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

FORM 10-Q

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

For the quarterly period ended September 30, 2023

or

o 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**	**27703**
(Address of principal executive offices)	(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	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 x No o

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 x No o

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	x	Accelerated filer	o
Non-accelerated filer	o	Smaller reporting company	o
		Emerging growth company	o

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. o

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

The number of shares of Common Stock, par value $0.01, of the Registrant outstanding as of October 31, 2023 was 204,809,380.

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 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), BCX10013, and early-stage discovery programs (including BCX17725, avoralstat, and our complement inhibitors), and our plans 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 financing;
- the timing or likelihood of regulatory filings or regulatory agreements, deferrals, approvals, and other decisions;
- our ability to manage our liquidity needs, including our ability to raise additional capital, to fund our operations or repay our recourse debt obligations;
- our financial performance;
- our ability to remediate any material weaknesses in our internal control over financial reporting; and

- competitive companies, technologies, and our industry.

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

- The ongoing novel coronavirus ("COVID-19") pandemic could create challenges in all aspects of our business, including, without limitation, delays, stoppages, difficulties, and increased expenses with respect to our and our partners' development, regulatory processes, and supply chains, negatively impact our ability to access the capital or credit markets to finance our operations, or have the effect of heightening many of the risks described below or in the "Risk Factors" section in Part II, Item 1A of this report.

- We have incurred losses since our inception, expect to continue to incur losses, and may never be profitable.

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

- Our success depends upon our ability to manage our product candidate pipeline, advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive and maintain regulatory approvals for the commercial sale of our product candidates. The development process and related regulatory processes are complex and uncertain, may be lengthy and expensive, and require, among other things, an indication that our products and product candidates are safe and effective. For example, applicable regulatory agencies could refuse to approve, or impose restrictions or warnings on, our product candidates, require us to conduct additional studies or adopt study designs that differ from our planned development strategies, suspend or terminate our clinical trials, withdraw approval for our products, or take other actions that could materially impact the cost, timing, and success of our planned development and commercialization strategies.

- We rely heavily upon third parties, including development partners, contractors, contract research organizations, and third-party suppliers, manufacturers, and distributors, for many important stages of our product candidate development and in the commercialization of certain of our products and product candidates. Our failure to establish and maintain these relationships, the failure of any such third party to perform its obligations under agreements with us, or the failure of such a relationship to meet our expectations could have a material adverse impact on our business, financial condition, and results of operations.

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

- 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, develop our product candidates, obtain collaborators, and raise capital.
- 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 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 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 incur damage or power is lost for a significant length of time, our business will suffer.
- A significant disruption in our or our third-party vendors' information technology systems or a cybersecurity breach could adversely affect our business.
- 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.
- We have identified material weaknesses in our internal control over financial reporting. These material weaknesses could divert management's attention and adversely affect our ability 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 may 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.

- 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.
- We are subject to legal proceedings, which could harm our reputation or result in other losses or unexpected expenditure of time and resources.
- If we fail to retain our existing key personnel or fail to attract and retain additional key personnel, the development of our product candidates, the commercialization of our products, and the related expansion of our business will be delayed or stopped.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

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

	September 30, 2023	December 31, 2022
Assets		
Cash and cash equivalents	$ 150,905	$ 304,767
Restricted cash	1,584	1,472
Investments	246,685	119,543
Trade receivables	53,646	50,599
Inventory, net	29,630	27,533
Prepaid expenses and other current assets	20,016	12,586
Total current assets	502,466	516,500
Property and equipment, net	8,999	8,617
Long-term investments	—	18,077
Other assets	11,459	6,806
Total assets	$ 522,924	$ 550,000
Liabilities and Stockholders' Deficit		
Accounts payable	$ 10,567	$ 14,356
Accrued expenses	77,392	87,565
Deferred revenue	522	1,224
Lease financing obligation	2,282	2,369
Total current liabilities	90,763	105,514
Lease financing obligation	9,966	5,804
Royalty financing obligations	535,186	501,655
Secured term loans	297,995	231,624
Stockholders' deficit:		
Preferred stock, $0.01 par value; shares authorized - 5,000; no shares issued and outstanding	—	—
Common stock, $0.01 par value; shares authorized - 450,000; shares issued and outstanding – 189,803 as of September 30, 2023 and 187,906 as of December 31, 2022	1,898	1,879
Additional paid-in capital	1,205,744	1,158,118
Accumulated other comprehensive income	800	26
Accumulated deficit	(1,619,428)	(1,454,620)
Total stockholders' deficit	(410,986)	(294,597)
Total liabilities and stockholders' deficit	$ 522,924	$ 550,000

See accompanying notes to condensed consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(In thousands, except per share data, Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
Revenues	$ 86,742	$ 75,827	$ 238,011	$ 191,282
Expenses:				
Cost of product sales	1,099	3,543	2,924	4,025
Research and development	46,879	52,740	146,514	180,090
Selling, general and administrative	50,648	36,919	149,512	109,218
Royalty	37	70	100	73
Total operating expenses	98,663	93,272	299,050	293,406
Loss from operations	(11,921)	(17,445)	(61,039)	(102,124)
Interest and other income	4,184	1,760	11,312	2,423
Interest expense	(27,345)	(24,775)	(83,656)	(72,634)
Foreign currency losses, net	(737)	(538)	(665)	(583)
Loss on extinguishment of debt	—	—	(29,019)	—
Loss before income taxes	(35,819)	(40,998)	(163,067)	(172,918)
Income tax expense	330	1,522	1,741	2,657
Net loss	$ (36,149)	$ (42,520)	$ (164,808)	$ (175,575)
Foreign currency translation adjustment	(185)	695	(67)	877
Unrealized gain (loss) on available for sale investments	295	(808)	841	(1,152)
Comprehensive loss	$ (36,039)	$ (42,633)	$ (164,034)	$ (175,850)
Basic and diluted net loss per common share	**$ (0.19)**	**$ (0.23)**	**$ (0.87)**	**$ (0.95)**
Weighted average shares outstanding	**189,644**	**186,180**	**189,095**	**185,566**

See accompanying notes to condensed consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands, Unaudited)

	Nine Months Ended September 30,	
	2023	**2022**
Cash flows from operating activities:		
Net loss	$ (164,808)	$ (175,575)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,232	1,041
Inventory obsolescence	236	—
Stock-based compensation expense	39,127	29,419
Non-cash interest expense on royalty financing obligations and secured term loan and amortization of debt issuance costs	66,286	55,119
Amortization of premium/discount on investments	(6,814)	(818)
Loss on extinguishment of debt	29,019	—
Changes in operating assets and liabilities:		
Receivables	(3,125)	(13,827)
Inventory	(2,357)	(11,263)
Prepaid expenses and other assets	(7,530)	(1,132)
Accounts payable and accrued expenses	(36,678)	(27,639)
Interest payable	—	6,345
Deferred revenue	(811)	329
Net cash used in operating activities	**(86,223)**	**(138,001)**
Cash flows from investing activities:		
Acquisitions of property and equipment	(1,614)	(825)
Purchase of investments	(357,419)	(244,283)
Sales and maturities of investments	256,008	39,655
Net cash used in investing activities	**(103,025)**	**(205,453)**
Cash flows from financing activities:		
Net proceeds from common stock issued under stock-based compensation plans	8,518	10,756
Net proceeds from secured term loans	300,000	73,072
Repayment of Athyrium secured term loans principal	(240,452)	—
Prepayment and repayment fees on Athyrium secured term loans	(21,261)	—
Payment of debt issuance costs on Pharmakon Tranche A term loan	(11,147)	—
Net cash provided by financing activities	**35,658**	**83,828**
Effect of exchange rate on cash, cash equivalents, and restricted cash	(160)	235
Decrease in cash, cash equivalents and restricted cash	(153,750)	(259,391)
Cash, cash equivalents and restricted cash at beginning of period	306,239	507,734
Cash, cash equivalents and restricted cash at end of period	**$ 152,489**	**$ 248,343**

