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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 June 30, 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 July 31, 2025, the registrant had 209,920,430 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 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;
- competitive companies, technologies, and our industry;
- business uncertainties, contractual restrictions on our operations, and additional expenses during the pending transaction with Neopharmed Gentili S.p.A. ("Neopharmed");
- adverse effects on our business and financial condition that may result if we fail to complete the pending transaction with Neopharmed; and
- our plans for use of the proceeds received in connection with the closing of the transaction with Neopharmed.

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.
- We face risks relating to the pending transaction with Neopharmed, including that the pending transaction may be delayed or terminated, which could have a material adverse impact on our business, financial condition, results of operations and stock price, and the pending transaction may disrupt our ongoing business operations and restrict our business activities.
- 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 may 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)

	June 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 88,033	$ 104,713
Restricted cash	492	210
Short-term investments	172,005	216,137
Trade receivables	91,177	79,069
Inventory, net	5,044	8,087
Prepaid expenses and other current assets	12,541	13,752
Current assets held for sale	29,170	—
Total current assets	398,462	421,968
Long-term inventory, net	22,205	23,187
Property and equipment, net	8,457	7,777
Long-term investments	10,101	20,323
Right of use assets	11,546	12,008
Other assets	3,592	5,157
Non-current assets held for sale	2,825	—
Total assets	$ 457,188	$ 490,420
Liabilities and Stockholders' Deficit		
Current liabilities:		
Accounts payable	$ 8,589	$ 11,644
Accrued expenses	107,114	113,292
Operating lease liabilities	392	937
Finance lease liabilities	1,616	1,835
Royalty financing obligations	35,741	32,676
Current liabilities held for sale	23,708	—
Total current liabilities	177,160	160,384
Operating lease liabilities	8,489	7,924
Finance lease liabilities	2,043	2,124
Royalty financing obligations	447,842	481,053
Secured term loan	242,794	314,869
Non-current liabilities held for sale	454	—
Total liabilities	878,782	966,354
Stockholders' deficit:		
Preferred stock, $0.01 par value; shares authorized - 5,000; no shares issued and outstanding at June 30, 2025 and December 31, 2024	—	—
Common stock, $0.01 par value; shares authorized - 450,000; shares issued and outstanding – 209,905 at June 30, 2025 and 208,543 at December 31, 2024	2,099	2,085
Additional paid-in capital	1,339,584	1,291,100
Accumulated other comprehensive income	1,646	921
Accumulated deficit	(1,764,923)	(1,770,040)
Total stockholders' deficit	(421,594)	(475,934)
Total liabilities and stockholders' deficit	$ 457,188	$ 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 June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
Revenues	$ 163,353	$ 109,332	$ 308,887	$ 202,093
Expenses:				
Cost of product sales	2,798	1,699	7,366	2,964
Research and development	43,386	37,623	80,656	84,116
Selling, general and administrative	87,383	61,249	169,852	120,740
Total operating expenses	133,567	100,571	257,874	207,820
Income (loss) from operations	29,786	8,761	51,013	(5,727)
Other income (expense):				
Interest income	2,516	3,554	5,540	7,585
Interest expense	(21,582)	(24,733)	(45,076)	(49,239)
Foreign currency losses, net	(63)	(84)	(62)	(135)
Loss on extinguishment of debt	(4,171)	—	(4,171)	—
Total other expense	(23,300)	(21,263)	(43,769)	(41,789)
Income (loss) before income taxes	6,486	(12,502)	7,244	(47,516)
Income tax expense	1,401	172	2,127	537
Net income (loss)	$ 5,085	$ (12,674)	$ 5,117	$ (48,053)
Other comprehensive income (loss):				
Foreign currency translation adjustment	702	(140)	1,068	(393)
Unrealized loss on available for sale investments	(188)	(74)	(343)	(387)
Total other comprehensive income (loss)	514	(214)	725	(780)
Net comprehensive income (loss)	$ 5,599	$ (12,888)	$ 5,842	$ (48,833)
Net income (loss) per common share: basic	**$ 0.02**	**$ (0.06)**	**$ 0.02**	**$ (0.23)**
Weighted average shares of common stock outstanding: basic	**209,519**	**206,425**	**209,203**	**206,244**
Net income (loss) per common share: diluted	**$ 0.02**	**$ (0.06)**	**$ 0.02**	**$ (0.23)**
Weighted average shares of common stock outstanding: diluted	**219,886**	**206,425**	**217,574**	**206,244**

See accompanying notes to condensed consolidated financial statements.

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

	Six Months Ended June 30,	
	2025	**2024**
Cash flows from operating activities:		
Net income (loss)	$ 5,117	$ (48,053)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Depreciation and amortization	660	626
Inventory obsolescence	1,026	1
Stock-based compensation expense	42,672	26,825
Payment of Pharmakon PIK Interest	(5,492)	—
Non-cash interest expense on royalty financing obligations	26,916	28,450
Non-cash interest expense on secured term loan and amortization of debt issuance costs	1,003	10,591
Amortization of discount on investments, net	(2,533)	(6,674)
Loss on extinguishment of debt	4,171	—
Changes in operating assets and liabilities:		
Increase in receivables	(21,295)	(12,085)
Decrease (increase) in inventory	568	(481)
Decrease in prepaid expenses and other assets	28	2,851
Decrease in royalty financing obligations	(36,921)	(33,069)
Decrease in accounts payable and accrued expenses	(2,135)	(24,034)
Net cash provided by (used in) operating activities	13,785	(55,052)
Cash flows from investing activities:		
Acquisitions of property and equipment	(322)	(390)
Purchases of investments	(78,103)	(200,303)
Sales and maturities of investments	134,647	227,000
Net cash provided by investing activities	56,222	26,307
Cash flows from financing activities:		
Net proceeds from common stock issued under stock-based compensation plans	5,811	1,945
Common stock issued to directors in lieu of cash retainer	15	23
Repayment of Pharmakon term loan principal	(69,508)	—
Prepayment premium and fees on Pharmakon term loan	(2,250)	—
Principal payments on royalty financing obligations	(3,956)	—
Withholding taxes paid on stock-based awards	(2,217)	(4,190)
Principal payments on finance lease liabilities	(1,052)	(792)
Net cash used in financing activities	(73,157)	(3,014)
Effect of exchange rates on cash, cash equivalents and restricted cash	1,810	(483)
Decrease in cash, cash equivalents and restricted cash, including cash classified within current assets held for sale	(1,340)	(32,242)
Less: net increase in cash and cash equivalents classified within current assets held for sale	(15,058)	—
Net decrease in cash, and cash equivalents, and restricted cash	(16,398)	(32,242)
Cash, cash equivalents and restricted cash:		
Beginning of period	106,323	112,447
End of period	$ 89,925	$ 80,205
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 88,033	$ 78,410
Restricted cash	492	1,795
Restricted cash in other assets	1,400	—
Total cash, cash equivalents and restricted cash	$ 89,925	$ 80,205

Supplemental cash flow disclosure:				
Cash paid for interest	$	16,679	$	10,041
Cash paid for taxes	$	1,228	$	865
Purchases of property and equipment included in accounts payable	$	1,069	$	—
Taxes withheld on stock-based awards included in accrued expenses	$	343	$	189

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)**
Net income	—	—	—	—	5,085	5,085
Other comprehensive income	—	—	—	514	—	514
Exercise of stock options	560	6	3,415	—	—	3,421
Vesting of restricted stock units	136	1	(1)	—	—	—
Issuance of shares to directors in lieu of cash retainer	1	—	9	—	—	9
Stock-based compensation expense	—	—	21,304	—	—	21,304
Balance at June 30, 2025	**209,905**	**$ 2,099**	**$ 1,339,584**	**$ 1,646**	**$ (1,764,923)**	**$ (421,594)**

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, 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, net	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)**
Net loss	—	—	—	—	(12,674)	(12,674)
Other comprehensive income	—	—	—	(214)	—	(214)
Exercise of stock options, net	80	1	263	—	—	264
Vesting of restricted stock units	200	2	(2)	—	—	—
Issuance of shares to directors in lieu of cash retainer	2	—	12	—	—	12
Stock-based compensation expense	—	—	13,173	—	—	13,173
Balance at June 30, 2024	**206,629**	**$ 2,066**	**$ 1,250,983**	**$ 557**	**$ (1,729,212)**	**$ (475,606)**

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 June 30, 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 13—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.

Assets Held for Sale

The Company classifies its long-lived assets to be sold as held for sale when the six criteria in ASC 360, *Property, Plant and Equipment* are met. This generally occurs when an agreement to sell exists, or when management has committed to a plan to sell the assets within one year. Assets and liabilities to be disposed of together as a group in a single transaction ("disposal groups") are classified as held for sale if their carrying amounts are principally expected to be recovered through a sale transaction rather than through continuing use.

The long-lived assets included in a disposal group are reported at the lower of their carrying value or fair value less cost to sell, beginning in the period the held for sale criteria are met. Prior to disposal, losses are recognized for any initial or subsequent write-down to fair value less cost to sell. Any gains or losses not previously recognized that result from the sale of a disposal group shall be recognized at the date of sale. Upon designation as an asset held for sale, the Company stops recording depreciation and amortization expense on long-lived assets. The Company assesses the fair value of a long-lived asset less any costs to sell at each reporting period and until the asset is no longer classified as held for sale.

Revenue Recognition

The Company recorded the following revenues for the three and six months ended June 30, 2025 and 2024 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
Product sales, net	$ 162,430	$ 107,987	$ 306,217	$ 197,259
Collaborative and other revenues	923	1,345	2,670	4,834
Total revenues	**$ 163,353**	**$ 109,332**	**$ 308,887**	**$ 202,093**

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 $1,892 and $1,610 as of June 30, 2025 and December 31, 2024, respectively, and primarily consisted of $1,400 as of June 30, 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 June 30, 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 4—Investments*"). There were no liabilities measured at fair value on a recurring basis as of June 30, 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 12—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. The Company's inventory also 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 June 30, 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 comprised of compensation and benefits and related costs associated with sales and marketing, finance, human resources, legal, information technology, quality, safety and regulatory activities related to marketed products, and other general and administrative personnel. Additionally, selling, general and administrative expenses are comprised of market research, marketing, advertising and legal expenses, including patent costs, licenses and other 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 June 30, 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. 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. 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 7—Royalty Financing Obligations*") and the term loan borrowings under the Pharmakon Loan Agreement (see "*Note 8—Debt*") during the six months ended June 30, 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.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law in the U.S., which contains a broad range of tax reform provisions affecting businesses. The Company is evaluating the full effects of the legislation on the Company, including the Company's estimated annual effective tax rate and cash tax position, but the Company expects that the legislation will not have a material impact on the Company's financial statements. As the legislation was signed into law after the close of second quarter, the financial impacts are not included within the Condensed Consolidated Statement of Comprehensive Income (Loss).

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 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 and six months ended June 30, 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. RAPIVAB is also generally distributed through the same specialty pharmacy in the United States. 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 86% and 85% of total net revenues for the three and six months ended June 30, 2025, respectively. Revenue where the specialty pharmacy is considered the customer was approximately 88% of total net revenues for both the three and six months ended June 30, 2024.

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 six months ended June 30, 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 — Assets and Liabilities Held for Sale

On June 27, 2025, the Company entered into a definitive agreement (the "Stock Purchase Agreement") with BioCryst Ireland Limited ("BioCryst Ireland"), a wholly owned subsidiary that operates the European ORLADEYO business, and Neopharmed Gentili S.p.A. ("Neopharmed"). Under the terms of the Stock Purchase Agreement, the Company has agreed to sell to Neopharmed all of its equity interests in BioCryst Ireland, which, together with its subsidiaries, holds certain assets, rights and employees related to our European ORLADEYO business. In connection with the sale, the Company will enter into several agreements with Neopharmed, including an Amended & Restated Intellectual Property License Agreement, a Trademark License Agreement, a Global Brand and Support Agreement, a Supply Agreement, and a Transition Services Agreement.

