standard indemnification arrangements under office leases (as described in Note 5, *Leases* to these consolidated financial statements) that require it to indemnify the landlord against all costs, expenses, fines, suits, claims, demands, liabilities, and actions directly resulting from any breach, violation, or nonperformance of any covenant or condition of the Company's lease. Through December 31, 2025, the Company had not experienced any losses related to these indemnification obligations and no claims were outstanding. The Company does not expect significant claims related to these indemnification obligations, and consequently, concluded that the fair value of these obligations is negligible, and no related reserves were established.

Stock-Based Compensation

The Company accounts for all stock option awards granted to employees and non-employees using a fair value method. Stock-based compensation is measured at the grant date fair value of the stock option grants and is recognized over the requisite service period of the awards (usually the vesting period) on a straight-line basis. For equity awards that have a performance condition, the Company recognizes compensation expense based on its assessment of the probability that the performance condition will be achieved. The Company accounts for forfeitures as they occur.

(Loss) Earnings Per Share

Basic earnings per share is computed by dividing undistributed net income attributable to the Company by the weighted-average number of common shares outstanding during the period. Diluted earnings per share is computed based on the treasury method by dividing net income by the weighted-average number of common shares outstanding during the period plus potentially dilutive common equivalent shares outstanding.

Recently Issued and Adopted Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board 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 financial statements or disclosures.

In December 2023, the FASB issued Accounting Standards Update ("ASU") 2023-09, *Improvements to Income Tax Disclosures* ("ASU 2023-09"), which intends to enhance the transparency, as well as the usefulness, of

income tax disclosures, primarily related to the rate reconciliation and income taxes paid. The Company has adopted the guidance relevant to ASU 2023-09 prospectively, expanding its applicable income tax disclosures.

In November 2024, the FASB issued ASU 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* (ASU “2024-03”). Among other items, the requirements include expanded disclosures around employee compensation and selling expenses. ASU 2024-03 will be effective for the Company for the year ending December 31, 2027. The Company is still evaluating the impact of this new guidance on its unaudited consolidated financial statements but expects the adoption to result in disclosure changes only.

4. 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 axatilimab as Niktimvo™ (axatilimab-csfr), which was approved in August 2024. The Company and Incyte share equally the profits and losses from co-commercialization efforts in the United States.

Incyte is responsible for leading all aspects of commercialization of axatilimab in the United States. The Company and Incyte will share equally the profits and losses from co-commercialization efforts in the United States. The Company and Incyte have agreed to co-develop axatilimab and to share development costs associated with global and 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 non-U.S. countries. All development costs related to the collaboration with Incyte will be subject to a joint development plan.

Under the terms of the Incyte Agreements, the Company is eligible 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.

The Incyte Agreement and the Incyte Share Purchase Agreement were executed on the same date and negotiated simultaneously. Management therefore concluded that the Incyte Agreements are to be combined for accounting purposes and therefore allocated the total consideration to the units of account identified. The common stock issued to Incyte was recorded at fair value of \$24.8 million.

The Company evaluated the terms of the Incyte Agreement and determined it is within the scope of Accounting Standard Update 2018-18, *Collaborative Arrangements (Topic 808)*, and has elements that are within the scope of *Topic 606* and *Topic 808*.

The Company identified the following promises in the Incyte Agreements that were evaluated under the scope of *Topic 606*: (i) delivery of a license for axatilimab to develop, commercialize, and conduct medical affairs and (ii) services to be performed in accordance with the development plan. The Company also evaluated whether certain options outlined within the Incyte Agreements represented material rights that would give rise to a performance obligation and concluded that none of the options convey a material right to Incyte and therefore are not considered separate performance obligations within the Incyte Agreements.

The Company assessed the above promises and determined that the license for axatilimab represents the only performance obligation within the scope of *Topic 606*. The license for axatilimab is considered functional intellectual property and distinct from other promises under the contract as Incyte can benefit from the license on its own or together with other readily available resources. The license represents a separate performance obligation within a contract with a customer under the scope of *Topic 606* at contract inception.

The Company considers the collaborative research and development activities and manufacturing activities to be separate units of account within the scope of *Topic 808* and are not deliverables under *Topic 606*. The Company and Incyte are both active participants in the activities and are exposed to significant risks and rewards that are dependent on the commercial success of the activities in the arrangement.

The Company used the most likely amount method to estimate variable consideration and estimated that the most likely amount for each potential preclinical, development, and regulatory variable consideration milestone payment under this agreement is zero, as achievement of those milestones is uncertain and highly susceptible to factors outside the Company's control. Accordingly, all such milestone payments were excluded from the transaction price. Management will reevaluate the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur, will adjust the transaction price as necessary. Sales based royalties, including milestones based on the level of sales, were also excluded from the transaction price, as the license is deemed to be the predominant item to which the royalties relate. The Company will recognize such revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

The Company considers the collaborative research and development activities and manufacturing activities to be separate units of account within the scope of *Topic 808* and are not deliverables under *Topic 606*. The Company and Incyte are both active participants in the activities and are exposed to significant risks and rewards that are dependent on the commercial success of the activities in the arrangement.

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 the third quarter of 2024. A \$5.0 million milestone receivable was recognized in the fourth quarter of 2025 upon the achievement of \$150.0 million of net sales of Niktimvo.

As of December 31, 2025, the Company has recorded \$28.1 million as a collaboration receivable due from Incyte related to the Company's portion of the profit share, commercialization and development costs under the Incyte Agreements and has recorded approximately \$4.4 million as a collaboration payable due to Incyte for development and commercialization costs incurred by Incyte as of December 31, 2025.