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
Balance at December 31, 2022	**$ 1,879**	**$ 1,158,118**	**$ 26**	**$ (1,454,620)**	**$ (294,597)**
Net loss	—	—	—	(53,333)	(53,333)
Other comprehensive income	—	—	559	—	559
Employee stock purchase plan sales, 176 shares, net	2	1,573	—	—	1,575
Exercise of stock awards, 801 shares, net	8	3,494	—	—	3,502
Stock-based compensation expense	—	14,007	—	—	14,007
Balance at March 31, 2023	**$ 1,889**	**$ 1,177,192**	**$ 585**	**$ (1,507,953)**	**$ (328,287)**
Net loss	—	—	—	(75,326)	(75,326)
Other comprehensive income	—	—	105	—	105
Exercise of stock awards, 608 shares, net	6	1,948	—	—	1,954
Stock-based compensation expense	—	12,841	—	—	12,841
Balance at June 30, 2023	**$ 1,895**	**$ 1,191,981**	**$ 690**	**$ (1,583,279)**	**$ (388,713)**
Net loss	—	—	—	(36,149)	(36,149)
Other comprehensive income	—	—	110	—	110
Employee stock purchase plan sales, 162 shares, net	2	1,018	—	—	1,020
Exercise of stock awards, 149 shares, net	1	466	—	—	467
Stock-based compensation expense	—	12,279	—	—	12,279
Balance at September 30, 2023	**$ 1,898**	**$ 1,205,744**	**$ 800**	**$ (1,619,428)**	**$ (410,986)**

BIOCRYST PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' DEFICIT

(In thousands, Unaudited)

	Common Stock	Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Deficit
Balance at December 31, 2021	**$ 1,843**	**$ 1,098,498**	**$ 177**	**$ (1,207,504)**	**$ (106,986)**
Net loss	—	—	—	(74,196)	(74,196)
Other comprehensive income	—	—	9	—	9
Employee stock purchase plan sales, 115 shares, net	1	1,503	—	—	1,504
Exercise of stock awards, 1,108 shares, net	12	5,841	—	—	5,853
Stock-based compensation expense	—	9,601	—	—	9,601
Balance at March 31, 2022	**$ 1,856**	**$ 1,115,443**	**$ 186**	**$ (1,281,700)**	**$ (164,215)**
Net loss	—	—	—	(58,859)	(58,859)
Other comprehensive loss	—	—	(171)	—	(171)
Exercise of warrants, 253 shares	3	—	—	—	3
Exercise of stock awards, 51 shares, net	—	145	—	—	145
Stock-based compensation expense	—	9,865	—	—	9,865
Balance at June 30, 2022	**$ 1,859**	**$ 1,125,453**	**$ 15**	**$ (1,340,559)**	**$ (213,232)**
Net loss	—	—	—	(42,520)	(42,520)
Other comprehensive loss	—	—	(113)	—	(113)
Employee stock purchase plan sales, 145 shares, net	1	1,356	—	—	1,357
Exercise of stock awards, 390 shares, net	4	1,892	—	—	1,896
Stock-based compensation expense	—	9,953	—	—	9,953
Balance at September 30, 2022	**$ 1,864**	**$ 1,138,654**	**$ (98)**	**$ (1,383,079)**	**$ (242,659)**

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 complement-mediated 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 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 pharmaceuticals through the process known as structure-guided drug design.

The Company's marketed products include oral, once-daily ORLADEYO® for the prevention of hereditary angioedema ("HAE") attacks and RAPIVAB® (peramivir injection) for the treatment of acute uncomplicated influenza in the United States. ORLADEYO received regulatory approval in the United States in December 2020. ORLADEYO has also received regulatory approvals in multiple global markets. The Company is commercializing ORLADEYO in each of these territories directly or through distributors, except in Japan where Torii Pharmaceutical Co., Ltd. ("Torii"), the Company's collaborative partner, has the exclusive right to commercialize ORLADEYO for the prevention of HAE attacks in exchange for certain milestone and royalty payments to the Company. In addition to its approval in the United States, peramivir injection has received regulatory approvals in Canada, Australia, Japan, Taiwan and Korea.

Based on the Company's expectations for revenue and operating expenses, the Company believes its financial resources available at September 30, 2023 will be sufficient to fund its operations for at least the next 12 months. The Company has sustained operating losses for the majority of its corporate history and expects that its 2023 expenses will exceed its 2023 revenues. The Company expects to continue to incur operating losses and negative cash flows until revenues reach a level sufficient to support ongoing operations. The Company's liquidity needs will largely be determined by the success of operations in regard to the successful commercialization of its products and the progression of its product candidates in the future. The Company regularly evaluates other opportunities to fund future operations, including: (1) out-licensing rights to certain of its products or product candidates, pursuant to which the Company would receive cash milestone payments; (2) raising additional capital through equity or debt financings or from other sources, including 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 its overhead structure; and/or (6) securing U.S. Government funding of its programs, including obtaining procurement contracts. The Company may issue securities, including common stock, preferred stock, depositary shares, purchase contracts, warrants, debt securities and units, through private placement transactions or registered public offerings in the future. 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'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.

The Company has made certain presentation changes relative to its revenue, which management considers fundamental to understanding the Company's current business and financial performance related to its primary product, ORLADEYO, including expanded international sales of ORLADEYO, relative to the Company's other sources of revenue. Accordingly, certain disaggregated revenue information has been provided in this Note 1 and "Note 2—Revenue" to these condensed consolidated financial statements. These presentation changes have been applied to prior year revenue amounts for consistency and comparability.

These financial statements should be read in conjunction with the financial statements for the year ended December 31, 2022 and the notes thereto included in the Company's 2022 Annual Report on Form 10-K. Interim operating results are not necessarily indicative of operating results for the full year. The condensed consolidated balance sheet as of December 31, 2022 has been 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, the ORLADEYO and Factor D inhibitors royalty financing obligations, inventory reserves, certain accruals, primarily related to the Company's research and development expenses, the valuation of stock options and the valuation allowance for deferred tax assets resulting from net operating losses. These estimates are based on historical experience and on various other assumptions that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results could differ from those estimates.

Revenue Recognition

The Company recorded the following revenues for the three and nine months ended September 30, 2023 and 2022:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
Product sales, net	$ 85,288	$ 75,213	$ 233,957	$ 189,647
Collaborative and other revenues	1,454	614	4,054	1,635
Total revenues	**$ 86,742**	**$ 75,827**	**$ 238,011**	**$ 191,282**

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, sales of peramivir to the Company's licensing partners and sales of RAPIVAB to the U.S. Department of Health and Human Services ("HHS") under the Company's procurement contract. In the United States, the Company ships ORLADEYO directly to patients through a single specialty pharmacy, which is considered its customer. In the European Union, United Kingdom and elsewhere, the Company sells ORLADEYO to specialty distributors as well as hospitals and pharmacies, which collectively are considered its customers.

