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

FORM 10-Q

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

For the quarterly period ended March 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: 000-23186

BIOCRYST PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**62-1413174**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
4505 Emperor Blvd., Suite 200 **Durham, North Carolina** (Address of principal executive offices)	**27703** (Zip Code)

+1-919-859-1302
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.01	BCRX	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

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

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

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

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

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

As of April 30, 2025, the registrant had 209,250,274 shares of common stock outstanding.

BIOCRYST PHARMACEUTICALS, INC.

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When used in this report, unless otherwise indicated, "we," "our," "us," the "Company," and "BioCryst" refer to BioCryst Pharmaceuticals, Inc. and, where appropriate, its subsidiaries.

Cautionary Note Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q (this "report") includes forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), which are subject to the "safe harbor" created in Section 21E. In particular, statements about our expectations, beliefs, plans, objectives or assumptions of future events or performance are contained or incorporated by reference in this report. All statements other than statements of historical facts contained herein are forward-looking statements. These forward-looking statements can generally be identified by the use of words such as "may," "will," "intends," "plans," "believes," "anticipates," "expects," "estimates," "predicts," "potential," the negative of these words or similar expressions. Statements that describe our future plans, strategies, intentions, expectations, objectives, goals or prospects are also forward-looking statements. Discussions containing these forward-looking statements are principally contained in the "*Risk Factors*" and "*Management's Discussion and Analysis of Financial Condition and Results of Operations*" sections of this report, as well as any amendments we make to those sections in filings with the U.S. Securities and Exchange Commission ("SEC"). These forward-looking statements include, but are not limited to, statements about:

- the preclinical development, clinical development, commercialization, or post-marketing studies of our products and product candidates, including ORLADEYO® (berotralstat), BCX17725, avoralstat, and early-stage discovery programs (including our complement inhibitors), and our plans and anticipated timing regarding the same;
- our discovery and commercialization of best-in-class and first-in-class medicines;
- the timing and success of our commercialization of ORLADEYO in the United States and elsewhere and expectations regarding the commercial market for ORLADEYO;
- the potential for government stockpiling orders of our products and product candidates, including the timing or likelihood of entering into any U.S. Government stockpile order, the likelihood of the U.S. Government exercising any options under our current procurement contract, and our ability to execute any such order;
- additional regulatory approvals, or milestones, royalties or profit from sales of our products by us or our partners;
- the implementation of our business model, strategic plans for our business, products, product candidates and technology;
- our ability to establish and maintain collaborations or out-license rights to our products and product candidates;
- plans, programs, progress and potential success of our collaborations, including with Torii Pharmaceutical Co., Ltd. ("Torii") for ORLADEYO in Japan and Shionogi & Co., Ltd. ("Shionogi") and Green Cross Corporation ("Green Cross") for peramivir in their territories;
- our and our subsidiary guarantors' ability to satisfy obligations under the Pharmakon Loan Agreement (as defined below) and to comply with the covenants as set forth in the agreements governing our debt obligations;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our products, product candidates, and technology;
- our ability to operate our business without infringing the intellectual property rights of others;
- estimates of our revenues, expenses, capital requirements, annual cash utilization, and our needs for additional capital or financing;
- the timing or likelihood of regulatory filings (including with respect to the ORLADEYO pediatric program) or regulatory agreements, deferrals, approvals, and other decisions;
- our ability to manage our liquidity needs to fund our operations or repay our recourse debt obligations;
- our financial performance;

- statements and projections regarding financial goals, including timing for achieving profitability or positive cash flow; and
- competitive companies, technologies, and our industry.

We have based any forward-looking statements on our current expectations about future events or performance. While we believe these expectations are reasonable, forward-looking statements are inherently subject to known and unknown risks and uncertainties, many of which are beyond our control. Actual results may differ materially from those suggested or implied by these forward-looking statements for various reasons, including those discussed in this report under the heading "*Risk Factors*" in Part II, Item 1A, some of which are summarized in the "*Risk Factor Summary*" below. Any forward-looking statement is subject to these and other risks, uncertainties, and assumptions relating to our operations, results of operations, industry, and future growth. Given these risks and uncertainties, you are cautioned not to place undue reliance on our forward-looking statements. The forward-looking statements included in this report are made only as of the date hereof. We do not undertake, and specifically decline, any obligation to update, revise or correct any of these statements or to publicly announce the results of any such revisions to any forward-looking statements to reflect future events or developments, except as may be required by U.S. federal securities laws.

Risk Factor Summary

An investment in the Company involves risks. You should carefully read this entire report and consider the uncertainties and risks discussed in the "*Risk Factors*" section in Part II, Item 1A of this report, which may adversely affect our business, financial condition, or results of operations, along with the other information included in our other filings with the SEC, before making an investment decision in the Company. A summary of the principal factors that make an investment in the Company speculative or risky is set forth below.

- We may never achieve sustained profitability, and we may need to raise additional capital in the future. If we are unable to raise capital if and when needed, we may need to adjust our operations.
- Our success depends upon our ability to manage our product candidate pipeline, advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive and maintain regulatory approvals for the commercial sale of our product candidates. The development process and related regulatory processes are complex and uncertain, may be lengthy and expensive, and require, among other things, an indication that our products and product candidates are safe and effective. For example, applicable regulatory agencies could refuse to approve, or impose restrictions or warnings on, our product candidates, require us to conduct additional studies or adopt study designs that differ from our planned development strategies, suspend or terminate our clinical trials, withdraw approval for our products, or take other actions that could materially impact the cost, timing, and success of our planned development and commercialization strategies.
- We rely heavily upon third parties, including development partners, contractors, contract research organizations, and third-party suppliers, manufacturers, and distributors, for many important stages of our product candidate development and in the commercialization of certain of our products and product candidates. Our failure to establish and maintain these relationships, the failure of any such third party to perform its obligations under agreements with us, or the failure of such a relationship to meet our expectations could have a material adverse impact on our business, financial condition, and results of operations.
- If we fail to obtain additional financing or acceptable partnership arrangements if and when needed, we may be unable to complete the development and commercialization of our products and product candidates or continue operations.
- If the U.S. Food and Drug Administration or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, the sales of our products could be adversely affected.
- The commercial viability of any approved product could be compromised if the product is less effective than expected, causes undesirable side effects that either were not previously identified or were worse than expected, or fails to achieve market acceptance by physicians, patients, third-party payors, health authorities, and others.
- There can be no assurance that our or our partners' commercialization efforts, methods, and strategies for our products or technologies will succeed, and our future revenue generation is uncertain.

- We have expanded, and may continue expanding, our development and regulatory capabilities and are implementing sales, marketing, and distribution capabilities, and as a result, we may encounter difficulties managing our growth, which could disrupt our operations.
- We face intense competition, and if we are unable to compete effectively, the demand for our products may be reduced. In addition, developments by others may render our products, product candidates, or technologies obsolete or noncompetitive.
- We are subject to various laws and regulations related to our products and product candidates, and if we or our employees, consultants, or partners do not comply with these laws and regulations, we could face substantial penalties, and our reputation could be harmed. In addition, we and our partners may be subject to new legislation, regulatory proposals, and healthcare payor initiatives that may increase our costs of compliance and adversely affect our or our partners' ability to market our products or develop our product candidates.
- If we fail to adequately protect or enforce our intellectual property rights, the value of those rights would diminish. Legal proceedings to protect or enforce our patents, the patents of our partners, or our other intellectual property rights could be expensive, time-consuming, and unsuccessful. If we fail to secure the rights to patents of others, this could adversely affect our business.
- We face an inherent risk of liability in the event that the use or misuse of our products or product candidates results in personal injury or death, and our product liability insurance coverage may be insufficient.
- If we fail to reach milestones or to make annual minimum payments or otherwise breach our obligations under our license agreements, our licensors may terminate our agreements with them and/or seek additional remedies.
- The Pharmakon Loan Agreement (as defined below) contains conditions and restrictions that limit our flexibility in operating our business. We may be required to make a prepayment or repay our outstanding indebtedness under the Pharmakon Loan Agreement earlier than we expect if a prepayment event or an event of default occurs, including, but not limited to, a material adverse change with respect to us, which could have a material adverse effect on our business.
- International expansion of our business exposes us to business, legal, regulatory, political, operational, financial, and economic risks. For example, our actual or perceived failure to comply with European governmental laws and regulations and other obligations related to privacy, data protection, and information security could harm our business. In addition, the United Kingdom's withdrawal from the European Union could result in increased regulatory and legal complexity, which may make it more difficult for us to do business in Europe and impose additional challenges in securing regulatory approval of our product candidates in Europe.
- If our facilities, or the facilities of our third-party vendors, incur damage or power is lost for a significant length of time, our operations will be disrupted, which will adversely affect our business.
- Cyber incidents and related disruptions in our or our third-party vendors' information technology systems, as well as challenges with properly managing artificial intelligence, could adversely affect our business.
- Our business, operations, clinical development or commercialization plans and timelines, and access to capital could be adversely affected by unpredictable and unstable market and economic conditions.
- If we fail to retain our existing key personnel, or fail to attract and retain additional key personnel, the development of our product candidates, the commercialization of our products, and the related expansion of our business will be delayed or stopped.
- Future acquisitions, strategic investments, partnerships, alliances, or divestitures could fail to meet our expectations and/or adversely affect our operating results and financial condition.
- Our existing principal stockholders hold a substantial amount of our common stock and may be able to influence significant corporate decisions, which may conflict with the interests of other stockholders.
- Our stock price has been, and is likely to continue to be, highly volatile, which could cause the value of an investment in our common stock to decline significantly.

- If we fail to maintain effective internal control over financial reporting, we may not be able to produce accurate and timely financial statements, which may adversely affect investor confidence in us and our financial reporting, adversely affect our business and operating results and negatively impact the trading price of our common stock.
- Natural disasters, epidemic or pandemic disease outbreaks, trade wars, armed conflicts, political unrest, or other events could disrupt our business or operations, or those of our development partners, manufacturers, regulators, or third parties with whom we conduct business now or in the future.
- We are subject to legal proceedings, which could harm our reputation or result in other losses or unexpected expenditure of time and resources.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

BIOCRYST PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands, except par value amounts, Unaudited)

	March 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 105,190	$ 104,713
Restricted cash	291	210
Short-term investments	189,902	216,137
Trade receivables	93,394	79,069
Inventory, net	6,873	8,087
Prepaid expenses and other current assets	14,327	13,752
Total current assets	409,977	421,968
Long-term inventory, net	25,097	23,187
Property and equipment, net	7,593	7,777
Long-term investments	20,548	20,323
Right of use assets	11,982	12,008
Other assets	4,850	5,157
Total assets	$ 480,047	$ 490,420
Liabilities and Stockholders' Deficit		
Current liabilities:		
Accounts payable	$ 10,049	$ 11,644
Accrued expenses	92,635	113,292
Operating lease liabilities	1,071	937
Finance lease liabilities	1,795	1,835
Royalty financing obligations	34,305	32,676
Total current liabilities	139,855	160,384
Operating lease liabilities	7,779	7,924
Finance lease liabilities	2,314	2,124
Royalty financing obligations	466,613	481,053
Secured term loan	315,413	314,869
Total liabilities	931,974	966,354
Stockholders' deficit:		
Preferred stock, $0.01 par value; shares authorized - 5,000; no shares issued and outstanding at March 31, 2025 and December 31, 2024	—	—
Common stock, $0.01 par value; shares authorized - 450,000; shares issued and outstanding – 209,208 at March 31, 2025 and 208,543 at December 31, 2024	2,092	2,085
Additional paid-in capital	1,314,857	1,291,100
Accumulated other comprehensive income	1,132	921
Accumulated deficit	(1,770,008)	(1,770,040)
Total stockholders' deficit	(451,927)	(475,934)
Total liabilities and stockholders' deficit	$ 480,047	$ 490,420

See accompanying notes to condensed consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)
(In thousands, except per share amounts, Unaudited)

	Three Months Ended March 31,	
	2025	**2024**
Revenues	$ 145,534	$ 92,761
Expenses:		
Cost of product sales	4,568	1,265
Research and development	37,270	46,493
Selling, general and administrative	82,469	59,491
Total operating expenses	124,307	107,249
Income (loss) from operations	21,227	(14,488)
Other income (expense):		
Interest income	3,024	4,031
Interest expense	(23,494)	(24,506)
Foreign currency gains (losses), net	1	(51)
Total other expense	(20,469)	(20,526)
Income (loss) before income taxes	758	(35,014)
Income tax expense	726	365
Net income (loss)	$ 32	$ (35,379)
Other comprehensive income (loss):		
Foreign currency translation adjustment	366	(253)
Unrealized loss on available for sale investments	(155)	(313)
Total other comprehensive income (loss)	211	(566)
Net comprehensive income (loss)	$ 243	$ (35,945)
Net income (loss) per common share: basic	**$ 0.00**	**$ (0.17)**
Weighted average shares of common stock outstanding: basic	**208,882**	**206,064**
Net income (loss) per common share: diluted	**$ 0.00**	**$ (0.17)**
Weighted average shares of common stock outstanding: diluted	**215,261**	**206,064**

See accompanying notes to condensed consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands, Unaudited)

	Three Months Ended March 31,	
	2025	**2024**
Cash flows from operating activities:		
Net income (loss)	$ 32	$ (35,379)
Adjustments to reconcile net income (loss) to net cash used in operating activities:		
Depreciation and amortization	331	306
Inventory obsolescence	209	—
Stock-based compensation expense	21,368	13,652
Non-cash interest expense on royalty financing obligations and secured term loan and amortization of debt issuance costs	14,058	19,440
Amortization of discount on investments, net	(1,383)	(3,181)
Changes in operating assets and liabilities:		
Receivables	(13,987)	(3,839)
Inventory	(870)	(1,220)
Prepaid expenses and other assets	(132)	1,106
Accounts payable and accrued expenses	(47,143)	(44,569)
Net cash used in operating activities	(27,517)	(53,684)
Cash flows from investing activities:		
Acquisitions of property and equipment	(143)	(235)
Purchases of investments	(48,762)	(88,009)
Maturities of investments	76,000	117,000
Net cash provided by investing activities	27,095	28,756
Cash flows from financing activities:		
Net proceeds from common stock issued under stock-based compensation plans	2,390	1,681
Common stock issued to directors in lieu of cash retainer	6	11
Withholding taxes paid on stock-based awards	(1,351)	(2,350)
Principal payments on finance lease liabilities	(516)	(390)
Net cash provided by (used in) financing activities	529	(1,048)
Effect of exchange rates on cash, cash equivalents and restricted cash	451	(340)
Increase (decrease) in cash, cash equivalents and restricted cash	558	(26,316)
Cash, cash equivalents and restricted cash:		
Beginning of period	106,323	112,447
End of period	$ 106,881	$ 86,131
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 105,190	$ 84,333
Restricted cash	291	1,798
Restricted cash in other assets	1,400	—
Total cash, cash equivalents and restricted cash	$ 106,881	$ 86,131
Supplemental cash flow disclosure:		
Cash paid for interest	$ 9,153	$ 4,987
Cash paid for taxes	$ 1	$ 186
Taxes withheld on stock-based awards included in accrued expenses	$ 276	$ 1,846

See accompanying notes to condensed consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' DEFICIT
(In thousands, Unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount				
Balance at December 31, 2024	**208,543**	**$ 2,085**	**$ 1,291,100**	**$ 921**	**$ (1,770,040)**	**$ (475,934)**
Net income	—	—	—	—	32	32
Other comprehensive income	—	—	—	211	—	211
Exercise of stock options	286	3	1,226	—	—	1,229
Vesting of restricted stock units	184	2	(2)	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(8)	—	(56)	—	—	(56)
Employee stock purchase plan sales	202	2	1,215	—	—	1,217
Issuance of shares to directors in lieu of cash retainer	1	—	6	—	—	6
Stock-based compensation expense	—	—	21,368	—	—	21,368
Balance at March 31, 2025	**209,208**	**$ 2,092**	**$ 1,314,857**	**$ 1,132**	**$ (1,770,008)**	**$ (451,927)**

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount				
Balance at December 31, 2023	**205,771**	**$ 2,058**	**$ 1,222,236**	**$ 1,337**	**$ (1,681,159)**	**$ (455,528)**
Net loss	—	—	—	—	(35,379)	(35,379)
Other comprehensive loss	—	—	—	(566)	—	(566)
Exercise of stock options	176	2	550	—	—	552
Vesting of restricted stock units	155	1	(1)	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(8)	—	(38)	—	—	(38)
Employee stock purchase plan sales	251	2	1,127	—	—	1,129
Issuance of shares to directors in lieu of cash retainer	2	—	11	—	—	11
Stock-based compensation expense	—	—	13,652	—	—	13,652
Balance at March 31, 2024	**206,347**	**$ 2,063**	**$ 1,237,537**	**$ 771**	**$ (1,716,538)**	**$ (476,167)**

See accompanying notes to condensed consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share amounts)
(Unaudited)

Note 1 — Significant Accounting Policies and Concentrations of Risk

The Company

BioCryst Pharmaceuticals, Inc. (the "Company") is a global biotechnology company with a deep commitment to improving the lives of people living with hereditary angioedema ("HAE") and other rare diseases. The Company leverages its expertise in structure-guided drug design with the goal of developing first-in-class or best-in-class oral small-molecule and injectable protein therapeutics to target difficult-to-treat rare diseases. The Company was founded in 1986 and incorporated in Delaware in 1991, and its headquarters is located in Durham, North Carolina. The Company integrates the disciplines of biology, crystallography, medicinal chemistry and computer modeling to discover and develop small molecule and protein therapeutics through the process known as structure-guided drug design.

The Company's marketed products include oral, once-daily ORLADEYO® for the prevention of HAE attacks and RAPIVAB® (peramivir injection) for the treatment of acute uncomplicated influenza in the United States. ORLADEYO has received regulatory approval in the United States and other global markets. The Company is commercializing ORLADEYO in each of these territories directly or through other parties. In addition to its approval in the United States, peramivir injection has received regulatory approvals in Canada (RAPIVAB), Australia (RAPIVAB), Japan (RAPIACTA), Taiwan (RAPIACTA) and Korea (PERAMIFLU).

Based on the Company's expectations for revenue and operating expenses, the Company believes its financial resources available at March 31, 2025 will be sufficient to fund its operations for at least the next 12 months. The Company may, in the future, issue securities, including common stock, preferred stock, depositary shares, purchase contracts, warrants, debt securities and units, through private placement transactions or registered public offerings. The Company's future liquidity needs, and ability to address those needs, will largely be determined by the success of its products and product candidates; the timing, scope and magnitude of its research and development and commercial expenses; and key developments and regulatory events and its decisions in the future.

Basis of Presentation

The condensed consolidated financial statements include the accounts of the Company and its subsidiaries. All intercompany transactions and balances among the consolidated entities have been eliminated from the condensed consolidated financial statements. The Company operates and manages its business as one reportable and operating segment (see "*Note 12—Segment Information*").

The Company's condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("U.S. GAAP") for interim financial reporting and the instructions to Form 10-Q and do not include all of the information and footnotes required by U.S. GAAP for complete financial statements. Such financial statements reflect all adjustments that are, in management's opinion, necessary to present fairly, in all material respects, the Company's condensed consolidated financial position, results of operations, and cash flows. There were no adjustments other than normal recurring adjustments. Certain prior year amounts have been reclassified to conform to the current year presentation.

These financial statements should be read in conjunction with the financial statements for the fiscal year ended December 31, 2024 and the notes thereto included in the Company's 2024 Annual Report on Form 10-K as filed with the SEC on February 25, 2025. Interim operating results are not necessarily indicative of operating results for the full fiscal year. The condensed consolidated balance sheet as of December 31, 2024 was derived from the audited consolidated financial statements included in the Company's most recent Annual Report on Form 10-K.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses, and related disclosure of contingent assets and liabilities. Significant estimates in the Company's condensed consolidated financial statements have

been made relative to the calculation of net product sales, royalty financing obligations, inventory reserves, certain accruals, primarily related to the Company's research and development expenses, the valuation of stock options and the valuation allowance for deferred tax assets resulting from net operating losses. These estimates are based on historical experience and on various other assumptions 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 could differ from those estimates.

Revenue Recognition

The Company recorded the following revenues for the three months ended March 31, 2025 and 2024 (in thousands):

	Three Months Ended March 31,	
	2025	**2024**
Product sales, net	$ 143,787	$ 89,272
Collaborative and other revenues	1,747	3,489
Total revenues	$ 145,534	$ 92,761

Pursuant to Accounting Standards Codification ("ASC") Topic 606, the Company recognizes revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To achieve this core principle, Topic 606 includes provisions within a five-step model that includes (i) identifying the contract with a customer, (ii) identifying the performance obligations in the contract, (iii) determining the transaction price, (iv) allocating the transaction price to the performance obligations, and (v) recognizing revenue when, or as, an entity satisfies a performance obligation.

At contract inception, the Company identifies the goods or services promised within each contract, assesses whether each promised good or service is distinct and determines those that are performance obligations. The Company recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when the performance obligation is satisfied.

Product Sales, Net

The Company's principal sources of product sales are sales of ORLADEYO, which the Company began shipping to patients in December 2020, and sales of peramivir (RAPIVAB/RAPIACTA/PERAMIFLU) to the Company's licensing partners and to the U.S. Department of Health and Human Services ("HHS"). In the United States, the Company generally ships ORLADEYO directly to patients through a single specialty pharmacy, which is considered its customer. Outside the United States, the Company sells ORLADEYO to specialty distributors and to hospitals and pharmacies, which collectively are considered its customers.

The Company recognizes revenue for sales when the customer obtains control of the product, which generally occurs upon delivery.

Net revenue from sales of ORLADEYO is recorded at net selling price (transaction price), which includes reserves for variable consideration such as (i) estimated government rebates, such as Medicaid and Medicare Part D reimbursements, and estimated managed care rebates, (ii) estimated chargebacks, (iii) estimated costs of co-payment assistance programs and (iv) product returns. These reserves, representing the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the applicable contracts and statutory requirements, are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable if no payments are required of the Company or a current liability if a payment is required of the Company. Actual amounts of consideration may differ from the Company's estimates. If actual results vary from estimates, these estimates are adjusted, which would affect net product revenue and earnings in the period such variances become known.

Government and Managed Care Rebates. The Company contracts with government agencies and managed care organizations or, collectively, third-party payors, so that ORLADEYO will be eligible for purchase by, or partial or full reimbursement from, such third-party payors. The Company estimates the rebates it will provide to third-party payors and deducts these estimated amounts from total gross product revenues at the time the revenues are recognized, resulting in a reduction of product revenue and the establishment of a current liability. The Company estimates the rebates that it will provide to third-party payors based upon (i) the Company's contracts with these third-party payors, (ii) the government

mandated discounts applicable to government-funded programs, and (iii) product distribution information obtained from the Company's specialty pharmacy regarding payor mix.

Chargebacks. Chargebacks are discounts that occur when certain contracted customers, pharmacy benefit managers, insurance companies, and government programs purchase directly from the Company's specialty pharmacy. These customers purchase the Company's product under contracts negotiated between them and the Company's specialty pharmacy. The specialty pharmacy, in turn, charges back to the Company the difference between the price that the specialty pharmacy paid and the negotiated price paid by the contracted customers, which may be higher or lower than the specialty pharmacy's purchase price with the Company. The Company estimates chargebacks and adjusts gross product revenues and establishes a current liability at the time revenues are recognized.

Co-payment assistance and patient assistance programs. Patients who have commercial insurance and meet certain eligibility requirements may receive co-payment assistance. Based upon the terms of the program and co-payment assistance utilization reports received from the specialty pharmacy, the Company estimates the co-payment assistance amounts, which are recorded in the same period in which the related revenue is recognized, resulting in a reduction of product revenue and establishment of a current liability. The Company also offers a patient assistance program that provides free drug product, for a limited period of time, to allow a patient's insurance coverage to be established. Based on patient assistance program utilization reports provided by the specialty pharmacy, the Company records gross revenue of the product provided and a full reduction of the revenue amount for the free drug discount.

Product returns. The Company does not provide contractual return rights to its customers, except in instances where the product is damaged or defective. Non-acceptance by the patient of shipped drug product by the specialty pharmacy is reflected as a reversal of sales in the period in which the sales were originally recorded. Reserves for estimated non-acceptances by patients are recorded as a reduction of revenue in the period that the related revenue is recognized, as well as a reduction to accounts receivable. Estimates of non-acceptance are based on quantitative information provided by the specialty pharmacy.

Collaborative and Other Revenues

The Company has collaboration and license agreements with a number of third parties. The Company's primary sources of revenue from these collaborative and other research and development arrangements are license, service and royalty revenues.

Revenue from license fees, royalty payments, milestone payments, and research and development fees are recognized as revenue when the earnings process is complete and the Company has no further continuing performance obligations or the Company has completed the performance obligations under the terms of the agreement.

Arrangements that involve the delivery of more than one performance obligation are initially evaluated as to whether the intellectual property licenses granted by the Company represent distinct performance obligations. If they are determined to be distinct, the value of the intellectual property licenses would be recognized up front while the research and development service fees would be recognized as the performance obligations are satisfied. For performance obligations based on services performed, the Company measures progress using an input method based on the effort it expends or costs it incurs toward the satisfaction of the performance obligation in relation to the total estimated effort or costs. Variable consideration is assessed at each reporting period as to whether it is not subject to significant future reversal and, therefore, should be included in the transaction price at the inception of the contract. If a contract includes a fixed or minimum amount of research and development support, this also would be included in the transaction price. Changes to collaborations, such as the extensions of the research term or increasing the number of targets or technology covered under an existing agreement, are assessed for whether they represent a modification or should be accounted for as a new contract. For contracts with multiple performance obligations, revenue is allocated to each performance obligation based on its relative standalone selling price. Standalone selling prices are based on observable prices at which the Company separately sells the products or services. If a standalone selling price is not directly observable, then the Company estimates the standalone selling price using either an adjusted market assessment approach or an expected cost plus margin approach, representing the amount that the Company believes the market is willing to pay for the product or service. Analyzing the arrangement to identify performance obligations requires the use of judgment, and each may be an obligation to deliver services, a right or license to use an asset, or another performance obligation.

Under certain of the Company's license agreements, the Company receives royalty payments based upon its licensees' net sales of covered products. Royalties are recognized at the later of when (i) the subsequent sale or usage occurs, or (ii) the performance obligation to which some or all of the sales-based or usage-based royalty has been satisfied.

Cash and Cash Equivalents

The Company generally considers cash equivalents to be all cash held in commercial checking accounts, money market accounts, or investments in debt instruments and certificates of deposit with maturities of three months or less at the time of purchase. The carrying value of cash and cash equivalents approximates fair value due to the short-term nature of these items.

Restricted Cash

Total restricted cash was $1,691 and $1,610 as of March 31, 2025 and December 31, 2024, respectively, and primarily consisted of $1,400 as of March 31, 2025 and December 31, 2024, for a letter of credit the Company is required to maintain associated with its Birmingham lease. The letter of credit associated with the Birmingham lease of $1,400 is reflected within other assets on the Condensed Consolidated Balance Sheets as of March 31, 2025.

Investments

The Company invests in high credit quality investments in accordance with its investment policy. The objectives of the Company's investment policy are to eliminate or greatly minimize the probability of a loss of principal value, maintain sufficient liquidity to meet cash flow requirements, and earn a competitive level of return. The Company places its excess cash with high credit quality financial institutions to limit the amount of its credit exposure. In accordance with its policy, the Company is able to invest in marketable debt securities that may consist of U.S. Treasury obligations, U.S government agency securities, money market funds, certificates of deposits and corporate notes and bonds. The Company's investment policy requires it to purchase high-quality marketable securities with a maximum individual maturity of two years and requires an average portfolio maturity of no more than 12 months. Some of the securities in which the Company invests may have market risk. This means that a change in prevailing interest rates may cause the principal amount of the investment to fluctuate. To minimize this risk, the Company schedules its investments with maturities that coincide with expected cash flow needs, thus avoiding the need to redeem an investment prior to its maturity date. Accordingly, the Company does not believe it has a material exposure to interest rate risk arising from its investments. The Company has not realized any significant losses from its investments.

The Company classifies all of its investments as available-for-sale. Available-for-sale investments are reported at fair value at each balance sheet date, and include any unrealized holding gains and losses in accumulated other comprehensive income, unless an unrealized loss is considered to be other than temporary, in which case the unrealized loss is charged to operations. The Company reviews its investments for other than temporary declines in fair value below cost basis at the end of each reporting period and whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Factors considered to determine whether an unrealized loss is temporary include whether a decline in fair value below the amortized cost basis is due to credit-related factors or non-credit-related factors, the financial condition and near-term prospects of the Company, and the Company's intent and ability to hold the investment to allow for an anticipated recovery in fair value. A credit-related impairment is recognized as an allowance on the balance sheet with a corresponding adjustment to earnings. Any impairment that is not credit-related is recognized in other comprehensive income, net of applicable taxes unless deemed other than temporary. Realized gains and losses are reflected in interest and other income in the Condensed Consolidated Statements of Comprehensive Income (Loss) and are determined using the specific identification method with transactions recorded on a settlement date basis. Investments with original maturities at date of purchase beyond three months and which mature at or less than 12 months from the balance sheet date are classified as current. Investments with a maturity beyond 12 months from the balance sheet date are classified as long-term.

Fair Value Measurements

Assets and liabilities recorded at fair value on a recurring basis on the Condensed Consolidated Balance Sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. 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 must maximize the use of observable inputs and minimize the use of unobservable inputs.

Assets measured at fair value on a recurring basis include investments (See "*Note 3—Investments*"). There were no liabilities measured at fair value on a recurring basis as of March 31, 2025 and December 31, 2024. The carrying amounts reflected in the Condensed Consolidated Balance Sheets for cash and cash equivalents, trade receivables, prepaid expenses

and other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Trade Receivables

The majority of the Company's trade receivables arise from product sales and primarily represent amounts due from its specialty pharmacy customer in the United States and other third-party distributors, hospitals and pharmacies in the European Union, United Kingdom and elsewhere and have standard payment terms that generally require payment within 30 to 90 days.

Receivables from collaborations are recorded for amounts due to the Company related to royalty receivables from the Company's partners, including Shionogi & Co., Ltd., Green Cross, and Torii (See "*Note 11—Collaborative and Other Relationships*").

The Company provides reserves against trade receivables for estimated losses that may result from a customer's inability to pay. Receivables are evaluated to determine if any reserve or allowance should be recorded based on consideration of the current economic environment, expectations of future economic conditions, specific circumstances and the Company's own historical collection experience. Amounts determined to be uncollectible are charged or written-off against the reserve.

Inventory

The Company's inventory primarily relates to ORLADEYO. Additionally, the Company's inventory includes peramivir.

The Company values its inventory at the lower of cost or estimated net realizable value. The Company determines the cost of its inventory on a first-in, first-out (FIFO) basis. Raw materials and work-in-process include all inventory costs prior to packaging and labeling, including raw material, active product ingredient, and the drug product. Finished goods include packaged and labeled products. The Company classifies inventory as long-term when consumption or sale of the inventory is not expected to occur within 12 months from the balance sheet date.

The Company's inventory is subject to expiration dating. At each reporting date, the Company evaluates the carrying value of its inventory and provides valuation reserves for any estimated excess, obsolete, short-dated or unmarketable inventory. In addition, the Company may experience spoilage of its raw materials and supplies. The Company's determination that a valuation reserve might be required, in addition to the quantification of such reserve, requires it to utilize significant judgment. Additionally, the Company's inventory is subject to strict quality control and monitoring that is performed throughout the manufacturing process, including release of work-in-process to finished goods. In the event that certain batches or units of product do not meet quality specifications, the Company will record a write-down of any potential unmarketable inventory to its estimated net realizable value and record the expense as cost of product in the Condensed Consolidated Statements of Comprehensive Income (Loss).

Prior to obtaining initial regulatory approval for an investigational product candidate, the Company expenses costs relating to production of pre-launch inventory as research and development expense in its Condensed Consolidated Statements of Comprehensive Income (Loss) in the period incurred. After regulatory approval has been received, the Company capitalizes inventory costs.

Property and Equipment

Property and equipment are recorded at cost. Depreciation is computed using the straight-line method over the estimated useful lives of the assets. Computer equipment and office equipment are depreciated over a life of three years. Laboratory equipment, software, and furniture and fixtures are depreciated over a life of five years. Leasehold improvements are amortized over their estimated useful lives or the expected lease term, whichever is less. Construction in progress reflects amounts incurred for construction or improvements of property and equipment that have not been placed in service.

The Company periodically reviews its property and equipment for impairment when events or changes in circumstances indicate that the carrying amount of such assets may not be recoverable. Determination of recoverability is based on an estimate of undiscounted future cash flows resulting from the use of the asset and its eventual disposition. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the assets, the assets are

written down to their estimated fair values. Property and equipment to be disposed of are reported at the lower of carrying amount or fair value less cost to sell.

Accrued Expenses

The Company enters into contractual agreements with third-party vendors who provide research and development, manufacturing, distribution, and other services in the ordinary course of business. Some of these contracts are subject to milestone-based invoicing, and services are completed over an extended period of time. The Company records liabilities under these contractual commitments when it determines an obligation has been incurred, regardless of the timing of the invoice. This process involves reviewing open contracts and purchase orders, communicating with applicable Company personnel to identify services that have been performed on its behalf and estimating the actual work completed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of actual cost. The majority of service providers invoice the Company monthly in arrears for services performed. The Company makes estimates of accrued expenses as of each balance sheet date in its financial statements based on the facts and circumstances, which can include assumptions such as expected patient enrollment, site activation and estimated project duration. The Company periodically confirms the accuracy of its estimates with the service providers and makes adjustments if necessary. Examples of estimated accrued expenses include (i) fees paid to clinical research organizations ("CROs") in connection with preclinical and toxicology studies and clinical trials; (ii) fees paid to investigative sites in connection with clinical trials; (iii) fees paid to contract manufacturers in connection with the production of the Company's raw materials, drug substance, drug products, and product candidates; and (iv) professional fees.