5. Leases

Leases

The Company accounts for leases in accordance with *ASC 842, Leases*, and determines whether an arrangement is a lease at inception. Operating lease right-of-use, or ROU, assets and lease liabilities are recognized based on the present value of the future minimum lease payments over the lease term at commencement date. Lease agreements with lease and non-lease components are accounted for separately. For leases that do not provide an implicit rate, the Company uses the incremental borrowing rate based on the information available at commencement date in determining the present value of future payments. The ROU asset also includes any lease payments made and excludes lease incentives and initial direct costs incurred. The lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Leases with an initial term of 12 months or less are not recorded on the balance sheet as the Company has elected to apply the short-term lease exemption. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term.

The Company identified three existing building leases under ASC 842 that are classified as operating leases. In September 2016, the Company entered into a five-year operating lease for 12,207 square feet of office space in Waltham, MA, with a lease commencement date of March 1, 2017. On August 17, 2021, the Company signed a 36-

month extension to the lease for the Waltham, MA office with aggregate payments of \$1.6 million, with a lease commencement date of March 1, 2022. As of December 31, 2025, the lease term has ended.

In December 2015, the Company entered into a 62-month operating lease for 4,039 square feet of space in New York, NY, which commenced on January 1, 2016. In February 2021, the Company signed an 18-month extension to the lease for the New York office, which commenced on March 1, 2021. In August 2022, the Company signed a 36-month extension to the lease for the New York office, with aggregate payments of \$0.7 million, with a lease commencement date of September 1, 2022. As of December 31, 2025, the lease term has ended.

In May 2023, the Company entered into a 27-month operating lease for an additional 12,217 square feet of space in New York, NY, with aggregate payments of \$1.6 million, with a lease commencement date of June 1, 2023. In October 2024, the Company signed a 36-month extension to the lease for the New York office, with aggregate payments of \$2.5 million, with a lease commencement date of October 23, 2024. The Company recognized \$1.5 million of ROU asset and lease liability upon the remeasurement of the lease as a result of the extension. The remaining lease term as of December 31, 2025 was 32 months.

As of December 31, 2025, the consolidated balance sheet includes a \$1.3 million operating lease ROU asset and a \$1.5 million ROU liability. The Company used an incremental borrowing rate of 10%-14% to calculate its lease obligations, and an increase or decrease in the rate does not have a significant impact on the ROU asset or ROU liability. The ROU asset is amortized on a straight-line basis over the remainder of the lease term. For the year ended December 31, 2025, the Company recorded approximately \$0.9 million in operating lease expense and made approximately \$0.8 million in lease payments.

Future minimum lease payments under the Company's operating leases, were as follows:

Maturity of lease liabilities (in thousands)	As of December 31, 2025
2026	656
2027	676
2028	403
Thereafter	—
Total lease payments	\$ 1,735
Less: imputed interest	(214)
Total operating lease liability	\$ 1,521

Future minimum lease payments under the Company's capital leases as of December 31, 2025 and 2024, were \$2,244 and \$9,435, respectively.

6. Loss per Share

Basic and diluted loss per share are calculated as follows (in 000s except share data):

	Years Ended December 31,		
	2025	2024	2023
Numerator - basic and diluted:			
Net loss - basic and diluted	\$ (285,422)	\$ (318,758)	\$ (209,360)
Net loss attributable to common stockholders - basic and diluted	\$ (285,422)	\$ (318,758)	\$ (209,360)
Denominator - basic and diluted:			
Weighted-average common shares outstanding - basic and diluted	86,625,610	85,622,065	70,370,519

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, in 000s):

		December 31,	
	2025	2024	2023
Options to purchase common	13,128	11,688	10,685
Non-vested RSUs	2,618	1,304	309
ESPP to purchase common stock	127	70	35

For additional information related to the Company's common stock see *Note 14, Common Stock* to these consolidated financial statements.

7. Significant Agreements

Vitae Pharmaceuticals, Inc.

In October 2017, the Company entered into a license agreement, or the Vitae License Agreement, with Vitae Pharmaceuticals, Inc., a subsidiary of AbbVie, or AbbVie, under which Vitae granted the Company an exclusive, sublicensable, worldwide license to a portfolio of preclinical, orally available, small molecule inhibitors of the interaction of menin with Mixed Lineage Leukemia, or MLL, protein, or the Menin Assets. The Company made a nonrefundable upfront payment of \$5.0 million to Vitae in the fourth quarter of 2017. Additionally, subject to the achievement of certain milestone events, the Company may be required to pay 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 the Company or any of its affiliates or sublicensees commercializes the Menin Assets, the Company will also be obligated to pay AbbVie low single to low double-digit percentage 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, the Company may be required to share a percentage of non-royalty income from sublicensees, subject to certain deductions, with AbbVie. 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 will at any time upon advance written notice to AbbVie. AbbVie 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. As a result, in 2017, the upfront payment of \$5.0 million was recorded as research and development expense in the consolidated statements of operations. Since the inception of the agreement, the Company achieved certain development and regulatory milestones resulting in \$38.0 million in expense, which includes an \$18.0 million milestone expense in 2024 and a \$12.0 million expense in 2025, related to the submission of a supplemental New Drug Application, or sNDA, for Revuforj and the first United States sale of Revuforj in its second indication.