The Company recognizes revenue for sales when its customers obtain control of the product, which generally occurs upon delivery. For ORLADEYO, the Company classifies payments to its specialty pharmacy customer for certain services

provided by its customer as selling, general and administrative expenses to the extent such services provided are determined to be distinct from the sale of its product.

Net revenue from sales of ORLADEYO is recorded at net selling price (transaction price), which includes estimates of variable consideration for which reserves are established for (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 are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable or as a current liability. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the applicable contract. The amount of variable consideration included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from estimates, the Company adjusts these estimates, 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. These reserves are recorded in the same period in which the revenue is 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, (iii) a range of possible outcomes that are probability-weighted for the estimated payor mix, and (iv) product distribution information obtained from the Company's specialty pharmacy.

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 products 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 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 from the Company. The Company estimates chargebacks and adjusts gross product revenues and accounts receivable based on the estimates 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 is able to 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. 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, as well as research and development agreements with certain government entities. 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.

Milestone payments are recognized as licensing revenue upon the achievement of specified milestones if (i) the milestone is substantive in nature and the achievement of the milestone was not probable at the inception of the agreement and (ii) the Company has a right to payment. Any milestone payments received prior to satisfying these revenue recognition criteria are recorded as deferred revenue.

Reimbursements received for direct out-of-pocket expenses related to research and development costs are recorded as revenue in the Condensed Consolidated Statements of Comprehensive Loss rather than as a reduction in expenses. Under the Company's contracts with the Biomedical Advanced Research and Development Authority within HHS ("BARDA/HHS") and the National Institute of Allergy and Infectious Diseases ("NIAID/HHS"), revenue is recognized as reimbursable direct and indirect costs are incurred.

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, certificates of deposit, money market accounts or investments in debt instruments 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 $1,584 and $1,472 as of September 30, 2023 and December 31, 2022, respectively, and primarily consisted of $1,466 and $1,449 as of September 30, 2023 and December 31, 2022, respectively, for a letter of credit the Company is required to maintain associated with the lease execution and build-out of its Birmingham research facilities.

Investments

The Company invests in high credit quality investments in accordance with its investment policy, which is designed to minimize the possibility of loss. The objective of the Company's investment policy is to ensure the safety and preservation of invested funds, as well as maintaining liquidity sufficient to meet cash flow requirements. The Company places its excess cash with high credit quality financial institutions, commercial companies, and government agencies in order 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. Government and government agency securities, money market and mutual fund investments, certificates of deposits, municipal and corporate notes and bonds, and commercial paper, among others. The Company's investment policy requires it to purchase high-quality marketable securities with a maximum individual maturity of three 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. Generally, the Company's investments are not collateralized. The Company has not realized any significant losses from its investments.

The Company classifies all of its investments as available-for-sale. Unrealized gains and losses on investments are recognized in comprehensive loss, unless an unrealized loss is considered to be other than temporary, in which case the unrealized loss is charged to operations. The Company periodically reviews its investments for other than temporary declines in fair value below cost basis and whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. The Company believes the individual unrealized losses represent temporary declines primarily resulting from interest rate changes. The Company does not have 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. Realized gains and losses are reflected in interest and other income in the Condensed Consolidated Statements of Comprehensive 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.

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 reimbursable research and development costs from HHS, and royalty receivables from the Company's partners, including Shionogi & Co., Ltd., Green Cross, and Torii.

Monthly invoices are submitted to HHS related to reimbursable research and development costs. The Company is also entitled to monthly reimbursement of indirect costs based on rates stipulated in the underlying contract. The Company's calculations of its indirect cost rates are subject to audit by the U.S. Government.

The Company does not adjust its receivables for the effects of a significant financing component at contract inception if it expects to collect the receivables in one year or less from the time of sale.

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 inventories primarily relate to ORLADEYO. Additionally, the Company's inventories include RAPIVAB and peramivir.

The Company values its inventories at the lower of cost or estimated net realizable value. The Company determines the cost of its inventories, which includes amounts related to materials, labor, manufacturing overhead and shipping and handling costs 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's inventories are subject to expiration dating. The Company regularly evaluates the carrying value of its inventories and provides valuation reserves for any estimated obsolete, short-dated or unmarketable inventories. 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. During the nine months ended September 30, 2023, the Company evaluated its inventory levels and associated expiration dating relative to the latest sales forecasts for ORLADEYO, RAPIVAB, and peramivir and estimated those

inventories at risk of obsolescence. Accordingly, the Company recorded an increase to the inventory valuation reserve of $170 for a total reserve of $1,347 as of September 30, 2023.

The Company expenses costs related to the production of inventories as research and development expenses in the period incurred until such time it is believed that future economic benefit is expected to be recognized, which generally is upon receipt of regulatory approval. Upon regulatory approval, the Company capitalizes subsequent costs related to the production of inventories.

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 is depreciated over a life of three years. Laboratory equipment, office equipment, and software are depreciated over a life of five years. Furniture and fixtures are depreciated over a life of seven years. Leasehold improvements are amortized over their estimated useful lives or the expected lease term, whichever is less.

In accordance with U.S. GAAP, 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 level of service performed 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. In accruing service fees, the Company estimates 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 the estimate, the Company will adjust the accrual accordingly. If the Company does not identify costs that it has begun to incur or if it underestimates or overestimates the level of these costs, actual expenses could differ from such estimates. As of September 30, 2023 and December 31, 2022, 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 and distributing inventories that are related to product revenue during the respective period, including freight. In addition, shipping and handling costs for product shipments are recorded as incurred. Finally, cost of product sales may also include costs related to excess or obsolete inventory adjustment charges.

Research and Development Expenses

The Company's research and development costs are expensed when incurred. Research and development expenses include all direct and indirect development costs related to the development of the Company's portfolio of product candidates. 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. Research and development expenses include, among other items, personnel costs, including salaries and benefits, manufacturing costs, clinical, regulatory, and toxicology services performed by 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 the Company's manufacturing and clinical and preclinical studies are performed by third-party CROs. Costs for studies performed by CROs are accrued by the Company over the service periods specified in the contracts, and estimates are adjusted, if required, based upon the Company's ongoing review of the level of services actually performed.

Additionally, the Company has license agreements with third parties which require fees related to sublicense agreements or maintenance fees. The Company expenses sublicense payments as incurred unless they are related to revenues that have been deferred, in which case the expenses are deferred and recognized over the related revenue recognition period. The Company expenses maintenance payments as incurred.

Deferred collaboration expenses represent sublicense payments paid to the Company's academic partners upon receipt of consideration from various commercial partners, and other consideration paid to the Company's academic partners for modification to existing license agreements. These deferred expenses would not have been incurred without receipt of such payments or modifications from the Company's commercial partners and are being expensed in proportion to the related revenue being recognized. The Company believes that this accounting treatment appropriately matches expenses with the associated revenue.