The Company bases its expenses related to clinical trials on its estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and CROs that conduct and manage clinical trials on the Company's behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. The Company accrues costs for clinical trial activities based upon estimates of the actual work completed in accordance with agreements established with third-party vendors. If the Company underestimates or overestimates the level of these costs, actual expenses could differ from such estimates. As of March 31, 2025 and December 31, 2024, the carrying value of accrued expenses approximates their fair value due to their short-term settlement.

Cost of Product Sales

Cost of product sales includes the cost of producing inventory that is related to product revenue during the respective period, including freight. Cost of product sales may also include costs related to excess or obsolete inventory adjustment charges.

Research and Development Expenses

Research and development expenses consist of costs associated with research activities as well as those with the Company's product development efforts, conducting preclinical trials, clinical trials and manufacturing activities. Research and development expenses are expensed as incurred. Most of the Company's clinical and preclinical studies are performed by third-party CROs. Costs for studies performed by CROs are accrued based upon estimates of the actual work completed in accordance with the third-party agreements. Advance payments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are recognized as expense when the related goods are delivered or the related services are performed.

The Company groups its research and development expenses into two major categories: direct expenses and indirect expenses. Direct expenses consist of compensation for research and development personnel and costs of outside parties to conduct laboratory studies, develop manufacturing processes and manufacture the product candidate, conduct and manage clinical trials, as well as other costs related to the Company's clinical and preclinical studies. Additionally, direct expenses consist of those costs necessary to discontinue and close out a development program, including termination fees and other commitments. These costs are accumulated and tracked by active program.

Indirect costs of the Company's clinical programs include lab supplies and services, facility expenses, depreciation of development equipment and an allocation of its general and administrative overhead costs that support the Company's research and development efforts.

Additionally, the Company has license agreements with third parties, such as Albert Einstein College of Medicine of Yeshiva University, Industrial Research, Ltd., and the University of Alabama at Birmingham ("UAB"), which require fees related to sublicense agreements. The Company accrues sublicense expenses as incurred.

Selling, General and Administrative Expenses

Selling, general and administrative expenses are primarily comprised of compensation and benefits associated with sales and marketing, finance, human resources, legal, information technology and other administrative personnel. Additionally, selling, general and administrative expenses are comprised of market research, marketing, advertising and legal expenses, including patent costs, licenses and other general and administrative costs.

All patent related costs are expensed to selling, general and administrative expenses when incurred as recoverability of such expenditures is uncertain.

Leases

The Company leases certain assets under operating and finance leases, which consist of real estate leases, laboratory equipment leases and office equipment leases as of March 31, 2025. The Company determines whether a contract is, or contains, a lease at inception. The Company accounts for lease obligations in accordance with ASU 2016-02: *Leases (Topic 842)*, which requires a lessee to recognize a right-of-use asset and a lease liability on its balance sheet for most leases. The Company elected the practical expedient that exempts leases with an initial lease term of twelve months or less, as well as the practical expedient that allows companies to select, by class of underlying asset, not to separate lease and non-lease components.

Certain of the Company's operating leases provide for renewal options, which can vary by lease. The right-of-use asset and lease liabilities on the Company's Condensed Consolidated Balance Sheets represent payments over the lease term, which include renewal options for certain real estate leases that the Company is likely to exercise. As part of the Company's assessment of the lease term, the Company elected the hindsight practical expedient, which allows companies to use current knowledge and expectations when determining the likelihood to extend lease options. Certain operating leases include rent escalation provisions, which the Company recognizes as expense on a straight-line basis. Lease expense for leases with an initial term of twelve months or less was not material.

The discount rate used to determine the Company's right-of-use asset and lease liability is the Company's incremental borrowing rate on a collateralized basis over a similar term and amount in a similar economic environment, as generally an implicit rate in the lease is not readily determinable.

The Company has not made any residual value guarantees related to its leases; therefore, the Company has no corresponding liability recorded on its Condensed Consolidated Balance Sheets.

Stock-Based Compensation

All share-based payments, including grants of stock option awards and restricted stock unit awards, are recognized in the Company's Condensed Consolidated Statements of Comprehensive Income (Loss) based on their fair values. Stock-based compensation cost is estimated at the grant date based on the fair value of the award and is recognized as expense on a straight-line basis over the requisite service period of the award. Determining the appropriate fair value model and the related assumptions for the model requires judgment, including estimating the stock price volatility and the expected term. The Company utilizes the Black-Scholes option-pricing model to value its stock option awards and recognize compensation expense on a straight-line basis over the requisite service period. The Company reduces stock-based compensation expense for estimated forfeitures. The estimation of share-based payment awards that will ultimately vest requires judgment, and to the extent actual results or updated estimates differ from the Company's current estimates, such amounts will be recorded as a cumulative adjustment in the period estimates are revised. In addition, the Company has outstanding performance-based restricted stock unit awards for which no compensation expense is recognized until it is probable that the performance condition will be achieved. Actual results, and future changes in estimates, may differ substantially from the Company's current estimates.

Interest Expense, Deferred Financing Costs and Royalty Financing Obligations

Interest expense primarily relates to the royalty financing obligations (see "*Note 6—Royalty Financing Obligations*") and the term loan borrowings under the Pharmakon Loan Agreement (see "*Note 7—Debt*") during the three months ended March 31, 2025 and 2024.

Costs directly associated with the borrowings have been capitalized and are netted against the corresponding debt liabilities on the Condensed Consolidated Balance Sheets. These costs are being amortized to interest expense over the terms of the corresponding borrowings using the effective interest rate method. When utilizing the effective interest method, in periods in which payment-in-kind ("PIK") interest was designated and added to the outstanding principal balance of the borrowing, the amortization of the deferred debt fees and issuance costs was accretive.

The royalty financing obligations are eligible to be repaid based on royalties from net sales of ORLADEYO. Interest expense is accrued using the effective interest rate method over the estimated period each of the related liabilities will be paid. This requires the Company to estimate the total amount of future royalty payments to be generated from product sales over the life of the agreement. The Company imputes interest on the carrying value of each of the royalty financing obligations and records interest expense using an imputed effective interest rate. The Company reassesses the expected royalty payments each reporting period and accounts for any changes through an adjustment to the effective interest rate on a prospective basis. The assumptions used in determining the expected repayment term of the debt and amortization period of the issuance costs require that the Company make estimates that could impact the carrying value of each of the liabilities, as well as the periods over which associated issuance costs will be amortized. A significant increase or decrease in forecasted net sales could materially impact each of the liability balances, interest expense and the time periods for repayment.

Income Taxes

The liability method is used in the Company's accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on differences between financial reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that are expected to be in effect when the differences are expected to reverse.

Significant management judgment is required in determining the Company's provision for income taxes, deferred tax assets and liabilities and any valuation allowance recorded against net deferred tax assets. The Company has recorded a valuation allowance against substantially all potential tax assets, due to uncertainties in its ability to utilize deferred tax assets, primarily consisting of certain net operating losses carried forward, before they expire. The valuation allowance is based on estimates of future earnings in each of the jurisdictions in which the Company operates and the period over which its deferred tax assets will be recoverable.

The Company accounts for uncertain tax positions in accordance with U.S. GAAP. Uncertain tax positions are recorded based upon certain recognition and measurement criteria. The Company re-evaluates uncertain tax positions and considers various factors, including, but not limited to, changes in tax law and the measurement of tax positions taken or expected to be taken in tax returns. The Company adjusts the amount of the liability to reflect any subsequent changes in the relevant facts and circumstances surrounding the uncertain tax positions. The Company recognizes interest and penalties related to income tax matters in income tax expense.

Beginning in fiscal year 2021, the Company began accruing for U.S. state taxes and foreign income taxes as a result of increased nexus in both U.S. state and foreign jurisdictions where historically the Company had no presence and where no net operating losses had historically been established.

In addition, starting in 2022, amendments to Section 174 of the Internal Revenue Code of 1986, as amended ("IRC"), no longer permit an immediate deduction for research and development expenditures in the tax year that such costs are incurred. Instead, these IRC Section 174 development costs must now be capitalized and amortized over either a five-year period for activities performed within the U.S. or a 15-year period for activities performed outside the U.S. The new amortization period begins with the midpoint of any taxable year that IRC Section 174 costs are first incurred, regardless of whether the expenditures were made prior to or after July 1, and runs until the midpoint of year six for activities conducted in the U.S. or year sixteen in the case of development conducted on foreign soil.

Certain countries in which the Company has operations have adopted legislation influenced by the Organization for Economic Cooperation and Development ("OECD") Pillar Two rules, including a minimum tax rate of 15%. It is uncertain whether the U.S. will enact legislation to adopt the Pillar Two framework. While the Company is currently not within the scope of the rules, it is continuing to review and evaluate additional guidance released by the OECD, along with the pending legislative adoption by additional individual countries where the Company operates.

Foreign Currency

The functional currency of each of the Company's foreign subsidiaries is primarily the local currency of the country in which the subsidiary operates. The Company's asset and liability accounts are translated at the current exchange rate as

of the balance sheet date. Revenue and expense accounts are translated at the average exchange rate over the period. Adjustments resulting from the translation of the financial statements of the Company's foreign subsidiaries into U.S. dollars are accumulated as a separate component of stockholders' equity within accumulated other comprehensive income. Gains or losses resulting from transactions denominated in foreign currencies are included in foreign currency gains (losses), net, within the Condensed Consolidated Statement of Comprehensive Income (Loss).

Net Income (Loss) Per Share

Basic net income (loss) per share is based upon the weighted average number of common shares outstanding during the period. Diluted net income (loss) per share is based upon the weighted average number of common shares outstanding and potentially dilutive common shares during the period as determined by using the treasury stock method.

Accumulated Other Comprehensive Income

Accumulated other comprehensive income is comprised of cumulative foreign currency translation adjustments and unrealized gains and losses on available-for-sale investments and is disclosed as a separate component of stockholders' equity. Realized gain and loss amounts on available-for-sale investments are reclassified from accumulated other comprehensive income and recorded as interest and other income on the Condensed Consolidated Statements of Comprehensive Income (Loss). There were no realized gains or losses reclassified out of accumulated other comprehensive income for the three months ended March 31, 2025 and 2024.

Significant Customers and Other Risks

Significant Customers

The Company's primary sources of revenue and cash flow are the sales of ORLADEYO in the United States.

ORLADEYO is generally distributed through an arrangement with a single specialty pharmacy in the United States. The specialty pharmacy subsequently sells ORLADEYO to its customers (pharmacy benefit managers, insurance companies, government programs and group purchasing organizations) and dispenses product to patients. The specialty pharmacy's inability or unwillingness to continue these distribution activities could adversely impact the Company's business, results of operations and financial condition. Revenue where the specialty pharmacy is considered the customer was approximately 84% and 87% of total net revenues for the three months ended March 31, 2025 and 2024, respectively.

The Company is distributing ORLADEYO in other global markets directly or through other parties.

Risks from Third-Party Manufacturing and Distribution Concentration

The Company relies on a single source manufacturer for active pharmaceutical ingredient and finished drug product manufacturing of product candidates in development and on a single specialty pharmacy for distribution of approved drug product in the United States. Delays or disruption in the manufacture or distribution of any product could adversely impact the future procurement stockpiling of the Company's commercial product, commercial revenue and product candidates.

Further, the Company's drug development activities are performed by a limited group of third-party vendors. If any of these vendors were unable to perform its services, this could significantly impact the Company's ability to complete its drug development activities.

Credit Risk

Cash equivalents and investments are financial instruments that potentially subject the Company to concentration of risk to the extent recorded on the Condensed Consolidated Balance Sheets. The Company deposits excess cash with major financial institutions in the United States. Balances may exceed the amount of insurance provided on such deposits. The Company believes it has established guidelines for investment of its excess cash relative to diversification and maturities that maintain safety and liquidity. To minimize the exposure due to adverse shifts in interest rates, the Company maintains a portfolio of investments with an average maturity of approximately 12 months or less.

The Company's receivables from sales of ORLADEYO are primarily due from one customer, resulting in a concentration of credit risk.

Recently Adopted Accounting Pronouncements

There have been no new accounting pronouncements adopted by the Company during the three months ended March 31, 2025.

New Accounting Pronouncements Not Yet Adopted

In December 2023, the Financial Accounting Standards Board ("FASB") issued ASU 2023-09, Income Taxes (Topic 740): Improvements to Income Tax Disclosures, which requires public entities, on an annual basis, to provide disclosure of specific categories in the rate reconciliation, as well as disclosure of income taxes paid disaggregated by jurisdiction. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024, with early adoption permitted. The Company expects adoption of this ASU will result in additional disclosures but does not expect it will have a material effect on the Company's consolidated balance sheet, statement of comprehensive income (loss), or statement of cash flows.

In November 2024, the FASB issued ASU 2024-03, Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses, which requires public entities, on an annual and interim basis, to provide disaggregated disclosure of certain income statement expenses into specified categories within the footnotes to the financial statements. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027, with early adoption permitted. The Company is evaluating the impact of adopting ASU 2024-03.

The Company does not expect any other recently issued accounting standards to have a material impact to its financial statements or disclosures.

Note 2 — Revenue

The Company recorded the following revenues for the three months ended March 31, 2025 and 2024 (in thousands):

	Three Months Ended March 31,	
	2025	**2024**
ORLADEYO:		
U.S.	$ 120,159	$ 79,966
Outside of U.S.	14,084	8,901
Total ORLADEYO	134,243	88,867
Other revenues	11,291	3,894
Total revenues	$ 145,534	$ 92,761

ORLADEYO revenues represent total revenues from product sales, collaborative revenues, and royalties. Other revenues primarily relate to the Company's product sales and royalties for peramivir.

No individual country outside of the U.S. exceeded 10% of total revenues for the three months ended March 31, 2025 and 2024.

Note 3 — Investments

Fair value is an exit price, representing the amount that would be received from the sale of an asset or paid to transfer a liability in an orderly transaction between market participants. Fair value is determined based on observable and unobservable inputs. Observable inputs reflect readily obtainable data from independent sources, while unobservable inputs reflect certain market assumptions. As a basis for considering such assumptions, U.S. GAAP establishes a three-tier value hierarchy, which prioritizes the inputs used to develop the assumptions and for measuring fair value as follows: (Level 1) observable inputs such as quoted prices in active markets for identical assets; (Level 2) inputs other than the quoted prices in active markets that are observable either directly or indirectly; and (Level 3) unobservable inputs for which there is little or no market data, which requires the Company to develop its own assumptions. This hierarchy requires the Company to use observable market data, when available, and to minimize the use of unobservable inputs when determining fair value.

The Company's financial instruments that are measured at fair value on a recurring basis consist of fixed income investments. These valuations are based on observable direct and indirect inputs, primarily quoted prices of similar, but not

identical, instruments in active markets or quoted prices for identical or similar instruments in markets that are not active. These fair values are obtained from independent pricing services.

Assets measured at fair value on a recurring basis were as follows (in thousands):

	March 31, 2025			
	Quoted Price in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Obligations of U.S. Government and its agencies	$ —	$ 210,450	$ —	$ 210,450
Total assets	$ —	$ 210,450	$ —	$ 210,450

	December 31, 2024			
	Quoted Price in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Obligations of U.S. Government and its agencies	$ —	$ 236,460	$ —	$ 236,460
Total assets	$ —	$ 236,460	$ —	$ 236,460

As of March 31, 2025, the Company had two securities with a total estimated fair value of $19,738 in an unrealized loss position. The Company believes the individual unrealized losses represent temporary declines primarily resulting from interest rate changes. The Company does not have an intent to sell these investments, and it is more likely than not that the investments will be held until recovery of their amortized cost basis. As such, no allowance was recognized.

The following tables summarize the fair value of the Company's investments by type (in thousands):

	March 31, 2025				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its agencies	$ 209,203	$ 964	$ 287	$ (4)	$ 210,450
Total investments	$ 209,203	$ 964	$ 287	$ (4)	$ 210,450

	December 31, 2024				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its agencies	$ 234,902	$ 1,121	$ 451	$ (14)	$ 236,460
Total investments	$ 234,902	$ 1,121	$ 451	$ (14)	$ 236,460

The following table summarizes the scheduled maturity for the Company's investments at March 31, 2025 and December 31, 2024 (in thousands):

	March 31, 2025	December 31, 2024
Maturing in one year or less	$ 189,902	$ 216,137
Maturing after one year through two years	20,548	20,323
Total investments	$ 210,450	$ 236,460

Note 4 — Trade Receivables

Product Sales

Receivables from product sales are recorded for amounts due to the Company related to sales of ORLADEYO and peramivir. At March 31, 2025 and December 31, 2024, receivables, net of reserves, related to sales of ORLADEYO were $90,487 and $76,282, respectively. At March 31, 2025 and December 31, 2024, receivables related to sales of peramivir were $1,202 and $564, respectively.

Collaborations

At March 31, 2025 and December 31, 2024 receivables from collaborations related to receivables from the Company's royalty partners and were $1,705 and $2,223, respectively.

Note 5 — Inventory

At March 31, 2025 and December 31, 2024, the Company's inventory primarily related to ORLADEYO. Inventory also included peramivir, which is primarily manufactured for the Company's partners.

The Company's inventories consisted of the following (in thousands):

	March 31, 2025	December 31, 2024
Raw materials	$ 8,742	$ 10,006
Work-in-process	17,426	16,152
Finished goods	7,887	7,765
Total inventory	34,055	33,923
Reserves	(2,085)	(2,649)
Total inventory, net	$ 31,970	$ 31,274

Note 6 — Royalty Financing Obligations

On December 7, 2020, the Company and RPI 2019 Intermediate Finance Trust ("RPI") entered into a Purchase and Sale Agreement (the "2020 RPI Royalty Purchase Agreement"), pursuant to which the Company sold to RPI the right to receive certain royalty payments from the Company for a purchase price of $125,000 in cash (the "2020 RPI Royalty Sale"). Under the 2020 RPI Royalty Purchase Agreement, RPI is entitled to receive tiered, sales-based royalties on net product sales of ORLADEYO in the United States and certain key European markets (collectively, the "Key Territories"), and other markets where the Company sells ORLADEYO directly or through distributors (collectively, the "Direct Sales") in an amount equal to: (i) 8.75% of aggregate annual net sales of ORLADEYO for annual net sales up to $350,000 and (ii) 2.75% of annual net sales for annual net sales between $350,000 and $550,000. No royalty payments are payable on annual Direct Sales over $550,000.

Under the 2020 RPI Royalty Purchase Agreement, RPI is also entitled to receive a tiered revenue share on ORLADEYO sublicense revenue or net sales by licensees outside of the Key Territories (the "Other Markets") equal to: (i) 20% of the proceeds received by the Company for upfront license fees and development milestones for ORLADEYO in the Other Markets, (ii) 20% of proceeds received on annual net sales of up to $150,000 in the Other Markets, and (iii) 10% of proceeds received by the Company on annual net sales between $150,000 and $230,000 in the Other Markets. No royalty payments are payable on annual net sales above $230,000 in the Other Markets.

On November 19, 2021, the Company and RPI entered into (i) a Purchase and Sale Agreement (the "2021 RPI Royalty Purchase Agreement" and together with the 2020 RPI Royalty Purchase Agreement, the "RPI Royalty Purchase Agreements"), pursuant to which the Company sold to RPI the right to receive certain royalty payments from the Company for a purchase price of $150,000 in cash, and (ii) a Purchase and Sale Agreement with OCM IP Healthcare Holdings Limited, an affiliate of OMERS Capital Markets ("OMERS") (the "OMERS Royalty Purchase Agreement" and collectively with the RPI Royalty Purchase Agreements, the "Royalty Purchase Agreements"), pursuant to which the Company sold to OMERS the right to receive certain royalty payments from the Company for a purchase price of an additional $150,000 in cash.

Under the 2021 RPI Royalty Purchase Agreement, RPI is entitled to receive tiered, sales-based royalties on Direct Sales in an amount equal to: (i) 0.75% of aggregate annual net sales of ORLADEYO for annual net sales up to $350,000 and (ii) 1.75% of annual net sales of ORLADEYO for annual net sales between $350,000 and $550,000. No royalty payments are payable on Direct Sales over $550,000. RPI is also entitled to receive a tiered revenue share on ORLADEYO sublicense revenue or net sales by licensees in the Other Markets in an amount equal to (i) 3.0% of proceeds received by the Company on annual net sales of up to $150,000 in the Other Markets and (ii) 2.0% of proceeds received by the Company on annual net sales between $150,000 and $230,000 in the Other Markets. No royalty payments are payable on annual net sales above $230,000 in the Other Markets.

The royalties payable under the 2021 RPI Royalty Purchase Agreement are in addition to the royalties payable to RPI under the 2020 RPI Royalty Purchase Agreement.

Under the OMERS Royalty Purchase Agreement, for the calendar quarter beginning October 1, 2023, OMERS was entitled to receive tiered, sales-based royalties on Direct Sales in an amount equal to: (i) 7.5% of aggregate annual net sales of ORLADEYO for annual net sales up to $350,000 and (ii) 6.0% of annual net sales of ORLADEYO for annual net sales between $350,000 and $550,000 (with no royalty payments payable on annual Direct Sales over $550,000). Commencing with the calendar quarter beginning January 1, 2024, OMERS is entitled to receive tiered, sales-based royalties on Direct Sales in an amount equal to: (i) 10.0% of aggregate annual net sales of ORLADEYO for annual net sales up to $350,000 and (ii) 3.0% of annual net sales of ORLADEYO for annual net sales between $350,000 and $550,000 (with no royalty payments payable on annual Direct Sales over $550,000).

Under the OMERS Royalty Purchase Agreement, OMERS is also entitled to receive a tiered revenue share on ORLADEYO sublicense revenue or net sales by licensees in the Other Markets in an amount equal to: (i) 20.0% of the proceeds received by the Company for upfront license fees and development milestones for ORLADEYO in the Other Markets, (ii) 20.0% of proceeds received by the Company on annual net sales of up to $150,000 in the Other Markets, and (iii) 10.0% of proceeds received by the Company on annual net sales between $150,000 and $230,000 in the Other Markets. No royalty payments are payable on annual net sales above $230,000 in the Other Markets. OMERS is also entitled to receive profit share amounts of up to 10% from certain other permitted sales in certain other markets.

Under the 2020 RPI Royalty Purchase Agreement, the Company is required to make royalty payments of amounts owed to RPI each calendar quarter following the first commercial sale of ORLADEYO in any country. Under the 2021 RPI Royalty Purchase Agreement, the Company is required to make payments to RPI in respect of net sales or sublicense revenue in each calendar quarter from and after October 1, 2021. Under the OMERS Royalty Purchase Agreement, the Company is required to make payments to OMERS in respect of net sales or sublicense revenue in each calendar quarter from and after October 1, 2023. OMERS will no longer be entitled to receive any payments on the date in which aggregate payments actually received by OMERS equals 155.0% of the $150,000 purchase price.

The transactions contemplated by each of the Royalty Purchase Agreements are referred to herein as the "Royalty Sales".

Under the Royalty Purchase Agreements, the Company has agreed to specified affirmative and negative covenants, including covenants regarding periodic reporting of information by the Company to RPI and OMERS, third-party audits of royalties paid under the Royalty Purchase Agreements, and restrictions on the ability of the Company or any of its subsidiaries to incur indebtedness other than certain royalty sales and as was permitted to be incurred under the terms of the Athyrium Credit Agreement (as defined in Note 7 herein) through its payoff and termination on April 17, 2023 or, subsequent to that date, the Pharmakon Loan Agreement (as defined in Note 7 herein), as applicable. See "*Note 7—Debt*" for further details on the Athyrium Credit Agreement and the Pharmakon Loan Agreement. The restrictions under the Royalty Purchase Agreements on the ability of the Company or any of its subsidiaries to incur indebtedness are eliminated after the achievement of certain specified milestones in the Royalty Purchase Agreements.

The cash consideration obtained pursuant to the Royalty Purchase Agreements is recorded in "Royalty financing obligations" on the Company's Condensed Consolidated Balance Sheets. The fair value for the royalty financing obligations at the time of the transactions was based on the Company's estimates of future royalties expected to be paid to the counterparty over the life of the arrangement. The Company subsequently records the obligations at their carrying value using the effective interest method. As of March 31, 2025 and December 31, 2024, the carrying value of the royalty financing obligations under the Royalty Purchase Agreements approximated fair value and was measured based on the Company's current estimates of future payments to RPI and OMERS over the lives of the agreements, which are considered Level 3 inputs. The Company utilizes the prospective method to account for subsequent changes in the estimated future royalties to be paid by the Company to the counterparty over the life of the arrangement. Under the prospective method, a new effective interest rate is determined based on the revised estimate of remaining cash flows. The new rate is the discount rate that equates the present value of the revised estimate of remaining cash flows with the carrying amount of the debt, and it will be used to recognize interest expense for the remaining periods. The Company periodically assesses the amount and timing of expected royalty payments using internal projections of future net product sales, which are based on key assumptions, including paid patients and price. To the extent such payments are greater or less than the Company's initial estimates or the timing of such payments is materially different than its original estimates, the Company will prospectively adjust the amortization of the royalty financing obligations and the effective interest rate. On a quarterly basis, the Company assesses the projected royalty payments relative to the projected interest accretion for the next twelve months to determine if the royalty liability balance is reduced relative to the current outstanding liability. In such case of excess payments relative to interest accretion for the next twelve months, the excess payments are considered to be a short-term liability and classified within current liabilities on the Company's Condensed Consolidated Balance Sheets.

During the three months ended March 31, 2025, there were no significant changes to the amount and timing of expected royalties under the Royalty Purchase Agreements based on the Company's latest forecasts related to ORLADEYO sales.

The following table shows the royalty financing obligations activity for the three months ended March 31, 2025 (in thousands) as well as the effective interest rate as of March 31, 2025:

	2020 RPI Royalty Agreement	2021 RPI Royalty Agreement	OMERS Royalty Agreement	Total
Balance as of December 31, 2024	$ 180,413	$ 181,140	$ 152,176	$ 513,729
Non-cash Interest expense on Royalty financing obligations	9,756	—	3,758	13,514
Royalty revenues paid and payable	(11,818)	(1,021)	(13,486)	(26,325)
Balance as of March 31, 2025	$ 178,351	$ 180,119	$ 142,448	$ 500,918
Effective interest rate	21.6 %	— %	10.0 %	

Cash paid for interest on the royalty financing obligations was $14,549 and $15,612 for the three months ended March 31, 2025 and 2024, respectively.

The Royalty financing obligations liabilities and the associated deferred issuance costs are amortized using the effective interest method over the term of the arrangement.

Concurrent with entering into the 2021 RPI Royalty Purchase Agreement, the Company and RPI entered into a Common Stock Purchase Agreement pursuant to which the Company sold common stock to RPI for a premium of $4,269. This premium has been deferred and is being amortized through interest expense using the effective interest method over the term of the applicable arrangement.

Note 7 — Debt

Pharmakon Loan Agreement

On April 17, 2023, the Company entered into a $450,000 Loan Agreement (the "Pharmakon Loan Agreement") with BioPharma Credit Investments V (Master) LP and BPCR Limited Partnership, as lenders, and BioPharma Credit PLC, as collateral agent for the lenders. Certain of the Company's wholly-owned subsidiaries are guarantors to the Pharmakon Loan Agreement. The Pharmakon Loan Agreement provides for an initial term loan in the principal amount of $300,000

(the "Tranche A Loan") funded on April 17, 2023 (the "Tranche A Closing Date"). The Company used a portion of the proceeds from the Tranche A Loan to repay the $241,787 of outstanding indebtedness (principal and interest due as of April 17, 2023) under the then-existing credit facility with Athyrium Opportunities III Co-Invest 1 LP (the "Athyrium Credit Agreement") and to pay associated transaction costs and fees, and used the remaining net proceeds of $25,805 for other general corporate purposes.

The Pharmakon Loan Agreement also provided for three additional term loan tranches, at the Company's option, in principal amounts of $50,000 each (each a "Subsequent Tranche Loan" and, collectively with the Tranche A Loan, the "Pharmakon Term Loans" and each, a "Pharmakon Term Loan"), which could have been requested on or prior to September 30, 2024. The Company chose not to request any Subsequent Tranche Loans and the options have since expired. The maturity date of the Pharmakon Loan Agreement is April 17, 2028 (the "Maturity Date"), the fifth anniversary of the Tranche A Closing Date.

The Pharmakon Loan Agreement provides for quarterly interest-only payments until the Maturity Date, with the unpaid principal amount of the outstanding Pharmakon Term Loans due and payable on the Maturity Date. During the first 18 months following the Tranche A Closing Date, the Company had the option to make a portion of the applicable interest payment on the Tranche A Loan in-kind (a "Pharmakon PIK Interest Payment") by capitalizing as principal up to 50% of the amount of interest accrued on the Tranche A Loan during the applicable interest period. The Pharmakon Term Loans bear interest at a rate equal to the three-month Secured Overnight Financing Rate ("SOFR"), which shall be no less than 1.75%, plus 7.00%, per annum or, for each interest period in which a Pharmakon PIK Interest Payment was made, with respect to the Tranche A Loan, SOFR plus 7.25%, per annum.

The Tranche A Loan accrued interest at an effective interest rate of 12.31% and 13.34% for the three months ended March 31, 2025 and 2024, respectively.

The Company is required to make a mandatory prepayment of the Pharmakon Term Loans (i) upon the occurrence of a change of control and (ii) prior to any repayment of any convertible debt that the Company may issue in the future, subject to certain exceptions. The Company may make voluntary prepayments in whole or in part, in minimum $25,000 increments. Prepayments are subject to a prepayment premium equal to, (i) with respect to any prepayment made prior to the second anniversary of the applicable Pharmakon Term Loan borrowing date, the sum of (1) 3.00% of the principal amount of the Pharmakon Term Loan being prepaid plus (2) the aggregate amount of all interest that would have accrued on the principal amount of the Pharmakon Term Loan being prepaid from the date of prepayment through and including the second anniversary of the date of the borrowing of such Pharmakon Term Loan; (ii) with respect to any prepayment made on or after the second anniversary and prior to the third anniversary of the applicable Pharmakon Term Loan borrowing date, 3.00% of the principal amount of the Pharmakon Term Loan being prepaid; (iii) with respect to any prepayment made on or after the third anniversary and prior to the fourth anniversary of the applicable Pharmakon Term Loan, 2.00% of the principal amount of the Pharmakon Term Loan being prepaid; and (iv) with respect to any prepayment made on or after the fourth anniversary of the applicable Pharmakon Term Loan borrowing date and before the Maturity Date, 1.00% of the principal amount of the Pharmakon Term Loan being prepaid. In addition, if the Company had requested any Subsequent Tranche Loans, certain funding fees would have been required to be paid.

The Pharmakon Loan Agreement also contains representations and warranties and affirmative and negative covenants customary for financings of this type, as well as customary events of default. Certain of the customary negative covenants limit the ability of the Company and certain of its subsidiaries to, among other things, dispose of assets, engage in mergers, acquisitions, and similar transactions, incur additional indebtedness, grant liens, make investments, pay dividends or make distributions or certain other restricted payments in respect of equity, prepay other indebtedness, enter into restrictive agreements, undertake fundamental changes or amend certain material contracts, among other customary covenants, in each case subject to certain exceptions.

A failure to comply with the covenants in the Pharmakon Loan Agreement, or an occurrence of any other event of default, could permit the lenders under the Pharmakon Loan Agreement to declare the borrowings thereunder, together with accrued interest and fees, and any applicable prepayment premium, to be immediately due and payable.

The Company's obligations under the Pharmakon Loan Agreement are secured by a security interest in, subject to certain exceptions, substantially all of the Company's assets.

As of March 31, 2025, the Company had total borrowings of $300,000 under the Pharmakon Loan Agreement. Interest expense on the Tranche A Loan for the three months ended March 31, 2025 was $9,153, all of which was paid at the end of the quarterly period. As of March 31, 2025, borrowings, including the prior period Pharmakon PIK Interest Payments, totaled $323,704.

As of March 31, 2024, the Company had total borrowings of $300,000 under the Pharmakon Loan Agreement. Interest expense on the Tranche A Loan for the three months ended March 31, 2024 was $9,974. As allowable under the Pharmakon Loan Agreement, the Company designated and accounted for 50% of the quarterly interest payment for the three months ended March 31, 2024 as a Pharmakon PIK Interest Payment and the total amount of $4,987 was added to the outstanding principal balance of the borrowing. The remaining 50% of the total quarterly interest payment of $4,987 was paid at the end of the quarterly period. As of March 31, 2024, borrowings, including the Pharmakon PIK Interest Payments, totaled $318,650.

The fair value of the debt approximates its carrying value based on prevailing interest rates as of the balance sheet date and is considered as Level 2 in the fair value hierarchy.

Incurred debt fees and issuance costs associated with the Tranche A Loan under the Pharmakon Loan Agreement totaled $11,147 and have been deferred and are being amortized as interest expense on an effective interest rate method over the remaining term of the Tranche A Loan. Deferred financing amortization of $544 and $266 was recognized for the three months ended March 31, 2025 and 2024, respectively.

On April 18, 2025, the Company made a prepayment on the Pharmakon Term Loan. See *"Note 15—Subsequent Events"* for additional information.

Note 8 — Lease Obligations

The Company leases certain assets under operating leases, which primarily consist of real estate leases, and finance leases, which generally consist of laboratory equipment leases and office equipment leases, as of March 31, 2025. The Company's real estate agreements expire at various times between 2025 through 2030 and include renewal options that are three years in length.