UCB Biopharma Sprl

In July 2016, the Company entered into a license agreement, or the UCB License Agreement, 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 Investigational New Drug Application-ready anti-CSF-1R monoclonal antibody. The Company made a nonrefundable upfront payment of \$5.0 million to UCB in the third quarter of 2016. Additionally, subject to the achievement of certain milestone events, the Company 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. 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 percentage 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 will be solely responsible for the development and commercialization of axatilimab, except that UCB is 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 will 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. 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 \$41.0 million as research and development expense, which includes a \$15.0 million milestone paid during the third quarter of 2024 upon the approval of Niktimvo, a \$10 million milestone recognized in the first quarter of 2025 for the first patient dosed in a Phase III study with the compound in a combination with another agent. The Company has also recognized a \$10.0 million commercial milestone expense in the fourth quarter of 2025 upon the achievement of \$150.0 million net sales of Niktimvo.

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

In March 2007, the Company entered into a license agreement, or the Bayer 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. Under the terms of the Bayer Agreement, the Company paid a nonrefundable up-front license fee of \$2.0 million and is responsible for the development and marketing of entinostat. The Company recorded the \$2.0 million license fee as research and development expense during the year ended December 31, 2007, as it had no alternative future use. 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.

8. Other Receivables, net

In April 2024, entinostat received marketing approval in China, and as a result, the Company recorded \$3.5 million of milestone revenue in 2024. As of December 31, 2025, the Company had recorded a \$3.7 million receivable related to milestones (plus accrued interest) under the license, development, and commercialization agreement, or the Eddingpharm License Agreement, with Eddingpharm Investment Company Limited, or Eddingpharm. As the receivable remains outstanding and the Company has assessed the amount as non-recoverable, the Company has recorded a full reserve against the asset as a selling, general and administrative expense as of December 31, 2025.

In April 2025, the Company submitted an sNDA for Revuforj with the FDA. In October 2025, the Company paid a \$1.3 million Prescription Drug User Fee Act (PDUFA) fee, while awaiting an FDA-decision on its submitted waiver of such fee. As Revuforj is an orphan drug, it is exempt from the PDUFA fee. As of December 2025, the Company has recorded a \$1.3 million receivable for the fee refund, and in February 2026, the refund was received from the FDA.

In December 2025, the Company recorded \$5.0 million of milestone revenue as a result of achieving \$150.0 million of Niktimvo net sales. As of December 31, 2025, the Company had recorded \$5.0 million in milestone receivable under the Incyte Collaboration and License Agreement.

9. Inventory, net

Inventory consisted of the following (in thousands):

	December 31,	
	2025	2024
Raw materials	\$ 29,027	\$ —
Work-in-process	2,112	330
Finished goods	1,615	36
Total Inventory	<u>\$ 32,754</u>	<u>\$ 366</u>

Inventories are stated at the lower of cost or net realizable value, as determined on a first-in, first-out basis.

Prior to the FDA's approval of Revuforj in November 2024, all costs for the manufacturing of product to support clinical development and commercial launch, including pre-launch inventory, were expensed as research and development costs. The pre-launch inventory will continue to be used in commercial production until it is depleted.

We have recorded a reserve of \$0.2 million for inventory expected to expire in the first quarter of 2026. This reserve is reflected as a reduction of the carrying value of inventory.

10. Property and Equipment, net

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

	December 31,	
	2025	2024
Equipment	\$ 271	\$ 84
Leasehold improvements	167	167
Furniture and fixtures	134	134
Office and computer equipment	34	34
Total property and equipment	606	419
Accumulated depreciation	(425)	(419)
Property and equipment, net	<u>\$ 181</u>	<u>\$ —</u>

Depreciation expense was \$6,000 and \$8,000 for the years ended December 31, 2025 and 2024, respectively.

11. 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):

	Total Carrying Value	Fair Value Measurements Using		
		Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
<u>December 31, 2025</u>				
Assets:				
Cash equivalents	\$ 115,358	\$ 115,358	\$ —	\$ —
Short-term investments	259,140	—	259,140	—
Long-term investments	—	—	—	—
Total assets	<u>\$ 374,498</u>	<u>\$ 115,358</u>	<u>\$ 259,140</u>	<u>\$ —</u>
<u>December 31, 2024</u>				
Assets:				
Cash equivalents	\$ 154,083	\$ 129,187	\$ 24,896	\$ —
Short-term investments	418,801	—	418,801	—
Long-term investments	119,520	—	119,520	—
Total assets	<u>\$ 692,404</u>	<u>\$ 129,187</u>	<u>\$ 563,217</u>	<u>\$ —</u>

There have been no material impairments of our assets measured and carried at fair value during the years ended December 31, 2025, and 2024. In addition, there have been no changes in valuation techniques during the years ended December 31, 2025, and 2024. 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 cash equivalents, short- and long-term investments and Royalty interest financing was determined other than quoted prices in active markets, which are either directly or indirectly observable as of the reporting date and fair value is determined using models or other valuation methodologies.

The short- and long-term investments are classified as available-for-sale securities. As of December 31, 2025, the remaining contractual maturities of the available-for-sale securities were 1 to 12 months, and the balance in the Company's accumulated other comprehensive income 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 years ended December 31, 2025. As a result, the Company did not reclassify any amounts out of accumulated other comprehensive income for the same periods. The Company has a limited number of available-for-sale securities in insignificant loss positions as of December 31, 2025, which the Company does not intend to sell and has concluded will not be required to sell before recovery of the amortized cost for the investment at maturity.