Under the terms of the Stock Purchase Agreement, Neopharmed will pay $250,000 in upfront consideration for the European assets and rights related to ORLADEYO, with up to an additional $14,000 in contingent milestone payments based on future sales performance in Central and Eastern Europe. Neopharmed will also pay a $15,000 royalty release fee to RPI 2019 Intermediate Finance Trust. The transaction is expected to close in the fourth quarter of 2025, subject to customary closing conditions. The Company intends to use the transaction proceeds to retire all remaining term debt.

As of June 30, 2025, the Company concluded that the criteria under ASC 360-10-45-9 for classification as held for sale were met for BioCryst Ireland. Accordingly, the assets and liabilities of BioCryst Ireland are classified as a disposal group held for sale in the Company's condensed consolidated balance sheet. The disposal group is not considered a discontinued operation under ASC 205-20, as it does not represent a strategic shift that will have a major effect on the Company's operations or financial results. Therefore, the results of operations for BioCryst Ireland are included in income from continuing operations for all periods presented.

No impairment loss was recognized in connection with the classification of the disposal group as held for sale, as the estimated fair value less costs to sell exceeded its carrying value.

The following table summarizes the assets and liabilities of BioCryst Ireland that are classified as held for sale on the unaudited condensed consolidated balance sheet as of June 30, 2025 (in thousands):

	June 30, 2025
Assets	
Current assets:	
Cash and cash equivalents	$ 15,058
Trade receivables	10,403
Inventory, net	2,043
Prepaid expenses and other current assets	1,666
Current assets held for sale	29,170
Non-current assets held for sale	2,825
Total assets held for sale	$ 31,995
Liabilities	
Current liabilities:	
Accounts payable	$ 1,266
Accrued expenses	22,120
Other current liabilities	322
Current liabilities held for sale	23,708
Non-current liabilities held for sale	454
Total liabilities held for sale	$ 24,162

Note 3 — Revenue

The Company recorded the following revenues for the three and six months ended June 30, 2025 and 2024 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
ORLADEYO:				
U.S.	$ 140,338	$ 95,909	$ 260,497	$ 175,875
Outside of U.S.	16,499	12,379	30,583	21,280
Total ORLADEYO	156,837	108,288	291,080	197,155
Other revenues	6,516	1,044	17,807	4,938
Total revenues	$ 163,353	$ 109,332	$ 308,887	$ 202,093

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 and six months ended June 30, 2025 and 2024.

Note 4 — 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):

	June 30, 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	$ —	$ 182,106	$ —	$ 182,106
Total assets	$ —	$ 182,106	$ —	$ 182,106

	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 June 30, 2025, the Company had six securities with a total estimated fair value of $59,117 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):

	June 30, 2025				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its agencies	$ 180,771	$ 1,241	$ 114	$ (20)	$ 182,106
Total investments	$ 180,771	$ 1,241	$ 114	$ (20)	$ 182,106

	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 June 30, 2025 and December 31, 2024 (in thousands):

	June 30, 2025	December 31, 2024
Maturing in one year or less	$ 172,005	$ 216,137
Maturing after one year through two years	10,101	20,323
Total investments	$ 182,106	$ 236,460

Note 5 — Trade Receivables

Product Sales

Receivables from product sales are recorded for amounts due to the Company related to sales of ORLADEYO and peramivir. At June 30, 2025 and December 31, 2024, receivables, net of reserves, related to sales of ORLADEYO were $87,575 and $76,282, respectively. At June 30, 2025 and December 31, 2024, receivables related to sales of peramivir were $381 and $564, respectively.

At June 30, 2025, there was $10,403 of accounts receivable, net of reserves, included in current assets held for sale. See "*Note 2—Assets and Liabilities Held for Sale*" for further details.

Collaborations

At June 30, 2025 and December 31, 2024 receivables from collaborations related to receivables from the Company's royalty partners were $3,221 and $2,223, respectively.

Note 6 — Inventory

At June 30, 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):

	June 30, 2025	December 31, 2024
Raw materials	$ 7,252	$ 10,006
Work-in-process	15,943	16,152
Finished goods	5,965	7,765
Total inventory	29,160	33,923
Reserves	(1,911)	(2,649)
Total inventory, net	$ 27,249	$ 31,274

As of June 30, 2025, there was $2,043 of inventory, net in current assets held for sale and $549 of inventory, net in non-current assets held for sale. See "*Note 2—Assets and Liabilities Held for Sale*" for further details.

Note 7 — 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 8 herein) through its payoff and termination on April 17, 2023 or, subsequent to that date, the Pharmakon Loan Agreement (as defined in Note 8 herein), as applicable. See "*Note 8—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 June 30, 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 June 30, 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 and six months ended June 30, 2025 (in thousands) as well as the effective interest rate as of June 30, 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 Payable	(11,818)	(1,021)	(13,486)	(26,325)
Balance as of March 31, 2025	$ 178,351	$ 180,119	$ 142,448	$ 500,918
Non-cash Interest expense on Royalty financing obligations	9,804	—	3,598	13,402
Royalty Revenues Payable	(13,798)	(1,193)	(15,746)	(30,737)
Balance as of June 30, 2025	$ 174,357	$ 178,926	$ 130,300	$ 483,583
Effective interest rate	21.7 %	— %	10.1 %	

Cash paid for interest on the royalty financing obligations was $22,372 and $36,921 for the three and six months ended June 30, 2025, respectively. Cash paid for interest on the royalty financing obligations was $17,457 and $33,069 for the three and six months ended June 30, 2024, respectively.

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 8 — 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 12.29% and 13.31% for the three months ended June 30, 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.

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. Additionally, unamortized deferred financing costs of $1,921 associated with the Pharmakon Term Loan were written-off at the time of repayment. Collectively, the prepayment and unamortized deferred financing costs totaled $4,171 are reflected as a one-time loss on extinguishment of debt on the Condensed Consolidated Statements of Comprehensive Income (Loss).

As of June 30, 2025, borrowings, including Pharmakon PIK Interest Payments, totaled $248,704 under the Pharmakon Loan Agreement. Interest expense on the Tranche A Loan for the three and six months ended June 30, 2025 was $7,526 and $16,679, respectively, all of which was paid at the end of the quarterly period.

As of June 30, 2024, borrowings, including the Pharmakon PIK Interest Payments, totaled $323,704 under the Pharmakon Loan Agreement. Interest expense on the Tranche A Loan for the three and six months ended June 30, 2024 was $10,108 and $20,082, respectively. As allowable under the Pharmakon Loan Agreement, the Company designated and accounted for 50% of the quarterly interest payment for the six months ended June 30, 2024 as a Pharmakon PIK Interest Payment and the total amount of $10,041 was added to the outstanding principal balance of the borrowing. The remaining 50% of the total quarterly interest payment of $10,041 was paid at the end of the quarterly period.

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.

Debt fees and issuance costs incurred 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 $459 and $1,003 was recognized for the three

and six months ended June 30, 2025, respectively. Deferred financing amortization of $284 and $550 was recognized for the three and six months ended June 30, 2024, respectively.

On July 24, 2025, the Company made a partial prepayment on the Pharmakon Term Loan. See *"Note 16—Subsequent Events"* for additional information.

Note 9 — 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 June 30, 2025. The Company's real estate agreements expire at various times between 2026 through 2033 and include renewal options that range from three to five years in length.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Operating lease expense	$ 526	$ 582	$ 1,070	$ 1,172
Finance lease expense:				
Amortization of right-of-use assets	$ 554	$ 416	$ 1,092	$ 823
Interest on lease liabilities	90	73	184	150
Total finance lease expense	$ 644	$ 489	$ 1,276	$ 973

Other supplemental information related to leases was as follows:

	June 30, 2025	December 31, 2024
Weighted average remaining lease term:		
Operating leases	9.9 years	9.0 years
Finance leases	2.6 years	2.6 years
Weighted average discount rate:		
Operating leases	10.71 %	10.91 %
Finance leases	9.42 %	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	June 30, 2025	December 31, 2024
Operating lease assets:			
Operating lease assets, net	*Right of use assets*	$ 7,916	$ 8,061
Operating lease liabilities:			
Current operating lease liabilities	*Operating lease liabilities – current liabilities*	$ 392	$ 937
Non-current operating lease liabilities	*Operating lease liabilities – long-term liabilities*	8,489	7,924
Total operating lease liabilities		$ 8,881	$ 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	June 30, 2025	December 31, 2024
Finance lease assets:			
Finance lease assets, net	*Right of use assets*	$ 3,630	$ 3,947
Finance lease liabilities:			
Current finance lease liabilities	*Finance lease liabilities – current liabilities*	$ 1,616	$ 1,835
Non-current finance lease liabilities	*Finance lease liabilities – long-term liabilities*	2,043	2,124
Total finance lease liabilities		$ 3,659	$ 3,959

Operating lease assets are recorded net of accumulated amortization of $2,265 and $6,065 as of June 30, 2025 and December 31, 2024, respectively. Finance lease assets are recorded net of accumulated amortization of $4,988 and $4,059 as of June 30, 2025 and December 31, 2024, respectively.

Maturities of lease liabilities as of June 30, 2025 are as follows (in thousands):

	Operating Leases	Finance Leases
2025 (remaining)	$ 464	$ 1,082
2026	1,153	1,512
2027	1,462	1,042
2028	1,506	478
2029	1,537	23
Thereafter	9,024	—
Total lease payments	15,146	4,137
Less imputed interest	(6,265)	(478)
Total	$ 8,881	$ 3,659

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

	Six Months Ended June 30,	
	2025	2024
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows for finance leases	$ 184	$ 150
Operating cash flows for operating leases	$ 903	$ 1,084
Operating lease assets obtained in exchange for operating lease liabilities:	$ 280	$ 156
Finance lease assets obtained in exchange for finance lease liabilities:	$ 844	$ 155
Non-cash increase to operating lease assets due to remeasurement of operating lease liabilities:	$ 786	$ 365

Note 10 — Stockholders' Equity

Shares Reserved for Future Issuance of Common Stock

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

	June 30, 2025	December 31, 2024
Shares reserved for exercises of outstanding stock options	42,093	44,240
Shares reserved for vesting of restricted stock units	9,798	10,112
Shares reserved for future issuance under the Stock Incentive Plan	13,332	1,065
Shares reserved for future issuance under the Inducement Equity Incentive Plan	1,725	1,699
Shares reserved for future issuance under the Employee Stock Purchase Plan	4,841	5,042
Total shares reserved for future issuance	71,789	62,158

Note 11 — Stock-Based Compensation

As of June 30, 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 and approved by the Company's stockholders 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 June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Incentive Plan	$ 19,377	$ 10,934	$ 38,609	$ 22,500
Inducement Plan	1,741	2,084	3,673	4,007
ESPP	186	155	390	318
Stock-based compensation expense	**$ 21,304**	**$ 13,173**	**$ 42,672**	**$ 26,825**

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	233	10.32		
Exercised	(599)	5.43		$ 2,407
Cancelled or Forfeited	(1,326)	10.43		
Outstanding at June 30, 2025	37,390	$ 7.93	6.35	$ 61,951
Exercisable at June 30, 2025	23,804	$ 7.92	5.03	$ 42,079

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	246	9.55
Vested	(197)	8.90
Forfeited	(422)	7.63
Unvested at June 30, 2025	8,916	$ 7.75

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 six months of 2025 and 2024 was $9.55 and $5.80, 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.

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	(247)	5.65		$ 981
Cancelled or Forfeited	(231)	11.55		
Outstanding at June 30, 2025	4,703	$ 8.43	6.45	$ 10,636
Exercisable at June 30, 2025	3,269	$ 7.82	5.82	$ 9,095

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	254	8.58
Vested	(123)	8.78
Forfeited	(72)	8.24
Unvested at June 30, 2025	882	$ 8.53

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 six months of 2025 and 2024 was $8.58 and $5.36, 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.

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 six months ended June 30, 2025 and 2024.

	Six Months Ended June 30,	
	2025	**2024**
Expected Life in Years	5.9	5.8
Expected Volatility	77.1 %	83.7 %
Expected Dividend Yield	0.0 %	0.0 %
Risk-Free Interest Rate	4.1 %	4.3 %
Weighted average grant date fair value per share	$ 7.12	$ 4.56

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 six months ended June 30, 2025 and 2024.