Lease expense under operating and finance leases was as follows (in thousands):

	Three Months Ended March 31,	
	2025	**2024**
Operating lease expense	$ 544	$ 590
Finance lease expense:		
Amortization of right-of-use assets	$ 538	$ 407
Interest on lease liabilities	94	77
Total finance lease expense	$ 632	$ 484

Other supplemental information related to leases was as follows:

	March 31, 2025	**December 31, 2024**
Weighted average remaining lease term:		
Operating leases	8.9 years	9.0 years
Finance leases	2.7 years	2.6 years
Weighted average discount rate:		
Operating leases	10.92 %	10.91 %
Finance leases	9.10 %	8.66 %

The following table summarizes the presentation in the Condensed Consolidated Balance Sheets of the Company's operating leases (in thousands):

	Balance Sheet Location	March 31, 2025	December 31, 2024
Operating lease assets:			
Operating lease assets, net	*Right of use assets*	$ 7,891	$ 8,061
Operating lease liabilities:			
Current operating lease liabilities	*Operating lease liabilities – current liabilities*	$ 1,071	$ 937
Non-current operating lease liabilities	*Operating lease liabilities – long-term liabilities*	7,779	7,924
Total operating lease liabilities		$ 8,850	$ 8,861

The following table summarizes the presentation in the Condensed Consolidated Balance Sheets of the Company's finance leases (in thousands):

	Balance Sheet Location	March 31, 2025	December 31, 2024
Finance lease assets:			
Finance lease assets, net	*Right of use assets*	$ 4,091	$ 3,947
Finance lease liabilities:			
Current finance lease liabilities	*Finance lease liabilities – current liabilities*	$ 1,795	$ 1,835
Non-current finance lease liabilities	*Finance lease liabilities – long-term liabilities*	2,314	2,124
Total finance lease liabilities		$ 4,109	$ 3,959

Operating lease assets are recorded net of accumulated amortization of $2,504 and $6,065 as of March 31, 2025 and December 31, 2024, respectively. Finance lease assets are recorded net of accumulated amortization of $4,445 and $4,059 as of March 31, 2025 and December 31, 2024, respectively.

Maturities of lease liabilities as of March 31, 2025 are as follows (in thousands):

	Operating Leases	Finance Leases
2025 (remaining)	$ 1,509	$ 1,692
2026	1,790	1,487
2027	1,705	1,017
2028	1,401	451
2029	996	6
Thereafter	6,922	—
Total lease payments	14,323	4,653
Less imputed interest	(5,473)	(544)
Total	$ 8,850	$ 4,109

Supplemental cash flow information related to leases was as follows (in thousands):

	Three Months Ended March 31,	
	2025	2024
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows for finance leases	$ 94	$ 77
Operating cash flows for operating leases	$ 385	$ 525
Financing cash flows for finance leases	$ 516	$ 390
Operating lease assets obtained in exchange for operating lease liabilities:	$ 115	$ 156
Finance lease assets obtained in exchange for finance lease liabilities:	$ 679	$ 103
Non-cash increase to operating lease assets due to remeasurement of operating lease liabilities:	$ —	$ 365

Note 9 — Stockholders' Equity

Sales of Common Stock

On February 27, 2024, the Company filed an automatic shelf registration statement on Form S-3 with the SEC. This shelf registration statement became effective automatically upon filing and allows the Company to sell an indeterminate number of securities, including common stock, preferred stock, depositary shares, purchase contracts, warrants, debt securities, and units, from time to time at prices and on terms to be determined at the time of sale.

Shares Reserved for Future Issuance of Common Stock

The Company had reserved shares of common stock for issuance as follows (in thousands):

	March 31, 2025	December 31, 2024
Shares reserved for exercises of outstanding stock options	43,085	44,240
Shares reserved for vesting of restricted stock units	9,909	10,112
Shares reserved for future issuance under the Stock Incentive Plan	1,940	1,065
Shares reserved for future issuance under the Inducement Equity Incentive Plan	1,711	1,699
Shares reserved for future issuance under the Employee Stock Purchase Plan	4,841	5,042
Total shares reserved for future issuance	61,486	62,158

Note 10 — Stock-Based Compensation

As of March 31, 2025, the Company had three stock-based employee compensation plans: the Amended and Restated Stock Incentive Plan ("Incentive Plan"), the Amended and Restated Inducement Equity Incentive Plan ("Inducement Plan") and the Amended and Restated Employee Stock Purchase Plan ("ESPP"). The Incentive Plan was most recently amended and restated on April 21, 2025, subject to stockholder approval at the Company's annual meeting of stockholders to be held on June 12, 2025. The Inducement Plan was most recently amended and restated by the Company's Board of Directors on October 26, 2023. The ESPP was most recently amended and restated by the Company's Board of Directors on July 7, 2023.

The Company recorded the following stock-based compensation expense (in thousands):

	Three Months Ended March 31,	
	2025	2024
Incentive Plan	$ 19,232	$ 11,566
Inducement Plan	1,932	1,923
ESPP	204	163
Stock-based compensation expense	$ 21,368	$ 13,652

Stock Incentive Plan

The following table summarizes stock option activity under the Incentive Plan:

	Shares (in thousands)	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2024	39,082	$ 7.96		
Granted	5	7.90		
Exercised	(213)	4.37		$ 803
Cancelled or Forfeited	(803)	10.59		
Outstanding at March 31, 2025	38,071	$ 7.93	6.55	$ 28,844
Exercisable at March 31, 2025	23,980	$ 7.96	5.17	$ 23,398
Vested and expected to vest at March 31, 2025	35,953	$ 7.92	6.42	$ 28,058

The following table summarizes restricted stock unit activity under the Incentive Plan:

	Shares (in thousands)	Weighted Average Grant Date Fair Value
Unvested at December 31, 2024	9,289	$ 7.73
Granted	144	8.96
Vested	(119)	10.13
Forfeited	(221)	7.79
Unvested at March 31, 2025	9,093	$ 7.71

For restricted stock unit awards granted under the Incentive Plan, the fair value of the awards is determined based on the market value of the Company's shares on the grant date. The weighted average grant date fair value of these awards granted during the first three months of 2025 and 2024 was $8.96 and $5.30, respectively. The fair value of the restricted stock unit awards is amortized to expense over the vesting periods using a straight-line expense attribution method.

As of March 31, 2025, total unrecognized compensation cost related to unvested restricted stock unit awards granted under the Incentive Plan was $49,129, which is expected to be recognized over a weighted average period of 1.6 years.

Inducement Equity Incentive Plan

The following table summarizes stock option activity under the Inducement Plan:

	Shares (in thousands)	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2024	5,158	$ 8.44		
Granted	23	7.90		
Exercised	(73)	4.06		$ 276
Cancelled or Forfeited	(94)	12.04		
Outstanding at March 31, 2025	5,014	$ 8.44	6.72	$ 7,043
Exercisable at March 31, 2025	3,394	$ 7.92	6.01	$ 6,295
Vested and expected to vest at March 31, 2025	4,681	$ 8.33	6.62	$ 6,910

The following table summarizes restricted stock unit activity under the Inducement Plan:

	Shares (in thousands)	Weighted Average Grant Date Fair Value
Unvested at December 31, 2024	823	$ 8.53
Granted	93	8.35
Vested	(65)	10.46
Forfeited	(35)	9.52
Unvested at March 31, 2025	816	$ 8.31

For restricted stock unit awards granted under the Inducement Plan, the fair value of the awards is determined based on the market value of the Company's shares on the grant date. The weighted average grant date fair value of these awards granted during the first three months of 2025 and 2024 was $8.35 and $5.25, respectively. The fair value of the restricted stock unit awards is amortized to expense over the vesting periods using a straight-line expense attribution method.

As of March 31, 2025, total unrecognized compensation cost related to unvested restricted stock unit awards granted under the Inducement Plan was $4,802, which is expected to be recognized over a weighted average period of 1.6 years.

Weighted Average Assumptions for Stock Option Awards Granted to Employees and Directors under the Incentive and Inducement Plans

For stock option awards granted under the Incentive Plan and the Inducement Plan, the fair value is estimated on the date of grant using a Black-Scholes option pricing model and the assumptions noted below. The fair value of the stock option awards is amortized to expense over the vesting periods using a straight-line expense attribution method.

Stock Incentive Plan

The following table summarizes the key assumptions used by the Company to value the stock option awards granted under the Incentive Plan during the three months ended March 31, 2025. There were no stock option awards granted under the Incentive Plan during the three months ended March 31, 2024.

	Three Months Ended March 31, 2025
Expected Life in Years	5.9
Expected Volatility	82.7 %
Expected Dividend Yield	0.0 %
Risk-Free Interest Rate	4.4 %
Weighted average grant date fair value per share	$ 5.73

As of March 31, 2025, total unrecognized compensation cost related to unvested stock option awards granted under the Incentive Plan was $51,515, which is expected to be recognized over a weighted average period of 1.4 years.

Inducement Equity Incentive Plan

The following table summarizes the key assumptions used by the Company to value the stock option awards granted under the Inducement Plan during the three months ended March 31, 2025 and 2024:

	Three Months Ended March 31,	
	2025	2024
Expected Life in Years	5.9	5.8
Expected Volatility	82.7 %	82.8 %
Expected Dividend Yield	0.0 %	0.0 %
Risk-Free Interest Rate	4.4 %	4.1 %
Weighted average grant date fair value per share	$ 5.73	$ 3.77

As of March 31, 2025, total unrecognized compensation cost related to unvested stock option awards granted under the Inducement Plan was $7,117, which is expected to be recognized over a weighted average period of 1.3 years.

Employee Stock Purchase Plan

The Company has reserved a total of 7,975 shares of common stock to be purchased under the ESPP, of which 4,841 shares remain available for purchase as of March 31, 2025. During the three months ended March 31, 2025 and March 31, 2024, the Company issued 202 and 251 shares under the ESPP, respectively.

Note 11 — Collaborative and Other Relationships

ORLADEYO

Torii Pharmaceutical Co., Ltd. ("Torii")

On November 5, 2019, the Company entered into a Commercialization and License Agreement with Torii (the "Original Torii Agreement"), granting Torii the exclusive right to commercialize ORLADEYO for the prevention of HAE attacks in Japan. Under the Original Torii Agreement, the Company received an upfront, non-refundable payment of $22,000. The Company received an additional milestone payment of $15,000 in the second quarter of 2021 upon receipt from the Japanese National Health Insurance System of a reimbursement price approval for ORLADEYO. In addition, the Company was entitled to receive tiered royalty payments, ranging from 20% to 40% of annual net sales of ORLADEYO in Japan during each calendar year. Torii's royalty payment obligations were subject to customary reductions in certain circumstances, but could not be reduced by more than 50% of the amount that otherwise would have been payable to the Company in the applicable calendar quarter.

The Company identified performance obligations under the Original Torii Agreement related to (i) the license to develop and commercialize ORLADEYO, (ii) regulatory approval support, and (iii) reimbursement pricing approval

support. These were each determined to be distinct from the other performance obligations. The Company allocated the $22,000 upfront consideration to the identified performance obligations using estimation approaches to determine the standalone selling prices under ASC Topic 606. Specifically, in determining the value related to the license, a valuation approach utilizing risk adjusted discounted cash flow projections was used, and an expected cost plus margin approach was utilized for the other performance obligations.

On November 30, 2023, the Company entered into an Amended and Restated Commercialization and License Agreement with Torii (as amended, the "Torii Agreement"). Under the Torii Agreement, the Company is entitled to receive tiered royalty payments, ranging from 20% to 80% of annual net sales of ORLADEYO in Japan during each calendar year. The Company is now responsible for all commercial promotion activities to support ORLADEYO sales in Japan, and Torii is responsible for HAE disease awareness activities in Japan. The Company will receive a 20% royalty on annual Japanese sales below a prespecified threshold and an 80% royalty on annual Japanese sales above the prespecified threshold.

Torii's updated royalty payment obligations commenced on November 30, 2023 and expire upon the later of (i) the tenth anniversary of the date of first commercial sale of ORLADEYO in Japan, (ii) the expiration of the Company's patents covering ORLADEYO, and (iii) the expiration of regulatory exclusivity for ORLADEYO in Japan.

The Company determined that the Torii Agreement represented a contract modification to be accounted for as if it were part of the Original Torii Agreement under ASC Topic 606. As the performance obligations under the Original Torii Agreement had been fully satisfied, the Company was not required to adjust revenue previously recognized.

Peramivir Injection (RAPIVAB, RAPIACTA, PERAMIFLU)

U.S. Department of Health and Human Services ("HHS")

In September 2024, the HHS awarded the Company up to a $69,388 contract for the procurement of up to 95.6 thousand doses over a five-year period of RAPIVAB (peramivir injection) for the treatment of influenza. The contract, awarded by the HHS Office of the Administration for Strategic Preparedness and Response ("ASPR"), will supply the Center for the Strategic National Stockpile, the nation's largest supply of life-saving pharmaceuticals and medical supplies for use in a public health emergency. The contract is structured with a 12-month base ordering period and four optional 12-month ordering periods, which the government can exercise on an annual basis. ASPR executed the first ordering period for $13,878 and the Company plans to supply 19.1 thousand doses to fulfill this option by September 29, 2025. The Company delivered 8.3 thousand doses of peramivir under this contract in the first quarter of 2025 and recorded revenue of $5,997 for the three months ended March 31, 2025. As the contract was entered into in September 2024, there were no doses delivered and no revenue recorded for the three months ended March 31, 2024.

Shionogi & Co., Ltd. ("Shionogi")

In February 2007, the Company entered into an exclusive license agreement with Shionogi to develop and commercialize peramivir in Japan for the treatment of seasonal and potentially life-threatening human influenza. Under the terms of the agreement, Shionogi obtained rights to injectable formulations of peramivir in Japan. In October 2008, the Company and Shionogi amended the license agreement to expand the territory covered by the agreement to include Taiwan. Shionogi has commercially launched peramivir under the commercial name RAPIACTA in Japan and Taiwan. The Company developed peramivir under a license from UAB and owes sublicense payments to UAB on any future milestone payments and/or royalties received by the Company from Shionogi.

Green Cross Corporation ("Green Cross")

In June 2006, the Company entered into an agreement with Green Cross to develop and commercialize peramivir in Korea. Under the terms of the agreement, Green Cross is responsible for all development, regulatory, and commercialization costs in Korea and the Company is entitled to share in profits resulting from the sale of peramivir in Korea, including the sale of peramivir to the Korean government for stockpiling purposes. Furthermore, Green Cross will pay the Company a premium over its cost to supply peramivir for development and any future marketing of peramivir products in Korea.

Other Collaborations

Clearside Biomedical, Inc. ("Clearside")

On November 3, 2023, the Company announced that it entered into a license agreement (the "Clearside Agreement") with Clearside, enabling the Company to develop its investigational plasma kallikrein inhibitor, avoralstat, with Clearside's SCS Microinjector® to deliver avoralstat to the back of the eye through the suprachoroidal space to treat patients with diabetic macular edema.

Under the Clearside Agreement, Clearside received a $5,000 upfront license fee from the Company, which was recognized in research and development expenses during the year ended December 31, 2023. Clearside is eligible to receive up to an additional $30,000 in clinical and regulatory milestone payments, and up to a total of $47,500 in three post-approval sales-based milestone payments as annual global net sales progress to $2,000,000. The Company will pay Clearside tiered mid-single digit royalties on annual global net product sales, at three tiers, including a top tier of >$1,500,000.

Note 12 — Segment Information

The Company operates as one reportable and operating segment, centered around its commercialized product, ORLADEYO, and its pipeline with the goal of developing first-in-class or best-in-class oral small-molecule and injectable protein therapeutics to target difficult-to-treat rare diseases. The determination of a single segment is consistent with the consolidated financial information regularly provided to the Company's chief operating decision maker ("CODM"). The Chief Executive Officer, as the CODM, uses consolidated, single-segment financial information for purposes of evaluating performance, making operating decisions, allocating resources, and planning and forecasting for future periods.

The CODM assesses performance and decides how to allocate resources based on consolidated net income (loss). This measure is used to monitor budget versus actual results to evaluate the performance of the segment. The CODM uses consolidated cash, cash equivalents and investments as the measure of segment assets. As of March 31, 2025 and December 31, 2024, the Company's cash, cash equivalents, and investments were $315,640 and $341,173, respectively.

The following table illustrates information about segment revenues, significant segment expenses, and segment net income (loss) for the three months ended March 31, 2025 and 2024 (in thousands):

	Three Months Ended March 31,	
	2025	2024
Revenues	$ 145,534	$ 92,761
Less[1]:		
Cost of product sales	4,568	1,265
Research and development		
Berotralstat	7,825	11,958
Factor D Program	1,489	10,480
BCX17725	7,308	7,759
Other research, preclinical and development costs	20,648	16,296
Selling, general and administrative	82,469	59,491
Foreign currency (gains) losses, net	(1)	51
Interest income	(3,024)	(4,031)
Interest expense	23,494	24,506
Income tax expense	726	365
Segment net income (loss)	**32**	**(35,379)**
Reconciliation of segment profit or loss:		
Adjustments and reconciling items	—	—
Consolidated net income (loss)	**$ 32**	**$ (35,379)**

[1] The significant expense categories and amounts align with the segment-level information that is regularly provided to the CODM.

All material long-lived assets of the Company reside in the U.S. For geographic information about the Company's product revenues, see "*Note 2—Revenue*".

Note 13 — Commitments and Contingencies

Abbreviated New Drug Application

In January 2025, the Company received a Paragraph IV notice of certification (the "Notice Letter") from Annora Pharma Private Limited ("Annora") advising that Annora has submitted an Abbreviated New Drug Application ("ANDA") to the FDA seeking approval to manufacture, use or sell a generic version of ORLADEYO in the United States prior to the expiration of three patents listed in the FDA's Orange Book: U.S. Patent Nos. 10,662,160; 11,117,867; and 11,618,733 (the "Challenged Patents"). The Notice Letter alleges that the Challenged Patents, which expire in 2039, are invalid, unenforceable and/or will not be infringed by the commercial manufacture, use or sale of the generic product described in Annora's ANDA. The Notice Letter does not challenge the following six ORLADEYO Orange Book patents that expire in 2035: U.S. Patent Nos. 10,125,102; 10,329,260; 10,689,346; 11,230,530; 11,708,333; and 12,116,346.

On March 10, 2025, the Company filed a patent infringement lawsuit in the United States District Court for the District of Delaware against Annora, Hetero Labs Limited, Hetero USA, Inc., and Camber Pharmaceuticals, Inc. (collectively, the "Defendants"), asserting infringement of the Challenged Patents arising from Annora's ANDA filing with the FDA. The Company is seeking, among other remedies, equitable relief enjoining the Defendants from infringing the Challenged Patents, as well as an order that the effective date of any FDA approval of the ANDA would be a date no earlier than the expiration of the Challenged Patents (including any regulatory extensions). While the Company intends to vigorously defend its intellectual property rights protecting ORLADEYO, this matter is in the early stages of litigation and no assessment can be made as to the likely outcome of this matter or whether it will be material to the Company. Accordingly, an estimate of the potential loss, or range of loss, if any, to the Company relating to this matter is not possible at this time.

Note 14 — Net Income (Loss) Per Share

Basic and diluted net income (loss) per share for the three months ended March 31, 2025 and 2024 were calculated as follows (in thousands, except per share amounts):

	Three Months Ended March 31,	
	2025	2024
Numerator:		
Net income (loss)	$ 32	$ (35,379)
Denominator:		
Weighted average shares of common stock outstanding: basic	208,882	206,064
Net income (loss) per common share: basic	$ 0.00	$ (0.17)
Effect of dilutive securities:		
Stock options to purchase common stock	4,401	—
Unvested restricted stock unit awards	1,961	—
Shares issuable under the employee stock purchase plan	17	—
Dilutive potential common shares	6,379	—
Weighted average shares of common stock outstanding: diluted	215,261	206,064
Net income (loss) per common share: diluted	$ 0.00	$ (0.17)

The Company's potentially dilutive securities include outstanding stock options, unvested restricted stock units and shares issuable under the employee stock purchase plan for the three months ended March 31, 2025 and 2024.

For the three months ended March 31, 2025, the dilutive effect of outstanding stock options, restricted stock unit awards, and shares issuable under the employee stock purchase plan was calculated using the treasury method, whereby all such awards are assumed to be exercised at the beginning of the period. The hypothetical proceeds from such exercises, including the average unrecognized stock compensation expense for outstanding stock options, restricted stock units and shares issuable under the employee stock purchase plan, were assumed to be used to purchase outstanding common stock at the average price during the period. The net share impact of dilutive securities was added to the weighted average basic common shares outstanding to calculate weighted average diluted shares outstanding.

For the three months ended March 31, 2024, during which the Company recorded a net loss, all potentially dilutive securities have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share, and thus they are considered "anti-dilutive." For this period, the weighted average number of shares of common stock outstanding used to calculate both basic and diluted net loss per share of common stock is the same.

The Company excluded the following potential common shares, presented based on amounts outstanding as of March 31, 2025 and March 31, 2024, from the computation of diluted net income (loss) per share attributable to common stockholders for the three months ended March 31, 2025 and 2024 because including them would have had an anti-dilutive effect:

	Three Months Ended March 31,	
	2025	2024
Outstanding stock options	30,555	39,580
Unvested restricted stock unit awards	1,422	6,124
Total	31,977	45,704

Note 15 — Subsequent Events

On April 18, 2025, as allowable under the Pharmakon Loan Agreement, the Company made a $75,000 partial prepayment on the outstanding principal amount under the Pharmakon Term Loan. In conjunction with the partial

prepayment, the Company incurred a $2,250 prepayment premium and paid $424 of interest accrued through the payment date.

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

The following Management's Discussion and Analysis ("MD&A") is intended to help the reader understand our results of operations and financial condition. This MD&A is provided as a supplement to, and should be read in conjunction with, our unaudited condensed consolidated financial statements and the accompanying notes to the financial statements and other disclosures included in this report (including the "Cautionary Note Regarding Forward-Looking Statements" at the beginning of this report and the "Risk Factors" section in Part II, Item 1A of this report).

Overview

We are a global biotechnology company with a deep commitment to improving the lives of people living with hereditary angioedema ("HAE") and other rare diseases. We leverage our expertise in structure-guided drug design with the goal of developing first-in-class or best-in-class oral small-molecule and injectable protein therapeutics to target difficult-to-treat rare diseases. In addition to our discovery and development efforts, our business strategy includes the successful commercialization of these drugs, as well as self-funding all of these efforts by achieving and increasing profitability. By focusing primarily on rare disease markets, we believe that we can more effectively control the costs of, and our strategic allocation of financial resources toward, post-approval commercialization.

Products and Product Candidates

ORLADEYO® (berotralstat)

ORLADEYO is an oral capsule, once-daily therapy discovered and developed by us for the prevention of HAE attacks. ORLADEYO is approved in the United States and other global markets for the prevention of HAE attacks in adults and pediatric patients 12 years and older. In addition, the ongoing APeX-P clinical trial, which is complete through the primary endpoint, is continuing to assess an oral granule formulation of ORLADEYO in pediatric patients who are 2 to 11 years of age.

We have built out our U.S. commercial infrastructure to support the launch and continued commercialization of ORLADEYO in the United States and are continuing to build our commercial infrastructure to support launches in other markets. Based on proprietary analyses of HAE prevalence and market research studies with HAE patients, physicians, and payors in the United States and Europe, and four full years of commercialization experience with ORLADEYO, we anticipate that the global commercial market for ORLADEYO has the potential to reach a global peak of $1 billion in annual net ORLADEYO revenues. We expect approximately 80 percent of our revenue at peak to come from the United States. These expectations are subject to numerous risks and uncertainties that may cause our actual results, performance, or achievements to be materially different. There can be no assurance that our commercialization methods and strategies will succeed, or that the market for ORLADEYO will develop in line with our current expectations. See "*Risk Factors—Risks Relating to Our Business—Risks Relating to Drug Development and Commercialization—There can be no assurance that our or our partners' commercialization efforts, methods, and strategies for our products or technologies will succeed, and our future revenue generation is uncertain*" in Part II, Item 1A of this report for further discussion of these risks.

Revenue from sales of ORLADEYO for the three months ended March 31, 2025 is discussed under "*Results of Operations*" in this MD&A. Revenue from sales of ORLADEYO in future periods is subject to uncertainties and will depend on several factors, including the success of our and our partners' commercialization efforts in the United States and elsewhere, the number of new patients switching to ORLADEYO, patient retention and demand, the number of physicians prescribing ORLADEYO, the rate of monthly prescriptions, reimbursement from third-party and government payors, the number of patients receiving free product, our pricing strategy, and market trends. We monitor and analyze this data on an ongoing basis as we continue to commercialize ORLADEYO. For example, during the three months ended March 31, 2025, we converted ORLADEYO patients from free product to paid during the prescription reauthorization period at a much faster rate than expected, on top of continued strong patient demand, generating higher than expected ORLADEYO revenue that we expect will continue through the full year.

BCX17725 (Netherton syndrome)

BCX17725 is a potent and selective investigational protein therapeutic KLK5 inhibitor designed to provide best-in-class, potentially disease-modifying, treatment for people with Netherton syndrome. Netherton syndrome is a serious, rare, lifelong genetic disorder causing disruption of the skin barrier with premature separation of the skin layers, chronic inflammation and vulnerability to serious infections, caused by lack of normal function of a natural inhibitor of KLK5. People with Netherton syndrome often have itchy, red, scaly, inflamed skin, fragile hair, and are more likely to develop severe food allergies, asthma and eczema. Netherton syndrome can be life-threatening, especially during infancy when

patients are vulnerable to dehydration and recurrent infections. Currently, there are no approved treatments that target the underlying cause of Netherton syndrome. BCX17725 is designed to replace missing functions of the natural KLK5 inhibitor, which could restore the normal skin barrier and result in improved skin function, including protection from severe inflammatory and infectious complications of the disease.

Avoralstat

We are developing our investigational plasma kallikrein inhibitor, avoralstat, with Clearside Biomedical, Inc.'s SCS Microinjector® to deliver avoralstat to the back of the eye through the suprachoroidal space to treat patients with diabetic macular edema ("DME"). DME is an important cause of vision loss in diabetes and is due to leakage of fluid from the blood vessels in the retina. While current treatments focus on vascular endothelial growth factor ("VEGF") inhibition, DME can develop from other mechanisms, such as the kallikrein-bradykinin pathway. This is supported by observations that many DME patients have an incomplete response to intravitreal anti-VEGF therapies that are administered every four to eight weeks. Avoralstat targets the kallikrein-bradykinin system on the retinal vascular endothelial cells and may result in less vascular leakage and less edema. Avoralstat, delivered to the suprachoroidal space, is designed to provide long-lasting exposure to the retinal vessels, which could result in less frequent injections and a reduced burden on patients and the healthcare system.

Complement Program

The goal of our overall complement program is to advance first-in-class and/or best-in-class compounds across multiple pathways in the complement system to treat complement-mediated diseases. We are pursuing oral medicines and protein therapeutics directed at targets across the classical, lectin, terminal, and alternative pathways of the complement system, including the therapies listed below.

Oral C5 Inhibitor. We are developing an oral C5 inhibitor that could be the first targeted oral therapy with competitive efficacy to currently-approved injected and infused anti-C5 therapies, such as eculizumab and ravulizumab. A drug with this profile could enable patients to switch from infused therapy and address their disease earlier in the treatment paradigm.

Oral C2 Inhibitor. We are developing a classical and lectin pathway complement inhibitor. An oral C2 inhibitor developed by us could be first-in-class and allow patients to switch from infused therapy and address their disease earlier in the treatment paradigm.

Bifunctional Complement Inhibitor. We are developing a bifunctional complement inhibitor anti-C2 monoclonal antibody that could be a first-in-class combined inhibitor of the classical, lectin and alternative pathways of the complement system to treat complex complement-mediated diseases that are influenced by multiple complement pathways.

RAPIVAB®/RAPIACTA®/PERAMIFLU® (peramivir injection)

RAPIVAB (peramivir injection) is approved in the United States for the treatment of acute uncomplicated influenza for patients six months and older. Since the 2009 H1N1 pandemic, RAPIVAB has been an important component of the U.S. Government's influenza preparedness efforts. Peramivir injection is also approved in Canada (RAPIVAB), Australia (RAPIVAB), Japan (RAPIACTA), Taiwan (RAPIACTA), and Korea (PERAMIFLU).

Revenues and Expenses

Our revenues are difficult to predict and depend on several factors, including those discussed in the "*Risk Factors*" section in Part II, Item 1A of this report. For example, our revenues depend, in part, on regulatory approval decisions for our products and product candidates, the effectiveness of our and our collaborative partners' commercialization efforts, market acceptance of our products, particularly ORLADEYO, and the resources dedicated to our products and product candidates by us and our collaborative partners, as well as entering into or modifying licensing agreements for our product candidates. Furthermore, revenues related to our collaborative development activities are dependent upon the progress toward, and the achievement of, developmental milestones by us or our collaborative partners.

Our operating expenses are also difficult to predict and depend on several factors, including research and development expenses, drug manufacturing, clinical research activities, the ongoing requirements of our development programs, the costs of commercialization, the availability of capital and direction from regulatory agencies, which are difficult to predict, and the factors discussed in the "*Risk Factors*" section in Part II, Item 1A of this report. Management may be able to control the timing and level of research and development and selling, general and administrative expenses,

but many of these expenditures will occur irrespective of our actions due to contractually committed activities and/or payments.

As a result of these factors, we believe that period-to-period comparisons are not necessarily meaningful, and you should not rely on them as an indication of future performance. Due to the foregoing factors, it is possible that our operating results will be below the expectations of market analysts and investors. In such event, the prevailing market price of our common stock could be materially adversely affected.

Recent Developments

ORLADEYO (berotralstat)

On February 12, 2025, we announced that Infarmed in Portugal has recommended ORLADEYO for the routine prevention of recurrent attacks of HAE in eligible patients 12 years and older. With this recommendation, ORLADEYO is now reimbursed in all major countries in Western Europe, except the Netherlands, which is expected in the first half of 2025.

On February 24, 2025, we announced that a new market tracking survey of 60 HAE treaters showed that 97 percent are considering prescribing ORLADEYO and 59 percent (up from 26 percent 18 months prior) of current prescribers indicate they are extremely likely to prescribe for more of their patients. In addition, we announced that additional real-world studies with ORLADEYO show statistically significant HAE attack rate reductions experienced by patients with C1-inhibitor deficiency and normal C1-inhibitor levels and function. Patient-reported outcomes also showed willingness to change long-term prophylaxis and improved treatment satisfaction across varying levels of attack frequency and severity after ORLADEYO initiation.

On February 24, 2025, we also announced that we are on track to submit a new drug application ("NDA") in 2025 to the U.S. Food and Drug Administration ("FDA") to expand the ORLADEYO label to children with HAE aged 2 to 11 using an oral granule formulation. In addition, we announced positive results from an interim analysis of the ongoing APeX-P clinical trial evaluating an oral granule formulation of ORLADEYO in pediatric patients with HAE aged 2 to 11.

On May 5, 2025, we announced that the percentage of U.S. HAE patients who describe a strong preference for an oral prophylaxis therapy increased to 70 percent, up from 50 percent in 2023, in our latest market survey of HAE patients.

On May 5, 2025, we announced that we have submitted an NDA to the FDA to expand the ORLADEYO label to children with HAE aged 2 to 11 using an oral granule formulation. We also expect to submit regulatory filings in 2025 in global territories, including Europe, Japan and Canada. ORLADEYO would be the first targeted oral prophylactic therapy for children with HAE.

BCX17725 (Netherton syndrome)

On February 24, 2025, we reaffirmed that BCX17725 has advanced into clinical trials and that we expect initial data from the program in 2025.

On May 5, 2025, we announced that the FDA has cleared our investigational new drug application, which will enable our clinical trial of BCX17725 to enroll patients in the United States. This phase 1 trial is also open in Australia. We also reaffirmed that we expect initial data from the program in 2025.

Avoralstat

On February 24, 2025, we reaffirmed our expectation to advance avoralstat into a clinical trial of patients with DME in 2025 and announced that initial clinical data from the avoralstat program is targeted by the end of 2025.

On May 5, 2025, we announced that the first clinical trial with suprachoroidal delivery of avoralstat has been granted authorization to proceed in Australia. We also reaffirmed that we expect initial data from patients with DME in 2025.

Pharmakon Loan Agreement

On April 18, 2025, we made a partial prepayment of $75.0 million of the outstanding principal amount under the Pharmakon Loan Agreement.

Results of Operations (three months ended March 31, 2025 compared to the three months ended March 31, 2024)

For the three months ended March 31, 2025, total revenues were $145.5 million compared to $92.8 million for the three months ended March 31, 2024. The increase in total revenues was due to a $45.4 million increase in ORLADEYO net revenue, excluding royalties, primarily due to an increase in direct sales of ORLADEYO due to both an increase in volume, driven by strong patient demand and an increase in the rate of paid shipments, and an increase in price. The increase in total revenues was also due to an increase in other revenues of $7.3 million, primarily due to an increase in direct sales of peramivir.

Cost of product sales for the three months ended March 31, 2025 and 2024 was $4.6 million and $1.3 million, respectively. The increase in cost of product sales was primarily due to the increase in peramivir and ORLADEYO sales.

The following table summarizes our research and development expenses for the periods indicated (in thousands). Certain prior period amounts have been reclassified for consistency with the current year presentation. These reclassifications had no effect on the total research and development expenses.

	Three Months Ended March 31,	
	2025	2024
Research and development expenses by program:		
Berotralstat	$ 7,825	$ 11,958
Factor D Program	1,489	10,480
BCX17725	7,308	7,759
Other research, preclinical and development costs	20,648	16,296
Total research and development expenses	$ 37,270	$ 46,493

Research and development expenses decreased to $37.3 million for the three months ended March 31, 2025 from $46.5 million for the three months ended March 31, 2024, primarily due to decreased expenses driven by the discontinuation and close-out of the Factor D programs, BCX10013 and BCX9930, the completion of the majority of Berotralstat clinical development activities in 2024, and a change in general and administrative expense allocations. These decreases were partially offset by an increase in other research, preclinical and development costs, comprised of avoralstat and other early-phase pipeline programs, primarily due to investigational new drug application-enabling activities. Further, there was an increase in stock-based compensation expense as a result of the retirement policy adopted in July 2024 and an increase in restricted stock unit awards outstanding.