The following table summarizes the available-for-sale securities (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized (Losses)	Fair Value
December 31, 2025				
Commercial paper	\$ 86,066	\$ 15	\$ —	\$ 86,081
Corporate bonds	28,227	30	—	28,257
US Treasury	144,395	407	—	144,802
	<u>\$ 258,688</u>	<u>\$ 452</u>	<u>\$ —</u>	<u>\$ 259,140</u>
December 31, 2024				
Commercial paper	\$ 263,952	\$ —	\$ (56)	\$ 263,896
Corporate bonds	40,261	—	—	40,261
US Treasury	233,945	219	—	234,164
	<u>\$ 538,158</u>	<u>\$ 219</u>	<u>\$ (56)</u>	<u>\$ 538,321</u>

12. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following (in thousands):

	December 31,	
	2025	2024
Prepaid insurance	\$ 1,590	\$ 1,276
Interest receivable on investments	2,146	2,634
Prepaid subscriptions	2,221	1,605
Prepaid state and local taxes	13	298
Prepaid rent	55	107
Prepaid inventory	6,252	2,009
Other	1,219	612
Total prepaid expenses and other current assets	<u>\$ 13,496</u>	<u>\$ 8,541</u>

13. Accrued Expenses and Other Current Liabilities

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

	December 31,	
	2025	2024
Product revenue allowances	\$ 9,810	\$ 849
Accrued clinical study and trial costs	18,977	23,869
Accrued selling, general, and administrative costs	6,316	4,258
Accrued compensation and related costs	24,352	14,477
Accrued professional fees	-	385
Accrued milestone costs	10,000	10,600
Accrued royalty payable	7,372	307
Accrued interest - debt	25,516	4,930
Other	721	421
Total accrued expense and other current liabilities	<u>\$ 103,064</u>	<u>\$ 60,096</u>

14. Common Stock

The Company is authorized to issue 200,000,000 shares of common stock. The holders of each share of common stock are entitled to one vote per share held and are entitled to receive dividends, if and when declared by

the Board, and to share ratably in the Company's assets available for distribution to stockholders, in the event of liquidation.

In May 2023, the Company entered into a sales agreement with 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 new sales agreement, shares will be sold pursuant to the previous shelf registration statement on Form S-3ASR (Registration No. 333-254661), which became automatically effective upon filing on March 24, 2021. 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. In the year ended December 31, 2023, the Company sold 2,719,744 common shares of common stock under the 2023 ATM Program, with net proceeds of approximately \$42.1 million.

In December 2021, the Company issued 3,802,144 shares of common stock and pre-funded warrants to purchase 1,142,856 shares of common stock. The offering price for the securities was \$17.50 per share or \$17.4999 for each Pre-Funded Warrant, with net proceeds of approximately \$81.2 million. On January 24, 2023, 86,000 pre-funded warrants were exercised for 85,998 shares of common stock, under a cashless exercise. On April 12, 2023, 273,000 pre-funded warrants were exercised for 272,996 shares of common stock, under a cashless exercise. On May 9, 2023, 498,142 pre-funded warrants were exercised for 498,137 shares of common stock, under a cashless exercise. As of December 31, 2025, 285,714 pre-funded warrants were considered issued and outstanding.

In December 2023, the Company issued 12,432,431 shares of common stock. The offering price for the securities was \$18.50 per share, with gross proceeds of approximately \$230.0 million, offset by \$14.0 million of issuance cost.

The Company has reserved for future issuance the following shares of common stock pursuant to the following outstanding securities or equity plans:

	December 31, 2025
Common stock issuable under pre-funded warrants	285,714
At-the-market program	7,280,256
RSUs and Options to purchase common stock	15,745,868
Equity plans	5,021,284
2015 Employee Stock Purchase Plan (the "ESPP")	2,002,859
Total	30,335,981

15. Stock-Based Compensation

In September 2015, the Company's board of directors adopted its 2015 Omnibus Incentive Plan, or 2015 Plan, which was subsequently approved by its stockholders and became effective upon the closing of the IPO on March 8, 2016. The 2015 Plan replaced the 2007 Stock Plan, or 2007 Plan and allows for the granting of incentive stock options, nonqualified stock options, stock appreciation rights, restricted stock, unrestricted stock, stock units, dividend equivalent rights, performance awards, annual incentive awards, and other equity-based awards to the Company's executives and other employees, non-employee members of the board of directors, and consultants of the Company. Any options or awards outstanding under the Company's 2007 Plan remain outstanding and continue to govern the terms of any equity grants that remain outstanding under the 2007 Plan. Any shares of common stock related to awards outstanding under the 2007 Plan that thereafter terminate by expiration, forfeiture, cancellation or otherwise without the issuance of such shares will be added to, and included in, the 2015 Plan reserve amount. The Company initially reserved 1,750,000 shares of its common stock for the issuance of awards under the 2015 Plan. The 2015 Plan provides that the number of shares reserved and available for issuance under the 2015 Plan will automatically increase each January 1, beginning on January 1, 2017, by 4% of the outstanding number of shares of common stock on the immediately preceding December 31 or such lesser number of shares as determined by the Company's board of directors. On January 1, 2026, the shares available for issuance under the 2015 Plan was increased to 3,496,239. The 2015 Plan will expire in March 2026.

In February 2023, the Company's board of directors adopted its 2023 Inducement Plan, or the 2023 Inducement Plan, which became effective March 1, 2023. The 2023 Inducement Plan allows for the granting of nonqualified stock options, restricted stock units, and other awards under the 2023 Inducement Plan to persons not previously an employee or director of the Company, or following a bona fide period of non-employment, an inducement material to such persons entering into employment with the Company. The Company initially reserved 1,900,000 shares of its common stock for the issuance of awards under the 2023 Inducement Plan. In December 2024 and 2023, the Company's board of directors approved the increase of the reserve by 1,200,000 and 1,100,000 shares respectively, effective the following year. There is no increase to the 2023 plan in January 2026.

As of December 31, 2025, there were 4,452,162 shares available for issuance under the 2015 Plan and 569,122 shares available for issuance under the 2023 Inducement Plan.