	Six Months Ended June 30,	
	2025	**2024**
Expected Life in Years	5.9	5.8
Expected Volatility	82.7 %	83.3 %
Expected Dividend Yield	0.0 %	0.0 %
Risk-Free Interest Rate	4.4 %	4.4 %
Weighted average grant date fair value per share	$ 5.73	$ 3.90

Note 12 — 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. The Company delivered 8.6 thousand and 16.8 thousand doses of peramivir under this contract for the three and six months ended June 30, 2025, respectively. The Company recorded revenue of $6,208 and $12,206 for the three and six months ended June 30, 2025, respectively. As the contract was entered into in September 2024, there were no doses delivered and no revenue recorded for the three months ended June 30, 2024. On May 15, 2025, ASPR notified the Company of its intent to not exercise any additional optional ordering periods available under the agreement.

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 13 — 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 June 30, 2025 the Company's cash, cash equivalents, and investments were $285,197, of which $15,058 was considered held for sale (see *Note 2 - Assets Held for Sale"*). As of December 31, 2024 the Company's cash, cash equivalents, and investments were $341,173.

The following table illustrates information about segment revenues, significant segment expenses, and segment net income (loss) for the three and six months ended June 30, 2025 and 2024 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenues	$ 163,353	$ 109,332	$ 308,887	$ 202,093
Less[1]:				
Cost of product sales	2,798	1,699	7,366	2,964
Research and development				
Berotralstat	12,073	13,146	19,898	25,104
Factor D Program	—	5,557	1,400	16,037
BCX17725	8,381	5,250	15,689	13,009
Avoralstat	8,307	4,137	15,687	8,173
Other research, preclinical and development costs	14,625	9,533	27,982	21,793
Selling, general and administrative	87,383	61,249	169,852	120,740
Foreign currency losses, net	63	84	62	135
Interest income	(2,516)	(3,554)	(5,540)	(7,585)
Interest expense	21,582	24,733	45,076	49,239
Income tax expense	1,401	172	2,127	537
Loss on extinguishment of debt	4,171	—	4,171	—
Segment net income (loss)	**5,085**	**(12,674)**	**5,117**	**(48,053)**
Reconciliation of segment profit or loss:				
Adjustments and reconciling items	—	—	—	—
Consolidated net income (loss)	**$ 5,085**	**$ (12,674)**	**$ 5,117**	**$ (48,053)**

[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 3—Revenue*".

Note 14 — 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 15 — Net Income (Loss) Per Share

Basic and diluted net income (loss) per share for the three and six months ended June 30, 2025 and 2024 were calculated as follows (in thousands, except per share amounts):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Numerator:				
Net income (loss)	$ 5,085	$ (12,674)	$ 5,117	$ (48,053)
Denominator:				
Weighted average shares of common stock outstanding: basic	209,519	206,425	209,203	206,244
Net income (loss) per common share: basic	$ 0.02	$ (0.06)	$ 0.02	$ (0.23)
Effect of dilutive securities:				
Stock options to purchase common stock	6,360	—	5,380	—
Unvested restricted stock unit awards	3,973	—	2,966	—
Shares issuable under the employee stock purchase plan	34	—	25	—
Dilutive potential common shares	10,367	—	8,371	—
Weighted average shares of common stock outstanding: diluted	219,886	206,425	217,574	206,244
Net income (loss) per common share: diluted	$ 0.02	$ (0.06)	$ 0.02	$ (0.23)

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 and six months ended June 30, 2025 and 2024.

For the three and six months ended June 30, 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 and six months ended June 30, 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 following table summarizes potential shares of common stock that were excluded from the computation of diluted net income (loss) per share attributable to common stockholders as they were anti-dilutive:

	As of June 30, 2025	As of June 30, 2024
Outstanding stock options	21,790	39,013
Unvested restricted stock unit awards	248	5,986
Total	22,038	44,999

Note 16 — Subsequent Events

On July 24, 2025, as allowable under the Pharmakon Loan Agreement, the Company made a $50,000 partial prepayment on the outstanding principal amount under the Pharmakon Term Loan. In conjunction with the partial prepayment, the Company incurred a $1,500 prepayment premium and paid $376 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 at enrollment.

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 over four 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 and six months ended June 30, 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 our pending transaction with Neopharmed, as defined and discussed below, for the sale of our European ORLADEYO Business (as described further under "*Recent Developments—Neopharmed Gentili S.p.A. Stock Purchase Agreement*" below), 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 and adjust our forecasts accordingly.

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, 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 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 submitted a new drug application ("NDA") 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. We also announced our expectation 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.

On May 14, 2025, we announced that the FDA accepted our NDA for the use of ORLADEYO in pediatric patients with HAE aged 2 to 11 years. In addition, we announced that the FDA granted priority review of our NDA, with a Prescription Drug User Fee Act ("PDUFA") target action date of September 12, 2025. We also announced that we filed a line extension application for the use of ORLADEYO oral granules in patients with HAE aged 2 to 11 years with the European Medicines Agency. On June 23, 2025, the FDA extended the PDUFA goal date for our NDA for the use of once-daily ORLADEYO oral granules in pediatric patients with HAE aged 2 to 11 years due to our submission of additional final reports and formulation data, which resulted in a standard three-month extension to the original goal date to provide time for a full review of the submission. The new PDUFA target action date for our NDA is December 12, 2025.

On May 16, 2025, we announced new real-world evidence on the use of ORLADEYO in adolescents and people with severe HAE showing significant and sustained reductions in HAE attack rates through 18 months of follow-up after beginning treatment with ORLADEYO in both patient populations.

On May 30, 2025, we announced new data highlighting the reduction in the percentage of days with HAE symptoms among young children initiating berotralstat in our APeX-P trial. We also announced the broad safety and efficacy outcomes observed across all age groups of patients taking ORLADEYO to prevent HAE attacks.

On June 6, 2025, we announced that, following a positive recommendation from the Zorginstituut Nederland, ORLADEYO was approved in the Netherlands for the routine prevention of HAE attacks in patients aged 12 years and older. We also announced that this reimbursement approval in the Netherlands marks national reimbursement for ORLADEYO across all major European countries, providing HAE patients with access to the first oral, once-daily preventive therapy.

On June 13, 2025, we announced that the National Institute of Drug and Food Surveillance in Colombia granted approval for ORLADEYO for the prophylaxis of HAE attacks in adults and pediatric patients 12 years of age or older. We have an exclusive collaboration with Pint Pharma GmbH ("Pint Pharma") to register and promote ORLADEYO in the pan-Latin America region. Under the terms of the agreement, Pint Pharma is responsible for obtaining and maintaining all marketing authorizations and for commercializing ORLADEYO in the region.

On June 16, 2025, we announced new data on the long-term efficacy and safety of ORLADEYO for the prophylactic treatment of HAE in patients across all age groups, demonstrating sustained reductions in HAE attacks and consistent safety profile.

On August 4, 2025, we announced that new real-world data from over 350 patients with HAE with normal C1 inhibitor showed substantial reductions in attack rates with ORLADEYO, which we believe reinforces its value for a historically underserved patient segment and provides strong evidence to close gaps in both treatment and reimbursement.

BCX17725 (Netherton syndrome)

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. In

addition, on July 30, 2025, we were notified that the FDA has granted Fast Track designation for BCX17725 for the treatment of Netherton syndrome. On August 4, 2025, we announced that we continue to expect initial data from the program by the end of the year.

Avoralstat

On May 5, 2025, we announced that the first clinical trial with suprachoroidal delivery of avoralstat was granted authorization to proceed in Australia. On August 4, 2025, we announced that we continue to expect initial data from this program by the end of the year.

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. On July 24, 2025, we made an additional partial prepayment of $50.0 million of the outstanding principal amount under the Pharmakon Loan Agreement.

Neopharmed Gentili S.p.A. Stock Purchase Agreement

On June 27, 2025, we entered into a stock purchase agreement (the "Stock Purchase Agreement") with BioCryst Ireland Limited ("BioCryst Ireland"), a private limited company incorporated under the laws of Ireland and a wholly owned subsidiary of the Company, and Neopharmed Gentili S.p.A., a corporation organized under the laws of Italy ("Neopharmed").

Under the terms of the Stock Purchase Agreement, we have agreed to sell to Neopharmed all of our equity interests in BioCryst Ireland, which, together with its subsidiaries, holds certain assets, rights, and employees related to our European ORLADEYO business (the "European ORLADEYO Business"). Concurrent with the closing of the transactions contemplated by the Purchase Agreement, we and BioCryst Ireland will amend and restate our existing intellectual property license agreement pursuant to which we will continue to grant to BioCryst Ireland certain rights with respect to ORLADEYO in the territory (the "IP License Agreement"). The terms of the IP License Agreement may also extend to the pediatric line extension of ORLADEYO, subject to certain regulatory approvals. Upon closing of the transaction, Neopharmed will pay us $250.0 million in cash, subject to customary purchase price adjustments. In addition, Neopharmed has agreed to pay us up to $14.0 million if certain revenue milestones are achieved prior to December 31, 2032. Neopharmed will also pay a $15.0 million royalty release fee to RPI 2019 Intermediate Finance Trust. The transaction is expected to close in October 2025, and we plan to use the proceeds from the transaction to retire all remaining outstanding term debt under the Pharmakon Loan Agreement.

Leadership Changes

On July 31, 2025, we announced that our President and Chief Executive Officer, Jon Stonehouse, had informed the Board of Directors of his intention to retire on December 31, 2025. The Board appointed Charlie Gayer, the Company's Chief Commercial Officer, as the Company's next President, effective August 1, 2025, and the next Chief Executive Officer, effective January 1, 2026. In addition, on July 7, 2025, we announced the appointment of Babar Ghias as the Company's Chief Financial Officer and Head of Corporate Development.

Results of Operations (three months ended June 30, 2025 compared to the three months ended June 30, 2024)

For the three months ended June 30, 2025, total revenues were $163.4 million compared to $109.3 million for the three months ended June 30, 2024. The increase in total revenues was due to a $48.6 million increase in ORLADEYO net revenue, excluding royalties, primarily due to an increase in direct sales of ORLADEYO due to an increase in volume, which was driven by strong patient demand, and an increase in the rate of paid shipments. The increase in total revenues was also due to an increase in other revenues of $5.5 million, primarily due to an increase in direct sales of peramivir.

Cost of product sales for the three months ended June 30, 2025 and 2024 was $2.8 million and $1.7 million, respectively. The increase in cost of product sales was primarily due to the increase in peramivir sales and an increase in inventory reserves.

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 June 30,	
	2025	2024
Research and development expenses by program:		
Berotralstat	$ 12,073	$ 13,146
Factor D Program	—	5,557
BCX17725	8,381	5,250
Avoralstat	8,307	4,137
Other research, preclinical and development costs	14,625	9,533
Total research and development expenses	$ 43,386	$ 37,623

Research and development expenses increased to $43.4 million for the three months ended June 30, 2025 from $37.6 million for the three months ended June 30, 2024, primarily due to an increase in preclinical and early clinical work for avoralstat, BCX17725, and 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 granted. These increases were partially offset by the discontinuation and close-out of the Factor D programs, and a decrease in berotralstat-related regulatory, safety and quality expenses as efforts shifted to selling, general, and administrative related activities, reflecting the program's commercial progression.

Research and development expenses include all direct and indirect expenses relating to research and development 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 June 30, 2025 were $87.4 million compared to $61.2 million for the three months ended June 30, 2024. This increase was primarily driven by $5.9 million of transaction costs related to the sale of our European ORLADEYO Business to Neopharmed, 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 granted. Further, there was an increase in ORLADEYO related regulatory, safety and quality activities as efforts shifted from development to supporting commercial products, reflecting the program's commercial progression, as well as increases in variable costs driven by the increase in ORLADEYO sales.

Interest expense for the three months ended June 30, 2025 was $21.6 million compared to $24.7 million for the three months ended June 30, 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 the result of the $75.0 million partial prepayment on the outstanding principal amount under the Pharmakon Term Loan in April 2025, and a decrease in the effective interest rate related to the Pharmakon Loan Agreement.