Research and development expenses include all direct and indirect expenses and are allocated to specific programs at the point of development of a lead product candidate. Direct expenses are charged directly to the program to which they relate, and indirect expenses are allocated based upon internal direct labor hours dedicated to each respective program. Direct expenses consist of compensation for research and development personnel and costs of outside parties to conduct laboratory studies, develop manufacturing processes and manufacture the product candidates, and conduct and manage clinical trials, as well as other costs related to our clinical and preclinical studies. Additionally, direct expenses consist of those costs necessary to discontinue and close out a development program, including termination fees and other commitments. Indirect expenses consist of lab supplies and services, facility expenses, depreciation of development equipment and other overhead of our research and development efforts. Research and development expenses vary according to the number of programs in clinical development and the stage of development of our clinical programs. Later stage clinical programs tend to cost more than earlier stage programs due to the longer length of time of the clinical trials and the higher number of patients enrolled in these clinical trials.

Selling, general and administrative expenses for the three months ended March 31, 2025 were $82.5 million compared to $59.5 million for the three months ended March 31, 2024. This increase was primarily driven by an increase in commercial expenses to support our growing ORLADEYO revenue, our newly launched regions and expanded international operations, and global commercial support activities across finance, human resources, information technology, and supply chain. Further, there was an increase in stock-based compensation expense as a result of the retirement policy adopted in July 2024 and an increase in restricted stock unit awards outstanding, and an increase in general and administrative expenses resulting from a change in general and administrative expense allocations.

Interest expense for the three months ended March 31, 2025 was $23.5 million compared to $24.5 million for the three months ended March 31, 2024. Interest expense is primarily comprised of non-cash interest expense due to the amortization of interest associated with the royalty financing obligations and interest expense associated with the borrowings under the Pharmakon Loan Agreement (as defined below), including the amortization of the deferred financing costs, associated with the borrowings under the Pharmakon Loan. The decrease in interest expense was primarily due to the decrease in the effective interest rate related to the Pharmakon Loan Agreement.

For the three months ended March 31, 2025, interest income was $3.0 million compared to $4.0 million for the three months ended March 31, 2024. Net foreign currency gains were less than $0.1 million for the three months ended March 31, 2025 compared to losses of $0.1 million for the three months ended March 31, 2024.

For the three months ended March 31, 2025, income tax expense was $0.7 million compared to $0.4 million for the three months ended March 31, 2024. The increase in income tax expense was primarily driven by the increase in net income for the three months ended March 31, 2025 compared to the net loss incurred in the three months ended March 31, 2024.

Liquidity and Capital Resources

Sources of Liquidity

Our operations have principally been funded through public offerings and private placements of equity securities; our credit facilities; revenues from ORLADEYO; royalty financing transactions; and cash from collaborative and other research and development agreements, including U.S. Government contracts. In addition to the above, we have received funding from other sources, including other collaborative and other research and development agreements, government grants, research grants, and interest income on our investments.

On April 17, 2023, we entered into a $450.0 million Loan Agreement (the "Pharmakon Loan Agreement") with BioPharma Credit Investments V (Master) LP and BPCR Limited Partnership, as lenders, and BioPharma Credit PLC, as collateral agent for the lenders. The Pharmakon Loan Agreement provides for an initial term loan in the principal amount of $300.0 million (the "Tranche A Loan"), which was funded on April 17, 2023. We utilized the proceeds from the Tranche A Loan to repay the approximate $241.8 million of outstanding indebtedness under the then-existing credit facility with Athyrium Opportunities III Co-Invest 1 LP (the "Athyrium Credit Agreement") and to pay transaction costs and fees, and we used the remaining net proceeds of approximately $25.8 million for other general corporate purposes. The Pharmakon Loan Agreement also provided for three additional term loan tranches in principal amounts of $50.0 million each, which we could have requested, at our option, on or prior to September 30, 2024. We chose not to request any of the additional term loan tranches and the options have now expired. The maturity date of the Pharmakon Loan Agreement is April 17, 2028. On April 18, 2025, we made a partial prepayment of $75.0 million of the outstanding principal amount under the Pharmakon Loan Agreement.

The Pharmakon Loan Agreement contains representations and warranties and affirmative and negative covenants customary for financings of this type, as well as customary events of default. Certain of the customary negative covenants limit the ability of the Company and certain of its subsidiaries to, among other things, dispose of assets; engage in mergers, acquisitions, and similar transactions; incur additional indebtedness; grant liens; make investments; pay dividends or make distributions or certain other restricted payments in respect of equity; prepay other indebtedness; enter into restrictive agreements; undertake fundamental changes; or amend certain material contracts, among other customary covenants, in each case subject to certain exceptions. These covenants could cause us to be unable to pursue business opportunities that we or our stockholders may consider beneficial without the lenders' permission or without repaying all obligations outstanding under the Pharmakon Loan Agreement. A breach of any of these covenants could result in an event of default under the Pharmakon Loan Agreement. As of March 31, 2025, we were in compliance with the negative covenants under the Pharmakon Loan Agreement. See "*Note 7—Debt*" in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information about our obligations under the Pharmakon Loan Agreement.

In 2020 and 2021, we entered into the Royalty Purchase Agreements (as defined in "*Note 6—Royalty Financing Obligations*" in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report) with RPI 2019 Intermediate Finance Trust ("RPI") and OCM IP Healthcare Holdings Limited, an affiliate of OMERS Capital Markets ("OMERS"). Under the Royalty Purchase Agreements, RPI and OMERS are entitled to receive tiered, sales-based royalties on net product sales of ORLADEYO in the United States and certain key European markets (collectively, the "Key Territories"), and other markets where we sell ORLADEYO directly or through distributors. In addition, RPI and OMERS are entitled to receive a tiered revenue share on amounts generally received by us on account of ORLADEYO sublicense revenue or net sales by licensees outside of the Key Territories. Our required payments to OMERS commenced with the

calendar quarter beginning October 1, 2023. See "*Note 6—Royalty Financing Obligations*" in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information about these financing transactions.

Our principal sources of liquidity at March 31, 2025 were approximately $105.2 million in cash and cash equivalents and approximately $210.5 million in available-for-sale investments.

Cash Flows

The following table summarizes our cash flows for each period presented (in thousands):

	Three Months Ended March 31,	
	2025	**2024**
Net cash (used in) provided by:		
Operating activities	$ (27,517)	$ (53,684)
Investing activities	27,095	28,756
Financing activities	529	(1,048)
Effect of exchange rates on cash, cash equivalents and restricted cash	451	(340)
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 558	$ (26,316)

Operating Activities

During the three months ended March 31, 2025, net cash used in operating activities of $27.5 million consisted primarily of $62.1 million of changes in operating assets and liabilities, primarily due to a decrease in accounts payable and accrued expenses and an increase in receivables, partially offset by $34.6 million of non-cash items, including $14.1 million of non-cash interest expense and $21.4 million of stock-based compensation expense.

During the three months ended March 31, 2024, net cash used in operating activities of $53.7 million consisted primarily of a net loss of $35.4 million and $48.5 million of changes in operating assets and liabilities, primarily due to a decrease in accounts payable and accrued expenses and an increase in accounts receivable, partially offset by $30.2 million of non-cash items, including $19.4 million of non-cash interest expense and $13.7 million of stock-based compensation expense.

Investing Activities

During the three months ended March 31, 2025, net cash provided by investing activities of $27.1 million primarily related to maturities of investment securities, partially offset by purchases of investment securities.

During the three months ended March 31, 2024, net cash provided by investing activities of $28.8 million primarily related to maturities of investment securities, partially offset by purchases of investment securities.

Financing Activities

During the three months ended March 31, 2025, net cash provided by financing activities of $0.5 million primarily consisted of net proceeds from common stock issued under stock-based compensation plans, partially offset by withholding taxes paid on stock-based awards and principal payments on finance lease liabilities.

During the three months ended March 31, 2024, net cash used in financing activities of $1.0 million consisted of withholding taxes paid on stock-based awards and principal payments on finance lease liabilities, partially offset by net proceeds from common stock issued under stock-based compensation plans.

Plan of Operation and Future Funding Requirements

We intend to contain costs and cash flow requirements by closely managing our third-party costs and headcount, leasing scientific equipment and facilities, and contracting with other parties to conduct certain research and development projects. We may incur additional expenses, potentially resulting in significant losses, as we continue to pursue our research and development activities, commercialize ORLADEYO, and hire additional personnel. We may incur additional

expenses related to the filing, prosecution, maintenance, defense, and enforcement of patent and other intellectual property claims and additional regulatory costs as our clinical programs advance through later stages of development or as regulatory exclusivity for our products expires. The objective of our investment policy is to ensure the safety and preservation of invested funds, as well as to maintain liquidity sufficient to meet cash flow requirements. We place our excess cash with high credit quality financial institutions, commercial companies, and government agencies in order to limit the amount of our credit exposure. We have not realized any significant losses on our investments.

In the future, we may finance our needs principally from the following:

- our existing capital resources and interest earned on that capital;
- revenues from product sales;
- payments under current or future collaborative and licensing agreements with corporate partners;
- lease, royalty, or loan financing; and
- public or private equity and/or debt financing.

Our current and planned clinical trials, plus the related development, manufacturing, regulatory approval process requirements, and additional personnel resources and testing required for the continuing development of our product candidates and the commercialization of our products will consume significant capital resources and could increase our expenses.

Our expenses, revenues and cash utilization rate could vary significantly depending on many factors, including the development progress of our collaborative agreements for our product candidates, the amount of funding or assistance, if any, we receive from new partnerships with third parties for the development and/or commercialization of our products and product candidates, the amount and timing of funding we receive, if any, from U.S. Government contracts, the progress and results of our current and proposed clinical trials for our most advanced product candidates, the progress made in the manufacturing of our lead product candidates, the success of our commercialization efforts for, and market acceptance of, our products, and the overall progression of our other programs.

Based on our expectations for revenue and operating expenses, we believe our financial resources will be sufficient to fund our operations for at least the next 12 months. We have no immediate need to access the capital markets, and we did not draw down the additional debt available to us under the Pharmakon Loan Agreement. Our liquidity needs will be largely determined by the success of operations in regard to the successful commercialization of our products and the future progression of our product candidates. From time to time, we evaluate other opportunities to fund future operations, including: (1) out-licensing rights to certain of our products or product candidates, pursuant to which we would receive cash milestone payments; (2) royalty or other monetization transactions; (3) obtaining additional product candidate regulatory approvals, which would generate revenue, milestone payments and cash flow; (4) reducing spending on one or more research and development programs, including by discontinuing development; (5) restructuring operations to change our overhead structure; and/or (6) securing U.S. Government funding of our programs, including obtaining procurement contracts. We may, in the future, issue securities, including common stock, preferred stock, depositary shares, purchase contracts, warrants, debt securities, and units, through private placement transactions or registered public offerings. Our future liquidity needs, and our ability to address those needs, will largely be determined by the success of our products and product candidates; the timing, scope, and magnitude of our research and development and commercial expenses; and key developments and regulatory events and our decisions in the future.

Our long-term capital requirements and the adequacy of our available funds will depend upon many factors, including:

- sustained market acceptance of approved products and successful commercialization of such products by either us or our partners;
- our ability to perform under any government contracts and to receive reimbursement and stockpiling procurement contracts;
- the progress and magnitude of our research, drug discovery and development programs;
- changes in existing collaborative relationships;
- our ability to establish additional collaborative relationships with academic institutions, biotechnology or pharmaceutical companies, and governmental agencies or other third parties;
- the extent to which our partners will share in the costs associated with the development of our programs or run the development programs themselves;
- our ability to negotiate favorable development and marketing strategic alliances for certain products and product candidates;

- any decision to build or expand internal development and commercial capabilities;
- the scope and results of preclinical studies and clinical trials to identify and develop product candidates;
- our ability to engage sites and enroll subjects in our clinical trials;
- the scope of manufacturing of our products to support our commercial operations and of our product candidates to support our preclinical research and clinical trials;
- increases in personnel and related costs to support the development and commercialization of our products and product candidates;
- the scope of manufacturing of our drug substance and product candidates required for future NDA filings;
- competitive and technological advances;
- the time and costs involved in obtaining regulatory approvals;
- post-approval commitments for ORLADEYO, peramivir, and other products that receive regulatory approval; and
- the costs involved in all aspects of intellectual property strategy and protection, including the costs involved in preparing, filing, prosecuting, maintaining, defending, and enforcing patent claims.

We may, in the future, be required to raise additional capital to complete the development and commercialization of our products and product candidates, and we may seek to raise capital in the future, including to take advantage of favorable opportunities in the capital markets. Additional funding may not be available when needed or in the form or on terms acceptable to us. Our future working capital requirements, including the need for additional working capital, will largely be determined by the advancement of our portfolio of product candidates and the commercialization of ORLADEYO. More specifically, our working capital requirements will be dependent on the number, magnitude, scope and timing of our development programs; regulatory approval of our product candidates; obtaining funding from collaborative partners; the cost, timing and outcome of regulatory reviews, regulatory investigations, and changes in regulatory requirements; the costs of obtaining patent protection for our product candidates; the timing and terms of business development activities; the rate of technological advances relevant to our operations; the efficiency of manufacturing processes developed on our behalf by third parties; the timing, scope and magnitude of commercial spending; and the level of required administrative support for our daily operations. See "*Risk Factors—Risks Relating to Our Business—Financial and Liquidity Risks*" and "*Risk Factors—Risks Relating to Our Business—Risks Relating to Drug Development and Commercialization—If we fail to obtain additional financing or acceptable partnership arrangements if and when needed, we may be unable to complete the development and commercialization of our products and product candidates or continue operations*" in Part II, Item 1A of this report for further discussion of the risks related to obtaining additional capital.

Critical Accounting Estimates

We have established various accounting policies that govern the application of U.S. GAAP, which were utilized in the preparation of our condensed consolidated financial statements. Certain accounting policies involve significant judgments and assumptions by management that have or are reasonably likely to have a material impact on the carrying value of certain assets and liabilities. Management considers such accounting policies to be critical accounting policies. The judgments and assumptions used by management are based on historical experience and other factors, which are believed to be reasonable under the circumstances. Because of the nature of the judgments and assumptions made by management, actual results could differ from these judgments and estimates, which could have a material impact on the carrying values of assets and liabilities and the results of operations.

While our significant accounting policies are more fully described in "*Note 1—Significant Accounting Policies and Concentrations of Risk*" in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our reported financial results and affect the more significant judgments and estimates that we use in the preparation of our financial statements.

Revenue Recognition

Pursuant to Accounting Standards Codification ("ASC") Topic 606, we recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To achieve this core principle, Topic 606 includes provisions within a five-step model that includes (i) identifying the contract with a customer, (ii) identifying the performance obligations in the contract, (iii) determining the transaction price, (iv) allocating the transaction price to the performance obligations, and (v) recognizing revenue when, or as, an entity satisfies a performance obligation.

At contract inception, we identify the goods or services promised within each contract, assess whether each promised good or service is distinct, and determine those that are performance obligations. We recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when the performance obligation is satisfied.

Product Sales, Net

Our principal sources of product sales are sales of ORLADEYO, which we began shipping to patients in December 2020, and sales of peramivir. In the United States, we generally ship ORLADEYO directly to patients through a single specialty pharmacy, which is considered our customer. Outside the United States, we sell ORLADEYO to specialty distributors and to hospitals and pharmacies, which collectively are considered our customers.

We recognize revenue for sales when the customer obtains control of the product, which generally occurs upon delivery.

Net revenue from sales of ORLADEYO is recorded at net selling price (transaction price), which includes reserves for variable consideration such as (i) estimated government rebates, such as Medicaid and Medicare Part D reimbursements, and estimated managed care rebates, (ii) estimated chargebacks, (iii) estimated costs of co-payment assistance programs, and (iv) product returns. These reserves, representing our best estimates of the amount of consideration to which we are entitled based on the terms of the applicable contracts and statutory requirements, are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable if no payments are required of us or a current liability if a payment is required of us. Actual amounts of consideration may differ from our estimates. If actual results vary from estimates, these estimates are adjusted, which would affect net product revenue and earnings in the period such variances become known.

Government and Managed Care Rebates. We contract with government agencies and managed care organizations or, collectively, third-party payors, so that ORLADEYO will be eligible for purchase by, or partial or full reimbursement from, such third-party payors. We estimate the rebates we will provide to third-party payors and deduct these estimated amounts from total gross product revenues at the time the revenues are recognized, resulting in a reduction of product revenue and the establishment of a current liability. We estimate the rebates that we will provide to third-party payors based upon (i) our contracts with these third-party payors, (ii) the government mandated discounts applicable to government-funded programs, and (iii) product distribution information obtained from our specialty pharmacy regarding payor mix.

Chargebacks. Chargebacks are discounts that occur when certain contracted customers, pharmacy benefit managers, insurance companies, and government programs purchase directly from our specialty pharmacy. These customers purchase our product under contracts negotiated between them and our specialty pharmacy. The specialty pharmacy, in turn, charges back to us the difference between the price that the specialty pharmacy paid and the negotiated price paid by the contracted customers, which may be higher or lower than the specialty pharmacy's purchase price with us. We estimate chargebacks and adjust gross product revenues and establish a current liability at the time revenues are recognized.

Co-payment assistance and patient assistance programs. Patients who have commercial insurance and meet certain eligibility requirements may receive co-payment assistance. Based upon the terms of the program and co-payment assistance utilization reports received from the specialty pharmacy, we estimate the co-payment assistance amounts, which are recorded in the same period in which the related revenue is recognized, resulting in a reduction of product revenue and establishment of a current liability. We also offer a patient assistance program that provides free drug product, for a limited period of time, to allow a patient's insurance coverage to be established. Based on patient assistance program utilization reports provided by the specialty pharmacy, we record gross revenue of the product provided and a full reduction of the revenue amount for the free drug discount.

Product returns. We do not provide contractual return rights to our customers, except in instances where the product is damaged or defective. Non-acceptance by the patient of shipped drug product by the specialty pharmacy is reflected as a reversal of sales in the period in which the sales were originally recorded. Reserves for estimated non-acceptances by patients are recorded as a reduction of revenue in the period that the related revenue is recognized, as well as a reduction to accounts receivable. Estimates of non-acceptance are based on quantitative information provided by the specialty pharmacy.

Collaborative and Other Revenues

We have collaboration and license agreements with a number of third parties. Our primary sources of revenue from these collaborative and other research and development arrangements are license, service and royalty revenues.

Revenue from license fees, royalty payments, milestone payments, and research and development fees are recognized as revenue when the earnings process is complete and we have no further continuing performance obligations or we have completed the performance obligations under the terms of the agreement.

Arrangements that involve the delivery of more than one performance obligation are initially evaluated as to whether the intellectual property licenses granted by us represent distinct performance obligations. If they are determined to be distinct, the value of the intellectual property licenses would be recognized up front while the research and development service fees would be recognized as the performance obligations are satisfied. For performance obligations based on services performed, we measure progress using an input method based on the effort we expend or costs we incur toward the satisfaction of the performance obligation in relation to the total estimated effort or costs. Variable consideration is assessed at each reporting period as to whether it is not subject to significant future reversal and, therefore, should be included in the transaction price at the inception of the contract. If a contract includes a fixed or minimum amount of research and development support, this also would be included in the transaction price. Changes to collaborations, such as the extensions of the research term or increasing the number of targets or technology covered under an existing agreement, are assessed for whether they represent a modification or should be accounted for as a new contract. For contracts with multiple performance obligations, revenue is allocated to each performance obligation based on its relative standalone selling price. Standalone selling prices are based on observable prices at which we separately sell the products or services. If a standalone selling price is not directly observable, then we estimate the standalone selling price using either an adjusted market assessment approach or an expected cost plus margin approach, representing the amount that we believe the market is willing to pay for the product or service. Analyzing the arrangement to identify performance obligations requires the use of judgment, and each may be an obligation to deliver services, a right or license to use an asset, or another performance obligation.

Under certain of our license agreements, we receive royalty payments based upon our licensees' net sales of covered products. Royalties are recognized at the later of when (i) the subsequent sale or usage occurs, or (ii) the performance obligation to which some or all of the sales-based or usage-based royalty has been satisfied.

Inventory

Our inventory primarily relates to ORLADEYO. Additionally, our inventory includes peramivir.

We value our inventory at the lower of cost or estimated net realizable value. We determine the cost of our inventory on a first-in, first-out (FIFO) basis. Raw materials and work-in-process include all inventory costs prior to packaging and labeling, including raw material, active product ingredient, and the drug product. Finished goods include packaged and labeled products. We classify inventory as long-term when consumption or sale of the inventory is not expected to occur within 12 months from the balance sheet date.

Our inventory is subject to expiration dating. At each reporting date, we evaluate the carrying value of our inventory and provide valuation reserves for any estimated excess, obsolete, short-dated or unmarketable inventory. In addition, we may experience spoilage of our raw materials and supplies. Our determination that a valuation reserve might be required, in addition to the quantification of such reserve, requires us to utilize significant judgment. Additionally, our inventory is subject to strict quality control and monitoring that is performed throughout the manufacturing process, including release of work-in-process to finished goods. In the event that certain batches or units of product do not meet quality specifications, we will record a write-down of any potential unmarketable inventory to its estimated net realizable value and record the expense as cost of product in the Condensed Consolidated Statements of Comprehensive Income (Loss).

Prior to obtaining initial regulatory approval for an investigational product candidate, we expense costs relating to production of pre-launch inventory as research and development expense in our Condensed Consolidated Statements of Comprehensive Income (Loss) in the period incurred. After regulatory approval has been received, we capitalize inventory costs.

Research and Development Expenses and Related Accruals

Research and development expenses include, among other items, personnel costs, including salaries and benefits, manufacturing costs, clinical, regulatory, and toxicology services performed by clinical research organizations ("CROs"),

materials and supplies, and overhead allocations consisting of various administrative and facilities related costs, as well as termination fees and other commitments associated with discontinued programs. Most of our manufacturing and clinical and preclinical studies are performed by third-party CROs. Our research and development costs are charged to expense when incurred. Research and development expenses include all direct and indirect development costs related to the development of our portfolio of product candidates.

Additionally, we have license agreements with third parties, such as Albert Einstein College of Medicine of Yeshiva University, Industrial Research, Ltd., and the University of Alabama at Birmingham ("UAB"), which require fees related to sublicense agreements. We accrue sublicense expenses as incurred.

We group our research and development expenses into two major categories: direct expenses and indirect expenses. Direct expenses consist of compensation for research and development personnel and costs of outside parties to conduct laboratory studies, develop manufacturing processes and manufacture the product candidate, conduct and manage clinical trials, as well as other costs related to our clinical and preclinical studies. Additionally, direct expenses consist of those costs necessary to discontinue and close out a development program, including termination fees and other commitments. These costs are accumulated and tracked by active program. Indirect expenses consist of lab supplies and services, facility expenses, depreciation of development equipment and other overhead of our research and development efforts. These costs apply to our discovery research efforts.

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 open contracts and purchase orders, communicating with applicable Company personnel to identify services that have been performed on its behalf and estimating the actual work completed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. When evaluating the adequacy of accrued expenses, we consider facts and circumstances known to us at the time, which can include assumptions such as expected patient enrollment, site activation and estimated project duration. Examples of estimated accrued research and development expenses include (i) fees paid to CROs in connection with preclinical and toxicology studies and clinical trials, (ii) fees paid to investigative sites in connection with clinical trials, (iii) fees paid to contract manufacturers in connection with the production of our raw materials, drug substance, drug products, and product candidates, and (iv) professional fees.

The financial terms of our agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of milestones. We record liabilities under these contractual commitments when we determine an obligation has been incurred, regardless of the timing of the invoice. In expensing service fees, we estimate the time period over which services will be performed and the level of effort expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we will adjust the accrual accordingly. If we do not identify costs that we have begun to incur or if we underestimate or overestimate the level of these costs, our actual expenses could differ from our estimates.

Stock-Based Compensation

All share-based payments, including grants of stock option awards and restricted stock unit awards, are recognized in our Condensed Consolidated Statements of Comprehensive Income (Loss) based on their fair values. Stock-based compensation cost is estimated at the grant date based on the fair value of the award and is recognized as expense on a straight-line basis over the requisite service period of the award. Determining the appropriate fair value model and the related assumptions for the model requires judgment, including estimating the stock price volatility, and the expected term. We utilize the Black-Scholes option-pricing model to value our stock option awards and recognize compensation expense on a straight-line basis over the requisite service period. We reduce stock-based compensation expense for estimated forfeitures. The estimation of share-based payment awards that will ultimately vest requires judgment, and to the extent actual results or updated estimates differ from our current estimates, such amounts will be recorded as a cumulative adjustment in the period estimates are revised. In addition, we have outstanding performance-based restricted stock unit awards for which no compensation expense is recognized until it is probable that the performance condition will be achieved. Actual results, and future changes in estimates, may differ substantially from our current estimates.

Interest Expense and Royalty Financing Obligations

The royalty financing obligations are eligible to be repaid based on royalties from net sales of ORLADEYO. Interest expense is accrued using the effective interest rate method over the estimated period each of the related liabilities will be

paid. This requires us to estimate the total amount of future royalty payments to be generated from product sales over the life of the agreement. We impute interest on the carrying value of each of the royalty financing obligations and record interest expense using an imputed effective interest rate. We reassess the expected royalty payments each reporting period and account for any changes through an adjustment to the effective interest rate on a prospective basis. The assumptions used in determining the expected repayment term of the debt and amortization period of the issuance costs requires that we make estimates that could impact the carrying value of each of the liabilities, as well as the periods over which associated issuance costs will be amortized. A significant increase or decrease in forecasted net sales could materially impact each of the liability balances, interest expense and the time periods for repayment.

Income Taxes

The liability method is used in our accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on differences between financial reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that are expected to be in effect when the differences are expected to reverse.

Significant management judgment is required in determining our provision for income taxes, deferred tax assets and liabilities and any valuation allowance recorded against net deferred tax assets. We have recorded a valuation allowance against substantially all potential tax assets, due to uncertainties in our ability to utilize deferred tax assets, primarily consisting of certain net operating losses carried forward, before they expire. The valuation allowance is based on estimates of future earnings in each of the jurisdictions in which we operate and the period over which our deferred tax assets will be recoverable.

We account for uncertain tax positions in accordance with U.S. GAAP. Uncertain tax positions are recorded based upon certain recognition and measurement criteria. We re-evaluate uncertain tax positions and consider various factors, including, but not limited to, changes in tax law and the measurement of tax positions taken or expected to be taken in tax returns. We adjust the amount of the liability to reflect any subsequent changes in the relevant facts and circumstances surrounding the uncertain tax positions. We recognize interest and penalties related to income tax matters in income tax expense.

Recent Accounting Pronouncements

"*Note 1—Significant Accounting Policies and Concentrations of Risk*" in the Notes to Condensed Consolidated Financial Statements included in Part I, Item 1 of this report discusses accounting pronouncements recently issued or proposed but not yet required to be adopted.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk

We are subject to interest rate risk on our investment portfolio and borrowings under our Pharmakon Loan Agreement. The Tranche A Loan under the Pharmakon Loan Agreement accrues interest each quarter at a rate equal to the three-month Secured Overnight Financing Rate ("SOFR"), which is capped to be no less than 1.75%, plus 7.00% or, for each quarterly interest period in which a Pharmakon PIK Interest Payment (as defined in "*Note 7—Debt*" in the Notes to the Condensed Consolidated Financial Statements included in Part I, Item 1 of this report) was made, SOFR plus 7.25%. Accordingly, increases in interest rates will increase the associated interest payments that we are required to make on the Tranche A Loan. For the three months ended March 31, 2025, interest was accrued at an effective rate of 12.31% on the $300.0 million borrowing under the Pharmakon Loan Agreement.

We invest in marketable securities in accordance with our investment policy. The primary objectives of our investment policy are to preserve capital, maintain proper liquidity to meet operating needs and earn a competitive level of return. Our investment policy specifies credit quality standards for our investments and limits the amount of credit exposure to any single issue, issuer or type of investment. We place our excess cash with high credit quality financial institutions, commercial companies, and government agencies in order to limit the amount of credit exposure. Some of the securities we invest in may have market risk. This means that a change in prevailing interest rates may cause the principal amount of the investment to fluctuate.

Our investment exposure to market risk for changes in interest rates relates to the increase or decrease in the amount of interest income we can earn on our portfolio, changes in the market value due to changes in interest rates and other market factors, as well as the increase or decrease in any realized gains and losses. Our investment portfolio includes only marketable securities and instruments with active secondary or resale markets to help ensure portfolio liquidity. A

hypothetical 100 basis point increase or decrease in interest rates along the entire interest rate yield curve would not significantly affect the fair value of our interest sensitive financial instruments, including our borrowings, but may affect our future earnings and cash flows. We generally have the ability to hold our fixed-income investments to maturity and, therefore, do not expect that our operating results, financial position or cash flows will be materially impacted due to a sudden change in interest rates. However, our future investment income may fall short of expectations due to changes in interest rates, or we may suffer losses in principal if forced to sell securities which have declined in market value due to changes in interest rates or other factors, such as changes in credit risk related to the securities' issuers. To minimize this risk, we schedule our investments to have maturities that coincide with our expected cash flow needs, thus avoiding the need to redeem an investment prior to its maturity date. Accordingly, we do not believe that we have material exposure to interest rate risk arising from our investments. Generally, our investments are not collateralized. We have not realized any significant losses from our investments.

We do not use interest rate derivative instruments to manage exposure to interest rate changes. We ensure the safety and preservation of invested principal funds by limiting default risk, market risk and reinvestment risk. We reduce default risk by investing exclusively in investment grade securities.

Foreign Currency Risk

Most of our revenues and expenses are denominated in U.S. dollars. Our commercial sales in Europe are primarily denominated in Euros and the British Pound, and our royalties from Torii are in Japanese Yen. We also had other transactions denominated in foreign currencies during the three months ended March 31, 2025, primarily related to operations in Europe, contract manufacturing and ex-U.S. clinical trial activities, and we expect to continue to do so. Our limited foreign currency exposure relative to our European operations is to fluctuations in the Euro, British Pound, Swiss Franc, Danish Krone, Swedish Krona, and Norwegian Krone. Additionally, we have operations in Canada and have foreign currency exposure relative to the Canadian Dollar.

We do not anticipate that foreign currency transaction gains or losses will be significant at our current level of operations. However, transaction gains or losses may become significant in the future as we continue to expand our operations internationally. We have not engaged in foreign currency hedging during the three months ended March 31, 2025; however, we may do so in the future.

Inflation Risk

Inflation generally impacts us by potentially increasing our operating expenses, including cost of product sales, clinical trial costs and selling activities. We do not believe that inflation has had a material impact on our business or results of operations during the periods for which the condensed consolidated financial statements are presented in this report. Significant adverse changes in inflation could negatively impact our future results of operations.

Item 4. Controls and Procedures

We maintain a set of disclosure controls and procedures that are designed to ensure that information relating to the Company required to be disclosed in our periodic filings under the Exchange Act is recorded, processed, summarized and reported in a timely manner under the Exchange Act. We carried out an evaluation, under the supervision and with the participation of management, including our Chief Executive Officer and Interim Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures. Based upon that evaluation, the Chief Executive Officer and Interim Chief Financial Officer concluded that, as of March 31, 2025, the Company's disclosure controls and procedures are effective to ensure that information required to be disclosed by the Company in the reports filed or submitted by it under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and include controls and procedures designed to ensure that information required to be disclosed by the Company in such reports is accumulated and communicated to the Company's management, including the Chief Executive Officer and Interim Chief Financial Officer of the Company, as appropriate to allow timely decisions regarding required disclosure.

There have been no changes in our internal control over financial reporting that occurred during the three months ended March 31, 2025 that have materially affected, or are reasonably likely to materially affect, the Company's internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

As previously disclosed, in January 2025, we received a Paragraph IV notice of certification (the "Notice Letter") from Annora Pharma Private Limited ("Annora") advising that Annora has submitted an Abbreviated New Drug Application ("ANDA") to the FDA seeking approval to manufacture, use or sell a generic version of ORLADEYO in the United States prior to the expiration of three patents listed in the FDA's Orange Book: U.S. Patent Nos. 10,662,160; 11,117,867; and 11,618,733 (the "Challenged Patents"). The Notice Letter alleges that the Challenged Patents, which expire in 2039, are invalid, unenforceable and/or will not be infringed by the commercial manufacture, use or sale of the generic product described in Annora's ANDA. The Notice Letter does not challenge the following six ORLADEYO Orange Book patents that expire in 2035: U.S. Patent Nos. 10,125,102; 10,329,260; 10,689,346; 11,230,530; 11,708,333; and 12,116,346.

On March 10, 2025, we filed a patent infringement lawsuit in the United States District Court for the District of Delaware against Annora, Hetero Labs Limited, Hetero USA, Inc., and Camber Pharmaceuticals, Inc. (collectively, the "Defendants"), asserting infringement of the Challenged Patents arising from Annora's ANDA filing with the FDA. We are seeking, among other remedies, equitable relief enjoining the Defendants from infringing the Challenged Patents, as well as an order that the effective date of any FDA approval of the ANDA would be a date no earlier than the expiration of the Challenged Patents (including any regulatory extensions). We intend to vigorously defend our intellectual property rights protecting ORLADEYO.

Item 1A. Risk Factors

An investment in our stock involves risks. You should carefully read this entire report and consider the following uncertainties and risks, which may adversely affect our business, financial condition or results of operations, along with all of the other information included in our other filings with the SEC, before making an investment decision regarding our common stock. Additionally, while some of the factors, events and contingencies described herein may have occurred in the past, the disclosures herein are not representations as to whether or not they have occurred and are instead provided because future occurrences thereof could adversely affect the Company.

Risks Relating to Our Business

Financial and Liquidity Risks

We may never achieve sustained profitability.

Since our inception, we have not achieved sustained profitability. Our expectations as to when we may achieve sustained profitability may change based upon our ability to execute our commercialization goals and operational initiatives and whether or not the assumptions underlying our projected revenues and expenses are correct. Our beliefs and projections regarding the attainment of our financial goals may differ from actual results based on market factors like competition, patient and physician acceptance of our products, reimbursement levels, or on our ability to execute our operational and budget plans, including management's ability to properly forecast our capital allocation needs. To achieve and maintain profitability, we, or our collaborative partners, must successfully manufacture and develop products and product candidates, receive regulatory approvals, and successfully commercialize our products and/or enter into profitable commercialization arrangements with other parties. Even if we are able to successfully commercialize our existing products, or to develop or otherwise acquire new commercially viable products, certain obligations we have to third parties, including, without limitation, our obligation to pay RPI and OMERS, as applicable, royalties on certain revenues from ORLADEYO under the Royalty Purchase Agreements (as defined in "*Note 6— Royalty Financing Obligations*" in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report), may reduce the profitability of such products.