The Company recognized stock-based compensation expense related to the issuance of stock option awards to employees and non-employees and related to the Employee Stock Purchase Plan in the consolidated statements of operations as follows (in thousands):

	Years Ended December 31,		
	2025	2024	2023
Research and development	\$ 16,032	\$ 19,934	\$ 14,147
Selling, general and administrative	\$ 31,471	23,092	16,804
Total	<u>\$ 47,503</u>	<u>\$ 43,026</u>	<u>\$ 30,951</u>

Stock Options and Restricted Stock Units

As of December 31, 2025, there was \$78.7 million of unrecognized compensation cost related to employee and non-employee unvested stock options and RSUs granted under the 2015 Plan and the 2023 Inducement Plan, which is expected to be recognized over a weighted-average remaining service period of 2.4 years. For the years ended December 31, 2025, and 2024, the Company capitalized \$460 and \$53 of stock compensation expense to inventory. There was no stock based compensation capitalized for the year ended December 31, 2023.

The Company's stock-based awards are subject to either service or performance-based vesting conditions. Compensation expense related to awards to employees, directors and non-employees with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards to employees with performance-based vesting conditions is recognized based on the grant date fair value over the requisite service period using the straight-line method to the extent achievement of the performance condition is probable.

In 2022, the Company granted to certain employees 140,000 performance-based stock options, or 2022 Performance Awards, primarily related to the achievement of certain regulatory development milestones related to product candidates. Recognition of stock-based compensation expense associated with these performance-based stock options commences when the performance condition is considered probable of achievement, using management's best estimates, which consider the inherent risk and uncertainty regarding the future outcomes of the milestones.

In the first quarter of 2022, management estimated one of the milestones, for the 2022 Performance Awards, was probable of achievement. The second performance milestone was achieved in November 2024 and 40,000 options were vested. The Company recorded no stock compensation expense in the year ended December 31, 2025, and \$0.4 million, and \$0.3 million of stock compensation expense for these awards for the years ended December 31, 2024, and 2023, respectively. As of December 31, 2025, 40,000 stock options outstanding were unvested, and 60,000 options had been cancelled related to employee terminations.

In the second quarter of 2023, the Company granted to certain employees 129,550 performance-based RSUs, or 2023 Performance Awards, primarily related to the achievement of certain regulatory development milestones related to product candidates. The Company recorded approximately \$0.8 million and \$1.0 million of stock compensation related to the achievement of the performance milestones during the year ended December 31, 2025, and 2024, respectively. The Company recorded no stock compensation expense for these awards in the year ended December 31, 2023. As of December 31, 2025, these awards are fully vested.

In the first quarter of 2024, the Company granted to certain employees 35,500 performance-based RSUs, or 2024 Performance Awards, primarily related to the achievement of certain regulatory development milestones related to product candidates. The Company recorded approximately \$0.1 million and \$0.5 million of stock compensation related to the achievement of the performance milestones during the years ended December 31, 2025, and 2024, respectively. As of December 31, 2025, these awards are fully vested.

In the first quarter of 2024, the Company granted to certain executives 141,725 performance-based RSUs, or 2024 Executive Performance Awards, primarily related to the achievement of revenue targets for Revuforj. The Company recorded approximately \$1.5 million of stock compensation related to the achievement of the performance milestones during the year ended December 31, 2025. The Company recorded no stock compensation expense for these awards in the year ended December 31, 2024. The remaining awards will vest upon the first and second year anniversaries of the performance target being achieved.

In the first quarter of 2025, the Company granted to certain executives 252,800 performance-based RSUs, or 2024 Executive Performance Awards, related to the achievement of revenue targets and regulatory development milestones for Revuforj. The Company recorded approximately \$0.3 million of stock compensation related to the achievement of the performance milestones during the year ended December 31, 2025. The awards will vest upon compensation committee approval in February 2028. As of December 31, 2025, it is probable that two thirds of these awards will vest and are being expensed as such.

In connection with the termination of eight employees in 2024 and five employees in 2025, the Company entered into severance and consulting agreements. Under these agreements, the Company extended the vesting term for unvested options, which would not have otherwise vested, and extended the exercise period of the vested options post termination of the consulting agreement. For one employee, the Company accelerated the vesting of unvested options that otherwise would have vested in the following twelve month period and extended the exercise period of the vested options post termination of employment. The Company accounted for the change as a modification of an equity award under ASC 718. As a result of the modifications, the Company recognized approximately \$1.1 million and \$1.2 million of incremental stock compensation expense in the years ended December 31, 2025, and 2024, respectively.

The fair value of each option award is estimated on the date of grant using the Black-Scholes option-pricing model with the weighted-average assumptions noted in the table below. Expected volatility for the Company's common stock was determined based on an average of the historical volatility of the Company's public stock price. The Company estimated the expected term of its employee stock options using the "simplified" method, whereby, the expected term equals the average of the vesting term and the original contractual term of the option. The

contractual life of the option was used for the estimated life of the non-employee grants. The assumed dividend yield is based upon the Company's expectation of not paying dividends in the foreseeable future. The risk-free interest rate for periods within the expected life of the option is based upon the U.S. Treasury yield curve in effect at the time of grant. The Company accounts for forfeitures when they occur. The grant date fair values of options issued to employees and non-employees were estimated using the Black-Scholes option-pricing model with the following assumptions:

	Years Ended December 31,		
	2025	2024	2023
Expected term (in years)	6.01	6.05	6.03
Volatility rate	68.01%	71.64%	75.13%
Risk-free interest rate	4.08%	4.15%	3.65%
Expected dividend yield	0.00%	0.00%	0.00%

A summary of employee and non-employee option activity under the Company's equity award plans is presented below (in thousands, except share data):

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding—January 1, 2025	11,688,079	\$ 18.63	6.6	\$ 9,746
Granted	3,976,819	\$ 13.64		
Exercised	(1,135,977)	\$ 10.67		
Cancelled, forfeited or expired	(1,400,615)	\$ 19.62		
Outstanding—December 31, 2025	<u>13,128,306</u>	\$ 17.74	7.0	\$ 56,881
Exercisable—December 31, 2025	<u>7,525,866</u>		5.6	\$ 30,174

The weighted-average grant date fair value of options granted during the years ended December 31, 2025, 2024 and 2023, was \$8.74, \$14.37, and \$16.20 per share, respectively. The fair value is being expensed over the vesting period of the options (usually three to four years) on a straight-line basis as the services are being provided.