For the three months ended June 30, 2025, interest income was $2.5 million compared to $3.6 million for the three months ended June 30, 2024. Net foreign currency losses were less than $0.1 million for the three months ended June 30, 2025 and 2024.

In April 2025, the Company made a $75.0 million partial prepayment on the outstanding principal amount under the Pharmakon Term Loan resulting in a one-time loss on extinguishment of debt of $4.2 million for the three months ended June 30, 2025.

For the three months ended June 30, 2025, income tax expense was $1.4 million compared to $0.2 million for the three months ended June 30, 2024. The increase in income tax expense was primarily driven by the increase in net income for the three months ended June 30, 2025 compared to the net loss incurred in the three months ended June 30, 2024.

Results of Operations (six months ended June 30, 2025 compared to the six months ended June 30, 2024)

For the six months ended June 30, 2025, total revenues were $308.9 million compared to $202.1 million for the six months ended June 30, 2024. The increase in total revenues was due to a $93.9 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, was 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 $12.9 million, primarily due to an increase in direct sales of peramivir.

Cost of product sales for the six months ended June 30, 2025 and 2024 was $7.4 million and $3.0 million, respectively. The increase in cost of product sales was primarily due to the increase in peramivir and ORLADEYO sales, and an increase in inventory reserves.

The following table summarizes our research and development expenses for the periods indicated (in thousands):

	Six Months Ended June 30,	
	2025	**2024**
Research and development expenses by program:		
Berotralstat	$ 19,898	$ 25,104
Factor D Program	1,400	16,037
BCX17725	15,689	13,009
Avoralstat	15,687	8,173
Other research, preclinical and development costs	27,982	21,793
Total research and development expenses	$ 80,656	$ 84,116

Research and development expenses decreased to $80.7 million for the six months ended June 30, 2025 from $84.1 million for the six months ended June 30, 2024, primarily due to a decrease in Factor D program expenses attributed to the discontinuation and close-out of the Factor D programs, a decrease in berotralstat-related regulatory, safety and quality expenses as efforts shifted to selling, general, and administrative related activities, reflecting the program's commercial progression, and a change in general and administrative expense allocations. These reductions were partially offset by increased spending on preclinical and early clinical work for avoralstat, BCX17725, and 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 granted.

Research and development expenses include all direct and indirect expenses relating to research and development 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 six months ended June 30, 2025 were $169.9 million compared to $120.7 million for the six months ended June 30, 2024. The increase was primarily driven by $6.4 million of transaction costs related to the sale of our European ORLADEYO Business, an increase from a change in general and administrative expense allocations, 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 granted. Further, there was an increase in ORLADEYO related regulatory, safety and quality activities as efforts shifted from development to supporting commercial products, reflecting the program's commercial progression, as well as increases in variable costs driven by the increase in ORLADEYO sales.

Interest expense for the six months ended June 30, 2025 was $45.1 million compared to $49.2 million for the six months ended June 30, 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 the result of the $75.0 million partial prepayment on the outstanding principal amount under the Pharmakon Term Loan in April 2025, and the decrease in the effective interest rate related to the Pharmakon Loan Agreement.

For the six months ended June 30, 2025, interest income was $5.5 million compared to $7.6 million for the six months ended June 30, 2024. The decrease in interest income was primarily the result of an overall decrease in our investment portfolio. Net foreign currency losses were less than $0.1 million for the six months ended June 30, 2025 compared to losses of $0.1 million for the six months ended June 30, 2024.

In April 2025, the Company made a $75.0 million partial prepayment on the outstanding principal amount under the Pharmakon Term Loan resulting in a one-time loss on extinguishment of debt of $4.2 million for the six months ended June 30, 2025.

For the six months ended June 30, 2025, income tax expense was $2.1 million compared to $0.5 million for the six months ended June 30, 2024. The increase in income tax expense was primarily driven by the increase in net income for the six months ended June 30, 2025 compared to the net loss incurred in the six months ended June 30, 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. On July 24, 2025, we made an additional partial prepayment of $50.0 million of the outstanding principal amount under the Pharmakon Loan Agreement. In connection with the closing of the transaction with Neopharmed described above, we plan to use the proceeds from the transaction to retire all remaining outstanding term debt 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 June 30, 2025, we were in compliance with the negative covenants under the Pharmakon Loan Agreement. See "*Note 8—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 7—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 7—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 June 30, 2025 were approximately $285.2 million in cash and cash equivalents and available-for-sale investments, of which $15.1 million was considered held for sale as of June 30, 2025.

Cash Flows

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

	Six Months Ended June 30,	
	2025	**2024**
Net cash provided by (used in):		
Operating activities	$ 13,785	$ (55,052)
Investing activities	56,222	26,307
Financing activities	(73,157)	(3,014)
Effect of exchange rates on cash, cash equivalents and restricted cash	1,810	(483)
Decrease in cash, cash equivalents and restricted cash, including cash classified within current assets held for sale	(1,340)	(32,242)
Less: net increase in cash and cash equivalents classified within current assets held for sale	(15,058)	—
Net decrease in cash, and cash equivalents, and restricted cash	$ (16,398)	$ (32,242)

Operating Activities

During the six months ended June 30, 2025, net cash provided by operating activities of $13.8 million consisted primarily of a net income of $5.1 million and $59.8 million of changes in operating assets and liabilities, primarily due to a decrease in royalty financing obligations and an increase in receivables, offset by $68.4 million of non-cash items, including $26.9 million of non-cash interest expense on royalty financing obligations and $42.7 million of stock-based compensation expense.

During the six months ended June 30, 2024, net cash used in operating activities of $55.1 million consisted primarily of a net loss of $48.1 million and $66.8 million of changes in operating assets and liabilities, primarily due to a decrease in accounts payable and accrued expenses, a decrease in royalty financing obligations, and an increase in accounts receivable, partially offset by $59.8 million of non-cash items, including $28.5 million in non-cash interest expense on royalty financing obligations and $26.8 million of stock-based compensation expense.

Investing Activities

During the six months ended June 30, 2025, net cash provided by investing activities of $56.2 million primarily related to sales and maturities of investment securities, partially offset by purchases of investment securities.

During the six months ended June 30, 2024, net cash provided by investing activities of $26.3 million primarily related to maturities of investment securities, partially offset by purchases of investment securities.

Financing Activities

During the six months ended June 30, 2025, net cash used in financing activities of $73.2 million primarily consisted of repayment of Pharmakon term loan principal and related prepayment premium and fees, and principal payments on royalty financing obligations, partially offset by net proceeds from common stock issued under stock-based compensation plans.

During the six months ended June 30, 2024, net cash used in financing activities of $3.0 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. Our inventory also 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. 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 8—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 June 30, 2025, interest was accrued at an effective rate of 12.29% on the Tranche A Loan 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 six months ended June 30, 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 six months ended June 30, 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 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 Chief Financial Officer concluded that, as of June 30, 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 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 June 30, 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 sustained 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 7— 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 in a given reporting period, we may not be able to sustain or increase profitability on a quarterly or annual basis. If we are unable to achieve sustained 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 the Pending Transaction with Neopharmed Gentili S.p.A.

The pendency of the proposed sale of our European ORLADEYO Business or the failure to complete the proposed sale could adversely affect our business and the market price of our common stock.

On June 27, 2025, we announced entry into the Stock Purchase Agreement with Neopharmed, whereby Neopharmed has agreed to acquire our European ORLADEYO Business. The announcement and pendency of the proposed sale of our European ORLADEYO Business could cause disruptions in and create uncertainty surrounding our European ORLADEYO Business, including affecting our relationships with our existing and future customers, patients, physicians, payors and employees, which could have a significant negative impact on our future revenues and results of operations, regardless of whether the sale is completed. In particular, management and financial resources have been diverted and will continue to be diverted towards the completion of the sale, which could have a negative impact on our future revenues and/or results of operations.

We are also subject to restrictions, without the consent of Neopharmed, on the conduct of our European ORLADEYO Business prior to the consummation of the sale. These restrictions could potentially hinder the ability of our European ORLADEYO Business to adapt to changing market conditions or take necessary actions to improve performance, and may otherwise have a significant negative impact on the future revenues and/or results of operations of our European ORLADEYO Business.

There is also no assurance that the closing of the sale will occur. Consummation of the sale is subject to various conditions, including the receipt of antitrust approval, certain third party consents, the accuracy of the Company's and BioCryst Ireland's representations and warranties except, generally, for any inaccuracies that have not had a material adverse effect with respect to our European ORLADEYO Business, the absence of any law or injunction restraining or

otherwise prohibiting the consummation of the transactions, the absence of a material adverse effect on our European ORLADEYO Business and delivery of a customary payoff letter relating to the repayment of certain of our indebtedness from the proceeds of the sale. The failure to satisfy all of the required conditions could delay the completion of the sale for a significant period of time or prevent it from occurring at all. There can be no assurance that the conditions to the completion of the sale will be satisfied or waived or that the transaction will be completed. In addition, either we or Neopharmed may terminate the Stock Purchase Agreement under certain circumstances, including if the sale is not completed by December 31, 2025. We have also incurred, and will continue to incur, significant costs, expenses and fees for professional services and other transaction costs in connection with the proposed sale, as well as the diversion of management resources towards the sale, for which we will have received little or no benefit if the closing does not occur. A failed transaction may also result in negative publicity, a negative impression of us in the investment community and a decline in our share price.

The amount of the milestone payments we may receive following the sale of our European ORLADEYO Business is subject to various risks and uncertainties.

In connection with the sale, Neopharmed will acquire our European ORLADEYO Business and will pay to us:

- a cash payment, payable upon closing, of $250 million, subject to customary purchase price adjustments; and
- future milestone payments of up to $14 million associated with revenue of ORLADEYO® in Central and Eastern Europe.

The milestone payments are subject to various risks and uncertainties. The milestone payments will be based on the achievement of specified net sales targets for the sale of ORLADEYO® in Central and Eastern Europe. It is not possible to determine with precision as of the date of this Quarterly Report on Form 10-Q the amount or timing of sales of ORLADEYO® in Central and Eastern Europe in the future and, therefore, it is possible that the milestone payments will not be earned or will be limited by lower net sales than anticipated. The specified net sales targets for ORLADEYO® in Central and Eastern Europe were based on certain assumptions about the future financial performance of ORLADEYO® in Central and Eastern Europe, and there can be no assurance that such projections will be achieved or that the milestone payments will become payable.

In addition, the cash payment we receive is subject to certain customary purchase price adjustments. If we miscalculate certain of these purchase price adjustments, the total amount of net proceeds we receive could be negatively impacted.

We will incur significant expenses in connection with the sale of our European ORLADEYO Business, regardless of whether the sale is completed.

We expect to incur significant expenses related to the sale of our European ORLADEYO Business. These expenses include, but are not limited to, legal fees, accounting fees and expenses, certain employee expenses and other related fees and expenses. Many of these expenses will be payable by us regardless of whether the sale is completed.

Further, if the sale is completed, we will enter into the Transition Services Agreement, which will provide for certain transition services to be provided to BioCryst Ireland by us, for the periods of time and compensation set forth therein, in order to facilitate the transition of the European ORLADEYO Business to Neopharmed. Performance of our obligations under the Transition Services Agreement may incur significant financial costs, and the focus and attention of our management and employee resources may be diverted, which could have a negative impact on our future revenues and/or results of operations.

We may not be able to realize the anticipated benefits of the sale of our European ORLADEYO Business.

We may not be able to realize the anticipated benefits from the sale of our European ORLADEYO Business. Our ability to realize the anticipated benefits of the sale and the success of the remaining company is subject to various risks and uncertainties, including the possibility that we may not be able to successfully sustain being a higher margin business or that we may not increase profitability through sustainable ORLADEYO® growth, our pipeline, and potential external opportunities.