Because of the numerous risks and uncertainties associated with developing our product candidates, launching new products, and their potential for commercialization, we are unable to predict the extent of any future losses. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. If we are unable to achieve and sustain profitability on our anticipated timeline, or at all, the market value of our common stock will likely decline.

We may need to raise additional capital in the future. If we are unable to raise capital if and when needed, we may need to adjust our operations.

We have sustained operating losses for the majority of our corporate history. Even if we are able to achieve sustained profitability, in order to continue future operations, progress our drug discovery and development programs, and commercialize our current products and product candidates, we may be required to raise additional capital in the future. In addition to seeking strategic partnerships and transactions, we may access the equity or debt markets, incur additional borrowings, pursue royalty or other monetization transactions, or seek other sources of funding to meet liquidity needs at any time, including to take advantage of attractive opportunities in the capital markets. Additional funding, whether through additional sales of securities, additional borrowings, royalty or other monetization transactions, collaborative arrangements with partners, or from other sources, may not be available if or when needed or in a form or on terms acceptable to us. The issuance of preferred or common stock or convertible securities, with terms and prices significantly more favorable than those of our currently outstanding common stock, could have the effect of diluting or adversely affecting the holdings or rights of our existing stockholders. Additional borrowings may subject us to more restrictive covenants than are currently applicable to us under the Pharmakon Loan Agreement (as defined below). In addition, collaborative arrangements may require us to transfer certain material rights to our corporate partners. Insufficient funds or lack of an acceptable partnership may require us to delay, scale-back or eliminate certain of our research and development programs. See "*Risks Relating to Our Business—Risks Relating to Drug Development and Commercialization—If we fail to obtain additional financing or acceptable partnership arrangements if and when needed, we may be unable to complete the development and commercialization of our products and product candidates or continue operations*" in this section for further discussion of the capital requirements for our development and commercialization efforts.

Our liquidity needs will largely be determined by the success of operations in regard to the commercialization of our products, particularly ORLADEYO, the progression of our product candidates in the future, and our ability to execute our budget plans. Our current plans for managing our liquidity needs primarily include controlling the timing and spending on our research and development programs and commercializing our approved products. See "*Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources*" in Part I, Item 2 of this report for additional information about our liquidity needs, capital requirements, potential funding alternatives, and adequacy of available funds.

There can be no assurance that any of our plans will be successful or that additional capital will be available to us on reasonable terms, or at all, if needed. If we are unable to obtain sufficient additional capital if and when needed, we may be forced to adjust or curtail our operations; delay, reduce, or stop ongoing clinical trials or commercialization efforts; cease operations altogether; or file for bankruptcy.

Risks Relating to Drug Development and Commercialization

Our success depends upon our ability to manage our product candidate pipeline, advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive regulatory approvals for the commercial sale of our product candidates.

The success of our business depends upon our ability to manage our product candidate pipeline, including through expanding the pipeline, as appropriate, through our internal identification and discovery of product candidates or otherwise in-licensing or acquiring products or product candidates and integrating them into our business effectively and efficiently; advancing our product candidates through the various stages of development; and receiving regulatory approvals for the commercial sale of our product candidates. Identifying, selecting, and in-licensing or acquiring products or product candidates requires substantial expense and technical and financial expertise, and if we are unable to effectively manage our pipeline or integrate viable products or product candidates into our business on acceptable terms, or at all, our business and drug development efforts could suffer.

To receive the regulatory approvals necessary for the commercial sale of our product candidates, we or our partners must demonstrate through preclinical studies and clinical trials that each product candidate is safe and effective. The development process and related regulatory process are complex and uncertain. The preclinical and clinical development of our product candidates is susceptible to the risk of failure inherent at any stage of drug development, including failure to demonstrate efficacy and safety, failure to demonstrate adequate benefit-risk balance, failure to achieve a commercially attractive and competitive product label, failure to achieve approval in commercially attractive indications, the occurrence of adverse events that are severe or medically or commercially unacceptable, our or our partners' failure to comply with trial protocols, applicable regulatory requirements, or industry standards, or a determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or be approved in accordance with our

development plans or at all. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. For example, any successful results of our preclinical and early clinical work for avoralstat, BCX17725 and our early-stage discovery programs do not guarantee the success of later clinical trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and for some product candidates, there may not be an ideal model for preclinical testing. We also cannot guarantee that any preclinical studies and clinical trials will be conducted as planned or completed on schedule, if at all, or that the results of such trials will be sufficient to support regulatory approval for our product candidates.

Progression of our product candidates through the clinical development process is dependent upon our trials indicating that our product candidates have adequate safety and efficacy in the patients being treated by achieving predetermined safety and efficacy endpoints according to the clinical trial protocols, as well as an adequate benefit-risk profile. Failure to achieve any of these endpoints or to show adequate benefit-risk profile in any of our programs (including the therapies in our pipeline described in "*Management's Discussion and Analysis of Financial Condition and Results of Operations—Overview—Products and Product Candidates*" in Part I, Item 2 of this report), has in the past, and could again in the future, result in delays in, modifications to, or discontinuations of our trials or require the performance of additional unplanned trials. If any of our product candidates is associated with adverse events or undesirable side effects or has properties that are unexpected, we may need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a benefit-risk perspective. Product candidates that initially show promise in clinical or preclinical testing could later be found to be associated with or to cause undesirable or unexpected side effects that could result in substantial modifications or delays in the development plans for our product candidates, significant unexpected costs, or the termination of programs.

In addition, the development plans for our product candidates, including our clinical trials, may not be adequately designed or executed, which could negatively affect the outcome and analysis of study results. Because of the cost and duration of clinical trials, we have decided in the past, and may in the future decide, to discontinue development of product candidates for various reasons, including, but not limited to, that they are unlikely to show favorable results in clinical trials, unlikely to help advance a product to the point of a meaningful collaboration, or unlikely to have reasonable commercial potential.

Undesirable or inconclusive data in our preclinical studies and clinical trials or side effects in humans could result in the FDA or foreign regulatory authorities (including, e.g., the European Medicines Agency ("EMA"), the Ministry of Health, Labor and Welfare ("MHLW") in Japan, or the United Kingdom's Medicines and Healthcare products Regulatory Agency ("MHRA")) refusing to approve a product candidate for any targeted indications or imposing restrictions or warnings that could impact development or the ultimate commercial viability of a product candidate. In addition, the FDA or foreign regulatory authorities may determine that study data from our product candidates necessitates additional studies or study designs which differ from our planned development strategy, and such regulatory authorities may also require patient monitoring and testing or may implement restrictions or other conditions on our development activities, any of which could materially impact the cost and timing of our planned development strategy. We, our partners, the FDA, or foreign regulatory authorities have previously, and may again in the future, pause enrollment in, suspend, or terminate clinical trials at any time if we or they believe the trial participants face unacceptable health risks.

Our ability to complete the clinical development process successfully is dependent upon many factors, including, but not limited to:

- our or our partners' ability to secure suitable clinical sites and investigators and to enroll and maintain an adequate number of patients on a timely basis or at all;
- patients that enroll in a clinical trial may not comply with the clinical trial protocols or maintain contact with investigators to provide complete data during and after treatment;
- our product candidates may not prove to be either safe or effective for our targeted indications, or at all, or may produce unfavorable or inconclusive results;
- we or our partners may decide, or be required by regulatory authorities, to pause enrollment in, suspend, or terminate clinical research for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate, noncompliance with regulatory requirements or their standards of conduct or evolving guidance, or findings of undesirable effects caused by a chemically or mechanistically similar product or product candidate;

- regulatory authorities may disagree with our or our partners' clinical trial protocols or our or their interpretation of data from preclinical studies and clinical trials;
- clinical protocols or study procedures may not be adequately designed or followed by the investigators;
- formulation improvements may not work as expected, which could negatively impact commercial demand for our product candidates;
- regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we or our partners enter into agreements for clinical and commercial supplies;
- the supply or quantity of raw materials or manufactured product candidates or other materials necessary to conduct development activities may be insufficient, inadequate, or unavailable at an acceptable cost, and we or our partners may experience interruptions in supply;
- our or our partners' development plans may be delayed or changed as a result of changes in development strategy, the impact of new or different regulations, requirements, and guidelines, or other unexpected events or conditions;
- the cost of preclinical studies and clinical trials may be greater than we anticipate;
- we or our third-party contractors, including those manufacturing our product candidates or components or ingredients thereof, or conducting clinical trials or laboratory testing on our or our partners' behalf, may fail to comply with regulatory requirements and industry standards or meet contractual obligations in a timely manner or at all; and
- the impact of any global health pandemic, such as COVID-19, on one or more of the foregoing factors.

Clinical trials are lengthy and expensive. Many of the factors listed above could result in increased clinical development costs or longer clinical development times for any of our programs. We and our partners incur substantial expense for, and devote significant time to, preclinical testing and clinical trials, yet we cannot be certain that the tests and trials will ever result in the commercial sale of a product. Even if we or our partners successfully complete clinical trials for our product candidates, we or our partners might not file the required regulatory submissions in a timely manner or may not receive regulatory approval for the product candidates, which in either case would adversely impact or preclude our ability to generate any revenues from product sales or licensing arrangements. In addition, any product candidate, if approved, may be subject to restrictions on labeling, marketing, distribution, prescribing, and use, which could adversely impact the sales of such product.

If our development collaborations with third parties, such as our development partners, contractors and contract research organizations, fail, the development of our product candidates will be delayed or stopped.

We rely heavily upon third parties for many important stages of our product candidate development, including, but not limited to:

- discovery of natural proteins that cause or enable biological reactions necessary for the progression of the disease or disorder;
- execution of certain pharmacology preclinical studies and late-stage development for our compounds and product candidates;
- management of our phase 1, 2 and 3 clinical trials, including medical monitoring, laboratory testing, and data management;
- execution of toxicology studies that may be required to obtain approval for our product candidates;
- formulation improvement strategies and methods;
- manufacturing the starting materials and drug substance required to formulate our products and the product candidates to be used in our clinical trials, toxicology studies and any potential commercial product; and
- management of certain regulatory interactions outside of the United States.

Our failure to engage in successful collaborations at any one of these stages would greatly impact our business. If we do not license protein targets or inhibitors from academic institutions or from other biotechnology companies on acceptable terms, or at all, our drug development efforts could suffer. Similarly, if the contract research organizations or third-party contractors that conduct our initial or late-stage clinical trials, conduct our toxicology or other studies, manufacture our starting materials, drug substance and product candidates, provide laboratory testing or other services (including clinical operation services) in connection with our clinical trials, provide medical writing services, or assist with our regulatory function breach their obligations to us, perform their services inconsistent with industry standards, or fail to comply with regulatory requirements, this would delay or prevent both the development of our product candidates and the availability of any potential commercial product.

If we lose our relationship with any one or more of these parties, we could experience a significant delay in both identifying another comparable provider and then contracting for its services. We may be unable to retain an alternative provider on reasonable terms, if at all. Even if we locate an alternative provider, it is likely that this provider may need additional time to respond to our needs and may not provide the same type or level of service as the original provider. In addition, any provider that we retain will be subject to applicable FDA current Good Laboratory Practices, current Good Manufacturing Practices ("cGMP"), and current Good Clinical Practices, and comparable foreign standards. We do not have control over compliance with these regulations by these providers. Consequently, if these practices and standards are not adhered to by these providers, the development and commercialization of our product candidates could be delayed. If any of the foregoing risks is realized, our business, financial condition and results of operations could be materially adversely affected.

If we fail to obtain additional financing or acceptable partnership arrangements if and when needed, we may be unable to complete the development and commercialization of our products and product candidates or continue operations.

As our programs advance, our costs could increase. Our current and planned discovery, development, approval, and commercialization efforts may require significant capital. Our expenses, revenues and cash utilization rate could vary significantly depending on many factors, including: our ability to effectively manage our product candidate pipeline; our ability to obtain regulatory approvals for our product candidates; our ability to maintain regulatory approvals for, successfully commercialize, and achieve sustained market acceptance of our products, including ORLADEYO; our ability to raise additional capital if needed; our ability to secure partnerships with third parties for our product candidates when deemed advisable; the amount of funding we receive from partnerships with third parties for the development and commercialization of our products and product candidates; the commercial success of our products achieved by our partners; the progress and results of our current and proposed clinical trials for our product candidates; and the progress made in the manufacture of our lead products and the progression of our other programs.

In order to continue future operations, progress our drug discovery and development programs, and commercialize our current products and product candidates, we may be required to raise additional capital. Our ability to raise additional capital if and when needed may be limited and may greatly depend upon our sustained success in commercializing and achieving market acceptance of ORLADEYO and the success of our current drug development programs, including the progress, timeline and ultimate outcome of the development programs (including, but not limited to, formulation progress, long-term human safety studies, clinical trial investigations, and carcinogenicity, drug-drug interaction, toxicity, or other required studies) described in "*Management's Discussion and Analysis of Financial Condition and Results of Operations—Overview—Products and Product Candidates*" in Part I, Item 2 of this report, as well as any post-approval studies for our products. In addition, constriction and volatility in the equity and debt markets, including as a result of the impacts of inflation, increased interest rates, disruption or instability in the banking industry, geopolitical instability, or public health emergencies such as the COVID-19 pandemic, may restrict our future flexibility to raise capital if and when such needs arise. See "*Risks Relating to Our Business—Financial and Liquidity Risks—We may need to raise additional capital in the future. If we are unable to raise capital if and when needed, we may need to adjust our operations*" in this section and "*Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources*" in Part I, Item 2 of this report for additional information about our liquidity risks and capital requirements.

Furthermore, we have exposure to many different industries, financing partners and counterparties, including commercial banks, investment banks and partners (which include investors, licensing partners, distribution partners, and others), which may be unstable or may become unstable in the current economic and political environment, including as a result of the impacts of inflation, increased interest rates, disruption or instability in the banking industry, potential U.S. Government shutdowns, changes in presidential administration in the United States, geopolitical instability, actual or threatened public health emergencies, outbreaks of disease, epidemics or pandemics (such as the COVID-19 pandemic). Any such instability may impact these parties' ability to fulfill contractual obligations to us, or it might limit or place burdensome conditions upon future transactions with us. Also, it is possible that suppliers may be negatively impacted. Any such unfavorable outcomes in our current programs or unfavorable economic conditions have in the past and could again place severe downward pressure on the price of our common stock and may decrease opportunities to raise capital in the capital or credit markets, and further could reduce the return available on invested corporate cash, which, if severe and sustained, could have a material and adverse impact on our results of operations and cash flows and limit our ability to continue development and commercialization of our products and product candidates.

If we or our partners do not obtain regulatory approvals for our product candidates or maintain regulatory approvals for our products, we or our partners will not be able to commercialize and sell these products and potential products, which would significantly harm our business because we will receive no revenue.

We or our partners must obtain regulatory approvals before marketing or selling our products. If the FDA or a comparable foreign regulatory authority delays or denies regulatory approval of one of our product candidates, or revokes approval of a previously approved product, we would be unable to market or sell the product in the applicable jurisdiction and would not receive revenue from sales or licensing arrangements related thereto, which could have a material and adverse impact on our business.

The process of preparing for and obtaining regulatory approval in any jurisdiction may be lengthy and expensive, and approval is never certain. Because of the risks and uncertainties inherent to the development process, our product candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain approval. As discussed under "*Risk Factors—Risks Relating to Our Business—Risks Relating to Drug Development and Commercialization—Our success depends upon our ability to manage our product candidate pipeline, advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive regulatory approvals for the commercial sale of our product candidates*," we and our partners have experienced, and may again in the future experience, any number of unfavorable outcomes during or as a result of preclinical studies and clinical trials that could delay or prevent regulatory approval of our product candidates, or negatively impact our management's credibility, our value and our operating results.

Even if the FDA or foreign regulatory authorities approve a product candidate, the approval may limit the indicated uses for a product candidate, impose other restrictions on the product candidate, and/or may require post-approval studies that could impair the commercial viability of a product candidate. Even upon any approval to market our potential products, whether in the United States or internationally, we will continue to be subject to extensive regulatory requirements, as discussed under "*Risk Factors—Risks Relating to Our Business—Legal and Regulatory Risks—We are subject to various laws and regulations related to our products and product candidates, and if we or our partners do not comply with these laws and regulations, we could face substantial penalties*."

Our failure to comply with existing or future regulatory requirements for regulatory approval, or our loss of, or changes to, previously obtained approvals, could impair our ability to generate any revenues from product sales or licensing arrangements, which could have a material adverse effect on our business, financial condition, and results of operations.

We focus primarily on rare diseases, which may create additional risks and challenges, including that the target patient populations of our products and product candidates may be small.

Because we focus primarily on developing drugs as treatments for rare diseases, we may seek orphan drug, breakthrough therapy or fast track designations for our product candidates in the United States or the equivalent designations elsewhere in the world. Often, regulatory authorities have broad discretion in determining whether or not to grant such designations. We cannot guarantee that our product candidates will receive orphan drug status from the FDA or equivalent designations from other regulatory authorities. Even with an orphan drug designation for our current and potential future product candidates, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for an existing or future product candidate, that exclusivity may not effectively protect the product from competition. See "*Business—Government Regulation—FDA Regulation—Orphan Drugs*" in Part I, Item 1 of our most recent Annual Report on Form 10-K.

We also cannot guarantee that we will receive breakthrough therapy, fast track, or equivalent designations, which provide certain potential benefits such as more frequent meetings with the applicable regulatory authorities to discuss development plans, intensive guidance on efficient drug development programs, and potential eligibility for rolling review or priority review. Even if we are successful in obtaining any such designations for our product candidates, such designations may not lead to faster development or regulatory review or approval and do not increase the likelihood that our product candidates will receive marketing approval. We may not be able to obtain or maintain these designations for our product candidates that receive them, and our competitors may obtain these designations for their product candidates, which could impact our ability to develop and commercialize our products and product candidates or compete with such competitors, which may adversely impact our business, financial condition or results of operations.

Given the small number of patients who have the diseases that we are targeting, it is important to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. Our projections of both

the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our products and product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. Additionally, the potentially addressable patient population for each of our products and product candidates may be limited or may not be amenable to treatment with our products and product candidates, and new patients may become increasingly difficult to identify or access. Further, even if we obtain significant market share for our products and product candidates, because the potential target populations are small, we may never become or remain profitable nor generate sufficient revenue growth to sustain our business.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of data or market exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the drug covered thereby becomes a "reference-listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations." Manufacturers may seek marketing approval of generic versions of reference-listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States, as described in "*Business—Government Regulation—FDA Regulation—Abbreviated New Drug Applications for Generic Drugs*" in Part I, Item 1 of our most recent Annual Report on Form 10-K. Generic drugs may be significantly less costly to bring to market than the reference-listed drug and companies that produce generic drugs are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference-listed drug is typically lost to the generic drug.

The FDA may not approve an ANDA for a generic drug until any applicable period of non-patent exclusivity for the reference-listed drug has expired, as described in "*Business—Government Regulation—FDA Regulation—Abbreviated New Drug Applications for Generic Drugs*" in Part I, Item 1 of our most recent Annual Report on Form 10-K, but such exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the nonclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness. Manufacturers may seek to launch generic drugs following the expiration of the marketing exclusivity period, even if we still have patent protection for such drugs.

Competition that our products or product candidates may face from generic drugs could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates. Our future revenues, profitability and cash flows could also be materially and adversely affected and our ability to obtain a return on the investments we have made in those product candidates may be substantially limited if our products or, if and when approved, product candidates, are not afforded the appropriate periods of non-patent exclusivity.

The commercial viability of any approved product could be compromised if the product is less effective than expected, causes undesirable side effects that either were not previously identified or were worse than expected, or fails to achieve market acceptance within the medical community.

If, after obtaining regulatory approval of a product, we or others discover that the product is less effective than previously believed or causes undesirable side effects that either were not previously identified or were worse than expected, any of the following adverse events could occur:

- regulatory authorities may withdraw their approval of, or impose marketing or manufacturing restrictions on, the product, or require us or our partners to create a medication guide outlining the risks of unidentified side effects for distribution to patients;
- we or our partners may be required to recall the product, change the way the product is administered, conduct additional clinical trials, or be subject to civil or criminal penalties; and
- the product may become less competitive and our reputation may suffer.

Even after receiving regulatory approval, any product could fail to gain sufficient, or any, market acceptance by physicians, patients, third-party payors, health authorities and others in the medical community. For example, physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch therapies due to lack of reimbursement for existing therapies. If an approved product does not achieve an adequate level of market

acceptance, it may not generate significant revenues. The occurrence of any of the foregoing could have a material and adverse impact on our business.

If we fail to successfully commercialize or establish collaborative relationships to commercialize certain of our products and product candidates, or if any partner terminates or fails to perform its obligations under agreements with us, potential revenues from commercialization of our products and product candidates could be reduced, delayed or eliminated.

Our business strategy includes increasing the asset value of our product and product candidate portfolio. We believe this is best achieved by retaining full product rights or through collaborative arrangements with third parties as appropriate. As needed, potential third-party relationships could relate to preclinical development, clinical development, regulatory approval, marketing, sales, and distribution of our products and product candidates.

Currently, we have established collaborative relationships with third-party distributors for ORLADEYO in certain markets and with each of Shionogi and Green Cross for the development and commercialization of peramivir. The process of establishing and implementing collaborative relationships is difficult, time-consuming and involves significant uncertainty, including:

- we or our partners may seek to renegotiate or terminate our relationships due to unsatisfactory commercial, regulatory or clinical results, including post-approval clinical commitments, a change in business strategy, a change of control or other reasons;
- our contracts for collaborative arrangements may expire;
- the possibility that expiration or termination of collaborative relationships, such as those with certain of our distribution partners, may trigger repurchase obligations of the Company for unsold product held by our partners;
- our partners may choose to pursue alternative technologies, including those of our competitors;
- we have had in the past, and in the future may have, disputes with a partner that could lead to litigation or arbitration, which could result in substantial costs and divert the attention of our management;
- we do not have day-to-day control over the activities of our partners and have limited control over their decisions;
- our ability to generate future event payments and royalties from our partners depends upon their abilities to establish the safety and efficacy of our product candidates, obtain regulatory approvals and achieve market acceptance of products developed from our product candidates;
- we or our partners may fail to properly initiate, maintain or defend our intellectual property rights, where applicable, or a party may utilize our proprietary information in such a way as to invite litigation that could jeopardize or potentially invalidate our proprietary information or expose us to potential liability;
- we or our partners may not devote sufficient capital or resources toward our products and product candidates; and
- we or our partners may not comply with applicable government regulatory requirements.

If we or our partners fail to fulfill our responsibilities in a timely manner, or at all, our development and commercialization efforts related to that collaboration could be reduced, delayed or terminated, or it may be necessary for us to assume responsibility for activities that would otherwise have been the responsibility of our partner. If we are unable to establish and maintain collaborative relationships on acceptable terms, we may have to delay or discontinue further development or commercialization of one or more of our products or product candidates, undertake commercialization activities at our own expense or find alternative sources of funding. Any delay in the development or commercialization of our products and product candidates would severely affect our business, because if our product candidates do not progress through the development process or reach the market in a timely manner, or at all, or if our products do not achieve market success, we may not receive any revenues from product sales or licensing arrangements.

The results of our partnership with Torii may not meet our current expectations.

We have a partnership agreement with Torii for ORLADEYO in Japan. Under our agreement with Torii, we are responsible for all field promotional activities with respect to ORLADEYO in Japan, which we conduct through our Japanese subsidiary, BioCryst Japan K.K. Furthermore, we remain responsible for regulatory activities with respect to ORLADEYO in Japan, and we use third parties to satisfy those regulatory responsibilities and certain other obligations in Japan. If any party fails to meet its obligations, the commercial success of ORLADEYO in Japan and the economic benefit expected could be negatively impacted.

There can be no assurance that our or our partners' commercialization efforts, methods, and strategies for our products or technologies will succeed, and our future revenue generation is uncertain.

There can be no assurance that our or our partners' commercialization efforts, methods and strategies will succeed. We may be unable to establish or sufficiently increase our sales, marketing and distribution capabilities for products we currently, or plan to, commercialize. Our ability to receive revenue from products we or our partners commercialize is subject to several risks, including:

- we or our partners may fail to complete clinical trials successfully, or satisfy post-marketing commitments, sufficient to obtain and maintain regulatory agency marketing approval;
- many competitors are more experienced and have significantly more resources, and their products could reach the market faster, be more cost effective or have a better efficacy or tolerability profile than our products and product candidates;
- we may fail to employ a comprehensive and effective intellectual property strategy, which could result in decreased commercial value of our Company, our products and product candidates, or royalties associated with such products (e.g., the loss of the peramivir patent in Korea, which may result in a reduced royalty from Green Cross);
- we may fail to employ a comprehensive and effective regulatory strategy, which could result in a delay or failure in commercialization of our products;
- our and our partners' ability to successfully commercialize our products is affected by the competitive landscape, which cannot be fully known at this time;
- revenue from product sales depends on our ability to obtain and maintain favorable pricing;
- reimbursement is constantly changing, which could greatly affect usage of our products;
- future revenue from product sales will depend on our ability to successfully complete clinical studies, obtain regulatory approvals, and manufacture, market, distribute and commercialize our approved drugs; and
- the impact of public health emergencies or the outbreak of disease, such as the COVID-19 pandemic, on us or our partners.

In addition, future revenue from sales of ORLADEYO is subject to uncertainties and will depend on several factors, including the success of our and our partners' commercialization efforts in the United States and elsewhere, the number of new patients switching to ORLADEYO, patient retention and demand, the number of physicians prescribing ORLADEYO, the rate of monthly prescriptions, reimbursement from third-party and government payors, the number of patients receiving free product, the conversion of patients from our clinical trials and early access programs to commercial customers, our pricing strategy, and market trends.

Even if we are able to successfully commercialize our existing products, or to develop new commercially viable products, certain obligations we have to third parties, including, without limitation, our obligations to pay royalties on certain revenues from ORLADEYO under the Royalty Purchase Agreements, may reduce the profitability of such products.

We have expanded, and may continue expanding, our development and regulatory capabilities and are implementing sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We have experienced, and may continue to experience, significant growth in the number of our employees and the scope of our operations in the United States and internationally, particularly in the areas of drug development, regulatory affairs, sales, marketing, and distribution. To manage our growth, we must continue to implement and improve our managerial, operational and financial systems and processes, expand our facilities and continue to recruit and train qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such growth, we may not be able to effectively manage the expansion of our operations, implement appropriate systems and processes in a timely manner or at all, or recruit, train, and retain qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. In addition, if a commercial launch for any product or product candidate for which we recruit a commercial team and establish marketing capabilities in any region is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

We depend on third-party vendors in the manufacture and distribution of our products, product candidates and the materials for our products and product candidates. If we cannot rely on existing third-party vendors, we will be required to incur significant costs and potential delays in finding new third-party vendors, which could adversely impact the development and commercialization timeframes for our products and product candidates.

We depend on third-party vendors, including third-party manufacturers, distributors, and specialty pharmacies, in the manufacture and distribution of our products, product candidates, and the materials for our products and product candidates. Often, especially in the early development and commercialization process, we have only one or limited sources for a particular product or service, such as manufacturing and/or distribution. We depend on these third-party vendors to perform their obligations in a timely manner and in accordance with applicable governmental regulations. Our third-party vendors, particularly our third-party manufacturers and distributors, each of which may be the only vendor we have engaged for a particular product, product candidate, or service or in a particular region, may encounter difficulties with meeting our requirements, including, but not limited to, problems involving, as applicable:

- insufficient resources being devoted in the manner necessary to satisfy our requirements within expected timeframes;
- inconsistent production yields;
- product liability claims or recalls of commercial product;
- difficulties in scaling production to commercial and validation sizes;
- interruption of the delivery of materials required for the manufacturing process;
- failure to distribute commercial supplies of our products to commercial vendors or end users in a timely manner;
- scheduling of plant time with other vendors or unexpected equipment failure;
- potential catastrophes that could strike their facilities or have an effect on infrastructure;
- potential impurities in our drug substance or products that could affect availability of product for our clinical trials or future commercialization;
- poor quality control and assurance or inadequate process controls;
- failure to provide us with accurate or timely information regarding inventory, the number of patients who are using our products, or serious adverse events and/or product complaints regarding our products;
- inability of third parties to satisfy their financial obligations to us or to others;
- potential breach of the manufacturing or distribution agreement by the third party;
- possible termination or non-renewal of a material agreement by the third party at a time that is costly or inconvenient to us; and
- lack of compliance or cooperation with regulations and specifications or requests set forth by the FDA or other foreign regulatory agencies or local customs.

Many additional factors could cause production or distribution interruptions with the manufacture and distribution of any of our products and product candidates, including human error, natural disasters, pandemics, labor disputes or shortages, acts of terrorism or war, equipment malfunctions, raw material shortages or supply chain issues. If our commercial distribution partners are not able to satisfy our requirements within the expected timeframe, or are unable to provide us with accurate or timely information and data, including with respect to inventory and sales, serious adverse events, and/or product complaints, our business, including our commercialization efforts for and sales of ORLADEYO, may be at risk. In addition, if specialty pharmacy services, including our third-party call center services, which provide patient support and financial services, prescription intake and distribution, reimbursement adjudication, and ongoing compliance support, are not effectively managed, the continuance of our commercialization efforts for and sales of ORLADEYO may be delayed or compromised.

In addition, our contract manufacturers may not be able to manufacture the materials required for our products or product candidates at a cost or in quantities necessary to make them commercially viable. Our raw materials, drug substances, products, and product candidates are manufactured by a limited group of suppliers, including some at a single facility. If any of these suppliers were unable to produce these items, this could significantly impact our supply of products and product candidate material for further preclinical testing and clinical trials. Our third-party manufacturers also may not meet our manufacturing requirements. Furthermore, changes in the manufacturing process or procedures, including a change in the location where the drug is manufactured or a change of a third-party manufacturer, may require prior review and approval in accordance with the FDA's cGMP and comparable foreign requirements. This review may be costly and time-consuming and could delay or prevent the launch of a product. The FDA or foreign regulatory authorities may at any time implement new standards, or change their interpretation and enforcement of existing standards, for manufacture, packaging or testing of products. If we or our contract manufacturers are unable to comply, we or they may be subject to

regulatory action, civil actions or penalties, any of which could be costly to us and could result in a delay or shortage of product.

We currently contract with a foreign CMO in China for the manufacturing of one of our product candidates. Foreign CMOs may be subject to U.S. legislation, including the proposed BIOSECURE Act, sanctions, trade restrictions and other foreign regulatory requirements, which could increase the cost or reduce the supply of material available to us or delay the procurement or supply of such material.

If we are unable to maintain current third-party relationships, or enter into new agreements with additional third parties on commercially reasonable terms, or at all, or if there is poor manufacturing or distribution performance or failure to comply with any regulatory agency on the part of any of our third-party vendors, we may not be able to complete development of, obtain timely approval of, or commercialize our products and product candidates.

Commercialization of our products by us and our partners is subject to the potential commercialization risks described herein and numerous additional risks. Any potential revenue benefits to us, including in the form of milestone payments, royalties or other consideration, are highly speculative.

Commercial success of our products is uncertain and is subject to all the risks and uncertainties disclosed in our other risk factors relating to drug development and commercialization. In addition, commercialization of our products is subject to further risks and may be negatively impacted by a number of factors, including, but not limited to, the following:

- our products may not prove to be adequately safe and effective for market approval in markets other than the markets in which they are currently approved;
- necessary funding for post-marketing commitments and further development of our products may not be available timely, at all, or in sufficient amounts;
- advances in competing products could substantially replace potential demand for our products;
- government and third-party payors may not provide sufficient coverage or reimbursement, which would negatively impact the demand for our products;
- we may not be able to supply commercial material to our partners and our partners may not be able to maintain or establish sufficient and acceptable commercial manufacturing, either directly or through third-party manufacturers;
- the commercial demand for and acceptance of our products by healthcare providers and by patients may not be sufficient to result in substantial product revenues to us or to our partners and may result in little to no revenue, milestone payments, or royalties to us;
- effectiveness of marketing and commercialization efforts for our products by us or our partners;
- market satisfaction with existing alternative therapies;
- perceived efficacy relative to other available therapies;
- disease prevalence;
- cost of treatment;
- our pricing and reimbursement strategy may not be effective;
- new legislative or regulatory proposals may influence our pricing and reimbursement strategy, which could impact product revenues;
- pricing and availability of imports or alternative products;
- marketing and sales activities of competitors;
- shifts in the medical community to new treatment paradigms or standards of care; and
- relative convenience and ease of administration.

Risks Relating to Competing in Our Industry

We face intense competition, and if we are unable to compete effectively, the demand for our products may be reduced.

The biotechnology and pharmaceutical industries are highly competitive and subject to rapid and substantial technological change. There are many companies seeking to develop products for the same indications that we currently target. Our competitors in the United States and elsewhere are numerous and include, among others, major multinational pharmaceutical and chemical companies and specialized biotechnology firms. Most of these competitors have greater resources than we do, including greater financial resources, larger research and development staffs and more experienced manufacturing, marketing, and sales organizations. In addition, most of our competitors have greater experience than we do in conducting clinical trials and obtaining FDA and other regulatory approvals. Accordingly, our competitors may succeed in obtaining FDA or other regulatory approvals of product candidates more rapidly than we do for products that compete with our products. Companies that complete clinical trials, obtain required regulatory approvals, and commence

commercial sale of their drugs before we do may achieve a significant competitive advantage, including patent and FDA exclusivity rights that would delay our ability to market products. We face, and will continue to face, competition in the commercialization of our products, licensing of potential product candidates for desirable disease targets, licensing of desirable product candidates, and development and marketing of our product candidates from academic institutions, government agencies, research institutions and biotechnology and pharmaceutical companies. Competition may also arise from, among other things:

- other drug development technologies;
- methods of preventing or reducing the incidence of disease, including vaccines; and
- new small molecule or other classes of therapeutic agents.