There were 1,135,977 options exercised for the year ended December 31, 2025, resulting in total proceeds of \$12.1 million; 717,413 options exercised for the year ended December 31, 2024, resulting in total proceeds of \$8.0 million; and 662,042 options exercised for the year ended December 31, 2023, resulting in total proceeds of \$5.1 million. The intrinsic value of options exercised during the years ended December 31, 2025, 2024 and 2023 was \$6.8 million, \$7.2 million, and \$9.5 million, respectively. In accordance with the Company's policy, the shares were issued from a pool of shares reserved for issuance under the 2015 Plan and 2023 Inducement Plan.

Restricted Stock Units

RSUs awarded to Board of Directors or employees vest on either i) one – year anniversary date of the related grant or ii) 25% on each anniversary for 4 years. The following table summarizes our RSU activity:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested—December 31, 2024	1,304,087	\$ 19.46
Granted ⁽¹⁾	2,001,335	\$ 13.37
Vested	(410,707)	\$ 21.31
Cancelled/Forfeited	(277,153)	\$ 15.13
Unvested—December 31, 2025	2,617,562	\$ 16.22

(1) RSUs granted in 2025 and 2024 had a weighted average grant date fair value of \$13.37 and \$18.80, respectively. The fair values of RSUs vested in 2025 and 2024 totaled \$8.8 million and \$1.8 million, respectively.

Employee Stock Purchase Plan

In September 2015, the Company's Board adopted the ESPP, which was subsequently approved by the Company's stockholders and became effective in 2016. The ESPP authorized the initial issuance of up to a total of 250,000 shares of common stock to the Company's employees. The number of shares of common stock available under the ESPP will automatically increase on January 1st of each year, continuing until the expiration of the ESPP, in an amount equal to the lesser of (a) 1% of the total number of shares of common stock outstanding on December 31st of the preceding calendar year, or (b) 250,000 shares. On January 1, 2026, the shares of common stock reserved for issuance under the ESPP was increased to 2,252,859. Under the terms of the ESPP, eligible employees can elect to acquire shares of the Company's common stock through periodic payroll deductions during a series of six-month offering periods. Purchases under the ESPP are affected on the last business day of each offering period at a 15% discount to the lower of closing price on that day or the closing price on the first day of the offering period. The Company issued 164,852 and 64,903 shares during 2025 and 2024, respectively. The ESPP plan will expire in March 2026.

The ESPP is considered a compensatory plan with the related compensation cost expensed over the six-month offering period. For the years ended December 31, 2025, 2024 and 2023 the Company recorded stock-based compensation expense related to the ESPP of \$0.8 million, \$0.6 million, and \$0.3 million respectively.

Employee Benefit Plan

The Company has a Section 401(k) defined contribution savings plan for its employees. The plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre- and after-tax basis, subject to legal limitations. Company contributions to the plan may be made at the discretion of the Board. For the years ended December 31, 2025, 2024 and 2023, the Company made \$3.2 million, \$2.7 million, and \$1.3 million contributions to the plan, respectively.

16. Purchase and Sale Agreement

On November 4, 2024, the Company entered into a Purchase and Sale Agreement, or the Purchase and Sale Agreement with Royalty Pharma Development Funding, LLC, or Royalty Pharma, pursuant to which Royalty Pharma purchased rights to certain revenue streams from net sales of products comprising or containing axatimab (including Niktimvo™) by the Company, its affiliates and its licensees in the United States and its respective territories, districts, commonwealths and possessions (including Guam and Puerto Rico) in exchange for an upfront fee of \$350.0 million.

Pursuant to the Purchase and Sale Agreement, Royalty Pharma purchased the right to receive a percentage of net sales equal to a royalty rate of 13.8% on quarterly net sales of Niktimvo in the United States and its respective territories; provided that the royalty rate is subject to certain adjustments based on future aggregate net sales of the Product in the Territory, or the Revenue Participation Right. Aggregate payments made to Royalty Pharma in respect of the Revenue Participation Right will be capped at \$822.5 million, or the Royalty Cap.

The Purchase and Sale Agreement contains customary representations, warranties and indemnities of the Company and Royalty Pharma and customary covenants relating to the royalty payments, including the grant of a back-up security interest in the purchased royalties and certain assets related to the Product and restrictions on the incurrence of additional indebtedness and on the existence of liens on the Company's assets related to the Product.

Upon a change of control, the Company will have the right, but not the obligation, to repurchase the Revenue Participation Right at a repurchase price set forth in the Purchase and Sale Agreement. In addition, the Purchase and Sale Agreement provides that if certain events of default occur, including certain bankruptcy events or certain termination events with respect to the Company's license agreement with UCB Biopharma Srl, Royalty Pharma may require the Company to repurchase Royalty Pharma's interests in the Revenue Participation Right at a repurchase price equal to the Royalty Cap.