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 such candidates 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 epidemic or 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 successfully consummate the pending transaction with Neopharmed, as contemplated under the Stock Purchase Agreement; 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 as a result of economic and political instability. 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 or develop 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, among others, third-party distributors for ORLADEYO in certain markets, with Torii for ORLADEYO in Japan, with each of Shionogi and Green Cross for the development and commercialization of peramivir, and with Clearside for the development of avoralstat with Clearside's SCS Microinjector®. 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 our pending transaction with Neopharmed for the sale of our European ORLADEYO Business, 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 at enrollment. 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 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. On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law in the United States. Although we are still evaluating the impact of the OBBBA on the provisions of the IRA and on our business, the OBBBA contains a variety of provisions that could impact our business and results of operations.

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 14—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;
- the 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, on May 15, 2025, ASPR notified the Company of its intent to not exercise any additional optional ordering periods available under the agreement.

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 June 30, 2025, we had an outstanding principal balance under the Pharmakon Loan Agreement of $248.7 million, inclusive of the Pharmakon PIK Interest Payments (as defined in "*Note 8—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 and July 24, 2025, we made a partial prepayment of $75.0 million and $50.0 million, respectively, 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, noncompliance with the GDPR or related national data protection laws, which may deviate from the GDPR, may result in significant fines of up to 4.0% 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.

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 responsive 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 the 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 may 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 may 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. See "*Risks Relating to the Pending Transaction with Neopharmed Gentili S.p.A.*" in this section for further discussion of the potential business disruptions, financial uncertainties, and operational risks that we face while the transaction with Neopharmed to sell our European ORLADEYO Business is pending.

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 42% 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 June 30, 2025, the 52-week range of the market price of our stock was from $6.02 to $11.31 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, divestitures (such as the pending transaction with Neopharmed), 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 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 July 31, 2025, there were 209,920,430 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 July 31, 2025, there were 46,659,214 stock options and restricted stock units outstanding and 12,976,044 shares available for issuance under our Amended and Restated Stock Incentive Plan, 5,615,859 stock options and restricted stock units outstanding and 1,681,195 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 the 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 June 30, 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
2.1	Stock Purchase Agreement, dated as of June 27, 2025, by and among BioCryst Pharmaceuticals, Inc., BioCryst Ireland Limited and Neopharmed Gentili S.p.A. Incorporated by reference to Exhibit 2.1 to the Company's Form 8-K filed June 30, 2025.
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. Incorporated by reference to Exhibit 10.1 to the Company's Form 10-Q filed May 6, 2025.
(10.2)*	Amendment No. 1 to the Consulting Agreement, effective May 31, 2025, by and between BioCryst Pharmaceuticals, Inc. and Anthony Doyle.
(10.3)*	Amended and Restated Employment Agreement, effective July 23, 2025, by and between BioCryst Pharmaceuticals, Inc. and Babar Ghias.
(10.4)*	Amendment No. 2 to the Employment Agreement, effective August 1, 2025, by and between BioCryst Pharmaceuticals, Inc. and Charles Gayer.
10.5*	BioCryst Pharmaceuticals, Inc. Stock Incentive Plan (as amended and restated as of April 21, 2025). Incorporated by reference to Exhibit 10.1 to the Company's Form 8-K filed June 16, 2025.
10.6*	Form of Notice of Grant of Stock Option and Standard Stock Option Agreement under the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan. Incorporated by reference to Exhibit 10.2 to the Company's Form 10-Q filed May 6, 2025.
10.7*	BioCryst Pharmaceuticals, Inc. Amended and Restated Non-Employee Director Compensation Policy, effective April 21, 2025. Incorporated by reference to Exhibit 10.3 to the Company's Form 10-Q filed May 6, 2025.
(31.1)	Certification of the Principal Executive 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.

(31.2)	Certification of the 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 Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
(32.2)**	Certification of the 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 and six months ended June 30, 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 5th day of August, 2025.

BIOCRYST PHARMACEUTICALS, INC.

By: /s/ Jon P. Stonehouse

Jon P. Stonehouse
Chief Executive Officer
(Principal Executive Officer)

By: /s/ Babar Ghias

Babar Ghias
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

AMENDMENT TO CONSULTING AGREEMENT

THIS FIRST AMENDMENT to the CONSULTING AGREEMENT (the "First Amendment") is made and entered into on this 31st day of May, 2025 (the "First Amendment Effective Date"), by and between BioCryst Pharmaceuticals, Inc., a Delaware Corporation and Anthony Doyle

RECITALS

WHEREAS, BioCryst and Anthony Doyle entered into a Consulting Agreement (the "Agreement") having an effective date of April 9, 2025; and

WHEREAS, both parties wish to extend the term of the Agreement.

NOW, THEREFORE, in consideration of the foregoing, and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, BioCryst and Anthony Doyle, intending to be legally bound, have agreed to amend the terms of the Agreement as follows:

> **The Term of the Agreement shall be extended through December 31, 2025, and shall automatically continue thereafter on a month-to-month basis until terminated by either party upon thirty (30) days' written notice.**

Except as expressly amended hereby, the Agreement shall remain in full force and effect in accordance with its terms.

IN WITNESS WHEREOF, the parties have executed this Amendment as of the first date above written.

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

Exhibit 10.3

AMENDED AND RESTATED EMPLOYMENT AGREEMENT

This **AMENDED AND RESTATED EMPLOYMENT AGREEMENT** ("***Amendment***") is made effective as of the 23rd day of July, 2025, between **Babar Ghias** ("***Employee***"), and **BioCryst Pharmaceuticals, Inc.**, a Delaware corporation, located at 4505 Emperor Boulevard, Suite 200, Durham NC 27703 ("***Company***"). The parties agree that any and all actions by a party pursuant to the Employment Agreement shall be deemed to have been made under this Amendment on the date such action was taken and nothing in this Amendment shall require duplication of any prior action by either party.

The Amendment is set forth as follows:

June 9, 2025

Via Electronic Mail

Babar Ghias
2738 North Magnolia,
Chicago, IL 60614

Dear Mr. Ghias,

On behalf of BioCryst Pharmaceuticals, Inc., a Delaware corporation (the "Company"), we are pleased to offer you the position of Chief Financial Officer. You will report directly to Jon Stonehouse, Chief Executive Officer. We, along with the other members of the Company's Board of Directors (the "Board"), and the Company's management team, are all very impressed with you and what you will bring to the Company. We look forward to your joining this Company and making significant contributions to its success.

Upon formal appointment by the Board of Directors as an Officer of the Company, this letter agreement (the "Agreement") will serve to confirm our agreement with respect to the terms and conditions of your employment.

1. Term of Employment.

(a) Subject to the terms and conditions of this Agreement, the Company hereby employs Babar Ghias ("Employee") as Chief Financial Officer. Employee shall devote substantially full-time attention to the business and affairs of the Company and its affiliates. Employee may (i) serve on corporate, civic, charitable or non-profit boards or committees, subject in all cases to the prior written approval in accordance with all applicable policies of the Company and its affiliates as in effect from time to time, and (ii) manage personal and family investments, participate in industry organizations and deliver lectures at educational institutions or events, so long as no such service or activity interferes, individually or in the aggregate, with the performance of Employee's responsibilities hereunder.

(b) The term of employment of Employee under this Agreement shall begin on July 7, 2025 (the "Effective Date") and shall continue until Employee is terminated in accordance with Section 4 of this Agreement.

2. Basic Full-Time Compensation and Benefits.

(a) Commencing as of the Effective Date, as basic compensation for services rendered under this Agreement, Employee shall be entitled to receive from the Company a salary of $560,000 per annum (the "Base Salary"), payable in accordance with the Company's standard payroll practices as in effect from time to time during the term of this Agreement. The Base Salary will be reviewed annually by the Board or a committee thereof and may be raised at the discretion of the Board or such committee.

(b) Employee shall be eligible to earn a cash bonus, payable as soon as reasonably practicable in the calendar year following each calendar year during the term of this Agreement, based on the Company's and/or Employee's achievement of performance related goals proposed by management and approved by the Board or a committee thereof for the Company's applicable fiscal year (the "Incentive Compensation"). The Incentive Compensation actually earned, if any, shall be determined in the sole discretion of the Board or a committee thereof and shall be based on a target amount equal to seventy percent (70%) of the Base Salary earned by Employee during such fiscal year (the "Target Amount"), which shall not be pro-rated for the first fiscal year of the term of this Agreement. The Board or a committee thereof may, in its discretion, approve an Incentive Compensation payment in excess of the Target Amount if the performance goals have been exceeded. Employee must be employed through April 1 of the next succeeding fiscal year in order to receive the Incentive Compensation payment for each fiscal year.

(c) Employee shall be provided with a one-time cash bonus of $160,000 ("Signing Bonus"), payable within thirty (30) days of the Effective Date. Any Incentive Compensation earned for the fiscal year in which the Effective Date occurs pursuant to Section 2(b) will be reduced by the Signing Bonus. In the event of Employee's termination of employment prior to payment of the applicable Incentive Compensation, Employee will promptly repay Signing Bonus.

(c) Employee shall be entitled to receive such other benefits and perquisites provided to similarly situated executive officers of the Company, subject to modification or termination at any time, which benefits may include, without limitation, reasonable paid time off (PTO), medical, dental and vision benefits, life insurance, and participation in profit sharing or retirement plans.

3. Equity Awards.

The Company shall grant to Employee 305,000 stock options and 147,000 RSUs pursuant to BioCryst's stock option plan (the "Initial Equity Grants"). The grant date of the Initial Equity Grants shall be July 24, 2025, or such other date as the parties mutually agree. The Initial Equity Grants shall be granted under and subject to the terms of the Company's Inducement Plan or the Company's Stock Incentive Plan, as applicable and as the same may be amended and restated from time to time. The Initial Equity Grants shall vest and become exercisable (contingent on Employee's continued provision of services to the Company on each respective vesting date) over a period of four (4) years on the anniversary of the grant date.

In addition, during the term of this Agreement, Employee shall be eligible to receive equity-based compensation as determined in the sole discretion of the Board or a committee thereof, which may be subject to the achievement of certain performance targets set by the Board or such committee. These equity-based awards shall be subject to the terms and conditions set forth in the Company's Stock Incentive Plan as in effect from time to time and award agreements issued thereunder.

4. Termination.

(a) If Employee's employment is terminated (i) by the Company for Cause, (ii) by Employee other than, following a Change of Control, pursuant to a Constructive Termination, or (iii) Employee's death or Disability, the Company shall pay Employee (A) any accrued and unpaid Base Salary, payable on the next payroll date; (B) reimbursement for any and all monies advanced or expenses incurred in connection with Employee's employment for reasonable and necessary expenses incurred by Employee on behalf of the Company for the period ending on the termination date, which amount shall be reimbursed within thirty (30) days of the Company's receipt of proper documentation from Employee; (C) any compensation that Employee had previously deferred (including any interest earned or credited thereon), in accordance with the terms and conditions of the applicable deferred compensation plans or arrangements then in effect, to the extent vested as of Employee's termination date, paid pursuant to the terms of such plans or arrangements; and (D) any vested amount or benefit payable under any welfare or retirement benefit plan or program in accordance with the terms thereof (the foregoing items in this Section 4(a), the "Accrued Obligations").

For all purposes under this Agreement, a termination for "Cause" shall mean a determination by the Board that Employee's employment be terminated for any of the following reasons: (i) failure or refusal to comply in any material respect with lawful policies, standards or regulations of Company; (ii) a violation of a federal or state law or regulation applicable to the business of the Company; (iii) conviction or plea of no contest to a felony under the laws of the United States or any State; (iv) fraud or misappropriation of property belonging to the Company or its affiliates; (v) a breach in any material respect of the terms of any confidentiality, invention assignment or proprietary information agreement with the Company or with a former employer, (vi) failure to satisfactorily perform Employee's duties after having received written notice of such failure and at least thirty (30) days to cure such failure, or (vii) misconduct or gross negligence in connection with the performance of Employee's duties.

For all purposes under this Agreement, "Disability" shall mean the inability of Employee to perform Employee's duties hereunder by reason of physical or mental incapacity for ninety (90) days, whether consecutive or not, during any consecutive twelve (12) month period.