Developments by others may render our products, product candidates, or technologies obsolete or noncompetitive.

We received FDA approval of ORLADEYO, an oral, once-daily therapy for the prevention of HAE attacks in adults and pediatric patients aged 12 years and older, in December 2020, and subsequently received regulatory approvals for ORLADEYO in other global markets. In addition, the ongoing APeX-P clinical trial, which is complete through the primary endpoint, is continuing to assess an oral granule formulation of ORLADEYO in pediatric patients who are 2 to 11 years of age. We are also performing research on or developing products for the treatment of several other rare or difficult-to-treat diseases, including Netherton syndrome, DME, and diseases of the complement system. We expect to encounter significant competition for our pharmaceutical products and product candidates. Companies that complete clinical trials, obtain required funding or government support, obtain required regulatory approvals and commence commercial sales or stockpiling orders of their products before their competitors may achieve a significant competitive advantage. In addition, various government entities throughout the world may offer incentives, grants and contracts to encourage additional investment into certain preventative and therapeutic agents, which may have the effect of further increasing the number of our competitors and/or providing advantages to certain competitors. See "*Business—Competition*" in Part I, Item 1 of our most recent Annual Report on Form 10-K for further discussion of our competitors, competitive products or programs, and the competitive conditions in these and other therapeutic areas.

If one or more of our competitors' products or programs, including potential competitors not currently identified, are successful, the market for our products may be reduced or eliminated.

Compared to us, many of our competitors and potential competitors have substantially greater:

- capital resources;
- research and development resources, including personnel and technology;
- regulatory experience;
- preclinical study and clinical testing experience;
- manufacturing, marketing, and sales experience; and
- production facilities.

Any of these competitive factors could impede our funding efforts, render our products, product candidates, or technologies noncompetitive or eliminate or reduce demand for our products and product candidates.

Legal and Regulatory Risks

We are subject to various laws and regulations related to our products and product candidates, and if we or our partners do not comply with these laws and regulations, we could face substantial penalties.

Our and our partners' activities related to approved products or, following their regulatory approval (if applicable), any of our product candidates under development, are subject to regulatory and law enforcement authorities in the United States (including the FDA, the Federal Trade Commission, the Department of Justice ("DOJ"), and state and local governments) and their foreign equivalents (including the EMA, MHLW, MHRA, and others).

We are responsible for reporting adverse drug experiences, have responsibility for certain post-approval studies, and may have responsibilities and costs related to a recall or withdrawal of our products from sale in the jurisdictions in which they are approved. We may also incur liability associated with product manufacturing contracted by us or in support of any of our partners. We are required to maintain records and provide data and reports to regulatory agencies related to our products (e.g., risk evaluation and mitigation strategies, track and trace requirements, and adverse events), and we may incur certain promotional regulatory and government pricing risks, all of which could have a material adverse impact on

our operations and financial condition. Similar responsibilities would apply upon regulatory approval of any of our other product candidates currently under development.

In addition, we are subject to the federal Physician Payment Sunshine Act and certain similar physician payment and drug pricing transparency legislation in various states. We are also subject to various federal and state laws pertaining to healthcare "fraud and abuse," including both federal and state anti-kickback and false claims laws. Outside of the United States, we may be subject to analogous foreign laws and regulations in the various jurisdictions in which we operate. These laws and regulations apply to our and our partners' operations, sales and marketing practices, price reporting, and relationships with physicians and other customers and third-party payors. Although we seek to comply with these statutes, it is possible that our practices, or those of our partners, might be challenged under healthcare fraud and abuse, anti-kickback, false claims or similar laws. Violations of the federal Physician Payment Sunshine Act and similar legislation or the fraud and abuse laws may be punishable by civil or criminal sanctions, including fines and civil monetary penalties, and future exclusion from participation in government healthcare programs.

The principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to certain regulatory authorities, including the FDA and comparable foreign regulatory authorities. Consequently, the FDA or other regulatory authority may conclude that a financial relationship between us and a principal investigator creates a conflict of interest or otherwise affects interpretation of the study. In the event of a conflict of interest with respect to a study, the integrity of the data generated at the applicable clinical trial site may be questioned or the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or other regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

The FDA and foreign regulatory authorities may also impose post-approval commitments on us for approved products, which we may not complete successfully or on time for any number of reasons, including, but not limited to, lack of funds to complete the studies and insufficient interest by appropriate sites, investigators or study subjects. We are currently subject to certain post-approval commitments and evolving FDA guidance. If we fail to comply with any post-approval legal and regulatory requirements, we could be subject to penalties, and our products could be subject to continual recordkeeping and reporting requirements, review and periodic inspections by the FDA and other regulatory bodies. Regulatory approval of a product may be subject to limitations on the indicated uses for which the product may be marketed or to other restrictive conditions of approval that limit our ability to promote, sell or distribute a product. Furthermore, the approval of our products and any other future product candidates may be subject to requirements for costly post-approval testing and surveillance to monitor their safety or efficacy or certain post-approval labeling, packaging and storage requirements.

Advertising and promotion are subject to stringent oversight from the FDA and foreign regulators, and as an NDA holder, we may be held responsible for any advertising and promotion that is not in compliance with applicable rules and regulations. Applicable regulatory authorities, competitors, and other third parties may take the position that we are not in compliance with such regulations. In addition to medical education efforts, we may offer patient support services to assist patients receiving treatment with our commercially approved products, and these support services have increasingly become the focus of government investigation.

Adverse event information concerning approved products must be reviewed, and as an NDA holder, we are required to make expedited and periodic adverse event reports to the FDA and other regulatory authorities. In addition, the research, manufacturing, distribution, sale and promotion of products are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services ("CMS"), other divisions of HHS, the DOJ and individual U.S. Attorney offices within the DOJ, state and local governments, and foreign equivalents of the foregoing. All of these activities are also potentially subject to healthcare false claims and fraud and abuse laws, as well as consumer protection and unfair competition laws.

If our operations with respect to our products that are subject to healthcare laws and regulations are found to be in violation of any of the healthcare fraud and abuse laws described above or in "*Business—Government Regulation*" in Part I, Item 1 of our most recent Annual Report on Form 10-K or any other governmental regulations that apply to us, we may be subject to liability and penalties, including civil and criminal penalties, damages, fines, debarment or exclusion from participating in government-funded healthcare programs such as Medicare or Medicaid, and the curtailment or restructuring of our operations. Any penalties, damages, fines, debarment, exclusion, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely

eliminated. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with all applicable fraud and abuse laws may be costly.

We cannot predict the likelihood, nature or extent of government regulation or other measures that may arise from future legislation or administrative or executive action, either in the United States or abroad.

The policies of the FDA and other regulatory authorities may change, including as a result of changes in presidential administration of the United States, and additional government regulations or executive orders may be enacted that could prevent, limit or delay regulatory approval of our product candidates, change our continuing compliance obligations, or otherwise adversely affect our business. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, and we may not achieve or sustain profitability. In addition, significant tariffs or other restrictions imposed and related countermeasures taken by impacted foreign countries could adversely affect our operations and financial results. We cannot predict the likelihood, nature or extent of government regulation or other measures that may arise from future legislation or administrative or executive action, either in the United States or abroad.

Our employees, consultants and partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are subject to the risk of fraud or other misconduct by our employees, consultants and partners, including intentional or unintentional failures to comply with FDA regulations or similar regulations of comparable other regulatory authorities, provide accurate information to the FDA or comparable other regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable other regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee and consultant misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter employee and consultant misconduct, whether intentional, reckless, negligent, or unintentional, 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 be in compliance with such laws, standards or regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

We and our partners may be subject to new legislation, regulatory proposals and healthcare payor initiatives that may increase our costs of compliance and adversely affect our or our partners' ability to market our products or develop our product candidates.

We are subject to new legislation, regulatory, and healthcare payor initiatives, including the Patient Protection and Affordable Care Act ("PPACA"), which made extensive changes to the delivery of healthcare in the United States, as discussed in "*Business—Government Regulation*" in Part I, Item 1 of our most recent Annual Report on Form 10-K. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare could result in decreased net revenues from our pharmaceutical products and decrease potential returns from our development efforts. In addition, pharmaceutical and device manufacturers are also required to report and disclose certain payments and transfers of value to, and investment interests held by, physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties for payments, transfers of value, or ownership or investment interests not reported in an annual submission. Compliance with the PPACA and state laws with similar provisions is difficult and time-consuming, and companies that do not comply with these state laws face civil penalties. Because of the breadth of these laws and the narrowness of the applicable safe harbors, it is possible that some of our business activities could be subject to challenge under one or more of such laws. Such a challenge could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

In addition, there have been a number of other legislative and regulatory proposals aimed at changing the pharmaceutical industry. For example, legislation has been enacted in certain states and at a federal level that requires development of an electronic pedigree to track and trace each prescription drug at the saleable unit level through the distribution system. Compliance with these electronic pedigree requirements may increase our operational expenses and impose significant administrative burdens. In addition, our compliance may be deemed insufficient and we could face a

material adverse effect on our business, financial condition, results of operations and growth prospects. As a result of these and other new proposals, we may determine to change our current manner of operation, provide additional benefits or change our contract arrangements, any of which could have a material adverse effect on our business, financial condition and results of operations.

Adequate coverage and reimbursement in the United States and other markets is essential to the commercial success of our approved products. Recently in the United States, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products. For example, the Inflation Reduction Act of 2022 ("IRA") implements a number of drug pricing measures intended to lower the cost of prescription drugs and related healthcare reforms, including limits on price increases and subjecting an escalating number of drugs to annual price negotiations with CMS. The IRA includes several provisions that will impact our business to varying degrees, including provisions that reduced the out-of-pocket spending cap for Medicare Part D beneficiaries to $2,000 starting in 2025; impose new manufacturer financial liability on all drugs in Medicare Part D; allow the U.S. Government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for drug prices that increase faster than inflation; and delay the rebate rule that would require pass through of pharmacy benefit manager rebates to beneficiaries. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication or indications are for that disease or condition. If a product receives multiple rare disease designations or has multiple approved indications for more than one disease or condition, it may not qualify for the orphan drug exemption.

We cannot be sure whether additional legislation or rule-making related to the IRA will be issued or enacted, how insurance pharmacy benefit managers and other insurance providers that manage benefits for Medicare recipients will react to the IRA, or what impact, if any, such additional changes will have on the insurance coverage and profitability of our products or any of our product candidates, if approved for commercial use, in the future. The full effect of the IRA on our business and the healthcare industry in general is not yet known. The IRA or other government efforts to reduce the price of prescription drugs or to limit the amount that governments pay for healthcare products and services could result in additional pricing pressure and have a significant impact on our business.

In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. Third-party payors are increasingly challenging the prices charged for medical products and services and, in some cases, imposing restrictions on the coverage of particular drugs. Many third-party payors negotiate the price of medical services and products and develop formularies which establish pricing and reimbursement levels. Exclusion of a product from a formulary can lead to its sharply reduced usage in the third-party payor's patient population. The process for obtaining coverage can be lengthy and costly, and we expect that it could take several months before a particular payor initially reviews a product and makes a decision with respect to coverage. For example, third-party payors may require cost-benefit analysis data from us in order to demonstrate the cost-effectiveness of our products or any other product we might bring to market. For any individual third-party payor, we may not be able to provide data sufficient to gain reimbursement on a similar or preferred basis to competitive products, or at all, which may have a material adverse effect on our business, financial condition and results of operations.

We may be subject to data privacy and security risks, and our actual or perceived failure to comply with regulations and other legal obligations related to privacy and data protection could harm our business.

We may be subject to legal obligations at the federal, state, and local level related to privacy and data protection, as described in "*Business—Government Regulation—Data Privacy and Security Laws*" in Part I, Item 1 of our most recent Annual Report on Form 10-K. Compliance with stringent and evolving U.S. data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use, and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. For example, we may be subject to the California Consumer Privacy Act ("CCPA"), which gives California residents expanded rights to access and require deletion of their personal data, opt out of certain personal data sharing, and receive detailed information about how their personal data is used. The CCPA provides for civil penalties of up to $7,500 per violation and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA exempts some data processed in the context of clinical trials, the CCPA may increase compliance costs and potential liability with respect to other personal data we may maintain about California residents.

We also may be subject to the General Data Protection Regulation ("GDPR") in the European Economic Area ("EEA") and similar legislation in the United Kingdom and Switzerland. See "*Business—Government Regulation—Data*

Privacy and Security Laws" in Part I, Item 1 of our most recent Annual Report on Form 10-K and "*Risks Relating to Our Business—Risks Relating to International Operations—Our actual or perceived failure to comply with European governmental laws and regulations and other legal obligations related to privacy, data protection and information security could harm our business*" in this section for additional discussion of privacy laws and regulations. Failure to comply with these laws and regulations could result in government enforcement actions, private litigation, or harm to our reputation and our business.

Despite our efforts, our personnel or third parties on whom we rely may fail to comply with such data privacy and security obligations, which could negatively impact our business operations. If we or the third parties on which we rely fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including, but not limited to, regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences.

If, because of our use of hazardous materials, we violate any environmental controls or regulations that apply to such materials, we may incur substantial costs and expenses in our remediation efforts.

Our research and development involves the controlled use of hazardous materials, chemicals and various radioactive compounds. We are subject to federal, state and local laws and regulations governing the use, storage, handling and disposal of these materials and some waste products. Accidental contamination or injury from these materials could occur. In the event of an accident, we could be liable for any damages that result, and any liabilities could exceed our resources. Compliance with environmental laws and regulations or a violation of such environmental laws and regulations could require us to incur substantial unexpected costs, which would materially and adversely affect our results of operations.

Intellectual Property Risks

If we fail to adequately protect or enforce our intellectual property rights, the value of those rights would diminish, and if we fail to secure the rights to patents of others, it could adversely affect our business.

Our success will depend in part on our ability and the abilities of our partners to obtain, protect and enforce viable intellectual property rights including, but not limited to, trade name, trademark and patent protection for our Company and its products, methods, processes and other technologies we may license or develop, to preserve our trade secrets, and to operate without infringing the proprietary rights of third parties both domestically and abroad. The patent position of biotechnology and pharmaceutical companies is generally highly uncertain, involves complex legal and factual questions and has recently been the subject of much litigation. Neither the United States Patent and Trademark Office ("USPTO"), the Patent Cooperation Treaty offices, nor the courts of the United States and other jurisdictions have consistent policies nor predictable rulings regarding the breadth of claims allowed or the degree of protection afforded under many biotechnology and pharmaceutical patents. Further, we may not have worldwide patent protection for all of our product candidates and our intellectual property rights may not be legally protected or enforceable in all countries throughout the world. In some jurisdictions, some of our product candidates in certain programs, including our HAE program, may have short or no composition of matter patent life and we may therefore rely on orphan drug exclusivity or data exclusivity. There can be no assurance that we will obtain orphan drug exclusivity or data exclusivity in every jurisdiction. Further, in some jurisdictions, we may rely on formulation patents or method of use patents. Both the ability to achieve issuance and the enforcement of formulation and method of use patents can be highly uncertain and can vary from jurisdiction to jurisdiction, and such patents may therefore not adequately prevent competitors and potential infringers in some jurisdictions. The validity, scope, enforceability, and commercial value of the rights protected by such patents, therefore, is highly uncertain.

We also rely on trade secrets to protect technology in cases when we believe patent protection is not appropriate or obtainable. However, trade secrets are difficult to protect. In addition, increasing restrictions on non-compete agreements could increase the difficulty of protecting certain proprietary information. If we cannot maintain the confidentiality of our technology and other confidential information in connection with our collaborators and advisors, our ability to receive patent protection or protect our proprietary information may be imperiled.

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights, or may design around our patent claims to produce competitive products that fall outside the scope of our patents. For example, a third party may develop a competitive drug that is similar to one or more of our products or product

candidates but that has a different composition that falls outside the scope of our patent protection. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive, time-consuming, and unsuccessful. An adverse result in any legal proceeding could put one or more of our patents at risk. Our success depends in part on avoiding the infringement of other parties' patents and other intellectual property rights as well as avoiding the breach of any licenses relating to our technologies and products. In the United States, patent applications filed in recent years are confidential for 18 months after the earliest effective filing date, while older applications are not published until the patent issues. As a result, avoiding patent infringement may be difficult and we may inadvertently infringe third-party patents or proprietary rights. These third parties could bring claims against us, our partners or our licensors that even if resolved in our favor, could cause us to incur substantial expenses and, if resolved against us, could additionally cause us to pay substantial damages. Further, if a patent infringement suit were brought against us, our partners or our licensors, we or they could be forced to stop or delay research, development, manufacturing or sales of any infringing product in the country or countries covered by the patent we infringe, unless we can obtain a license from the patent holder. Such a license may not be available on acceptable terms, or at all, particularly if the third party is developing or marketing a product competitive with the infringing product. Even if we, our partners or our licensors were able to obtain a license, the rights may be nonexclusive, which would give our competitors access to the same intellectual property.

In addition, as described under "*Business—Government Regulation—FDA Regulation—Abbreviated New Drug Applications for Generic Drugs*" in Part I, Item 1 of our most recent Annual Report on Form 10-K, third parties may not file an ANDA for a generic drug with the FDA until the expiration of five years following the original product approval unless the submission is accompanied by a Paragraph IV certification, in which case third parties may submit an ANDA four years following the original product approval (referred to as the "NCE-1 date"). As the NCE-1 date for ORLADEYO was in December 2024, we anticipate that third parties will challenge our applicable patents, which may result in our initiation of patent infringement litigation in response to such challenge. For example, in January 2025, we received a Paragraph IV notice of certification from Annora advising that Annora has submitted an ANDA to the FDA seeking approval to manufacture, use or sell a generic version of ORLADEYO in the United States prior to the expiration of three patents listed in the FDA's Orange Book, which expire in 2039. On March 10, 2025, we filed a patent infringement lawsuit in the United States District Court for the District of Delaware against the Defendants (as defined above) asserting infringement of the challenged patents arising from Annora's ANDA filing with the FDA. For further information, see the section titled "*Legal Proceedings*" included in Part II, Item 1 of this report and "*Note 13—Commitments and Contingencies"* in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report. We intend to vigorously defend our intellectual property rights protecting ORLADEYO. We cannot predict how any additional third party would address our listed patents, whether we would sue on any such patents, or the outcome of any such suit. However, litigation to enforce or defend intellectual property rights is complex, costly, and involves significant commitments of management's time.

If we or our partners are unable or fail to adequately initiate, protect, defend or enforce our intellectual property rights in any area of commercial interest or in any part of the world where we wish to seek regulatory approval for our products, methods, processes and other technologies, the value of our products and product candidates to produce revenue would diminish. Additionally, if our products, methods, processes, and other technologies or our commercial use of such products, processes, and other technologies, including, but not limited to, any trade name, trademark or commercial strategy infringe the proprietary rights of other parties, we could incur substantial costs. The USPTO and the patent offices of other jurisdictions have issued to us a number of patents for our various inventions, and we have in-licensed several patents from various institutions. We have filed additional patent applications and provisional patent applications with the USPTO. We have filed a number of corresponding foreign patent applications and intend to file additional foreign and U.S. patent applications, as appropriate. We have also filed certain trademark and trade name applications worldwide. We cannot assure you as to:

- the degree and range of protection any patents will afford against competitors with similar products;
- if and when patents will issue;
- if patents do issue, we cannot be sure that we will be able to adequately defend such patents and whether or not we will be able to adequately enforce such patents; or
- whether or not others will obtain patents claiming aspects similar to those covered by our patent applications.

If the USPTO or other foreign patent office upholds patents issued to others or if the USPTO grants patent applications filed by others, we may have to:

- obtain licenses or redesign our products or processes to avoid infringement;
- stop using the subject matter claimed in those patents; or

- pay damages.

We may initiate, or others may bring against us, litigation or administrative proceedings related to intellectual property rights, including proceedings before the USPTO or other foreign patent office. Any judgment adverse to us in any litigation or other proceeding arising in connection with a patent or patent application could materially and adversely affect our business, financial condition and results of operations. In addition, the costs of any litigation or administrative proceeding may be substantial whether or not we are successful.

Our success is also dependent upon the skills, knowledge and experience, none of which is patentable, of our scientific and technical personnel. To help protect our rights, we require all employees, consultants, advisors and partners to enter into confidentiality agreements that prohibit the disclosure of confidential information to anyone outside of our Company and require disclosure and assignment to us of their ideas, developments, discoveries and inventions. These agreements may not provide adequate protection for our trade secrets, know-how or other proprietary information in the event of any unauthorized use or disclosure or the lawful development by others of such information, and if any of our proprietary information is disclosed, our business will suffer because our revenues depend upon our ability to license or commercialize our products and product candidates and any such events would significantly impair the value of such products and product candidates.

We have diversified our pipeline to include the development of protein therapeutics, which may create additional risks and challenges.

We have diversified our pipeline beyond small-molecule medicines to develop protein therapeutics. The development of protein therapeutics may create additional risks and challenges, including, among others:

- patent protection for protein therapeutics may be narrower in scope than for our small-molecule medicines, and our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our protein therapeutic candidates or prevent others from designing around our claims;
- formulation issues with our protein therapeutic candidates may require redevelopment of the formulation, which may be time-consuming or unsuccessful;
- the patent applications that we own or in-license may fail to result in issued patents with claims that cover our protein therapeutic candidates in the United States or in other countries;
- our competitors may be able to more easily develop and seek patent protection on similar protein therapeutic candidates; and
- orally-administered drugs are often less expensive and present a reduced treatment burden as compared to protein therapeutics and therefore would have competitive advantages if they were developed and shown to be safe and effective for the indication that our protein therapeutic product candidates are targeting.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

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 biotechnology and pharmaceutical industries involves both technological and legal complexity. Therefore, obtaining and enforcing such patents is costly, time-consuming, and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to obtain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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

We employ certain individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Our efforts to vet our employees, consultants, and independent contractors and prevent their use of the proprietary information or know-how of others in their work for us may not be successful, and we may in the future be subject to claims that our employees, consultants, or

independent contractors have wrongfully used or disclosed confidential information of third parties. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and distract management and other employees.

Product Liability Risks

We face an inherent risk of liability in the event that the use or misuse of our products or product candidates results in personal injury or death, and our product liability insurance coverage may be insufficient.

If the use or misuse of any products we sell, or a partner sells, harms people, we may be subject to costly and damaging product liability claims brought against us by consumers, healthcare providers, pharmaceutical companies, third-party payors or others. The use of our product candidates in clinical trials, including post-marketing clinical studies, could also expose us to product liability claims. We cannot predict all of the possible harms or side effects that may result from the use of our products or the testing of product candidates, and therefore, the amount of insurance coverage we currently have may not be adequate to cover all liabilities or defense costs we might incur. A product liability claim or series of claims brought against us could give rise to a substantial liability that could exceed our resources. Even if claims are not successful, the costs of defending such claims and potential adverse publicity could be harmful to our business.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and face even greater risks upon commercialization by us of our products or product candidates. We have product liability insurance covering our clinical trials. Clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance or increase our existing coverage at a reasonable cost to protect us against losses that could have a material adverse effect on our business. An individual may bring a product liability claim against us if one of our products or product candidates causes, or is claimed to have caused, an injury or is found to be unsuitable for consumer use. Any product liability claim brought against us, with or without merit, could result in:

- liabilities that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available;
- an increase of our product liability insurance rates or the inability to maintain insurance coverage in the future on acceptable terms, or at all;
- withdrawal of clinical trial volunteers or patients;
- damage to our reputation and the reputation of our products, resulting in lower sales;
- regulatory investigations that could require costly recalls or product modifications;
- litigation costs; and
- the diversion of management's attention from managing our business.

Risks Relating to Contractual Arrangements

We face risks related to our U.S. Government contracts, which may create a disadvantage and additional risks to us.

In September 2024, we entered into a contract with ASPR for the procurement of up to 95,625 doses over a five-year period of RAPIVAB for the treatment of influenza. The contract is structured with a 12-month base ordering period and four optional 12-month ordering periods, which the U.S. Government can exercise on an annual basis. While ASPR executed the first ordering period, there is no guarantee that the U.S. Government will exercise any additional ordering periods. In addition, changes in U.S. Government budgets and agendas may result in the reduction, delay or elimination of funding or in a decreased emphasis on the procurement of RAPIVAB. Even if any optional ordering period is exercised, there can be no assurance that we or our manufacturers will be able to fully meet the demand for RAPIVAB with respect to this or any future arrangement.

We had contracts with the Biomedical Advanced Research and Development Authority within the U.S. Department of Health and Human Services ("BARDA/HHS") and the National Institute of Allergy and Infectious Diseases within HHS ("NIAID/HHS") for the development of galidesivir as a treatment for diseases caused by RNA pathogens, including Marburg virus disease, Yellow Fever and Ebola virus disease. In contracting with U.S. Government agencies, we became subject to various U.S. Government contract requirements, including general clauses for a cost-reimbursement research and development contract, which may limit our reimbursement. While all U.S. Government funding for galidesivir expired in 2022, we may still face risks related to our U.S. Government contracts pending final close out of these contracts.

U.S. Government contracts typically contain a number of extraordinary provisions that would not typically be found in commercial contracts, and which may create a disadvantage and additional risks to us as compared to competitors that do not have U.S. Government contracts. As a U.S. Government contractor, we are subject to an increased risk of investigations, criminal prosecution, civil fraud, whistleblower lawsuits and other legal actions and liabilities as compared to private sector commercial companies, and we could suffer serious harm to our reputation if allegations of impropriety were made against us. We could be subject to severe penalties, including legal actions and liabilities, in the event that we are unable to comply with delivery requirements or any other provision of a U.S. Government contract.

If we fail to reach milestones or to make annual minimum payments or otherwise breach our obligations under our license agreements, our licensors may terminate our agreements with them and/or seek additional remedies.

If we are unable or fail to meet payment obligations, performance milestones relating to the timing of regulatory filings, product supply obligations, post-approval commitments, or development and commercial diligence obligations; are unable or fail to make milestone payments or material data use payments in accordance with applicable provisions; or fail to pay the minimum annual payments under any of our in-licenses relating to our products or product candidates, our licensors may terminate the applicable license and/or seek other available remedies. As a result, our development of the respective product candidate or commercialization of the product would cease.

Because continuing events of default exist under the PhaRMA Notes, the holders of the PhaRMA Notes may be able to foreclose on the collateral securing the PhaRMA Notes and our equity interest in Royalty Sub. As a result, we may not realize the benefit of future royalty payments, if any, that might otherwise accrue to us following repayment of the PhaRMA Notes and we could otherwise be adversely affected.

In March 2011, JPR Royalty Sub LLC, our wholly-owned subsidiary ("Royalty Sub"), issued $30.0 million in aggregate principal amount of PhaRMA Senior Secured 14.0% Notes due on December 1, 2020 (the "PhaRMA Notes"). The PhaRMA Notes are secured principally by (i) certain royalty and milestone payments under our agreement with Shionogi, pursuant to which Shionogi licensed from us the rights to market peramivir in Japan and Taiwan and (ii) the pledge by us of our equity interest in Royalty Sub. Since September 1, 2014, payments from Shionogi have been insufficient for Royalty Sub to service its obligations under the PhaRMA Notes, resulting in a continuing event of default with respect to the PhaRMA Notes since that time. In addition, the PhaRMA Notes had a final legal maturity date of December 1, 2020, at which time the outstanding principal amount of the PhaRMA Notes of $30.0 million, together with accrued and unpaid interest of $20.6 million, was due in full. The failure by Royalty Sub to repay these amounts at the maturity date constituted an additional event of default under the PhaRMA Notes. As Royalty Sub has been unable to service its obligations under the PhaRMA Notes and continuing events of default exist under the PhaRMA Notes, the holders of the PhaRMA Notes may be able to foreclose on the collateral securing the PhaRMA Notes and our equity interest in Royalty Sub and may exercise other remedies available to them under the indenture or other documents related to the PhaRMA Notes. In such event, we may not realize the benefit of future royalty payments, if any, that might otherwise accrue to us following repayment of the PhaRMA Notes, we may incur legal costs, and we might otherwise be adversely affected.

We cannot predict whether holders of PhaRMA Notes will seek to pursue any remedies as a result of the continuing events of default with respect to the PhaRMA Notes. The PhaRMA Notes are the obligation of Royalty Sub. Due to the non-recourse nature of the PhaRMA Notes, in the event of any potential foreclosure, we believe the primary impact to us would be the loss of future royalty payments, if any, from Shionogi and the legal costs associated with retiring the PhaRMA Notes. As a result, we do not currently expect the continuing events of default on the PhaRMA Notes to have a significant impact on our future results of operations or cash flows. However, we cannot assure you that this will be the case or that we will not otherwise be adversely affected as a result of the continuing events of default under the PhaRMA Notes or the failure by Royalty Sub to repay the PhaRMA Notes at maturity.

While Royalty Sub continues to pay the holders of the PhaRMA Notes any royalty payments received from Shionogi, which are immaterial, we wrote off the balance due under the PhaRMA Notes to other income as a debt extinguishment as of December 31, 2021.

We have incurred significant indebtedness, which could adversely affect our business. Additionally, the Pharmakon Loan Agreement contains conditions and restrictions that limit our flexibility in operating our business. We may be required to make a prepayment or repay our outstanding indebtedness earlier than we expect if a prepayment event or

an event of default occurs, including a material adverse change with respect to us, which could have a material adverse effect on our business.

On April 17, 2023, we entered into the $450.0 million Pharmakon Loan Agreement (the "Pharmakon Loan Agreement") with BioPharma Credit Investments V (Master) LP and BPCR Limited Partnership, as lenders, and BioPharma Credit PLC, as collateral agent for the lenders, and closed on an initial term loan thereunder in the principal amount of $300.0 million. As of March 31, 2025, we had an outstanding principal balance under the Pharmakon Loan Agreement of $323.7 million, inclusive of the Pharmakon PIK Interest Payments (as defined in "*Note 7—Debt*" in the Notes to the Condensed Consolidated Financial Statements included in Part I, Item 1 of this report). Under the Pharmakon Loan Agreement, we will be required to pay to Pharmakon, for the account of the lenders, a prepayment premium or a make-whole premium, as applicable, plus certain fees or expenses set forth in the Pharmakon Loan Agreement in the event that we prepay, or are required to prepay, voluntarily or pursuant to a mandatory prepayment obligation under the Pharmakon Loan Agreement (e.g., upon a change of control of the Company and specified other events, subject to certain exceptions), all or part of the then-outstanding term loans under the Pharmakon Loan Agreement, in each case, subject to certain exceptions set forth in the Pharmakon Loan Agreement. For example, on April 18, 2025, we made a partial prepayment of $75.0 million of the outstanding principal amount under the Pharmakon Loan Agreement, in addition to a prepayment premium and certain other fees and expenses.

Our indebtedness could have important consequences to our stockholders. For example, it:

- increases our vulnerability to adverse general economic or industry conditions;
- limits our flexibility in planning for, or reacting to, changes in our business or the industry in which we operate;
- makes us more vulnerable to increases in interest rates, as borrowings under the Pharmakon Loan Agreement accrue interest at variable, uncapped rates, such that increases in interest rates will increase the associated interest payments that we are required to make on outstanding borrowings;
- requires us to dedicate a portion of our cash flow from operations to interest payments, limiting the availability of cash for other purposes;
- limits our ability to obtain additional financing or refinancing in the future for working capital or other purposes; and
- places us at a competitive disadvantage compared to our competitors that have less indebtedness.

Furthermore, the Pharmakon Loan Agreement contains various covenants that limit our ability to engage in specified types of transactions. Subject to certain exceptions, these covenants limit our ability to, among other things, dispose of assets; engage in certain mergers, acquisitions, and similar transactions; incur additional indebtedness; grant liens; make investments; pay dividends or make distributions or certain other restricted payments in respect of equity; prepay other indebtedness; enter into restrictive agreements; undertake fundamental changes; or amend certain material contracts.

The covenants contained in the Pharmakon Loan Agreement could cause us to be unable to pursue business opportunities that we or our stockholders may consider beneficial without the lenders' permission or without repaying all outstanding obligations under the Pharmakon Loan Agreement.

A breach of any of these covenants could result in an event of default under the Pharmakon Loan Agreement. An event of default will also occur if, among other things, we fail to pay amounts due under the Pharmakon Loan Agreement, we fail to repay certain other indebtedness having an aggregate principal amount in excess of a threshold amount, an insolvency event occurs with respect to us, judgments for the payment of money in excess of a threshold amount are entered into against us, a material adverse change in our business, assets, properties, liabilities, or condition occurs, or a material impairment of our ability to perform our obligations under the Pharmakon Loan Agreement occurs, certain negative regulatory events occur, including, without limitation, certain withdrawal events with respect to ORLADEYO, or we fail to make required payments under our Royalty Purchase Agreements. In the case of a continuing event of default under the Pharmakon Loan Agreement, the lenders under the Pharmakon Loan Agreement could elect to declare all amounts outstanding to be immediately due and payable, proceed against the collateral in which we granted to the lenders a security interest, or otherwise exercise the rights of a secured creditor. Amounts outstanding under the Pharmakon Loan Agreement are secured by a security interest in, subject to certain exceptions, substantially all of our assets. Because substantially all of our assets are pledged to secure the Pharmakon Loan Agreement obligations, our ability to incur additional secured indebtedness or to sell or dispose of assets to raise capital may be impaired, which could have an adverse effect on our financial flexibility.

Risks Relating to International Operations

International expansion of our business exposes us to business, legal, regulatory, political, operational, financial, and economic risks.