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 expenses consist of lab supplies and services, facility expenses, depreciation of development equipment and other overhead of the Company's research and development efforts. These costs apply to work on non-active product candidates and the Company's discovery research efforts.

Selling, General and Administrative Expenses

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

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

Leases

The Company leases certain assets, predominantly under operating leases, which consist of real estate leases, laboratory equipment leases and office equipment leases as of September 30, 2023. 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.

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

The discount rate used in the calculation of the Company's right-of-use asset and lease liability was determined based on the stated rate within each contract when available, or the Company's collateralized borrowing rate from lending institutions.

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 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 life of an award, 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 vesting periods. The estimation of share-based payment awards that will ultimately vest requires judgment, and to the extent actual results or updated estimates differ from the Company's current estimates, such amounts will be recorded as a cumulative adjustment in the period estimates are revised. In addition, the Company has outstanding performance-based stock options and restricted stock units for which no compensation expense is recognized until "performance" is deemed to have occurred. Significant management judgment is also required in determining estimates of future stock price volatility to be used in the valuation of the options. Actual results, and future changes in estimates, may differ substantially from the Company's current estimates.

Interest Expense and Deferred Financing Costs

Interest expense primarily relates to the royalty financing obligations (Note 6) and the secured term loan borrowings under the Athyrium Credit Agreement during 2022 and the term loan borrowings under both the Athyrium Credit Agreement and the Pharmakon Loan Agreement during 2023 (Note 7). On April 17, 2023, the Company entered into the Pharmakon Loan Agreement and received initial funding in the form of a term loan of $300,000, the proceeds of which were primarily used to repay the term loan borrowings under the Athyrium Credit Agreement. Accordingly, interest expense for both the three and nine months ended September 30, 2023 includes interest expense from both the Athyrium Credit Agreement and the Pharmakon Loan Agreement. 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. Amortization of deferred financing costs included in interest expense was $230 and $1,536 for the three and nine months ended September 30, 2023, respectively, and $258 and $597 for the three and nine months ended September 30, 2022, respectively. When utilizing the effective interest method, in periods in which payment-in-kind ("PIK") interest was designated and was added to the outstanding principal balance of the borrowing, the amortization of the deferred debt fees and issuance costs was accretive. The quarter ended December 31, 2022 was the last period eligible for the PIK Interest Payment designation under the Athyrium Credit Agreement.

Interest Expense and Royalty Financing Obligations

The royalty financing obligations are eligible to be repaid based on royalties from net sales of ORLADEYO and BCX10013. 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.

The Company accounts for uncertain tax positions in accordance with U.S. GAAP. 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 taxable income in each of the jurisdictions in which the Company operates and the period over which its deferred tax assets will be recoverable.

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.

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- or 15-year period, depending on the location of the activities performed. 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 five for activities conducted in the United States or year 15 in the case of development conducted on foreign soil.

Net Loss Per Share

Basic net loss per share is based upon the weighted average number of common shares outstanding during the period. Diluted net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding during the period, after giving consideration to the dilutive effect of potentially dilutive common shares. The Company has generated a net loss in all periods presented, so the diluted net loss per share is equivalent to basic net loss per share for all periods presented herein because common equivalent shares from unexercised stock options, warrants and common shares expected to be issued under the Company's equity compensation plans would be anti-dilutive. The Company excluded the following potential common shares, presented based on amounts outstanding as of September 30, 2023 and September 30, 2022, from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect:

	September 30, 2023	September 30, 2022
Outstanding stock options	35,160	32,647
Unvested restricted stock unit awards	4,589	2,760
Warrants to purchase common stock	15,023	15,023
Total	54,772	50,430

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 Loss. There were no realized gains or losses reclassified out of accumulated other comprehensive income for the three and nine months ended September 30, 2023 and 2022.

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 and other global markets and, for 2022, sales of RAPIVAB (peramivir injection) under the Company's procurement contract with the Assistant Secretary for Preparedness and Response within HHS.

ORLADEYO is distributed through an arrangement with a single specialty pharmacy in the United States, which represents the substantial majority of the ORLADEYO net product sales. The specialty pharmacy subsequently sells ORLADEYO to its customers (pharmacy benefit managers, insurance companies, government programs and group purchasing organizations) and dispenses product to patients. The specialty pharmacy's inability or unwillingness to

continue these distribution activities could adversely impact the Company's business, results of operations and financial condition.

The Company is distributing ORLADEYO in other global markets directly or through distributors, except in Japan where Torii, the Company's collaborative partner, has the exclusive right to commercialize ORLADEYO.

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.

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.

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. Sales of ORLADEYO from the Company to the specialty pharmacy only occur once an order of product has been received by the specialty pharmacy from one of its customers, which include pharmacy benefit managers, insurance companies, government programs and group purchasing organizations.

The majority of the Company's receivables from collaborations are due from the U.S. Government, for which there is no assumed credit risk.

Recently Adopted Accounting Pronouncements

There have been no new accounting pronouncements adopted by the Company or new accounting pronouncements issued by the Financial Accounting Standards Board during the nine months ended September 30, 2023, as compared to the recent accounting pronouncements described in Note 1 of the Company's Annual Report on Form 10-K for the year ended December 31, 2022, that the Company believes are of significance or potential significance to the Company.

Note 2 — Revenue

The Company recorded the following revenues (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
ORLADEYO:				
U.S.	$ 75,268	$ 60,106	$ 208,934	$ 162,743
Outside of U.S.	10,416	5,866	26,173	18,155
Total ORLADEYO	85,684	65,972	235,107	180,898
Other revenues	1,058	9,855	2,904	10,384
Total revenues	$ 86,742	$ 75,827	$ 238,011	$ 191,282

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 injection (RAPIVAB/RAPIACTA/PERAMIFLU) and galidesivir development contracts with BARDA/HHS and NIAID/HHS.

Note 3 — Investments

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

The Company's financial instruments that are measured at fair value on a recurring basis consist of fixed income investments. These valuations are based on observable direct and indirect inputs, primarily quoted prices of similar, but not identical, instruments in active markets or quoted prices for identical or similar instruments in markets that are not active. These fair values are obtained from independent pricing services.

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

	September 30, 2023			
	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	$ —	$ 243,638	$ —	$ 243,638
Corporate debt securities	—	2,074	—	2,074
Certificates of deposit	—	973	—	973
Total assets	$ —	$ 246,685	$ —	$ 246,685

	December 31, 2022			
	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	$ —	$ 129,371	$ —	$ 129,371
Corporate debt securities	—	6,092	—	6,092
Certificates of deposit	—	2,157	—	2,157
Total assets	$ —	$ 137,620	$ —	$ 137,620

There were no changes in valuation techniques during the three and nine months ended September 30, 2023 and 2022. There were no liabilities measured at fair value on a recurring basis as of September 30, 2023 and December 31, 2022.

As of September 30, 2023, the Company had 24 securities with a total estimated fair market value of $215,095 in an unrealized loss position. The Company anticipated a full recovery of the amortized cost basis of its debt securities at maturity and an allowance was not recognized.