(b) If Employee's employment is terminated by the Company without Cause, or, following a Change of Control, by Employee pursuant to a Constructive Termination, then Employee will receive any Accrued Obligations and, subject to Sections 4(c) and 6(f), Employee will receive the following: (i) continuation of Base Salary for one (1) year following the effective termination date, payable in accordance with the regular payroll practices of the Company; (ii) payment of one times Employee's annual target Incentive Compensation in effect for the fiscal year in which Employee's termination date occurs, payable in equal installments over the regularly scheduled payroll periods of the Company for the one year following the effective date of termination; and (iii) if Employee elects to continue health insurance coverage under the Consolidated Omnibus

Budget Reconciliation Act of 1985, as amended ("COBRA") following termination of employment, the Company shall pay the monthly premium under COBRA on the same basis as active employees until the earlier of (x) 12 months following the effective termination date, or (y) the date upon which Employee commences employment with an entity other than the Company. Employee will notify the Company in writing within five (5) days of Employee's receipt of an offer of employment with any entity other than the Company, and will accordingly identify the date upon which Employee will commence employment in such writing (clauses (i) through (iii), "Severance").

For all purposes under this Agreement, "Change of Control" shall mean: (i) the sale, transfer, or other disposition of all or substantially all of the assets of the Company in liquidation or dissolution of the Company; (ii) the consummation of a merger or consolidation of the Company with any other corporation or other entity, other than (I) a merger or consolidation (A) which results in the voting securities of the Company outstanding immediately prior to such merger or consolidation continuing to represent fifty percent (50%) or more of the combined voting power of the surviving entity or the ultimate parent thereof outstanding immediately after such merger or consolidation and (B) immediately following which the individuals who comprise the Board immediately prior thereto constitute fifty percent (50%) or more of the board of directors of the entity surviving such merger or consolidation or, if the Company or the entity surviving such merger or consolidation is then a subsidiary, the ultimate parent thereof, or (II) a merger or consolidation effected to implement a recapitalization of the Company (or similar transaction) in which no Person is or becomes the beneficial owner (within the meaning of Rule 13d-3 of the Securities Exchange Act of 1934, as amended (the "1934 Act")), directly or indirectly, of securities of the Company (not including in the securities beneficially owned by such Person any securities acquired directly from the Company or its affiliates) representing more than fifty percent (50%) of the combined voting power of the Company's then outstanding securities; (iii) any person or related group of persons (other than the Company or a person that directly or indirectly controls, is controlled by, or is under common control with, the Company) directly or indirectly acquires beneficial ownership (within the meaning of Rule 13d-3 of the 1934 Act) of securities possessing more than fifty percent (50%) of the total combined voting of the Company's outstanding securities pursuant to a tender or exchange offer made directly to the Company's stockholders; or (iv) a change in the composition of the Board over a period of twelve (12) consecutive months such that a majority of the Board members (rounded up to the next whole number) ceases to be comprised of individuals who either (A) have been Board members continuously since the beginning of such period or (B) have been elected or nominated for election as Board members during such period by at least two-thirds of the Board members described in clause (A) who were still in office at the time such election or nomination was approved by the Board.

For all purposes under this Agreement, "Constructive Termination" shall mean a resignation of employment within 30 days of the occurrence of any of the following events which occurs within 6 months following a Change of Control: (i) a material reduction in Employee's responsibilities; (ii) a material reduction in Employee's Base Salary, unless such reduction is comparable in percentage to, and is part of, a reduction in the base salary of all executives of the Company; or (iii) a relocation of Employee's principal office to a location more than 50 miles from the location of Employee's principal office immediately preceding a Change of Control.

(c) The Company's obligation to provide Severance is conditioned upon Employee returning to the Company all of its property and confidential information that is in Employee's possession

and Employee's execution and non-revocation of an enforceable release of claims (the "Release"). If Employee chooses not to execute the Release, revokes Employee's execution of the Release, or fails to comply with the terms of the Release, then the Company shall have no obligation to provide Severance and such Severance amount is subject to recoupment by the Company. The Release shall be provided to Employee no later than seven (7) days following Employee's separation from service and Employee must execute it within the time period specified in the Release (which shall not be longer than forty-five (45) days from the date of receipt). The Release shall not be effective until any applicable revocation period has expired.

5. Non-Competition; Proprietary Information and Inventions.

(a) Proprietary Information and Inventions Agreement; Non-Competition and Non-Solicitation Agreement. As a condition precedent to the employment of Employee by the Company pursuant to the terms of this Agreement, Employee shall execute (i) the Company's Proprietary Information and Inventions Agreement, attached hereto as Exhibit A, and (ii) the Company's Non-Competition and Non-Solicitation Agreement, attached hereto as Exhibit B.[1]

(b) Equitable Remedies. Employee acknowledges and recognizes that a violation of the Proprietary Information and Inventions Agreement or the Non-Competition and Non-Solicitation Agreement by Employee may cause irreparable and substantial damage and harm to the Company or its affiliates, could constitute a failure of consideration, and that money damages will not provide a full remedy for the Company for such violations. Employee agrees that in the event of Employee's breach of the Proprietary Information and Inventions Agreement or the Non-Competition and Non-Solicitation Agreement, the Company will be entitled, if it so elects, to institute and prosecute proceedings at law or in equity to obtain damages with respect to such breach, to enforce the specific performance of such agreement(s) by Employee, and to enjoin Employee from engaging in any activity in violation hereof.

6. Miscellaneous.

(a) Entire Agreement. This Agreement, including the exhibits hereto, constitutes the entire agreement between the parties relating to the employment of Employee by the Company and there are no terms relating to such employment other than those contained in this Agreement and supersedes all other agreements or understandings related to the subject matter contained herein. No modification or variation hereof shall be deemed valid unless in writing and signed by the parties hereto. No waiver by either party of any provision or condition of this Agreement shall be deemed a waiver of similar or dissimilar provisions or conditions at any time.

(b) Assignability. This Agreement may not be assigned without prior written consent of the parties hereto, except that the Company may assign this Agreement to a wholly-owned subsidiary of the Company without prior written consent of Employee. To the extent allowable pursuant to this Agreement, this Agreement shall be binding upon and shall inure to the benefit of each of the parties hereto and their respective executors, administrators, personal representatives, heirs, successors and assigns.

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[1] The originally executed Proprietary Information and Inventions Agreement (Exhibit A) and the Non-Competition and Non-Solicitation Agreement (Exhibit B) by Babar Ghias shall be appended hereto in PDF format.

(c) Notices. Any notice or other communication given or rendered hereunder by any party hereto shall be in writing and delivered personally or sent by registered or certified mail, postage prepaid, at the respective addresses of the parties hereto as set forth below.

(d) Captions. The section headings contained herein are inserted only as a matter of convenience and reference and in no way define, limit or describe the scope of this Agreement or the intent of any provision hereof.

(e) Taxes. All amounts to be paid to Employee hereunder are in the nature of compensation for Employee's employment by the Company, and shall be subject to withholding, income, occupation and payroll taxes and other charges applicable to such compensation.

(f) Section 409A. The parties intend for the payments and benefits under this Agreement to be exempt from Section 409A of the Internal Revenue Code of 1986, as amended (the "Code"), or, if not so exempt, to be paid or provided in a manner which complies with the requirements of such section, and intend that this Agreement shall be construed and administered in accordance with such intention. In the event the Company determines that a payment or benefit under this Agreement may not be in compliance with Section 409A of the Code, the Company shall reasonably confer with Employee in order to modify or amend this Agreement to comply with Section 409A of the Code and to do so in a manner to best preserve the economic benefit of this Agreement. Notwithstanding anything contained herein to the contrary, (i) in the event (A) any payments described in Section 4 would be "deferred compensation" subject to Section 409A of the Code; and (B) Employee is a "specified employee" (as defined in Code Section 409A(2)(B)(i)), such payments shall, to the extent required by Code Section 409A, be delayed for the minimum period and in the minimum manner necessary to avoid the imposition of the tax required by Section 409A of the Code; (ii) each amount to be paid or benefit to be provided under this Agreement shall be construed as a separately identified payment for purposes of Section 409A of the Code; (iii) any payments that are due within the "short term deferral period" as defined in Section 409A of the Code shall not be treated as deferred compensation unless applicable law requires otherwise; and (iv) amounts reimbursable to Employee under this Agreement shall be paid to Employee on or before the last day of the year following the year in which the expense was incurred and the amount of expenses eligible for reimbursement (and in-kind benefits provided to Employee) during any one (1) year may not affect amounts reimbursable or provided in any subsequent year. Notwithstanding anything in this Agreement to the contrary, in the event any payments hereunder could occur in one of two calendar years as a result of being dependent upon the Release becoming nonrevocable, then, to the extent required to avoid the imposition of taxes or penalties under Section 409A of the Code, such payments shall commence on the first regularly scheduled payroll date of the Company, following the date the Release becomes nonrevocable, that occurs in the second of such two calendar years.

(g) Golden Parachute Provisions. If it is determined that any payment or benefit provided by the Company to or for the benefit of Employee, whether paid or payable or distributed or distributable pursuant to the terms of this Agreement or otherwise, including, by example and not by way of limitation, acceleration by the Company or otherwise of the date of vesting or payment under any plan, program, arrangement or agreement of the Company would be subject to the excise tax imposed by Internal Revenue Code section 4999 or any interest or penalties with respect to such excise tax (such excise tax together with any such interest and penalties, shall be referred to as the "Excise Tax"), then the Company shall first make a calculation under

which such payments or benefits provided to Employee are reduced to the extent necessary so that no portion thereof shall be subject to the Excise Tax (the "4999 Limit"). The Company shall then compare (i) Employee's Net After-Tax Benefit (as defined below) assuming application of the 4999 Limit with (ii) Employee's Net After-Tax Benefit without application of the 4999 Limit. Employee shall be entitled to the greater of (i) or (ii). "Net After-Tax Benefit" shall mean the sum of (x) all payments that Employee receives or is entitled to receive that are contingent on a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company within the meaning of Internal Revenue Code section 280G(b)(2), less (y) the amount of federal, state, local, employment, and Excise Tax (if any) imposed with respect to such payments. Any reduction pursuant to this Section 6(g) shall be implemented by determining the Parachute Payment Ratio (as defined below) for each "parachute payment" and then reducing the "parachute payments" in order beginning with the "parachute payment" with the highest Parachute Payment Ratio. For "parachute payments" with the same Parachute Payment Ratio, such "parachute payments" shall be reduced based on the time of payment of such "parachute payments," with amounts having later payment dates being reduced first. For "parachute payments" with the same Parachute Payment Ratio and the same time of payment, such "parachute payments" shall be reduced on a pro rata basis (but not below zero) prior to reducing "parachute payments" with a lower Parachute Payment Ratio. "Parachute Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable "parachute payment" for purposes of Internal Revenue Code Section 280G and the denominator of which is the actual present value of such payment.

(h) Governing Law. This Agreement is made and shall be governed by and construed in accordance with the laws of the State of North Carolina without respect to its conflicts of law principles.

VOLUNTARILY ACCEPTED AND AGREED

By signing this Agreement, you confirm that you have received a copy of the PIIA and non-compete, Exhibit A and Exhibit B hereto, respectively, at least 14 days before your start date. You also acknowledge that you have been advised by the Company to seek the advice of an attorney prior to signing this Agreement.

IN WITNESS WHEREOF, this Amended and Restated Employment Agreement has been executed as of the day and year first above written.

BABAR GHIAS ("Employee"):
By: /s/ Babar Ghias
Name: Babar Ghias
Date: 23 July 2025

BIOCRYST PHARMACEUTICALS, INC. ("Company"):

By: /s/ Jon Stonehouse
Name: Jon Stonehouse
Title: Chief Executive Officer
Date: 23 July 2025

Exhibit A
(Proprietary Information and Inventions Agreement)

EMPLOYEE'S PROPRIETARY INFORMATION AND INVENTIONS AGREEMENT

I, Babar Ghias, recognize that BioCryst Pharmaceuticals, Inc., a Delaware corporation (hereinafter the "Company" and together with its subsidiaries, including existing and future
subsidiaries, the "Company Group"), is engaged in a continuous program of research, development, and production respecting its business, present and future, including fields generally related to its business.