The Company assessed the Purchase and Sale Agreement and identified it as a sale of future revenue in the form of a debt instrument to be accounted for as debt under ASC 470. The Company has elected to use the prospective method in its calculation of its effective interest rate and will update this calculation quarterly when there are changes in the projected sales. The debt is allocated on the balance sheet as short term and long term. The short term portion represents the royalty payments owed over the next 12 months. The long term portion is recorded on the balance sheet as net of issuance costs. Issuance costs pursuant to the Purchase and Sale Agreement consisted primarily of bank and legal fees and totaled \$6.3 million. These issuance costs were recorded as a direct deduction to the carrying amount of the liability and will be amortized under the effective interest method over the estimated period the liability will be repaid. As of December 31, 2025, the royalty interest financing balance is equal to its fair value, based on the adjusted quarterly interest rate calculation. The balance is a level 2 classified fair value. For the year ended December 31, 2025, the Company estimated an effective annual interest rate of approximately 13.02%. Over the course of the Purchase and Sale agreement, the annual interest rate will be affected by the amount and timing of net Niktimvo revenue recognized and change in timing of forecasted net Niktimvo revenue. On a quarterly basis, the Company reassesses the expected timing of the net Niktimvo revenue, recalculates the amortization and effective interest rate, and adjusts the accounting prospectively, as needed. The Company recognized interest expense of \$33.8 million and \$5.0 million in the years ended December 31, 2025, and 2024, respectively, related to the Purchase and Sale Agreement. The Company made \$13.2 million of payments on the Purchase and Sale agreement during the year ended December 31, 2025.

	December 31,	
	2025	2024
Current portion of royalty interest financing	\$ -	\$ 12,116
Royalty interest financing, less current portion	350,000	337,884
Debt issuance costs	(6,091)	(6,319)
Royalty interest financing, net	<u>\$ 343,909</u>	<u>\$ 343,681</u>

17. Income Taxes

For the years ending December 31, 2025, and December 31, 2024, the Company recognized current state income tax expense of \$0.4 million and \$0.3 million, respectively, recorded under selling, general and administrative expense. The Company's current year profit and historical losses before income tax for the periods presented was generated entirely in the United States.

Reconciliation of the differences between the effective income tax rate and federal statutory rate for the year ended December 31, 2025 (in thousands):

	Amount	Percent	
Income tax computed at federal statutory rate	\$ (59,830)	21.0	%
State taxes, net of federal benefit	342	(0.1)	
Tax Credits			
Research and development	2,529	(0.9)	
Orphan drug	(26,991)	9.5	
Nontaxable or Nondeductible items			
Stock option deduction	3,060	(1.1)	
Non-deductible officer compensation	3,234	(1.1)	
Other	202	(0.1)	
Other Adjustments			
Other	363	(0.1)	
Change in valuation allowance	77,524	(27.3)	
Effective tax rate	\$ 433	(0.2)	%

As previously disclosed for the years ended December 31, 2024, and 2023, prior to the adoption of ASU 2023-09, the following is a reconciliation of the difference between the effective income tax rate and the federal statutory rate:

	Years Ended December 31,	
	2024	2023
Income tax computed at federal statutory rate	21.0%	21.0%
State taxes, net of federal benefit	-0.1%	13.2%
General business credit carryovers	1.1%	1.5%
Non-deductible expenses	-1.0%	-1.0%
Change in valuation allowance	-17.3%	-34.9%
Return to provision true up	-3.8%	0.0%
Other	0.0%	0.0%
	<u>-0.1%</u>	<u>-0.2%</u>

The significant components of the Company's deferred tax are as follows (in thousands):

	Years Ended December 31,	
	2025	2024
Deferred tax assets (liabilities):		
Net operating loss carryforwards	\$ 135,517	\$ 43,362
Research and development credits	13,519	16,074
Orphan drug credits	26,991	—
Capitalized start-up and other costs	80,089	84,870
Capitalized research and development costs	59,475	97,780
Equity based compensation	15,348	13,687
Accruals	5,021	2,829
Disallowed interest	2,489	—
Other temporary differences	1,257	36
Deferred tax assets before valuation allowance	339,706	258,638
Valuation allowances	(339,706)	(258,638)
Net deferred tax assets	<u>\$ —</u>	<u>\$ —</u>

Supplemental disclosure of cash paid during the period for income taxes (net of refunds received):

	Year Ended December 31, 2025
Tennessee	\$ 0.07
New York	0.06
Massachusetts	0.01
Other	0.01
Total cash taxes paid, net of refunds received	\$ 0.15

The One Big Beautiful Bill Act, or OBBBA, was signed into law on July 4, 2025. The OBBBA includes significant changes affecting business taxpayers, with various effective dates. Among the provisions impacting the Company is Section 174A, which permits current expensing of domestic research and experimental, or R&E, expenditures. The Company elected to currently deduct domestic R&E costs incurred during the year and did not elect to accelerate deductions for domestic R&E costs capitalized in prior years. Foreign R&E expenditures continue to be amortized over 15 years under Section 174.

For the fiscal year ended December 31, 2025, the Company was subject to the Section 163(j) business interest expense limitation based on its average annual gross receipts (as determined under Section 448(c)). For tax years beginning after December 31, 2024, the OBBBA modifies the Section 163(j) Adjusted Taxable Income (“ATI”) calculation to allow an add-back for depreciation and amortization.

The Company has provided a valuation allowance for the full amount of the net deferred tax assets as the realization of the deferred tax assets is not determined to be more likely than not. The valuation allowance increased by \$81.1 million in 2025 due to the increase in deferred tax assets, primarily driven by the generation of net operating losses, and capitalized research expenses. The valuation allowance increased by \$52.7 million in 2024 due to the increase in deferred tax assets, primarily driven by the generation of net operating losses and capitalized research expenses, and capitalized start-up costs.