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

	September 30, 2023				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its agencies	$ 243,810	$ 37	$ 3	$ (212)	$ 243,638
Corporate debt securities	2,068	6	—	—	2,074
Certificates of deposit	980	6	—	(13)	973
Total investments	$ 246,858	$ 49	$ 3	$ (225)	$ 246,685

	December 31, 2022				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its agencies	$ 129,940	$ 427	$ —	$ (996)	$ 129,371
Corporate debt securities	6,093	37	—	(38)	6,092
Certificates of deposit	2,163	23	—	(29)	2,157
Total investments	$ 138,196	$ 487	$ —	$ (1,063)	$ 137,620

The following table summarizes the scheduled maturity for the Company's investments at September 30, 2023 and December 31, 2022 (in thousands):

	September 30, 2023	December 31, 2022
Maturing in one year or less	$ 246,685	$ 119,543
Maturing after one year through two years	—	18,077
Total investments	$ 246,685	$ 137,620

Note 4 — Trade Receivables

Product Sales

Receivables from product sales are recorded for amounts due to the Company related to sales of ORLADEYO and RAPIVAB. At September 30, 2023 and December 31, 2022, receivables net of reserves related to sales of ORLADEYO were $51,837 and $41,508, respectively. A returns reserve related to sales of ORLADEYO of $124 was recorded as of September 30, 2023. No reserve or allowance amounts were recorded as of December 31, 2022. At September 30, 2023 and December 31, 2022, receivables related to sales of RAPIVAB were $132 and $823, respectively.

Collaborations

Receivables from collaborations were as follows (in thousands):

	September 30, 2023		
	Billed	Unbilled	Total
U.S. Department of Health and Human Services, net	$ —	$ 113	$ 113
Royalty receivables from partners	1,564	—	1,564
Total receivables	$ 1,564	$ 113	$ 1,677

	December 31, 2022		
	Billed	Unbilled	Total
U.S. Department of Health and Human Services, net	$ 7,218	$ 284	$ 7,502
Royalty receivables from partners	741	—	741
Other collaborations	—	25	25
Total receivables	$ 7,959	$ 309	$ 8,268

As of September 30, 2023 and December 31, 2022, the Company maintained a reserve of $522 and $437, respectively, related to royalties associated with Green Cross.

Note 5 — Inventory

At September 30, 2023 and December 31, 2022, the Company's inventory primarily related to ORLADEYO. Additionally, inventory included RAPIVAB and peramivir, which is manufactured for the Company's partners.

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

	September 30, 2023	December 31, 2022
Raw materials	$ 6,656	$ 8,906
Work-in-process	17,739	14,990
Finished goods	6,582	4,814
Total inventory	$ 30,977	$ 28,710
Reserves	(1,347)	(1,177)
Total inventory, net	$ 29,630	$ 27,533

Note 6 — Royalty Monetizations

ORLADEYO and Factor D Inhibitors

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 3.0% of proceeds received by the Company on annual net sales of up to $150,000 in the Other Markets, and (iii) 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.

Under the 2021 RPI Royalty Purchase Agreement, RPI is also entitled to receive tiered, sales-based royalties on net product sales of BCX10013 in an amount equal to: (i) 3.0% of worldwide aggregate annual net sales up to $1,500,000 and (ii) 2.0% of worldwide aggregate annual net sales between $1,500,000 and $3,000,000. No royalty payments are payable on annual net sales above $3,000,000. RPI is also entitled to receive tiered profit share amounts of up to 3.0% from certain other permitted sales in certain 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 is 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). For each calendar quarter beginning on or after January 1, 2024, OMERS will be entitled to receive tiered, sales-based royalties on Direct Sales in an amount equal to: (i) 10.0% of aggregate annual net sales of ORLADEYO for annual net sales up to $350,000 and (ii) 3.0% of annual net sales of ORLADEYO for annual net sales between $350,000 and $550,000 (with no royalty payments payable on annual Direct Sales over $550,000).

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

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

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

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

The cash consideration obtained pursuant to the Royalty Purchase Agreements is recorded in “Royalty financing obligations” on the Company’s Condensed Consolidated Balance Sheets. The fair value for the royalty financing obligations at the time of the transactions was based on the Company’s estimates of future royalties expected to be paid to the counterparty over the life of the arrangement. The Company subsequently records the obligations at their carrying value

using the effective interest method. In order to amortize the royalty financing obligations, the Company utilizes the prospective method to estimate the 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 a combination of internal projections and forecasts from external sources. The estimates of future net product sales (and resulting royalty payments) are based on key assumptions including population, penetration, probability of success, and sales price, among others. 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.

During the three months ended September 30, 2023, the Company adjusted its forecasts related to its BCX10013 program and updated its ORLADEYO forecast based on actual results for the first nine months of 2023. The primary factors that impacted forecasts on the BCX10013 development program were development delays, reduced probability of success, reduced pricing assumptions and reduced market share assumptions. These adjustments impacted the amount and timing of expected royalties to be made under the RPI Royalty Purchase Agreements. As a result, the effective interest rate related to the 2020 RPI Royalty Purchase Agreement decreased from 22.2% to 22.1%, the effective interest rate related to the 2021 RPI Royalty Purchase Agreement decreased from 10.0% to 0.0%, and the effective interest rate related to the OMERS Royalty Purchase Agreement decreased from 10.6% to 10.2%.

The following table shows the activity within the Royalty financing obligations account (in thousands) as well as the effective interest rate as of September 30, 2023:

	2020 RPI Royalty Agreement	2021 RPI Royalty Agreement	OMERS Royalty Agreement	Total
Balance as of December 31, 2022	$ 164,981	$ 173,651	$ 163,023	$ 501,655
Non-cash Interest expense on Royalty financing obligations	9,309	5,680	4,329	19,318
Royalty revenues paid and payable	(6,038)	(524)	—	(6,562)
Balance as of March 31, 2023	$ 168,252	$ 178,807	$ 167,352	$ 514,411
Non-cash Interest expense on Royalty financing obligations	9,552	5,440	4,494	19,486
Royalty revenues paid and payable	(7,155)	(621)	—	(7,776)
Balance as of June 30, 2023	$ 170,649	$ 183,626	$ 171,846	$ 526,121
Non-cash Interest expense on Royalty financing obligations	9,647	3,068	4,557	17,272
Royalty revenues paid and payable	(7,553)	(654)	—	(8,207)
Balance as of September 30, 2023	$ 172,743	$ 186,040	$ 176,403	$ 535,186
Effective interest rate	22.1 %	— %	10.2 %	

The Royalty financing obligations liabilities and the associated deferred issuance costs are amortized using the effective interest method over the term of the arrangement, in accordance with the respective guidance.

Concurrent with entering into the 2021 RPI Royalty Purchase Agreement, the Company and RPI entered into a Common Stock Purchase Agreement (the "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. See "Note 9—Stockholders' Equity" for further details on the common stock sale premium.

Note 7 — Debt

Pharmakon Loan Agreement

On April 17, 2023, the Company entered into a $450,000 Loan Agreement (the "Pharmakon Loan Agreement") with BioPharma Credit Investments V (Master) LP and BPCR Limited Partnership, as lenders, and BioPharma Credit PLC, as collateral agent for the lenders. Certain of the Company's wholly-owned subsidiaries are guarantors to the Pharmakon Loan Agreement. The Pharmakon Loan Agreement provides for an initial term loan in the principal amount of $300,000 (the "Tranche A Loan") funded on April 17, 2023 (the "Tranche A Closing Date"). The Company used a portion of the proceeds from the Tranche A Loan to repay the $241,787 of outstanding indebtedness (principal and interest due as of April 17, 2023) under the then-existing Athyrium Credit Agreement and to pay associated transaction costs and fees, and intends to use the remaining net proceeds of $25,805 for other general corporate purposes.