I understand that:

A. As part of my employment by a member of the Company Group, I will faithfully and diligently serve and endeavor to further and safeguard the interests of the Company Group, and I recognize that I am expected to make new contributions and inventions of value to the Company Group;

B. My employment creates a relationship of confidence and trust between me and the Company Group with respect to any information:

    i. Applicable to the business of the Company Group; or

    ii. Applicable to the business of any client or customer of the Company Group which may be made known to me by the Company Group or by any client or customer of Company Group, or learned by me during the period of my employment.

C. The Company Group possesses and will continue to possess information that has been created, discovered or developed by, or assigned, disclosed or otherwise become known to, it (including without limitation information created, discovered, developed, disclosed or made known by me during the period of or arising out of my employment by any member of the Company Group), which information is not generally known to the public. All of the aforementioned information is hereinafter called "Proprietary Information." By way of illustration, but not limitation, Proprietary Information includes trade secrets, processes, formulas, data and know-how, improvements, inventions, techniques, marketing plans, financial information, strategies, forecasts, and customer lists.

In consideration of my employment or continued employment, as the case may be, by any member of the Company Group and the compensation received by me from the Company Group from time to time, I hereby agree as follows:

1. All Proprietary Information shall be the sole property of the Company and its assigns, and the Company and its assigns shall be the sole owner of all patents and other rights, title, and interest in connection therewith. I hereby assign to the Company any and all rights I may have or acquire in such Proprietary Information and/or patents. At all times, both during my employment by any member of the Company Group and after termination, I will keep in confidence and trust all Proprietary Information, and I will not use or disclose any Proprietary

Information or anything relating to it without the prior written consent of the Company Group, except as may be necessary in the ordinary course of performing my duties as an employee of the Company or any member of the Company Group. In the event I am required to disclose Proprietary Information pursuant to applicable law or court order, I shall, whenever legally permissible, promptly disclose such request to the Company, and cooperate with the Company to seek a protective order and to otherwise limit such disclosure from becoming public.

2. Notwithstanding anything set forth in this Agreement, or any other agreement that I have with the Company or its affiliates to the contrary, I shall not be prohibited from reporting possible violations of federal or state law or regulation to any governmental agency or entity, legislative body, or any self-regulatory organization, or making other disclosures that are protected under the whistleblower provisions of federal or state law or regulation, nor am I required to notify the Company regarding any such reporting, disclosure or cooperation with the government. Pursuant to 18 U.S.C. § 1833(b), I understand that I will not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret of the Company or its affiliates that (i) is made (A) in confidence to a federal, state, or local government official, either directly or indirectly, or to my attorney and (B) solely for the purpose of reporting or investigating a suspected violation of law; or (ii) is made in a complaint or other document that is filed under seal in a lawsuit or other proceeding. I understand that if I file a lawsuit for retaliation by any member of the Company Group for reporting a suspected violation of law, I may disclose the trade secret to my attorney and use the trade secret information in the court proceeding if I (x) file any document containing the trade secret under seal, and (y) do not disclose the trade secret, except pursuant to court order. Nothing in this Agreement, or any other agreement that I have with the Company or its affiliates, is intended to conflict with 18 U.S.C. § 1833(b) or create liability for disclosures of trade secrets that are expressly allowed by such section.

3. I agree that, during the period of my employment by any member of the Company Group, I will not, without the Company's express prior written consent, engage in any employment or consulting other than for the Company Group. In the event of the termination of my employment by me or by the Company Group for any reason or at any time upon Company’s request, I will promptly deliver to the Company all documents and data of any nature pertaining to my work with the Company Group and I will not take with me any documents or data of any description or any reproduction of any description containing or pertaining to any Proprietary Information.

4. I will promptly and fully disclose to the Company, or any persons designated by it, all improvements, inventions, formulas, processes, techniques, know how, and data, whether or not patentable, copyrightable, or otherwise protectible as intellectual property, made or conceived or reduced to practice or learned by me, either alone or jointly with others, during the period of my employment by the Company which are related to or useful in the business of the Company, or result from tasks assigned me by the Company or result from use of premises owned, leased, or contracted for by the Company (all said improvements, inventions, formulas, processes, techniques, know how, and data shall be collectively hereinafter called “Inventions”). I agree to keep complete, accurate, and authentic accounts, notes, data, and records of all Inventions in the manner and form requested by the Company, which accounts, notes, data, and records shall be and remain the sole property of the Company. I agree to surrender the same promptly to the Company upon its request or, in the absence of such a request, upon the termination of my employment by the Company.

5. I agree that all Inventions are and shall be the sole property of the Company and its assigns, and that the Company and its assigns shall be the sole owner of all intellectual property and other rights in connection therewith and by reason of my being employed by any member of the Company Group, to the extent permitted by law, all of the Inventions consisting of copyrightable subject matter is “work made for hire” as defined in the Copyright Act of 1976 (17 U.S.C. § 101). To the extent that any Invention is not a “work made for hire,” I hereby assign to the Company for no additional consideration any and all rights I may have or acquire in or to such Inventions, including the right to sue, counterclaim, and recover for all past, present, and future infringement, misappropriation, or dilution thereof, and all rights corresponding thereto throughout the world. I further agree as to all such Inventions to assist the Company in every proper way (but at the Company’s expense) to apply for, obtain, maintain and from time to time enforce such intellectual property rights, including patents and extensions and continuations of said patents, on said Inventions in any and all countries, and to that end I will execute all documents for use in applying for, obtaining and maintaining such intellectual property enforcing same, as the Company may desire, together with any further assignments thereof to the Company or persons designated by it. The foregoing obligation to assist the Company shall continue beyond the termination of my employment, but the Company shall compensate me at a reasonable rate after such termination for time actually spent by me at the Company’s request on such assistance.

6. As a matter of record I attach hereto a complete list of all Inventions or improvements relevant to the subject matter of my employment by the Company Group which have been conceived, made, or reduced to practice by me, alone or jointly with others, prior to my engagement by the Company Group which I desire to remove from the operation of this Agreement. I covenant that such list is complete. If no such list is attached to this Agreement, I represent that I have no such Inventions and improvements at the time of signing this Agreement.

7. I represent that my performance of all of the terms of this Agreement and as an employee of any member of the Company does not and will not breach any agreement to keep in confidence Proprietary Information of any third party acquired by me in confidence or in trust prior to my employment by the Company. I have not entered into, and I agree that I will not enter into, any agreement either written or oral in conflict herewith.

8. I understand that, as part of the consideration of the offer of employment extended to me by the Company or of my continued employment by the Company, as the case may be, I will not bring, have not brought, with me to the Company Group and I will not use, have not used, in the performance of my responsibilities at the Company materials or documents of a former employer, unless I have obtained written authorization from the former employer for their possession and use. Accordingly, this is to advise the Company that the only materials that I will bring to the Company or use in my employment are identified on the attached sheet (Exhibit A) and, as to each such item, I represent that I have obtained, prior to the effective date of my employment with the Company, written authorization for their possession and use in my employment with any member of the Company. I also understand that, in my employment with the Company, I am not to breach any obligation of confidentiality that I have to former employers, and I agree that I shall fulfill all such obligations during my employment with the Company.

9. This Agreement shall be effective as of the first day of my employment by the Company. I understand and agree that this Agreement is not a contract of employment.

10. This Agreement shall be binding upon me, my heirs, executors, assigns, administrators, and other legal representatives and shall inure to the benefit of the Company, its successors and assigns.

[SIGNATURE ON FOLLOWING PAGE]

IN WITNESS WHEREOF, the Company has caused this Proprietary Information and Inventions Agreement to be executed by its duly authorized officer and Employee has executed the same as of the dates set forth below.

BIOCRYST PHARMACEUTICALS, INC.

By: /s/ Jon Stonehouse
Jon Stonehouse
Chief Executive Officer

Date: 09 June 2025

EMPLOYEE

By: /s/ Babar Ghias
Babar Ghias

Date: 09 June 2025

Exhibit A to PIIA

Dear Sir/Madam:

I, ___Babar Ghias_____, propose to bring to my BioCryst employment the following tangible materials and previously unpublished documents, which materials and documents may be used in my BioCryst employment:

X No materials ____ See below _____ Additional sheets attached

The signature below by a representative of my current or former employer confirms that my continued possession and use of these materials is authorized.

AUTHORIZATION:

_____
Signature

_____
Title

_____
Employer

Very truly yours,

/s/ Babar Ghias_____

Exhibit B
(Non-Competition and Non-Solicitation Agreement)

This Non-Competition and Non-Solicitation Agreement (this "Agreement") is made and entered into as of July 7, 2025 (the "Effective Date) by and between Babar Ghias (the "Employee") and the member of the Company Group (as defined below) employing Employee (the "Company"). The Company and Employee are sometimes referred to in this Agreement individually as a "Party" and collectively as "Parties."

RECITALS

WHEREAS, the Company is a member of the Company Group which is comprised of BioCryst Pharmaceuticals, Inc. and its existing and future subsidiaries and affiliates (individually or collectively, "Company Group"). For the purposes of this Agreement, employment with the Company Group shall mean employment by any member of the Company Group. Throughout this Agreement, BioCryst Pharmaceuticals, Inc. may be referred to as "Parent;"

WHEREAS, Employee is beginning an employment relationship with the Company (the "Employment Agreement") as Chief Financial Officer which requires that Employee sign this Agreement as a condition of such employment, and is simultaneously entering into an Employee's Proprietary Information and Inventions Agreement (the "PIIA") with a member of the Company Group; and

WHEREAS, in consideration for Employee's promises and obligations set forth herein, the Company is offering Employee severance pay as specifically described in the Employment Agreement, including that portion of the severance pay set forth in Section 4(b)(ii) of the Employment Agreement to which Employee was not previously entitled.

NOW THEREFORE, in consideration of the foregoing recitals (which are incorporated herein by reference) and the mutual promises and obligations set forth below and other good and valuable consideration, the receipt and sufficiency of which the Parties acknowledge, the Company and Employee agree as follows:

1. **COMPANY BUSINESS AND PROTECTABLE INTERESTS.** Employee acknowledges that: (i) by virtue of Employee's position with the Company, Employee will have access to Proprietary Information, as that term is defined in the PIIA, which information has not become publicly available through no fault of Employee ("Confidential Information"); (ii) the Company Group is currently engaged primarily, but not exclusively, in the business of the discovery, development and commercialization of medicines and programs for rare diseases (the "Business"); (iii) during the course of Employee's employment, the Company Group's Business may expand or change, in which case, such expansions or changes shall correspondingly expand or (if abandoned) contract the definition of "Business" and Employee's obligations under this Agreement; (iv) due to the nature of the Business, Confidential Information developed by the Company Group in furtherance of the treatment for a particular rare disease would have commercial value to any other entity pursuing the development of medicines for the same disease regardless of the location of that entity, and the use of that information by such an entity would have a negative commercial impact on the Company Group; (v) the Company Group has clients, customers and collaborative partners throughout the United States and the world

and the specific location of a competing business is not necessarily relevant to the capacity of that business to compete with the Company Group; and (vi) the provisions of this Agreement are reasonably necessary to protect the Company Group's legitimate business interests, are reasonable as to time, territory and scope of activities which are restricted, do not interfere with public policy or public interest and are described with sufficient accuracy and definiteness to enable Employee to understand the scope of the restrictions imposed upon Employee.