As of December 31, 2025, the Company had approximately \$585.3 million and \$227.7 million in federal and state net operating losses, or NOLs, respectively, which may be available to offset future taxable income. The Company has generated federal NOLs of \$563.2 million and state NOLs of \$8.7 million which have indefinite carryforward period. The remaining \$22.1 million of federal NOLs and \$219.0 million the Company’s state NOLs will begin to expire at various dates starting in 2026. Effective January 1, 2024, the Company has been reclassified as an active trade or business for tax purposes. This resulted in a reclassification of capitalized start-up and other costs to net operating losses and valuation allowance in the 2025 tax statements.

As of December 31, 2025, the Company had available research and development and orphan drug tax credits of approximately \$9.8 million and \$27.0 million, respectively. Income tax credits began to expire in 2024.

Realization of future tax benefits is dependent on many factors, including the Company’s ability to generate taxable income within the net operating loss carryforward period. Under the Internal Revenue Code provisions, certain substantial changes in the Company’s ownership, including the sale of the Company or significant changes in ownership due to sales of equity, may have limited, or may limit in the future, the amount of net operating loss carryforwards which could be used annually to offset future taxable income. The Company last completed an analysis from January 1, 2023 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. The Company may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. As a result, its ability to use its pre- change NOLs to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability. 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. As of December 31, 2025, and 2024, the Company had uncertain tax positions of \$0.1 million related to research and development credits, which reduce the deferred tax assets with a corresponding decrease to the valuation

allowance. The Company has elected to recognize interest and penalties related to income tax matters as a component of income tax expense, of which no interest or penalties were recorded for the years ended December 31, 2025 and 2024. The Company expects none of the unrecognized tax benefits to decrease within the next 12 months related to expired statutes or settlement with the taxing authorities. Due to the Company's valuation allowance as of December 31, 2025, none of the Company's unrecognized tax benefits, if recognized, would affect the effective tax rate.

A reconciliation of the Company's unrecognized tax benefits is as follows (in thousands):

	Years Ended December 31,		
	2025	2024	2023
Unrecognized tax benefit--beginning of year	\$ 143	\$ 143	\$ 143
Decreases related to prior period positions	—	—	—
Unrecognized tax benefit--end of year	<u>\$ 143</u>	<u>\$ 143</u>	<u>\$ 143</u>

The Company files tax returns in the U.S. and various states. All tax years since inception (October 11, 2005) remain open to examination by major tax jurisdictions to which the Company is subject, as carryforward attributes generated in years past may still be adjusted upon examination by the Internal Revenue Service or state tax authorities if they have or will be used in a future period. The Company is currently not under examination by the Internal Revenue Service or any other jurisdictions for any tax years.

18. Commitments and Contingencies

License Agreements

In April 2013, the Company entered into Eddingpharm License Agreement and a Series B-1 purchase agreement with Eddingpharm. Under the terms of the Eddingpharm License Agreement, Eddingpharm, in exchange for rights to develop and commercialize entinostat in China and certain other Asian countries, purchased \$5.0 million of Series B-1 and agreed to make certain contingent milestone and royalty payments based on revenue targets. In certain cases, the Company is required to assist Eddingpharm, and Eddingpharm is required to reimburse the Company for any out-of-pocket expenses incurred in providing this assistance, including reimbursement for person-hours above a certain cap.

The Company is obligated to pay royalties pursuant to the Vitae License Agreement, as a percentage of net product sales for direct licensed products, such as Revuforj. The obligation to pay royalties expires, on a country-by-country basis and licensed product-by-licensed product basis until the later of expiration of the last valid claim of a patent right, the expiration of the applicable regulatory exclusivity for such licensed product in such county, or 10 years from the first commercial sale of a licensed product in each country. These fees were recorded as cost of product sales.

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 December 31, 2025, or 2024.

19. Segment Reporting

The Company manages its business activities on a consolidated basis and operate as a single operating and reportable segment: Syndax Pharmaceuticals. The Company derives revenue in the United States through milestone revenue and product sales on the recently approved products, Revuforj and Niktimvo. The accounting policies of the segment are the same as those described in Note 3 – Summary of Significant Accounting Policies.

To assess performance, the Company's Chief Operating Decision Maker, or CODM, the Chief Executive Officer, or CEO, Michael Metzger, uses consolidated net loss as the segment's measure of segment profit or loss.

The CODM uses net loss in the budget and forecasting process and considers budget-to actual variances on a quarterly bases when making decisions about the allocation of operating and capital resources.

The following table provides the operating financial results of our biopharmaceutical cancer therapeutics segment:

	Year Ended December 31,		
	2025	2024	2023
Total Revenue	\$ 172,352	\$ 23,680	\$ —
Less: Significant and other segment expenses			
Cost of product sales	6,970	826	—
Research and development expenses			
Revumenib-related costs	112,422	107,909	64,122
Axatilimab-related costs	48,289	49,638	32,114
Other R&D programs	4,154	2,564	5,441
Personnel cost and other expenses	77,887	61,602	47,208
General and administrative expenses			
Commercial related expenses	53,630	33,541	13,115
Personnel cost and other expenses	71,271	46,893	22,898
Other SG&A expenses	23,310	17,353	14,105
Stock-based compensation	47,503	43,026	30,951
Royalty interest expense	33,779	4,930	—
Interest (income) expense, net	(22,724)	(26,091)	(20,955)
Other expense, net	1,283	247	361
Segment net loss	<u>\$ (285,422)</u>	<u>\$ (318,758)</u>	<u>\$ (209,360)</u>



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