The Pharmakon Loan Agreement also provides 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 may be requested on or prior to September 30, 2024. 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 has 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 will bear interest at a rate equal to the three-month Secured Overnight Financing Rate ("SOFR") rate, 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 is 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 13.24% for the three months ended September 30, 2023.

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, upon the drawing of any Subsequent Tranche Loan, certain funding fees are required to be paid.

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

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

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

As of September 30, 2023, the Company had total borrowings of $300,000 under the Pharmakon Loan Agreement. Total quarterly interest expense on the Tranche A Loan for the nine months ended September 30, 2023 totaled $17,350. As allowable under the Pharmakon Loan Agreement, the Company has designated and accounted for 50% of the quarterly interest payments for the nine months ended September 30, 2023 as a Pharmakon PIK Interest Payment and the amount of $8,675 has been added to the outstanding principal balance of the borrowing. The remaining 50% of the quarterly interest payments of $8,675 have been paid at the end of each quarterly period. As of September 30, 2023, borrowings, including the Pharmakon PIK Interest Payments, totaled $308,675. The fair value of the debt approximates its carrying value based on prevailing interest rates as of the balance sheet date and is considered as Level 2 in the fair value hierarchy.

Incurred debt fees and issuance costs associated with the Tranche A Loan under the Pharmakon Loan Agreement totaled $11,147 and have been deferred and are being amortized as interest expense on an effective interest rate method over the remaining term of the Tranche A Loan. Deferred financing amortization of $230 and $467 was recognized for the three and nine months ended September 30, 2023, respectively.

Athyrium Credit Agreement

On December 7, 2020, the Company entered into a $200,000 Credit Agreement (the "Athyrium Credit Agreement") with Athyrium Opportunities III Co-Invest 1 LP ("Athyrium"), as lender and as administrative agent for the lenders. Certain of the Company's direct and indirect subsidiaries were guarantors to the Athyrium Credit Agreement. The Athyrium Credit Agreement provided for an initial term loan in the principal amount of $125,000 (the "Term A Loan"), which was received by the Company on December 7, 2020 and is recorded in "Secured term loans" on the Company's balance sheet. The Company used a portion of the proceeds from the Term A Loan to repay $43,298 of outstanding indebtedness, including accrued interest, under its prior credit facility with MidCap Financial Trust.

The Athyrium Credit Agreement also provided for two additional term loans, at the Company's option, in the respective principal amounts of $25,000 (the "Term B Loan") and $50,000 (the "Term C Loan" and, collectively with the Term A Loan and the Term B Loan, the "Athyrium Term Loans"). Having achieved all required revenue-based milestones, the Company exercised its option to draw upon the additional funding available under the Athyrium Credit Agreement, borrowing the principal amounts of $25,000 under the Term B Loan and $50,000 under the Term C Loan. Both the Term B Loan and the Term C Loan were funded on July 29, 2022 in the aggregate principal amount of $75,000. The Term B Loan and the Term C Loan were subject to all the provisions under the Athyrium Credit Agreement.

On November 19, 2021, the Company entered into an amendment to the Athyrium Credit Agreement to, among other things, (i) permit the Company to enter into the 2021 RPI Royalty Purchase Agreement, the OMERS Royalty Purchase Agreement, and the other definitive documentation related thereto and to perform its obligations thereunder; (ii) require the Company to pay to Athyrium, for the account of the lenders, a make-whole premium plus certain fees set forth in the Athyrium Credit Agreement in the event that the Company prepaid or repaid, or was required to prepay or repay, voluntarily or pursuant to mandatory prepayment obligations under the Athyrium Credit Agreement (e.g., with the proceeds of certain asset sales, certain ORLADEYO out-licensing or royalty monetization transactions (excluding the Royalty Sales), extraordinary receipts, debt issuances, or upon a change of control of the Company and specified other events, subject to certain exceptions), all of the then-outstanding Athyrium Term Loans, in each case, subject to certain exceptions set forth in the Athyrium Credit Agreement.

The Athyrium Credit Agreement provided for quarterly interest-only payments until the maturity date, with the unpaid principal amount of the outstanding Athyrium Term Loans due and payable on the maturity date. For each of the first eight full fiscal quarters following December 7, 2020, the Company had the option to make the applicable interest payment in-kind (an "Athyrium PIK Interest Payment") by capitalizing the entire amount of interest accrued during the applicable interest period with the unpaid original principal amount outstanding on the last day of such period. The Athyrium Term Loans accrued interest at a rate equal to the three-month LIBOR rate, which was no less than 1.75% and no more than 3.50% ("LIBOR"), plus 8.25%, or for each interest period in which an Athyrium PIK Interest Payment was made, LIBOR plus 10.25%. The quarter ended December 31, 2022 was the last period eligible for the Athyrium PIK Interest Payment designation.

The Athyrium Term Loans accrued interest at an effective interest rate of 12.68% during the period in which the debt was outstanding for the three months ended September 30, 2022.

Subject to certain exceptions, the Athyrium Credit Agreement would have required the Company to make mandatory prepayments of the Athyrium Term Loans with the proceeds of certain asset sales, certain ORLADEYO out-licensing or royalty monetization transactions (excluding the Royalty Sales), extraordinary receipts, debt issuances, or upon a change of control of the Company and specified other events. The Company could have made voluntary prepayments in whole or in part. Prepayments were subject to a premium equal to, (i) with respect to any voluntary prepayment and certain mandatory prepayments paid on or prior to the second anniversary of the applicable Athyrium Term Loan borrowing date, the amount, if any, by which (a) the sum of (1) 102.00% of the principal amount of the Athyrium Term Loan being prepaid plus (2) the present value of all interest that would have accrued on the principal amount of the Athyrium Term Loan being prepaid through and including the second anniversary of the date of the borrowing of such Athyrium Term Loan, plus 0.50%, exceeds (b) the principal amount of the Athyrium Term Loan being prepaid; (ii) with respect to any prepayment made between the second and third anniversaries of the applicable Athyrium Term Loan borrowing date, 2.00% of the principal amount of the Athyrium Term Loan being prepaid; (iii) with respect to any prepayment made between the third and fourth anniversaries of the applicable Athyrium Term Loan borrowing date, 1.00% of the principal amount of the Athyrium Term Loan being prepaid; and (iv) with respect to any prepayment made after the fourth anniversary of the applicable Athyrium Term Loan borrowing date, 0.00% of the principal amount of the Athyrium Term Loan being prepaid. Upon the prepayment or repayment, including at maturity, of all or any of the Athyrium Term Loans, the Company was obligated to pay an exit fee in an amount equal to 2.00% of the principal amount of the Athyrium Term Loans prepaid or repaid. In addition, each Athyrium Term Loan was subject to a 1.00% commitment fee at its respective borrowing date.