2. <u>COMPETITIVE BUSINESS ACTIVITIES.</u>

(a) Employee agrees that during the period of Employee's employment with the Company Group and for a period of time ending on the date occurring one year after the date such Employee is no longer employed by any member of the Company Group (irrespective of the circumstances of such termination), Employee will not:

i. on Employee's own or another's behalf, whether as an officer, director, manager, stockholder, partner, member, associate, owner, employee, consultant, or otherwise do any of the following or provide material assistance to any other party or entity to do so:

(A) engage in the Business with respect to medicines or programs with which Employee was materially involved on behalf of the Company Group during Employee's employment or with respect to which Employee obtained Confidential Information during Employee's employment;

(B) solicit or do business which is the same, similar to or otherwise in competition with the Business, from or with persons or entities: (a) who are clients, customers or collaborative partners of the Company Group; (b) with whom or which Employee or someone for whom Employee was responsible solicited, negotiated, contracted, serviced or had material contact with on the Company Group's behalf; (c) with respect to whom or which Employee obtained Confidential Information during and as a consequence of Employee's employment with the Company Group; or (d) who at any time during the last year of Employee's employment with the Company Group, were clients, customers or collaborative partners of the Company Group; nor shall Employee request, induce, or solicit such persons or entities to curtail or cancel their business with the Company Group;

(C) offer employment to, hire or otherwise solicit for employment any employee or other person who had been employed or retained by the Company Group during the last year of Employee's employment with the Company Group and with whom Employee had material contacts during the course of Employee's employment; nor shall Employee request, induce, or solicit any employee or independent contractor of the Company Group who had been employed or retained by the Company Group during the

last year of Employee's employment with the Company Group and with whom Employee had material contacts during the course of Employee's employment to terminate his or her employment or independent contractor relationship with the Company Group; or

ii. take any action, which is materially detrimental, or otherwise intended to be adverse to the Company Group's goodwill, name, business relations, prospects and operations.

(b) The restrictions set forth in Section 2(a)(i)(A) apply to the following separate and distinct geographical areas: (i) the world; (ii) North America (iii) Europe; (iv) the United States; (v) the United Kingdom; (vi) Japan; (vii) the State of North Carolina; (viii) the State of Alabama; (ix) within a 60-mile radius of any location of the Company Group in which Employee had an office or performed material services during Employee's employment with the Company Group; (x) any city, metropolitan area, county, state or country in which Employee's substantial services were provided, or for which Employee had substantial responsibility, or in which Employee worked on Company Group projects, while employed by the Company Group; (xi) any city, metropolitan area, county, state or country in which the Company Group is located or does or, during Employee's employment with the Company Group, did business.

(c) The restrictions set forth in Section 2(a)(i)(A) apply only to prohibit Employee from engaging in activities that are materially similar to the activities in which Employee engaged on behalf of the Company Group or with respect to which Employee would reasonably be expected to use Confidential Information.

(d) Notwithstanding the foregoing, Employee's ownership, directly or indirectly, of not more than one percent of the issued and outstanding stock of a corporation the shares of which are regularly traded on a national securities exchange or in the over-the-counter market shall not violate Section 2(a).

For the avoidance of doubt, and notwithstanding any provision of this Agreement to the contrary, a transfer or assignment of employment, or an assignment of this Agreement, from the Company to a member of the Company Group or from one member of the Company Group to another member of the Company Group (in one or multiple instances), shall not be a termination of employment for the purposes of triggering the one year post-employment competitive business restrictions set forth above.

3. REMEDIES. Employee acknowledges that Employee's failure to abide by this Agreement would cause irreparable harm to the Company Group for which legal remedies would be inadequate. Therefore, in addition to any legal or other relief to which the Company Group may be entitled by virtue of Employee's failure to abide by these provisions; the Company Group, or any member thereof, may seek equitable relief, including, but not limited to, preliminary and permanent injunctive relief, for Employee's actual or threatened failure to abide by these provisions, and Employee will indemnify the Company Group for all expenses including attorneys' fees in seeking to enforce these provisions.

4. TOLLING. The period during which Employee must refrain from the activities set forth in Section 2(a) shall be tolled during any period in which Employee fails to abide by such

provisions.

5. VIOLATION BY COMPANY. In the event that Employee alleges and proves a violation by the Company Group of any obligation of the Company Group to Employee by agreement or operation of law, such violation shall not excuse Employee from Employee's obligations pursuant to this Agreement, but rather Employee shall be entitled to remedies available for the specific violation alleged and proven.

6. OTHER AGREEMENTS. Nothing in this Agreement shall terminate, revoke, or diminish Employee's obligations or the Company Group's rights and remedies under law or pursuant to the PIIA, relating to trade secrets or proprietary information.

7. ENTIRE AGREEMENT. This Agreement, the PIIA, and the Employment Agreement together constitute the exclusive and complete agreement between the Parties with respect to the subject matter contained herein and therein, and supersedes any prior agreements or understandings regarding such subject matter.No change or modification of this Agreement shall be valid or binding upon the Parties unless such change or modification is in writing and is signed by the Parties.

8. WAIVER OF BREACH. The Company's or Employee's waiver of any breach of a provision of this Agreement shall not waive any subsequent breach by the other Party.

9. SEVERABILITY. If a court of competent jurisdiction holds that any provision or sub-part thereof contained in this Agreement is invalid, illegal, or unenforceable, that invalidity, illegality, or unenforceability shall not affect any other provision in this Agreement. Additionally, if any of the provisions of this Agreement are held unenforceable by a court of competent jurisdiction, then the Parties desire that such provision, clause, or phrase be "blue- penciled" or rewritten by the court to the extent necessary to render it enforceable.

10. THIRD-PARTY BENEFICIARIES; SUCCESSORS AND ASSIGNS.

(a) The Parties agree that members of the Company Group are intended third-party beneficiaries of this Agreement with rights to enforce the terms of this Agreement to the maximum extent allowed by law.

(b) The Parties agree that this Agreement is binding upon and shall inure to the benefit of the Company, its successors and assigns. The Company shall be entitled to freely assign, in whole or in part, this Agreement and/or any right hereunder to any member of the Company Group, or to any successor of all or substantially all of the business or assets of the Company or any member of the Company Group (and any such assignee shall be entitled to freely further so assign, in one or multiple instances). The assignee shall assume the Company's obligations attendant to the rights being assigned. In the event this Agreement is assigned to any member of the Company Group, or in the event a successor-in-interest to either the Company or any member of the Company Group becomes Employee's employer under this Agreement, then the following shall apply from and after the effective date of the assignment or transfer of rights to the successor-in-interest, as the case may be: all references in this Agreement to the

Company shall be deemed to mean the assignee or successor-in-interest, as the case may be, without any need for an amendment to accomplish such substitution.

(c) Employee irrevocably consents to any such assignment and the substitution of the assignee for the Company as to rights that are assigned, and Employee also irrevocably consents to the discharge of the Company as to any obligations or liabilities under or by reason of this Agreement arising on or after the date of the assignment. In the event of any assignment from the Company to a member of the Company Group, the Company shall be an intended third-party beneficiary of this Agreement with rights to enforce the terms of this Agreement to the maximum extent allowed by law.

11. PARTIES BOUND. The terms, provisions, covenants and agreements contained in this Agreement shall apply to, be binding upon and inure to the benefit of the Company's successors and assigns, and Employee's heirs, executors, administrators, and other legal representatives. Employee may not assign this Agreement.

12. GOVERNING LAW. This Agreement and the employment relationship created by it shall be interpreted and construed in accordance with the laws of the State of North Carolina, including its statutes of limitations, without giving effect to any conflict-of-laws rule that would result in the application of the laws of a different jurisdiction. The Parties consent to sole and exclusive jurisdiction in Wake County, North Carolina for the purpose of any litigation relating to this Agreement and agree that any litigation by or involving them relating to this Agreement shall be conducted in the state courts of North Carolina or the appropriate federal district court located in Wake County, North Carolina. Employee consents to the exercise of personal jurisdiction in any state or federal court located in Wake County, North Carolina and waives any objection based upon personal jurisdiction or *forum non conveniens* with respect to any action commenced in such courts.

13. COUNTERPARTS. This Agreement may be executed in counterparts, each of which shall be an original, with the same effect as if the signatures affixed thereto were upon the same instrument. Any such counterpart, to the extent delivered by .pdf or similar attachment to electronic mail shall be treated in all manner and respects as an original executed counterpart and shall be considered to have the same binding legal effect as if it were the original signed version thereof delivered in person.

14. EMPLOYEE 14. ACKNOWLEDGMENT. Employee understands and agrees that this Agreement is not a contract of employment for any particular term and that employment by the Company is, for all purposes, "at will."

[SIGNATURES ON FOLLOWING PAGE]

IN WITNESS WHEREOF, the Parties have entered into this Non-Competition and Non-Solicitation Agreement knowingly and voluntarily as of the day and year first written above.

EMPLOYEE:
Babar Ghias

Signature: /s/ Babar Ghias

Date: 09 June 2025

EMPLOYER:
BIOCRYST PHARMACEUTICALS, INC.

Signature: /s/ Jon Stonehouse
Name: Jon Stonehouse
Title: Chief Executive Officer

Date: 09 June 2025

SECOND AMENDMENT TO EMPLOYMENT AGREEMENT

This Second Amendment to Employment Agreement (this "Amendment"), dated August 1, 2025 (the "Effective Date"), is entered into by and between BioCryst Pharmaceuticals, Inc. (the "Company") and Mr. Charles Gayer ("Employee").

RECITALS

WHEREAS, the Company and Employee are parties to that Employment Agreement, dated as of January 14, 2020, as amended by the First Amendment dated as of September 24, 2021 (the "Employment Agreement"); and

WHEREAS, the parties wish to amend the terms of the Employment Agreement, as set forth herein, effective as of the date hereof.

NOW, THEREFORE, in consideration of the mutual covenants contained herein and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties agree as follows:

1. Title. Effective as of August 1, 2025, the Company shall employ Employee as President and Chief Commercial Officer.

2. Section 2(a). The first sentence of Section 2(a) is hereby amended and restated in its entirety as follows:

   "Commencing as of August 1, 2025, as basic compensation for services rendered under this Agreement, Employee shall be entitled to receive from the Company a salary of $675,000 per annum (the "Base Salary"), payable in accordance with the Company's standard payroll practices as in effect from time to time during the term of this Agreement."

3. Target Incentive Compensation. For Employee's Incentive Compensation for the 2025 fiscal year, the "Target Amount" shall be equal to (i) seventy percent (70%) of the Base Salary earned by Employee during the portion of the 2025 fiscal preceding August 1, 2025 and (ii) seventy-five percent (75%) of the Base Salary earned by Employee during the remainder of the 2025 fiscal year beginning August 1, 2025.

4. Section 3(b). A new Section 3(b) is added as follows.

   "(b) For the 2025 fiscal year, Employee's annual equity-based awards shall be determined by the Board or a committee thereof at levels commensurate with the role of President and Chief Executive Officer."

5. Continuation of Employment Agreement. Except as otherwise expressly provided herein, all of the terms and provisions of the Employment Agreement shall remain in full force and effect and this Amendment shall not amend or modify any other rights, powers, duties, or obligations of any party to the Employment Agreement.

6. Complete Agreement. This Amendment and the Employment Agreement contain the entire agreement between the parties hereto with respect to the matters contained herein and supersedes and replaces any prior agreement between the parties with respect to the matters set forth in this Amendment.

7. Counterparts. This Amendment may be executed in any number of counterparts and any such counterparts may be transmitted by electronic transmission, and each of such counterparts, whether an original or an electronic or ".pdf" of an original, shall be deemed to be an original and all of such counterparts together shall constitute a single agreement.

IN WITNESS WHEREOF, the parties hereto have executed this Amendment, on the date first set forth above.

EMPLOYEE

Charles Gayer

By: /s/ Charles Gayer
Charles Gayer

BIOCRYST PHARMACEUTICALS, INC.

By:
Jon Stonehouse
Chief Executive Officer

By: /s/ Jon Stonehouse
Jon Stonehouse
Chief Executive Officer

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: August 5, 2025

/s/ Jon P. Stonehouse

Jon P. Stonehouse

Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Babar Ghias, 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: August 5, 2025

/s/ Babar Ghias

Babar Ghias

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

Exhibit 32.1

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

In connection with the Quarterly Report of BioCryst Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended June 30, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Jon P. Stonehouse, Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

(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

Chief Executive Officer
(Principal Executive Officer)

Date: August 5, 2025

Exhibit 32.2

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 June 30, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Babar Ghias, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

(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/ Babar Ghias

Babar Ghias

Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)

Date: August 5, 2025