Our business strategy includes international expansion, including the commercialization of products outside of the United States. In addition, we currently conduct clinical studies and regulatory activities and have hired, and expect to continue hiring, employees outside of the United States. Doing business internationally involves a number of risks, including, but not limited to:

- multiple, conflicting, and changing laws and regulations such as privacy and data regulations, transparency regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- introduction of new health authority requirements and/or changes in health authority expectations;
- failure by us or our partners to obtain and maintain regulatory approvals for the use of our products in various countries;
- complexities and difficulties in obtaining and maintaining protection for, and enforcing, our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits on our ability to penetrate international markets;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products, and exposure to foreign currency exchange rate fluctuations, which have been increasingly prevalent alongside a fluctuating U.S. dollar;
- natural disasters and political and economic instability, including wars, terrorism, political unrest, results of certain elections and votes, actual or threatened public health emergencies and outbreak of disease, epidemics or pandemics (e.g., the COVID-19 pandemic), boycotts, adoption or expansion of government trade restrictions, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance;
- regulatory and compliance risks that relate to maintaining accurate information and control over commercial operations and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, including its books and records provisions or anti-bribery provisions, or the U.K. Bribery Act and similar foreign laws and regulations; and
- regulatory and compliance risks relating to doing business with any entity that is subject to sanctions administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury.

Any of these factors could significantly harm our international expansion of operations and adversely affect our business and results of operations.

Additionally, in some countries, such as Japan and the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control and access. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we or our partners may be required to conduct a clinical trial that compares the cost-effectiveness of our product to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

Foreign currency exchange rate fluctuations could have an adverse impact on our results of operations, financial position, and cash flows.

We conduct operations in many countries outside of the United States involving transactions in a variety of currencies other than the U.S. dollar. These transactions include, without limitation, commercial sales, contract manufacturing, and clinical trial activities. Although most of our revenues and expenses are denominated in U.S. dollars, our commercial sales in Europe are primarily denominated in Euros and British Pounds. We also have foreign currency exposure to fluctuations in other foreign currencies, such as the Swiss Franc, Danish Krone, Swedish Krona, Norwegian Krone, Japanese Yen and Canadian Dollar. Changes in the value of these currencies relative to the U.S. dollar may impact our condensed consolidated operating results, including our revenues and expenses, causing fluctuations in our operating results from period to period and/or resulting in foreign currency transaction losses that adversely impact our results of operations, financial position, and cash flows. As we continue to expand our operations internationally, our exposure to

foreign currency transaction gains or losses may become more significant. See "*Quantitative and Qualitative Disclosures about Market Risk—Foreign Currency Risk*" in Part I, Item 3 of this report for additional information about our foreign currency risk.

Our actual or perceived failure to comply with European governmental laws and regulations and other legal obligations related to privacy, data protection and information security could harm our business.

Outside the United States, an increasing number of laws and regulations may govern data privacy and security. EU member states, the United Kingdom, Switzerland and other countries have adopted data protection laws and regulations, which impose significant compliance obligations. These laws include the GDPR and similar national legislation within the EEA, the United Kingdom GDPR, Switzerland's Federal Data Protection Act, the EU Clinical Trials Regulation, and the e-Privacy Directive (2002/58/EC), and are discussed in more detail in "*Business—Government Regulation—Data Privacy and Security Laws*" in Part I, Item 1 of our most recent Annual Report on Form 10-K. Failure to comply with the requirements of these laws may result in significant fines. For example, the GDPR or related national data protection laws, which may deviate from the GDPR, may result in significant fines of up to 4% of global revenues, or €20.0 million, whichever is greater.

In addition to such fines, failure to comply with the requirements of the GDPR or similar national legislation may result in temporary or definitive bans on data processing and other corrective actions and subject us to litigation and/or adverse publicity, which could have material adverse effects on our reputation and business. As a result of the implementation of the GDPR, we are required to put in place additional mechanisms to ensure compliance with the data protection rules. For example, the GDPR requires us to make more detailed disclosures to data subjects, requires disclosure of the legal basis on which we can process personal data, makes it harder for us to obtain valid consent for processing, requires the appointment of a data protection officer where sensitive personal data (i.e., health data) is processed on a large scale, introduces mandatory data breach notification throughout the European Union, imposes additional obligations on us when we are contracting with service providers and requires us to adopt appropriate privacy governance including policies, procedures, training and data audits. We depend on a number of third parties in relation to the provision of our services, a number of which process personal data of EU individuals on our behalf. With each such provider, we are required to enter into contractual arrangements under which they are contractually obligated to only process personal data according to our instructions, and conduct diligence to ensure that they have sufficient technical and organizational security measures in place.

Compliance with evolving laws regarding the transfer of personal data to the United States and other countries also requires increased resources and may result in increased exposure to regulatory actions, 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. We are also subject to evolving European privacy laws on electronic marketing and cookies. The European Union is in the process of replacing the e-Privacy Directive (2002/58/EC) with a new set of rules taking the form of a regulation that will be directly implemented in the laws of each EU member state. While this e-Privacy Regulation was originally intended to be adopted on May 25, 2018, it is still going through the European legislative process and the timing of its adoption remains unclear.

Compliance with the requirements imposed by the GDPR and other such laws can be time-consuming, expensive and difficult, and may increase our cost of doing business or require us to change our business practices, and despite our efforts we may not be successful in achieving compliance if our personnel, collaborators, partners or vendors do not comply with applicable data protection obligations. Despite our efforts, our personnel or third parties on whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties on which we rely fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences.

The United Kingdom's decision to withdraw from the European Union could result in increased regulatory and legal complexity, which may make it more difficult for us to do business in Europe and impose additional challenges in securing regulatory approval of our product candidates in Europe.

The United Kingdom's exit from the European Union, or Brexit, has caused political and economic uncertainty, including in the regulatory framework applicable to our operations and product candidates, and this uncertainty may persist for years. Brexit could, among other outcomes, disrupt the free movement of goods, services and people between the United Kingdom and the European Union, and result in increased legal and regulatory complexities, as well as potential

higher costs of conducting business in Europe. The long-term effects of Brexit will depend in part on how the current and future trade agreements between the United Kingdom and the European Union take effect in practice. Changes in U.K. or EU regulations may cause disruption or delays in granting clinical trial authorization or opinions for marketing authorization, disruption of importation and export of active substance and other components of new drug formulations, and disruption of the supply chain for clinical trial product and final authorized formulations.

The cumulative effects of the disruption to the regulatory framework may add considerably to the development lead time to marketing authorization and commercialization of products in the European Union and/or the United Kingdom. It is possible that there will be increased regulatory complexities, which can disrupt the timing of our clinical trials and regulatory approvals. In addition, changes in, and legal uncertainty with regard to, national and international laws and regulations may present difficulties for our clinical and regulatory strategy. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenues and achieve and sustain profitability.

In addition, as a result of Brexit, other European countries may seek to conduct referenda with respect to their continuing membership with the European Union. Given these possibilities and others we may not anticipate, as well as the absence of comparable precedent, it is unclear what financial, regulatory and legal implications the withdrawal of the United Kingdom from the European Union will have and how such withdrawal will affect us, and the full extent to which our business could be adversely affected.

Risks Relating to Technology

If our facilities, or the facilities of our third-party vendors, incur damage or power is lost for a significant length of time, our business will suffer.

We and our third-party vendors store commercial product, clinical and stability samples, and manufacturing data at our facilities that could be damaged if the facilities incur physical damage or in the event of an extended power failure. We have backup power systems in addition to backup generators to maintain power to all critical functions, but any loss of these products or samples could result in significant delays in our commercialization or drug development process.

In addition, we store most of our preclinical and clinical data at our facilities. While duplicate copies of most clinical data are secured off-site, and a significant portion of our data is included in regular backups of our systems, we could lose important data if our facilities incur damage, or if our vendor data systems fail, suffer damage or are destroyed. Any significant degradation or failure of our computer systems could cause us to inaccurately calculate or lose our data. Loss of data could result in significant delays in our drug development process, and any system failure could harm our business and operations.

Cyber incidents and related disruptions in our or our third-party vendors' information technology systems could adversely affect our business.

We are increasingly dependent on information technology systems to operate our business. In addition, the FDA and comparable foreign regulatory authorities regulate, among other things, the record keeping and storage of data pertaining to potential pharmaceutical products. Like other companies in our industry, our information technology systems and infrastructure (as well as those of our third-party providers) and our lab equipment and operations technology may be vulnerable to cyber incidents, intrusions, and other similar activities that threaten the confidentiality, integrity, and availability of our information. These threats come from a variety of sources, including by computer hackers, foreign governments, foreign companies, or competitors, or may be breached by employee error, malfeasance or other disruption. These threats are prevalent, continue to rise, and are becoming increasingly difficult to detect. Recently, there have been reports of disruptions in billing and data systems in healthcare (e.g., the cybersecurity incident affecting Change Healthcare in February 2024). Such cybersecurity events which materially disrupt the healthcare system upon which our business relies could adversely affect our business if such disruption is widespread and continues for an extended period of time.

Cyber incidents could also include the use of artificial intelligence ("AI") and machine learning to launch more automated, targeted and coordinated attacks on targets. Cyber incidents may lead to operational outages, loss of intellectual property due to industrial espionage, malware, and financial or data attacks via social engineering. These risks have increased as we have experienced significant growth in the number of our employees and the scope of our operations and as virtual and remote working have become more widely used, and sensitive data is accessed by employees working in less secure, home-based environments. A breakdown, invasion, corruption, destruction, or interruption of information technology systems could negatively impact operations. If our systems are damaged, fail to function properly or otherwise

become unavailable, we may incur substantial costs to repair or replace them, and we may experience loss of critical data and interruptions or delays in our ability to perform critical functions, which could adversely affect our business, financial condition or results of operations.

In addition, we rely on third-party service providers and technologies to operate significant information technology systems and business infrastructure, and we currently use these providers to perform business critical information technology and business services. Supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been or will not be compromised.

We have experienced cybersecurity threats and incidents, which to date have not had a material impact on our reputation, business, financial condition, or operations; however; there is no assurance that such impacts will not be material in the future.

Any compromise of our data security could also result in a violation of applicable privacy and other laws, significant legal, regulatory, and financial exposure, damage to our reputation, loss or misuse of the information and a loss of confidence in our data security measures, which could harm our business. Loss or misuse of our intellectual property, clinical trial data, or commercially sensitive data could adversely impact our business. While we have implemented security measures designed to protect against security incidents and a significant portion of our data is included in regular backups of our systems, there can be no assurance that our efforts to protect our data and information technology systems will prevent breakdowns or breaches in our systems, or those of third parties with which we do business, and any such events could adversely affect our business.

From time to time, we use artificial intelligence in our business, and challenges with properly managing its use could adversely affect our business.

The increasing use of AI and machine learning technology in the biopharmaceutical industry, combined with an uncertain regulatory environment, presents new risks and challenges. From time to time, we adopt and integrate AI solutions into our systems for specific use cases reviewed by legal and information security, and applications of AI may become important in our operations over time. Our vendors may incorporate AI tools into their offerings without disclosing this use to us, and the providers of these tools may not meet existing or rapidly evolving regulatory or industry standards with respect to privacy and data protection. Moreover, the use of AI-based software may lead to the inadvertent release of confidential or proprietary information, which may adversely impact our ability to realize the benefit of our intellectual property, cause us to incur liabilities as the result of any breaches of confidentiality or impact our ability to comply with data security and privacy laws. Further, as the regulatory framework for these technologies evolves, it is possible that new laws and regulations will be adopted, or that existing laws and regulations may be interpreted in ways that would affect our business, including as a result of the cost to comply with such laws or regulations. Our competitors or other third parties may also incorporate AI into their businesses more efficiently than us, which could impair our ability to compete effectively and adversely affect our results of operations. The rapid innovation and developments surrounding AI, including potential government regulation of AI, may require significant resources to develop, test and maintain our implementations of AI.

Other Operational Risks

Health epidemics or pandemics could materially adversely affect our business, operations, clinical development or commercialization plans and timelines, or that of third parties with whom we conduct business, including, without limitation, our development partners, manufacturers, CROs, and others, as well as the regulatory and government agencies with whom we work.

A health epidemic or pandemic, such as the COVID-19 pandemic, and related government orders or evolving business policies and procedures, could cause disruptions to our business, operations, and clinical development or commercialization plans and timelines, as well as the business and operations of third parties with whom we conduct business.

If our operations or those of third parties with whom we conduct business, such as development partners, manufacturers, CROs and others, are impaired or curtailed as a result of such events, the development and commercialization of our products and product candidates could be stopped or delayed, or the costs of such development and commercialization activities could increase, any of which could have a material adverse impact on our business. For example, our suppliers or other vendors may be unable to meet their obligations to us or perform their services as expected. In such circumstances, we may not be able to enter into arrangements with alternative suppliers or vendors or do so on

commercially reasonable terms or in a timely manner. Such delays could adversely impact our ability to meet our desired clinical development and any commercialization timelines.

In addition, our clinical trials were affected by the COVID-19 pandemic and we may experience similar delays or interruptions due to health epidemics or pandemics in the future, which could adversely impact our clinical trial operations. Health epidemics or pandemics could also affect the operations of regulators and other health and governmental authorities, which could result in delays of reviews and approvals, inspections, or other regulatory activities, including as we continue to expand internationally and bring ORLADEYO to additional global markets.

The global impact of a health epidemic or pandemic, such as the COVID-19 pandemic, could also materially affect global economies and financial markets, which could reduce our ability to access the equity or debt capital markets or obtain other sources of capital if needed, which could negatively affect our liquidity. In addition, a recession or market correction could materially affect our business and the value of our common stock. Health epidemics or pandemics could also have the effect of heightening many of the other risks described in this report.

Our business, operations, clinical development or commercialization plans and timelines, and access to capital could be adversely affected by unpredictable and unstable market and economic conditions.

Our business, operations, clinical development or commercialization plans and timelines, and access to capital could be adversely affected by unpredictable and unstable market and economic conditions, including as a result of inflation, increased interest rates, disruption or instability in the banking industry, foreign exchange rate fluctuations, potential U.S. Government shutdowns, instability in connection with changes in presidential administration in the United States, geopolitical instability, actual or threatened public health emergencies, or outbreaks of disease, epidemics or pandemics (such as the COVID-19 pandemic). The magnitude, duration and long-term effect of each of these factors, as well as the effects of actions taken by governments to address them, are unknown at this time, but they could result in further significant disruption of the global economy and financial markets. Our business may be adversely affected by any related economic downturn, volatile geopolitical and business environment, or continued market instability.

Unstable market and economic conditions could materially affect our ability to access the equity or debt capital markets or obtain other sources of capital if needed in the future, which could negatively affect our liquidity. In addition, a recession or market correction could materially affect our business and the value of our common stock.

Market and economic conditions continue to evolve, with the ultimate impacts being uncertain and subject to change. These effects could be material, and we will continue to monitor the economic climate closely. We do not yet know the full extent and magnitude of the impacts that these developments will have on our business, on the healthcare system, or on the global economy. In addition, unstable market conditions could have the effect of heightening many of the other risks described in this report.

Insurance coverage is increasingly more costly and difficult to obtain or maintain.

While we currently have insurance for our business, property, directors and officers, and our products, insurance is increasingly more costly and narrower in scope, and we may be required to assume more risk in the future. If we are subject to claims or suffer a loss or damage in excess of our insurance coverage, we will be required to bear any loss in excess of our insurance limits. If we are subject to claims or suffer a loss or damage that is outside of our insurance coverage, we may incur significant uninsured costs associated with loss or damage that could have an adverse effect on our operations and financial position. Furthermore, any claims made on our insurance policies may impact our ability to obtain or maintain insurance coverage at reasonable costs or at all.

If we fail to retain our existing key personnel or fail to attract and retain additional key personnel, the development of our product candidates, the commercialization of our products, and the related expansion of our business will be delayed or stopped.

We are highly dependent upon our senior management and scientific team, the unexpected loss of whose services might impede the achievement of our development and commercial objectives. Competition for key personnel with the experience that we require is intense and is expected to continue to increase. Our inability to attract and retain the required number of skilled and experienced management, commercial, operational and scientific personnel would harm our business because we rely upon these personnel for many important functions of our business.

If our risk management committee and other compliance methods are not effective, our business, financial condition and operating results may be adversely affected.

Our ability to identify, manage and respond to the various risks related to our business is largely dependent on our established and maintained compliance, risk, audit and reporting systems and procedures. The Board of Directors has ultimate responsibility for risk oversight of the Company and carries out this duty through its committees. The Board of Directors may delegate oversight authority with respect to certain issues in a committee's applicable areas of expertise. At the Company level, our senior management team similarly monitors risk through the risk management committee and other sub-committees focused on specific areas of risk (e.g., cybersecurity, quality assurance). Membership of the risk management committee consists primarily of key department heads who are asked to bring to such committee relevant items for discussion that they or their teams have identified at the numerous sub-committees these individuals chair or attend. The risk management committee, along with the other sub-committees in the Company, identifies key risks and mitigation strategies which are reported directly to our senior management, the Audit Committee and to the full Board of Directors on a regular basis.

If our policies, procedures, and compliance systems, including our risk management committee, are not effective, or if we are not successful in monitoring or evaluating the risks to which we are or may be exposed, our business, reputation, financial condition and operating results could be materially adversely affected. We cannot provide assurance that our policies and procedures will always be effective, or that our management or the risk management committee would be able to identify any such ineffectiveness. If our compliance and risk management strategies are not effective, our business, financial condition and operating results may be adversely affected.

Future acquisitions, strategic investments, partnerships, alliances, or divestitures could be difficult to identify and integrate, divert the attention of management, disrupt our business, dilute stockholder value, materially change the risk profile of the Company and could fail to meet our expectations, any of which could adversely affect our operating results and financial condition.

We may in the future seek to acquire or invest in businesses, products or technologies that we believe could complement or expand our portfolio or otherwise offer growth opportunities. The pursuit of potential acquisitions may divert the attention of management and cause us to incur various expenses in identifying, investigating and pursuing businesses or products. In addition, we may not be able to find and identify desirable acquisition targets or be successful in entering into an agreement with any particular target or consummating any such agreement. Even if we do consummate an acquisition, in connection therewith we may be required to issue equity (thereby diluting our current stockholders) or debt, we may not be able to integrate successfully the acquired personnel, operations and technologies, or effectively manage the combined business following the acquisition, or the acquired business could otherwise fail to meet our expectations, which, in each case, could have a material adverse effect on our business projections, financial condition, results of operations and prospects.

In addition, we may divest or license all or a portion of certain business or product categories, which could cause a decline in revenue or profitability and may make our financial results more volatile. We may be unable to complete any such divestiture or license on terms favorable to us, within the expected timeframes, or at all. We may have continued financial exposure to divested or licensed businesses following the completion of any such transaction, including increased costs due to potential litigation, contingent liabilities and indemnification of the buyer or licensee related to, among other things, lawsuits, regulatory matters or tax liabilities. Such divestitures or licenses may also divert management's attention from our core businesses and lead to potential issues with employees, customers or suppliers.

Our business and operations could be negatively affected if we become subject to stockholder activism or hostile bids, which could cause us to incur significant expense, hinder execution of our business strategy and impact our stock price.

Stockholder activism, which takes many forms and arises in a variety of situations, has been increasingly prevalent. Stock price declines may also increase our vulnerability to unsolicited approaches. If we become the subject of certain forms of stockholder activism, such as proxy contests or hostile bids, the attention of our management and our Board of Directors may be diverted from execution of our strategy. Such stockholder activism could give rise to perceived uncertainties as to our future strategy, adversely affect our relationships with business partners and make it more difficult to attract and retain qualified personnel. Also, we may incur substantial costs, including significant legal fees and other expenses, related to activist stockholder matters. Our stock price could be subject to significant fluctuation or otherwise be adversely affected by the events, risks and uncertainties of any stockholder activism.

Risks Relating to Investing in Our Common Stock

Our existing principal stockholders hold a substantial amount of our common stock and may be able to influence significant corporate decisions, which may conflict with the interest of other stockholders.

Some of our stockholders own greater than 5% of our outstanding common stock. Our top ten stockholders own approximately 45% of our common stock and can individually, and as a group, influence our operations based upon their concentrated ownership and may also be able to influence the outcome of matters requiring approval of the stockholders, including the election of our directors and other corporate actions.

Our stock price has been, and is likely to continue to be, highly volatile, which could cause the value of an investment in our common stock to decline significantly.

The market prices for securities of biotechnology companies in general have been highly volatile and may continue to be highly volatile in the future. Moreover, our stock price has fluctuated frequently, and these fluctuations are often not related to our financial results. For the twelve months ended March 31, 2025, the 52-week range of the market price of our stock was from $4.03 to $9.50 per share. The following factors, in addition to other risk factors described in this section, may have, and in some cases have had, a significant impact on the market price of our common stock:

- announcements of technological innovations or new products by us or our competitors;
- developments or disputes concerning patents or proprietary rights;
- additional dilution through sales of our common stock or other derivative securities;
- status of new or existing licensing or collaborative agreements and government contracts;
- announcements relating to the status of our programs;
- us or our partners achieving or failing to achieve development milestones;
- publicity regarding actual or potential medical results relating to products under development by us or our competitors;
- publicity regarding certain public health concerns for which we are or may be developing treatments;
- regulatory developments in both the United States and foreign countries;
- public concern as to the safety of pharmaceutical products;
- actual or anticipated fluctuations in our operating results;
- changes in financial estimates or recommendations by securities analysts and the comparison of such estimates to our actual results;
- online automated financial platforms' treatment or classification of our financial information;
- changes in our public guidance;
- changes in the structure of healthcare payment systems, including developments in price control legislation;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, capital commitments or other monetization transactions;
- additions or departures of key personnel or members of our Board of Directors;
- purchases or sales of substantial amounts of our stock by existing stockholders, including officers or directors;
- economic and other external factors or other disasters or crises; and
- period-to-period fluctuations in our financial results.

This volatility could cause the value of an investment in our common stock to decline significantly. In addition, companies that have experienced volatility in the market price of their stock in the past have been subject to securities class action litigation. Securities litigation, and any other type of litigation, brought against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business and adversely affect our results of operations.

If we fail to maintain effective internal control over financial reporting, we may not be able to produce accurate and timely financial statements, which may adversely affect investor confidence in us and our financial reporting, adversely affect our business and operating results and negatively impact the trading price of our common stock.

As a public company, we are required to maintain effective internal control over financial reporting (as described in "*Controls and Procedures*" in Part I, Item 4 of this report), and effective disclosure controls and procedures. If we identify one or more material weaknesses in our internal control over financial reporting, we will not be able to assert that our internal controls and procedures are effective. A material weakness, as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), is a deficiency, or combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of annual or interim

financial statements will not be prevented or detected on a timely basis. In 2023, we identified and timely reported two material weaknesses in our internal control over financial reporting, which management determined to be subsequently remediated as of December 31, 2023 and September 30, 2024, respectively.

Although we believe the financial statements included in this report fairly present, in all material respects, our financial condition, results of operations and cash flows for the periods presented in conformity with U.S. GAAP, any failure to maintain effective internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If our internal control over financial reporting is not effective, investors may lose 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.

Future sales and issuances of securities may dilute the ownership interests of our current stockholders and cause our stock price to decline.

Future sales of our common stock by us or our current stockholders into the public market could cause the market price of our stock to fall. As of April 30, 2025, there were 209,250,274 shares of our common stock outstanding. We may from time to time issue securities in relation to a license arrangement, collaboration, merger or acquisition. We may also sell, for our own account, shares of common stock or other equity securities, from time to time at prices and on terms to be determined at the time of sale.

As of April 30, 2025, there were 46,840,842 stock options and restricted stock units outstanding and 13,242,654 shares available for issuance under our Amended and Restated Stock Incentive Plan (inclusive of the 11,000,000 shares that are subject to stockholder approval at our annual meeting of stockholders to be held on June 12, 2025), 5,794,692 stock options and restricted stock units outstanding and 1,724,932 shares available for issuance under our Amended and Restated Inducement Equity Incentive Plan, and 4,841,013 shares available for issuance under our Amended and Restated Employee Stock Purchase Plan. In addition, we could also make equity grants outside of our Amended and Restated Stock Incentive Plan or Amended and Restated Inducement Equity Incentive Plan. The shares underlying existing stock options, restricted stock units and possible future stock options, stock appreciation rights, restricted stock units and stock awards have been, or will be, registered pursuant to registration statements on Form S-8.

If some or all of such shares are sold or otherwise issued into the public market over a short period of time, our current stockholders' ownership interests may be diluted and the value of all publicly traded shares is likely to decline, as the market may not be able to absorb those shares at then-current market prices. Additionally, such sales and issuances may make it more difficult for us to sell equity securities or equity-related securities in the future at a time and price that our management deems acceptable, or at all.

We have anti-takeover provisions in our corporate charter documents that may result in outcomes with which you do not agree.

Our Board of Directors has the authority to issue up to 5,000,000 shares of undesignated preferred stock and to determine the rights, preferences, privileges and restrictions of those shares without further vote or action by our stockholders. The rights of the holders of any preferred stock that may be issued in the future may adversely affect the rights of the holders of common stock. The issuance of preferred stock could make it more difficult for third parties to acquire a majority of our outstanding voting stock.

In addition, our Certificate of Incorporation provides for staggered terms for the members of the Board of Directors and supermajority approval of the removal of any member of the Board of Directors and prevents our stockholders from acting by written consent. Our Certificate of Incorporation also requires supermajority approval of any amendment of these provisions. These provisions and other provisions of our Amended and Restated By-Laws and of Delaware law applicable to us could delay or make more difficult a merger, tender offer or proxy contest involving us.

We have never paid dividends on our common stock and do not anticipate doing so in the foreseeable future.

We have never paid cash dividends on our stock. We currently intend to retain all future earnings, if any, for use in the operation of our business. Accordingly, we do not anticipate paying cash dividends on our common stock in the foreseeable future.

Our Amended and Restated By-Laws provide that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for certain litigation that may be initiated by our stockholders, which may limit a stockholder's ability to obtain a favorable judicial forum for such disputes with us or our directors, officers or employees.

Our Amended and Restated By-Laws provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, stockholders, employees or agents to us or our stockholders, (iii) any action asserting a claim against us or any of our directors, officers, stockholders, employees or agents arising out of or relating to any provision of the General Corporation Law of Delaware or our Certificate of Incorporation or Amended and Restated By-Laws, or (iv) any action against us or any of our directors, officers, stockholders, employees or agents governed by the internal affairs doctrine of the State of Delaware. This exclusive forum provision does not apply to establish the Delaware Court of Chancery as the forum for actions or proceedings brought to enforce a duty or liability created by the Securities Act of 1933, as amended, or the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction.

This exclusive forum provision may limit a stockholder's ability to choose its preferred judicial forum for disputes with us or our directors, officers, employees or agents, which may discourage the filing of lawsuits with respect to such claims. If a court were to find this exclusive forum provision to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in another jurisdiction, which could adversely affect our business and financial condition.

General Risk Factors

Natural disasters, epidemic or pandemic disease outbreaks, trade wars, armed conflicts, political unrest or other events could disrupt our business or operations or those of our development partners, manufacturers, regulators or other third parties with whom we conduct business now or in the future.

A wide variety of events beyond our control, such as natural disasters (including as a result of climate change), epidemic or pandemic disease outbreaks (such as the COVID-19 pandemic), trade wars, armed conflict, political unrest, government shutdowns, instability in connection with changes in presidential administration in the United States, or other events could disrupt our business or operations or those of our development partners, manufacturers, regulatory authorities, or other third parties with whom we conduct business. These events may cause businesses and government agencies to be shut down, supply chains or trade to be interrupted, slowed, or rendered inoperable, and individuals to become ill, quarantined, or otherwise unable to work and/or travel due to health reasons or governmental restrictions. If our operations or those of third parties with whom we conduct business are impaired or curtailed as a result of these events, the development and commercialization of our products and product candidates could be impaired or halted, which could have a material adverse impact on our business. See, for example, "*Risk Factors—Risks Relating to Our Business—Other Operational Risks—Our business, operations, clinical development or commercialization plans and timelines, and access to capital could be adversely affected by unpredictable and unstable market and economic conditions*." In addition, other events, such as the Ukraine-Russia and Middle East conflicts, or rising tensions between China and Taiwan, could adversely impact our business. For example, the conflicts could lead to sanctions, embargoes, supply shortages, regional instability, geopolitical shifts, cyber-attacks, other retaliatory actions, and adverse effects on macroeconomic conditions, currency exchange rates, and financial markets, which could adversely impact our operations and financial results, as well as those of third parties with whom we conduct business.

We are subject to legal proceedings, which could harm our reputation or result in other losses or unexpected expenditure of time and resources.

From time to time, we may be involved in disputes, including, without limitation, disputes with our employees, collaborative partners, and third-party vendors. We may be called upon to initiate legal proceedings or to defend ourselves in such legal proceedings relating to our relationships with these parties, our decisions and actions or omissions with respect thereto, and our business. In addition, if our stock price is volatile, we may become involved in securities class action lawsuits in the future. Due to the inherent uncertainties in legal proceedings, we cannot accurately predict the ultimate outcome of any such proceeding. An unfavorable outcome in any such proceeding could have an adverse impact on our business, financial condition and results of operations. Any current or future dispute resolution or legal proceeding, regardless of the merits of any such proceeding, could harm our reputation and result in substantial costs and a diversion of management's attention and resources that are needed to successfully run our business.

Item 5. Other Information

Director and Officer Trading Arrangements

During the three months ended March 31, 2025, none of the Company's directors or officers adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement" (as each of those terms is defined in Item 408(a) of Regulation S-K).

Item 6. Exhibits

Number	Description
3.1	Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed December 22, 2006.
3.2	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed July 24, 2007.
3.3	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed May 7, 2014.
3.4	Certificate of Elimination of the Series B Junior Participating Preferred Stock. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed May 13, 2020.
3.5	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.2 to the Company's Form 8-K filed May 13, 2020.
3.6	Amended and Restated By-Laws of BioCryst Pharmaceuticals, Inc., effective January 16, 2024. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed January 18, 2024.
(10.1)*	Consulting Agreement, dated April 9, 2025, by and between BioCryst Pharmaceuticals, Inc. and Anthony Doyle.
(10.2)*	Form of Notice of Grant of Stock Option and Standard Stock Option Agreement under the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan.
(10.3)*	BioCryst Pharmaceuticals, Inc. Amended and Restated Non-Employee Director Compensation Policy, effective April 21, 2025.
(31.1)	Certification of the Principal Executive Officer and Principal Financial Officer Pursuant to Rule 13a-14(a) and Rule 15d-14(a) of the Securities Exchange Act of 1934, as Amended, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
(32.1)**	Certification of the Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
(101)	Financial statements from the Quarterly Report on Form 10-Q of BioCryst Pharmaceuticals, Inc. for the three months ended March 31, 2025, formatted in Inline XBRL: (i) Condensed Consolidated Balance Sheets, (ii) Condensed Consolidated Statements of Comprehensive Income (Loss), (iii) Condensed Consolidated Statements of Cash Flows, (iv) Condensed Consolidated Statements of Stockholders' Deficit, and (v) Notes to Condensed Consolidated Financial Statements, tagged as blocks of text and including detailed tags.
(104)	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).

( ) Filed herewith.

* Management contract.

** The certification is being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and will not be deemed “filed” for purposes of Section 18 of the Exchange Act, except to the extent that the Company specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized on this 6th day of May, 2025.

BIOCRYST PHARMACEUTICALS, INC.

By: /s/ Jon P. Stonehouse

Jon P. Stonehouse

President, Chief Executive Officer and Interim Chief Financial Officer

(Principal Executive Officer, Interim Principal Financial Officer and Interim Principal Accounting Officer)

CONSULTING AGREEMENT

This **CONSULTING AGREEMENT** (the "Agreement") is hereby entered into by and between Anthony Doyle ("Consultant") with an address at 317 Cypress Falls Drive, Cary, NC 27513 and BioCryst Pharmaceuticals, Inc. (together with its Affiliates, "BioCryst") a Delaware corporation, with offices at 4505 Emperor Boulevard, Suite 200, Durham, North Carolina 27703 and shall be effective as of April 9, 2025 (the "Effective Date"). With respect to either party, "Affiliate" means any entity or organization controlling, controlled by or under common control with such party. For the purposes of this definition "control" means the possession, direct or indirect, of the power to direct or cause the direction of the management and policies of an organization or entity, whether the ownership of voting securities, by contract or otherwise.

W I T N E S S E T H:

In consideration of the services rendered by Consultant to BioCryst, the compensation to be paid to Consultant by BioCryst, and the mutual promises and agreements hereinafter set forth, the parties hereto agree as follows:

1. **Term**. This Agreement will commence on the Effective Date and continue in effect through May 31, 2025 (the "Initial Term"). The Initial Term may be extended by mutual written agreement of the parties.

2. **Services**. Consultant agrees to render the requested services ("Services") to BioCryst using Consultant's knowledge, experience, skill and judgement. The Services shall include those set forth in Exhibit A and as agreed by the parties in writing from time to time. Consultant shall deliver to BioCryst, and BioCryst shall be the sole owner of, the Results of Consultant's work under this Agreement. The term "Results" means the work product resulting from Consultant's performance of Services under this Agreement and, includes, without limitation, all deliverables described in such exhibits and all documentation of work performed under this Agreement. Consultant shall report directly to Jon Stonehouse and shall provide his services in accordance with such reasonable instructions. Consultant agrees to keep complete, accurate and authentic accounts, notes, data and records of all Results made by Consultant in the course of this Agreement, and in the manner and form requested by BioCryst. Consultant shall not utilize any third party in the performance of the Services without the prior written consent of BioCryst.

   The Services rendered under this Agreement constitute services in accordance with the terms of the BioCryst Stock Incentive Plan (the "Incentive Plan") and the BioCryst Inducement Equity Incentive Plan (the "Inducement Plan" and together with the Incentive Plan, the "Plans"), and therefore, Consultant's equity awards received while in the employment of BioCryst will continue to vest during the term of the Agreement in accordance with the provisions of the applicable Plans and any related award agreements. Without limiting the foregoing, the parties agree that there has not been and will not be any lapse of "Services" rendered to BioCryst with respect to the transition of Consultant from an Employee of BioCryst immediately prior to the effectiveness of this Agreement, and the existing outstanding equity awards heretofore granted Consultant shall remain in full force and effect, notwithstanding the transition of Consultant from Employee status to Consultant or independent contractor status.