Quarterly interest payments under the Athyrium Credit Agreement for the nine months ended September 30, 2023 totaled $8,476. Quarterly interest payments under the Athyrium Credit Agreement for the nine months ended September 30, 2022 totaled $15,311 and were designated and accounted for as Athyrium PIK Interest Payments and added to the outstanding principal balance of the borrowing. From the Athyrium Term Loan inception through December 31, 2022, the quarterly interest payments were designated and accounted for as Athyrium PIK Interest Payments and added to the outstanding principal balance of the borrowing. The quarter ended December 31, 2022 was the last period eligible for the Athyrium PIK Interest Payment designation. Deferred financing amortization of $1,069 and $597, was recognized for the nine months ended September 30, 2023 and 2022, respectively.

On April 17, 2023, the outstanding principal of the Athyrium Term Loans, including the Athyrium PIK Interest Payments of $240,452 along with interest accrued of $1,355 for the first 17 days of the quarterly interest period ended June 30, 2023, was repaid with the funding received through the Pharmakon Loan Agreement.

Upon repayment of the Athyrium Term Loans, the Company incurred certain unaccrued prepayment and final payment fees to Athyrium of $17,261. Additionally, unamortized deferred financing costs of $11,758 associated with the Athyrium Term Loans were written-off at the time of repayment. Collectively, the prepayment and final payment fees and unamortized deferred financing costs totaled $29,019 and are reflected as a one-time loss on extinguishment of debt on the Condensed Consolidated Statements of Comprehensive Loss for the nine months ended September 30, 2023.

Note 8 — Lease Obligations

In June 2021, the Company entered into an operating sublease agreement for approximately 19,978 square feet of office space in Durham, North Carolina (the “Sublease Agreement”) with an expiration of December 2023. In March 2023, the Company signed a lease directly with the landlord commencing in January 2024 for the same space (the “Frontier Agreement”). The Company accounted for the Frontier Agreement as a new agreement and a separate transaction from the Sublease Agreement. In July 2023, the Company’s Sublease Agreement was terminated, and the Company amended the Frontier Agreement to move the commencement date from January 2024 to July 2023 to remain in continuous possession of the office space. The amendment was accounted for as a lease modification, and there was no impact as the lease commenced on the modification date. The Company recorded a right-of-use asset and lease liability of $2,063 in July 2023.

In February 2015, the Company entered into an operating lease agreement for approximately 31,601 square feet of office space in Birmingham, Alabama. In February 2020, the Company entered into an amendment to increase the existing space under the lease by approximately 2,846 rentable square feet (the “First Expansion Premises”). In December 2022, the Company entered into a second amendment to extend the term of the lease and to increase the existing space under the lease by 14,607 rentable square feet (the “Second Expansion Premises”). The second amendment allows for tenant improvement allowances not to exceed an aggregate of $515. The Company will capitalize all necessarily incurred leasehold improvement costs to bring the facilities to the condition for their intended use. In August 2023, the Second Expansion Premises commenced. The second amendment was accounted for as a lease modification, and the right-of-use asset and operating lease liability for the existing premises were remeasured at the modification date. The Company recorded a right-of-use asset of $5,327 and a lease liability of $5,925 in July 2023.

The Company leases certain assets, predominantly under operating leases, which consist of real estate leases, laboratory equipment leases and office equipment leases as of September 30, 2023. Renewal options for the Company's leases range from 1 to 3 years in length and begin from 2024 through 2030.

Aggregate lease expense was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Aggregate lease expense	$ 1,014	$ 634	$ 2,505	$ 1,854

Other supplemental information related to leases was as follows:

	As of September 30, 2023	As of December 31, 2022
Weighted average remaining lease term	8.0 years	8.1 years
Weighted average discount rate	10.0%	11.0%

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

	Balance Sheet Location	As of September 30, 2023	As of December 31, 2022
Assets:			
Lease assets, net	*Other Assets*	$ 11,380	$ 6,806
Liabilities:			
Current lease liabilities	*Lease financing obligation – current liabilities*	$ 2,282	$ 2,369
Non-current lease liabilities	*Lease financing obligation – long-term liabilities*	9,966	5,804
Total lease liabilities		$ 12,248	$ 8,173

Lease assets are recorded net of accumulated amortization of $6,350 and $4,349 as of September 30, 2023 and December 31, 2022, respectively.

Cash paid for amounts included in the measurement of lease liabilities was $834 and $2,293 for the three and nine months ended September 30, 2023, respectively. This compares to cash paid for amounts included in the measurement of lease liabilities of $615 and $1,796 for the three and nine months ended September 30, 2022, respectively.

Maturities of lease liabilities as of September 30, 2023, are as follows (in thousands):

2023 (remaining)	$ 853
2024	3,279
2025	2,332
2026	1,846
2027	1,531
Thereafter	9,292
Total lease payments	19,133
Less imputed interest	(6,885)
Total	$ 12,248

Note 9 — Stockholders' Equity

Sales of Common Stock

On March 1, 2021, the Company filed an automatic shelf registration statement on Form S-3 with the SEC. This shelf registration statement became effective automatically upon filing and allows the Company to sell an indeterminate number of securities, including common stock, preferred stock, depositary shares, purchase contracts, warrants, debt securities, and units, from time to time at prices and on terms to be determined at the time of sale.

On November 19, 2021, concurrent with the Company entering into the 2021 RPI Royalty Purchase Agreement, the Company and RPI entered into the Common Stock Purchase Agreement, pursuant to which the Company issued 3,846 shares of the Company's common stock to RPI for an aggregate purchase price of $50,000, at a price of $13.00 per share, calculated based on the 20-day volume weighted average price. The $13.00 per share price represented a premium of $1.11 over the closing price of $11.89 of the Company's common stock on November 19, 2021. The premium of $4,269 paid by RPI on the purchase of the Company's common stock has been deferred and is being amortized as a component of interest expense of the 2021 RPI royalty financing obligation.

Shares Reserved for Future Issuance of Common Stock

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

	As of September 30, 2023	As of December 31, 2022
Shares reserved for exercises of outstanding stock options	35,160	36,520
Shares reserved for vesting of restricted stock units	4,589	4,750
Shares reserved for exercises of warrants	15,023	15,023
Shares reserved for future issuance under the Stock Incentive Plan	11,934	4,206
Shares reserved for future issuance under the Inducement Equity Incentive Plan	200	947
Shares reserved for future issuance under the Employee Stock Purchase Plan	5,454	5,792
Total shares reserved for future issuance	**72,360**	**67,238**

Note 10 — Stock-Based Compensation

As of September 30, 2023, 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 24, 2023 and approved by the Company's stockholders on June 13, 2023. 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):

	Nine Months Ended September 30,	
	2023	2022
Incentive Plan	$ 30,934	$ 23,687
Inducement Plan	7,346	4,604
ESPP	847	1,130
Stock-based compensation expense	$ 39,127	$ 29,421

There was approximately $111,804 of total unrecognized compensation expense related to non-vested stock option and restricted stock unit awards granted by the Company as of September 30, 2023, which the Company expects to recognize over a weighted-average period of approximately 1.3 years. In addition, the Company has outstanding performance-based stock options and restricted stock unit awards for which no compensation expense is recognized until it is probable the performance conditions will be met.