3. **Payment and Expenses**. Consultant shall be compensated for Consulting services in accordance with and as described in Exhibit A as amended from time to time by the parties in writing. In addition, BioCryst shall reimburse Consultant for actual and reasonable out-of-pocket expenses approved by BioCryst in advance and incurred in the performance of the Services. The foregoing compensation and expense reimbursements are Consultant's sole compensation for rendering Services to BioCryst. Consultant shall submit expense reports via an invoice and will not be reimbursed for individual expenses exceeding $ 25.00 without a corresponding receipt. Approved expenses invoiced shall be paid within thirty (30) days of receipt by BioCryst. BioCryst shall not be responsible for paying amounts which are not billed within three (3) months of performing such service or incurring expenses.

4. **Proprietary Information and Inventions**. The Proprietary Information and Inventions Agreement previously entered into by the parties dated April 14, 2020 (the "PIIA") is hereby incorporated by reference and made a part hereof to the same extent and with the same force as if fully set forth herein and shall remain in effect for the term of this Agreement.

5. **Non-Solicitation and Non-Competition**. The Non-Competition and Non-Solicitation Agreement as entered into by Consultant and BioCryst dated March 29, 2020 (the "Non-Compete and Non-Solicitation") is hereby incorporated by reference and made a part hereof to the same extent and with the same force as if fully set forth herein. For purposes of clarification, the parties agree that the non-compete and non-solicitation obligations in the Non-Compete and Non-Solicitation shall apply for one (1) year past the termination of this Agreement.

6. **Representations and Warranties**. Consultant represents and warrants that:

   a. **Non-disclosure of Third-party's Confidential Information.** The performance of all the terms of this Agreement will not breach any agreement to keep in confidence proprietary information acquired by Consultant in confidence or in trust prior to the execution of this Agreement, and Consultant has not entered into, and Consultant agrees not to enter into, any agreement, either written or oral, that conflicts or might conflict with Consultant's performance of the Services and other obligations under this Agreement.

   b. **Compliance with Law**. Consultant will comply with all applicable federal, state and local laws, regulations, professional standards, and industry codes, ordinances and orders, as amended from time to time. In addition, Consultant will materially comply with all reasonable and applicable BioCryst policies and procedures as provided in writing to Consultant.

7. **Limitation of Liability**. NEITHER PARTY SHALL BE LIABLE WITH RESPECT TO ANY SUBJECT MATTER OF THIS AGREEMENT UNDER ANY CONTRACT, NEGLIGENCE, STRICT LIABILITY OR OTHER THEORY FOR ANY INDIRECT, INCIDENTAL, SPECIAL, EXEMPLARY OR CONSEQUENTIAL DAMAGES, INCLUDING WITHOUT LIMITATION, ANY LOSS OR REVENUES OR PROFITS.

8. **Termination**. This Agreement may be terminated by either BioCryst or the Consultant at any time, for any reason, with or without cause, by giving written notice to the other party; termination to be effective upon the other party's receipt of notice. Upon receipt of notice of termination, the receiving party shall cease performing services, unless otherwise notified. The parties will cooperate to ensure a smooth transition of the projects and Services in process at the time of Termination. Termination of this Agreement under the provisions of this section 8 shall not release either party from any obligation and payment becoming due prior to the effective date of termination, if such termination is not caused by the default of either party.

9. **Independent Contractor**. Nothing herein contained shall be deemed to create an agency, joint venture, partnership or franchise relationship between the parties hereto. Consultant acknowledges that he is an independent contractor, is not an agent or employee of BioCryst, is not entitled to any BioCryst employment rights or benefits and is not authorized to act on behalf of BioCryst. Consultant shall be solely responsible for any and all tax obligations of Consultant, including but not limited to, all city, state and federal income taxes, social security tax and other self-employment taxes incurred by Consultant.

10. **Performance**. Consultant's performance under this Agreement shall be conducted with due diligence and in full compliance with the highest professional standards of practice in the industry. Consultant shall comply with all applicable laws and BioCryst safety rules in the course of performing the Services. If Consultant's work requires a license, Consultant has obtained that license and the license is in full force and effect.

11. **Publicity/Material Non-Public Information**. Consultant shall not utilize or publicize the name "BioCryst Pharmaceuticals, Inc.," the ticker symbol "BCRX" or any trademarks of BioCryst, without the

express written consent of BioCryst. Consultant acknowledges that it is aware that the United States securities laws prohibit it or them from purchasing or selling securities of BioCryst while in possession of such material, non-public information.

12. **Privileged Communications**. It is expected that, in furtherance of this Agreement, the parties will, from time to time, disclose to one another privileged communications with counsel, including opinions, memoranda, letters, and other written, electronic, and verbal communications. Such disclosures are made with the understanding that they shall remain privileged and confidential and that they are made in connection with the shared community of legal interests existing between the Parties, including the community of legal interests in avoiding infringement of any valid, enforceable third-party patents and in obtaining patent protection for Sponsored Research IP.

13. **Survival**. Consultant agrees that obligations under Sections 4-6, 11-20 of this Agreement, and any sections which by their nature, shall continue in effect after termination of this Agreement.

14. **Severability**. If one or more provisions of this Agreement are held to be unenforceable under applicable law, such provisions shall be modified to the minimum extent necessary to comply with applicable law and the intent of the parties.

15. **Governing Law**. Consultant agrees that any dispute in the meaning, effect or validity of this Agreement shall be resolved in accordance with the laws of the State of North Carolina without regard to the conflict of laws provisions thereof, and Consultant submits to the exclusive jurisdiction and venue of the federal and state courts located in Wake County, North Carolina.

16. **Binding Nature; Assignment**. This Agreement shall inure to the benefit of and be binding upon the successors and permitted assigns of the parties hereto. Notwithstanding the foregoing, this Agreement may not be assigned, in whole or in part, by either party without the prior written consent of the other party. However, BioCryst may transfer or assign this Agreement, in whole or in part, without prior notice to or consent of Consultant, to an Affiliate of BioCryst or in connection with a merger, consolidation, or a sale or transfer of all or substantially all of the assets to which this Agreement relates, provided that all obligations of BioCryst are assumed by the assignee.

17. **Entire Agreement**. This Agreement together with all Exhibits hereto contains the entire understanding of the parties regarding its subject matter and supersedes all prior negotiations, understandings and agreements between the parties, whether oral or in writing, with respect to the subject matter hereof, and can only be modified by a subsequent written agreement executed by an authorized signatory of BioCryst.

18. **Headings**. The section headings in this Agreement are for purposes of reference only.

19. **Facsimile and Counterparts**. This Agreement may be executed via facsimile and in counterparts, each of which shall be deemed to be an original but all of which together shall constitute one and the same agreement.

Consultant	**BioCryst Pharmaceuticals, Inc.**
Signature: /s/ Anthony Doyle	Signature: /s/ Alane Phillips Barnes
Name: Anthony Doyle	Name: Alane Phillips Barnes
Title: Consultant	Title: Chief Legal Officer

BIOCRYST PHARMACEUTICALS, INC.
NOTICE OF GRANT OF STOCK OPTION

Notice is hereby given of the following stock option grant (the "Option") to purchase shares of the Common Stock of BioCryst Pharmaceuticals, Inc. (the "Company"):

Optionee:	_____
Grant Date:	_____
Option Price:	_____
Number of Optioned Shares:	_____
Expiration Date:	_____

Type of Option:

____ Incentive Stock Option
(up to tax code limits – the portion of the option covering Option Shares in excess of tax code limits shall be accounted for as a non-qualified stock option)

____ Non-Qualified Stock Option

Exercise Schedule: The Option will initially become exercisable for 25% of the Optioned Shares upon Optionee's completion of twelve (12) months of Service (as defined in the Standard Stock Option Agreement) measured from the Grant Date and will become exercisable for the balance of the Optioned Shares at the rate of 25% of the Optioned Shares upon Optionee's completion of each additional year of Service measured from the first anniversary of the Grant Date for the following three years until fully vested on the fourth anniversary of the grant. The Option shall not become exercisable for any additional Optioned Shares following the Optionee's cessation of Service.

Optionee understands that the Option is granted subject to and in accordance with the express terms and conditions of the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan (the "Plan") and agrees to be bound by and conform to the terms and conditions of the Plan, the Plan Prospectus, this Notice of Grant of Stock Option and its accompanying Standard Stock Option Agreement. Optionee acknowledges that notwithstanding anything to the contrary in any written employment agreement between Optionee and the Company, the exercisability of the Option shall not accelerate upon a Change in Control (or any equivalent term as set forth in any applicable written employment agreement) except to the extent provided in the Plan. Optionee acknowledges that copies of the Plan, the Plan Prospectus, and the Standard Stock Option Agreement are available to Optionee on the Company's intranet and have been made available to Optionee.

No Employment or Service Contract: Nothing in the Standard Stock Option Agreement or the Plan shall confer upon the Optionee the right to continue in the Service of the Company (or any parent or subsidiary corporation of the Company employing or retaining Optionee) for any period of specific duration or interfere with or otherwise restrict in any way the rights of the Company or the Optionee, which rights are hereby expressly reserved by each, to terminate Optionee's Service at any time for any reason whatsoever, with or without cause.

By my signature below, I hereby acknowledge receipt of this Option granted on the date shown above, which has been issued to me under the terms and conditions of the Plan.

OPTIONEE

_____
Signature

_____
Date Signed

BIOCRYST PHARMACEUTICALS, INC.

Jon P. Stonehouse
President & CEO

BIOCRYST PHARMACEUTICALS, INC.
STANDARD STOCK OPTION AGREEMENT
WITNESSETH:

RECITALS

A. The Board of Directors of the Company has adopted the Company's Stock Incentive Plan (the "Plan") for the purpose of attracting and retaining the services of selected key employees (including officers and directors), non-employee Board members and consultants and other independent contractors who contribute to the financial success of the Company or its parent or subsidiary corporations.

B. Optionee is an individual who is to render valuable services to the Company or its parent or subsidiary corporations, and this Agreement is executed pursuant to, and is intended to carry out the purposes of, the Plan in connection with the Company's grant of a stock option to Optionee.

NOW, THEREFORE, it is hereby agreed as follows:

1. **Grant of Option**. Subject to and upon the terms and conditions set forth in this Agreement, the Company hereby grants to Optionee, as of the grant date (the "Grant Date") specified in the accompanying Notice of Grant of Stock Option (the "Grant Notice"), a stock option to purchase up to that number of shares of the Company's Common Stock (the "Optioned Shares") specified in the Grant Notice. The Optioned Shares shall be purchasable from time to time during the option term at the option price per share (the "Option Price") specified in the Grant Notice.

2. **Option Term**. This option shall expire at the close of business on the Expiration Date specified in the Grant Notice, unless sooner terminated in accordance with Paragraph 5, 6 or 19 of this Agreement.

3. **Limited Transferability**. During the lifetime of the Optionee, this option (together with its tandem stock appreciation right), shall be exercisable only by the Optionee and shall not be assignable or transferable by the Optionee except for a transfer by will or by the laws of descent and distribution following the Optionee's death. Notwithstanding the foregoing, this option may, to the extent it is a non-statutory stock option, in connection with the Optionee's estate plan, be assigned in whole or in part during the during Optionee's lifetime either as (i) as a gift to one or more members of Optionee's immediate family, to a trust in which Optionee and/or one or more such family members hold more than fifty percent (50%) of the beneficial interest or an entity in which more than fifty percent (50%) of the voting interests are owned by Optionee and/or one or more such family members, or (ii) pursuant to a domestic relations order. The assigned portion shall be exercisable only by the person or persons who acquire a proprietary interest in the option pursuant to such assignment. The terms applicable to the assigned portion shall be the same as those in effect for this option immediately prior to such assignment and shall be set forth in such documents issued to the assignee as the Plan Administrator may deem appropriate.

4. **Exercisability**. This option shall become exercisable for the Optioned Shares in installments as is specified in the Grant Notice. As the option becomes exercisable for one or more installments, the installments shall accumulate and the option shall remain exercisable for the accumulated installments until the Expiration Date or the sooner termination of the option term under this Agreement.

5. **Acceleration; Termination**. The option term specified in Paragraph 2 shall terminate (and this option shall cease to be exercisable) prior to the Expiration Date should one of the following provisions become applicable:

(a) Except to the extent otherwise provided in subparagraphs (ii) through (v) below, should optionee cease to remain in Service at any time during the option term, then the period for exercising this option shall be reduced to a three (3)-month period commencing with the date of such cessation of Service, but in no event shall this option be exercisable at any time after the Expiration Date. Upon the expiration of such three (3) month period or (if earlier) upon the Expiration Date, this option shall terminate and cease to be outstanding. However, should Optionee die during the three

(3)-month period following his or her cessation of Service, the personal representative of the Optionee's estate or the person or persons to whom this option is transferred pursuant to the Optionee's will or in accordance with the laws of descent or distribution shall have a twelve (12)-month period following the date of the Optionee's death during which to exercise this Option, but in no event shall this option be exercisable at any time after the Expiration Date.

(b) Should Optionee, after completing five (5) full years of Service, die while in Service, then the exercisability of each of his or her outstanding options shall automatically accelerate so that each such option shall become fully exercisable with respect to the total number of Optioned Shares at the time subject to such option and may be exercised for all or any portion of such shares. The personal representative of the Optionee's estate or the person or persons to whom this option is transferred pursuant to the Optionee's will or in accordance with the laws of descent and distribution shall have a twelve (12)-month period following the date of the Optionee's death during which to exercise this option, but in no event shall this option be exercisable at any time after the Expiration Date.

(c) Should Optionee die while in Service prior to completing five (5) full years of Service, then the period for which each outstanding vested option held by the Optionee at the time of death shall be exercisable by the Optionee's estate or the person or persons to whom the option is transferred pursuant to the Optionee's will shall be limited to the twelve (12)-month period following the date of the Optionee's death, but in no event shall this option be exercisable at any time after the Expiration Date.

(d) Should Optionee become permanently disabled (as defined in Section 22(e)(3) of the Internal Revenue Code) and cease by reason thereof to remain in Service at any time during the option term, then the period for exercising this option shall be reduced to a twelve (12)-month period commencing with the date of such cessation of Service, but in no event shall this option be exercisable at any time after the Expiration Date. Upon the expiration of such twelve (12)-month period or (if earlier) upon the Expiration Date, this option shall terminate and cease to be outstanding.

(e) Should (A) the Optionee's Service be terminated for misconduct (including, but not limited to, any act of dishonesty, willful misconduct, fraud or embezzlement) or (B) the Optionee make any unauthorized use or disclosure of confidential information or trade secrets of the Company or its parent or subsidiary corporations, then in any such event this option (vested and unvested) shall terminate immediately and cease to be exercisable.

(f) During the limited period of exercisability applicable in accordance with subparagraphs (i) through (iv) above, this option may not be exercised for more than the number of the Optioned Shares (if any) for which this option is, at the time of the Optionee's cessation of Service, exercisable in accordance with the exercise provisions specified in this Agreement and the Grant Notice.

(g) For purposes of this Paragraph 5 and for all other purposes under this Agreement, the following definitional provisions shall be in effect:

(i) The Optionee shall be deemed to remain in Service for so long as the Optionee continues to render periodic services to the Company or any parent or subsidiary corporation, whether as an Employee, a non-employee member of the Company's Board of Directors or an independent consultant or advisor.

(ii) The Optionee shall be deemed to be an Employee and to continue in the Company's employ for so long as the Optionee remains in the employ of the Company or one or more of its parent or subsidiary corporations, subject to the control and direction of the employer entity as to both the work to be performed and the manner and method of performance.

(iii) A corporation shall be considered to be a subsidiary corporation of the Company if it is a member of an unbroken chain of corporations beginning with the Company, provided each such corporation in the chain (other than the last corporation) owns,

at the time of determination, stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain.

(iv) A corporation shall be considered to be a parent corporation of the Company if it is a member of an unbroken chain ending with the Company, provided each such corporation in the chain (other than the Company) owns, at the time of determination, stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain.

6. **Corporate Transactions and Change in Control**.

(a) In the event of a Corporate Transaction or Change in Control (each as defined in the Plan), this option shall be treated as set forth in Article Two, Section III of the Plan.

(b) This Agreement shall not in any way affect the right of the Company to adjust, reclassify, reorganize or otherwise make changes in its capital or business structure or to merge, consolidate, dissolve, liquidate or sell or transfer all or any part of its business or assets.

7. **Adjustment in Optioned Shares**.

(a) In the event any change is made to the Common Stock issuable under the Plan by reason of any stock split, stock dividend, recapitalization, combination of shares, exchange of shares, or other change affecting the outstanding Common Stock as a class without receipt of consideration, then appropriate adjustments shall be made to (i) the total number and/or class of Optioned Shares subject to this option and (ii) the Option Price payable per share in order to reflect such change and thereby preclude a dilution or enlargement of benefits hereunder.

(b) If this option is to be assumed or is otherwise to remain outstanding after a Corporate Transaction, then this option shall be appropriately adjusted to apply and pertain to the number and class of securities which would have been issuable to the Optionee in the consummation of such Corporate Transaction had the option been exercised immediately prior to such Corporate Transaction, and appropriate adjustments shall also be made to the Option Price payable per share, provided the aggregate Option Price payable hereunder shall remain the same.

8. **Privilege of Stock Ownership**. The holder of this option shall not have any shareholder rights with respect to the Optioned Shares until such individual shall have exercised the option and paid the Option Price.

9. **Manner of Exercising Option**.

(a) In order to exercise this option with respect to all or any part of the Optioned Shares for which this option is at the time exercisable, Optionee (or in the case of exercise after Optionee's death, the Optionee's executor, administrator, heir or legatee, as the case may be) must take the following actions:

(i) Provide the Plan Administrator (or its designee) with written notice of the option exercise (the "Exercise Notice") specifying the number of Optioned Shares for which the option is being exercised.

(ii) Pay the aggregate Option Price for the purchased shares in one of the following alternative forms:

(A) full payment in cash or check payable to the Company's order; or

(B) full payment in shares of Common Stock held by Optionee for the requisite period necessary to avoid a charge to the Company's reported earnings and valued at Fair Market Value on the Exercise Date; or

(C) full payment in a combination of shares of Common Stock held for the requisite period necessary to avoid a charge to the Company's earnings and

valued at Fair Market Value on the Exercise Date and cash or check drawn to the Company's order; or

(D) If the Company's outstanding Common Stock is registered under Section 12(g) of the Securities Exchange Act of 1934, as amended (the "1934 Act"), at the time this option is exercised, then payment of the Option Price may also be effected through a broker-dealer sale and remittance procedure pursuant to which Optionee (i) shall provide irrevocable written instructions to a designated brokerage firm to effect the immediate sale of the purchased shares and remit to the Company, out of the sale proceeds available on the settlement date, sufficient funds to cover the aggregate Option Price payable for the purchased shares plus all applicable Federal and state income and employment taxes required to be withheld by the Company by reason of such purchase and (ii) shall provide written directives to the Company to deliver the certificates for the purchased shares directly to such brokerage firm in order to complete the sale.

(iii) Furnish to the Company appropriate documentation that the person or persons exercising the option (if other than Optionee) have the right to exercise this option.

(b) For purposes of subparagraph (a) above and for all other valuation purposes under this Agreement, the Fair Market Value per share of Common Stock on any relevant date shall be determined in accordance with the following provisions:

(i) If the Common Stock is not at the time listed or admitted to trading on any national securities exchange but is traded in the over-the-counter market, the Fair Market Value shall be the mean between the highest bid and lowest asked prices (or, if such information is available, the closing selling price) per share of Common Stock on the date in question in the over-the-counter market, as such prices are reported by the National Association of Securities Dealers through the Nasdaq system or any successor system. If there are no reported bid and asked prices (or closing selling price) for the Common Stock on the date in question, then the mean between the highest bid price and lowest asked price (or the closing selling price) on the last preceding date for which such quotations exist shall be determinative of Fair Market Value.

(ii) If the Common Stock is at the time listed or admitted to trading on any national securities exchange, then the Fair Market Value shall be the closing selling price per share of Common Stock on the date in question on the securities exchange determined by the Plan Administrator to be the primary market for the Common Stock, as such price is officially quoted in the composite tape of transactions on such exchange. If there is no reported sale of Common Stock on such exchange on the date in question, then the Fair Market Value shall be the closing selling price on the exchange on the last preceding date for which such quotation exists.

(iii) If the Common Stock is on the date in question neither listed or admitted to trading on any stock exchange nor traded in the over-the-counter market, then the fair market value shall be determined by the Plan Administrator after taking into account such factors as the Plan Administrator shall deem appropriate.

(c) The Exercise Date shall be the date on which the Exercise Notice is delivered to the Plan Administrator. Except to the extent the sale and remittance procedure specified above is utilized for the exercise of the option, payment of the Option Price for the purchased shares must accompany such notice.

(d) As soon as practical after the Exercise Date, the Company shall issue to or on behalf of Optionee (or other person or persons exercising this option) the Purchased Options Shares via electronic means or through delivery of a certificate or certificates representing the purchased Optioned Shares.

(e) In no event may this option be exercised for any fractional share.

10. **Compliance with Laws and Regulations**.

(a) The exercise of this option (or of its tandem stock appreciation right) and the issuance of Common Stock hereunder shall be subject to compliance by the Company and the Optionee with all applicable requirements of law relating thereto and with all applicable regulations of any stock exchange or over-the-counter market on which shares of the Company's Common Stock may be listed or traded at the time of such exercise and issuance.

(b) In connection with the exercise of this option (or its tandem stock appreciation right), Optionee shall execute and deliver to the Company such representations in writing as may be requested by the Company in order for it to comply with the applicable requirements of federal and state securities laws.

11. **Successors and Assigns**. Except to the extent otherwise provided in Paragraph 3 or 6, the provisions of this Agreement shall inure to the benefit of, and be binding upon, the successors, administrators, heirs, legal representatives and assigns of Optionee and the successors and assigns of the Company.

12. **Liability of Company**.

(a) If the Optioned Shares covered by this Agreement exceed, as of the Grant Date, the number of shares of Common Stock which may without shareholder approval be issued under the Plan, then this option shall be void with respect to such excess shares unless shareholder approval of an amendment sufficiently increasing the number of shares of Common Stock issuable under the Plan is obtained in accordance with the provisions of the Plan.

(b) The inability of the Company to obtain approval from any regulatory body having authority deemed by the Company to be necessary to the lawful issuance and sale of any Common Stock pursuant to this Agreement shall relieve the Company of any liability with respect to the non-issuance or sale of the Common Stock as to which such approval shall not have been obtained. The Company, however, shall use its best efforts to obtain all such approvals.

13. **No Employment or Service Contract**. Nothing in this Agreement or in the Plan shall confer upon the Optionee any right to continue in the Service of the Company (or any parent or subsidiary corporation of the Company employing or retaining Optionee) for any period of time or interfere with or otherwise restrict in any way the rights of the Company (or any parent or subsidiary corporation of the Company employing or retaining Optionee) or the Optionee, which rights are hereby expressly reserved by each, to terminate the Optionee's Service at any time for any reason whatsoever, with or without cause.

14. **Notices**. Any notice required to be given or delivered to the Company under the terms of this Agreement shall be in writing and addressed to the Company in care of the Corporate Secretary at its principal corporate offices. Any notice required to be given or delivered to Optionee shall be in writing and addressed to Optionee at the address indicated below Optionee's signature line on the Grant Notice. All notices shall be deemed to have been given or delivered upon personal delivery or upon deposit in the U.S. mail, postage prepaid and properly addressed to the party to be notified**.**

15. **Construction**. This Agreement and the option evidenced hereby are made and granted pursuant to the Plan and are in all respects limited by and subject to the express terms and provisions of the Plan. All decisions of the Plan Administrator with respect to any question or issue arising under the Plan or this Agreement shall be conclusive and binding on all persons having an interest in this option.

16. **Governing Law**. The interpretation, performance, and enforcement of this Agreement shall be governed by the laws of the State of Delaware without resort to that State's conflict-of-laws rules.

17. **Additional Terms Applicable to an Incentive Stock Option**. In the event this option is designated as an incentive stock option in the Grant Notice, the following terms and conditions shall also apply to the grant:

(a) This option shall cease to qualify for favorable tax treatment as an incentive stock option under the federal tax laws if (and to the extent) this option is exercised for one or more Optioned Shares: (i) more than three (3) months after the date the Optionee ceases to be an Employee

for any reason other than death or permanent disability (as defined in Paragraph 5) or (ii) more than one (1) year after the date the Optionee ceases to be an Employee by reason of permanent disability.

(b) No installment under this option (whether annual or monthly) shall qualify for favorable tax treatment as an incentive stock option under the Federal tax laws if (and to the extent) the aggregate fair market value (determined at the Grant Date) of the Common Stock for which such installment first becomes exercisable hereunder will, when added to the aggregate fair market value (determined as of the respective date or dates of grant) of any earlier installments of Common Stock for which this option or one or more other incentive stock options granted to the Optionee prior to the Grant Date (whether under the Plan or any other option plan of the Company or any Parent or Subsidiary corporations) first become exercisable during the same calendar year, exceed One Hundred Thousand Dollars ($100,000) in the aggregate.

(c) Should the exercisability of this option be accelerated upon a Corporate Transaction or a Change in Control, then this option shall quality for favorable tax treatment as an incentive stock option under the Federal tax laws only to the extent the aggregate Fair Market Value (determined at the Grant Date) of the Common Stock for which this option first becomes exercisable in the calendar year in which the Corporate Transaction or Change in Control occurs does not, when added to the aggregate Fair Market Value (determined as of the respective date or dates of grant) of any earlier installments of Common Stock for which this option or one or more other incentive stock options granted to the Optionee prior to the Grant Date (whether under the Plan or any other option plan of the Company or any Parent or Subsidiary corporations) first become exercisable during the same calendar year, exceed One Hundred Thousand Dollars ($100,000) in the aggregate.

(d) To the extent this option should fail to qualify as an incentive stock option under the Federal tax laws, the Optionee will recognize compensation income in connection with the acquisition of one or more Optioned Shares hereunder, and the Optionee must make appropriate arrangements for the satisfaction of all Federal, State or local income tax withholding requirements and Federal social security employee tax requirements applicable to such compensation income.

18. **Additional Terms Applicable to a Non-Statutory Stock Option**. In the event this option is designated as a non-statutory stock option in the Grant Notice, Optionee hereby agrees to make appropriate arrangements with the Company or parent or subsidiary corporation employing Optionee for the satisfaction of any federal, state or local income tax withholding requirements and federal social security employee tax requirements applicable to the exercise of this option.

19. **Restrictions on Optioned Shares**. The Company may impose such restrictions, conditions or limitations as it determines appropriate as to the timing and manner of any resales by Optionee or other subsequent transfers by Optionee of any Optioned Shares, including without limitation (a) restrictions under an insider trading policy, (b) restrictions designed to delay and/or coordinate the timing and manner of sales by Optionee and other optionees and (c) restrictions as to the use of a specified brokerage firm for such resales or other transfers.

20. **Conflict with Plan**. In the event of a conflict between the terms and conditions of this Agreement and the Plan, the Plan controls.

21. **Electronic Delivery**. Optionee hereby consents to the delivery of information (including, without limitation, information required to be delivered to Recipient pursuant to applicable securities laws) regarding the Company and its subsidiaries and affiliates, the Plan, and the option via Company web site or other electronic delivery.

22. **Headings**. The headings preceding the text of the sections hereof are inserted solely for convenience of reference, and shall not constitute a part of this Agreement, nor shall they affect its meaning, construction or effect.

BIOCRYST PHARMACEUTICALS, INC.

AMENDED AND RESTATED
NON-EMPLOYEE DIRECTOR COMPENSATION POLICY

Effective April 21, 2025

Each member of the Board of Directors (the "***Board***") of BioCryst Pharmaceuticals, Inc. (the "***Company***") who is not also serving as an employee of the Company or any of its subsidiaries (each a "***Director***") will, automatically and without further action by the Board or the Compensation Committee of the Board (the "***Compensation Committee***"), receive the compensation described in this Non-Employee Director Compensation Policy (this "***Policy***"). A Director may decline all or any portion of his or her compensation by giving notice to the Company prior to the date cash is to be paid or equity awards are to be granted, as the case may be. This Policy may be amended at any time in the sole discretion of the Board, or by the Compensation Committee at the recommendation of the Board.

Annual Cash Retainer

Each Director will automatically, and without further action by the Board or Compensation Committee, receive cash compensation solely in the form of annual retainers, as set forth in the table below. The annual retainer amounts are payable in four equal installment payments paid in arrears on a quarterly basis. The annual retainer amount will be pro-rated for a Director who joins the Board or a committee other than at the beginning of the applicable quarter.

Board Retainers	
Annual Retainer	$45,000
Additional Retainer for Board Chair	$35,000
Additional Retainers - Committee Chairs	
Audit Committee	$20,000
Commercialization Committee	$15,000
Compensation Committee	$15,000
Corporate Governance and Nominating Committee	$10,000
Finance Committee	$15,000
Science Committee	$15,000
Additional Retainers – Committee Members (Non-Chair)	
Audit Committee	$10,000
Commercialization Committee	$7,500
Compensation Committee	$7,500
Corporate Governance and Nominating Committee	$5,000
Finance Committee	$7,500
Science Committee	$7,500

Each Director will be given the opportunity to elect to receive, in lieu of cash retainers, a number of shares of the Company's common stock ("***Common Stock***") equivalent in value to the retainer earned by such Director (excluding any committee retainers). Each Director may elect to receive either 50% or 100% of his or her cash retainer(s) (excluding any committee retainers) in the form of Common Stock. These shares will be distributed four times a year, in line with the quarterly cash retainer payments. The number of shares to be distributed will be determined using the closing price of Common Stock on the last business day of the applicable three-month period. Elections to receive shares of Common Stock in lieu of cash retainers for a year shall be made as of the date of each annual meeting of the Company's stockholders, effective until the subsequent annual meeting.

Equity Compensation

The equity compensation set forth below will be granted under the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan, as amended (the "***Plan***"). Capitalized terms not defined herein have the meaning specified in the Plan. All stock options granted under this Policy will be non-statutory stock options, with an exercise price per share equal to 100% of the fair market value per share of the Common Stock on the Grant Date, and a term of 10 years from the date of grant (subject to earlier termination in connection with a termination of service as provided in the award agreement).

1. Initial Grant: Each individual who first becomes a Director on or after the effective date of this Policy shall be granted on the date of initial election or appointment awards with an aggregate dollar value (based on the aggregate accounting value on the date of grant) of up to the product of (i) $500,000 and (ii) a fraction, the numerator of which is the number of months (rounded to the nearest whole number) remaining between the date such Board member first became a non-employee Board member and the Company's next scheduled Annual Stockholders Meeting (or the one year anniversary of the most recent Annual Stockholders Meeting if the next Annual Stockholders Meeting has not yet been scheduled), and the denominator of which is 12. Such award shall be made 60% in the form of non-statutory options and 40% in restricted stock units ("RSUs"). Subject to the proviso in Section VI of Article One of the Plan, (i) such option grant shall vest and become exercisable for the option shares in 36 equal monthly installments over a 3-year period measured from the grant date, and (ii) such RSUs shall vest in three equal annual installments beginning on the twelve (12)-month anniversary of the grant date.

2. Annual Grant: Immediately following each Annual Stockholders Meeting of the Company, each individual who is then serving as a Director (except for those individuals first elected or appointed to serve as non-employee Board members at such meeting or as of such date), shall be granted awards with an aggregate dollar value (based on the aggregate accounting value on the date of grant) of $325,000. Such award shall be made 60% in the form of non-statutory options and 40% in RSUs. Subject to the proviso in Section VI of Article One of the Plan, such option grant shall vest and become exercisable for the option shares, and such grant of RSUs shall vest, on the twelve (12)-month anniversary of the grant date.

Should the grantee cease to serve as a Board member for any reason while holding one or more vested option grants under this Director Compensation Policy, then such grantee shall have the remainder of the ten (10) year term of each such option in which to exercise each such vested option for any or all of the shares of Common Stock for which the option is exercisable at the time of such cessation of Board service. Each such unvested option shall immediately terminate and cease to be outstanding, at the time of such cessation of Board service, with respect to any shares for which the option is not otherwise at that

time exercisable. Upon the expiration of the ten (10)-year option term, the grant shall terminate and cease to be outstanding in its entirety. Upon the death of the grantee, whether before or after cessation of Board service, any option held by grantee at the time of grantee's death may be exercised, for any or all of the shares of Common Stock for which the option was vested and exercisable at the time of cessation of Board service by the grantee and which have not been theretofore exercised by the grantee, by the personal representative of the grantee's estate or by the person or persons to whom the option is transferred pursuant to the grantee's will or in accordance with the laws of descent and distribution. Any such exercise must occur during the remainder of the ten (10) year term of such option.

Reimbursement of Expenses

Upon presentation of documentation of such expenses reasonably satisfactory to the Company, each Director is entitled to be reimbursed for reasonable travel and other expenses incurred in connection with attending meetings of the Board and any Committee of the Board on which he or she serves and while representing the Company in conducting certain business.

Approved by the Board of Directors: April 21, 2025

Exhibit 31.1

CERTIFICATIONS

I, Jon P. Stonehouse, certify that:

1. I have reviewed this quarterly report on Form 10-Q of BioCryst Pharmaceuticals, Inc.;

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

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

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

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

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

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

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

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

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

Date: May 6, 2025

/s/ Jon P. Stonehouse

Jon P. Stonehouse

President, Chief Executive Officer and Interim Chief Financial Officer

(Principal Executive Officer and Interim Principal Financial Officer)

Exhibit 32.1

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

In connection with the Quarterly Report of BioCryst Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended March 31, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned officer, Jon P. Stonehouse, certifies to the best of his knowledge and in his respective capacities, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

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

/s/ Jon P. Stonehouse

Jon P. Stonehouse

President, Chief Executive Officer and Interim Chief Financial Officer

(Principal Executive Officer and Interim Principal Financial Officer)

Date: May 6, 2025