Stock Incentive Plan

The Company grants stock option awards, restricted stock and restricted stock units to its employees, directors, and consultants under the Incentive Plan. Under the Incentive Plan, stock option awards are granted with an exercise price equal to the market price of the Company's common stock at the date of grant. Stock option awards and restricted stock units granted to employees generally vest 25% each year until fully vested after four years.

In December 2014, the Company issued 1,250 performance-based stock options. These awards vest upon successful completion of specific development milestones. As of September 30, 2023, 85% of these grants have vested.

In January 2022, the Company issued 221 performance-based restricted stock unit awards. 21 of the awards met the performance objectives in 2022 and became eligible for vesting at 50% on the first anniversary of the grant date and 25% on each of the second and third anniversaries of the grant date, until fully vested after three years. The remaining awards were cancelled.

Stock option awards and restricted stock unit awards granted to non-employee directors of the Company generally vest over one year. Stock option awards granted to new non-employee directors when they first join the Company's Board of Directors generally vest, subject to the terms of the Incentive Plan, in 36 equal monthly installments over a three-year period measured from the grant date. All stock option awards have contractual terms of 10 years. Restricted stock unit awards granted to new non-employee directors when they first join the Company's Board of Directors generally vest, subject to the terms of the Incentive Plan, in three equal annual installments beginning on the first anniversary of the grant date. The vesting and exercise provisions of all awards granted under the Incentive Plan are subject to acceleration in the event of certain stockholder-approved transactions, or upon the occurrence of a change in control as defined in the Incentive Plan.

Related activity under the Incentive Plan is as follows (in thousands):

	Awards Available	Options Outstanding	Weighted Average Exercise Price
Balance December 31, 2022	4,206	31,179	$ 8.56
Plan amendment	7,000	—	—
Restricted stock unit awards granted	(314)	—	—
Restricted stock unit awards cancelled	491	—	—
Stock option awards granted	(618)	618	9.01
Stock option awards exercised	—	(856)	5.25
Stock option awards cancelled	1,169	(1,169)	9.93
Balance September 30, 2023	11,934	29,772	$ 8.61

For stock option awards granted under the Incentive Plan during the first nine months of 2023 and 2022, the fair value was estimated on the date of grant using a Black-Scholes option pricing model and the assumptions noted in the table following the next subsection. The weighted average grant date fair value of these awards granted during the first nine months of 2023 and 2022 was $6.40 and $7.78, respectively. The fair value of the stock option awards is amortized to expense over the vesting periods using a straight-line expense attribution method. For restricted stock unit awards granted under the Incentive Plan, the fair value of the awards was 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 nine months of 2023 and 2022 was $9.18 and $13.18, 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 Company has the ability to grant stock option and restricted stock unit awards to newly-hired employees as inducements material to each employee entering employment with the Company. Awards granted to newly hired employees generally vest 25% each year until fully vested after four years and are subject to the terms and conditions of the Inducement Plan. Each stock option has a term of 10 years. The vesting and exercise provisions of all awards granted under

the Inducement Plan are subject to acceleration in the event of certain stockholder-approved transactions, or upon the occurrence of a change in control as defined in the Inducement Plan.

Related activity under the Inducement Plan is as follows (in thousands):

	Awards Available	Options Outstanding	Weighted Average Exercise Price
Balance December 31, 2022	947	5,341	$ 8.80
Restricted stock unit awards granted	(457)	—	—
Restricted stock unit awards cancelled	93	—	—
Stock option awards granted	(992)	992	8.64
Stock option awards exercised	—	(336)	3.67
Stock option awards cancelled	609	(609)	10.36
Balance September 30, 2023	200	5,388	$ 8.91

For stock option awards granted under the Inducement Plan during the first nine months of 2023 and 2022, the fair value was estimated on the date of grant using a Black-Scholes option pricing model and the assumptions noted in the table below. The weighted average grant date fair value of these awards granted during the first nine months of 2023 and 2022 was $6.14 and $9.72, respectively. The fair value of the stock option awards is amortized to expense over the vesting periods using a straight-line expense attribution method. For restricted stock unit awards granted under the Inducement Plan, the fair value of the awards was 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 nine months of 2023 and 2022 was $8.35 and $13.38, 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.

The following table summarizes the key assumptions used by the Company to value the stock option awards granted under all plans during the first nine months of 2023 and 2022. Historically, the expected life was based on the average of the assumption that all outstanding stock option awards would be exercised at full vesting and the assumption that all outstanding stock option awards would be exercised at the midpoint of the current date (if already vested) or at full vesting (if not yet vested) and the full contractual term. Effective July 1, 2023, the expected life is based on the historical settlement of options by taking into account exercises and post-vesting terminations and weighing them based on the number of options settled. This change in approach did not have a significant impact on the value of the stock option awards granted. The expected volatility represents the historical volatility on the Company's publicly-traded common stock. The Company has assumed no expected dividend yield, as dividends have never been paid to stock or option holders and will not be paid for the foreseeable future. The weighted average risk-free interest rate is the implied yield currently available on zero-coupon government issues with a remaining term equal to the expected term.

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

	Nine Months Ended September 30,	
	2023	2022
Expected Life in Years	5.5	5.5
Expected Volatility	83.9 %	84.1 %
Expected Dividend Yield	0.0 %	0.0 %
Risk-Free Interest Rate	3.9 %	2.9 %

Employee Stock Purchase Plan

The Company has reserved a total of 7,975 shares of common stock to be purchased under the ESPP, of which 5,454 shares remain available for purchase as of September 30, 2023. Eligible employees may authorize up to 15% of their salary to purchase common stock at the lower of 85% of the beginning or 85% of the ending price during six-month purchase intervals. No more than three thousand shares may be purchased by any one employee at the six-month purchase dates, and

no employee may purchase stock having a fair market value at the commencement date of $25 or more in any one calendar year. During the nine months ended September 30, 2023 and September 30, 2022, the Company issued 338 and 260 shares under the ESPP, respectively. Compensation expense for shares purchased under the ESPP related to the purchase discount and the "look-back" option were determined using a Black-Scholes option pricing model.

Note 11 — Collaborative and Other Relationships

ORLADEYO

Torii Pharmaceutical Co., Ltd.

On November 5, 2019, the Company entered into a Commercialization and License Agreement with Torii (the "Torii Agreement"), granting Torii the exclusive right to commercialize ORLADEYO for the prevention of HAE attacks in Japan.

Under the Torii Agreement, the Company received an upfront, non-refundable payment of $22,000. The Japanese National Health Insurance System's ("NHI") approval of the addition of ORLADEYO to the NHI drug price list in April 2021 triggered a $15,000 milestone payment from Torii to the Company, which was received in May 2021.

In addition, under the Torii Agreement, the Company is 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 are subject to customary reductions in certain circumstances, but may not be reduced by more than 50% of the amount that otherwise would have been payable to the Company in the applicable calendar quarter. Torii's royalty payment obligations commenced upon the first commercial sale of ORLADEYO in Japan 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 is responsible for supplying Torii with its required amounts of ORLADEYO. The activities of the parties pursuant to the Torii Agreement are overseen by a joint steering committee, composed of an equal number of representatives from each party to coordinate the development and commercialization of ORLADEYO in Japan. Torii launched ORLADEYO in Japan on April 23, 2021.

The Company identified performance obligations 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.

Peramivir Injection (RAPIVAB, RAPIACTA, PERAMIFLU)

Shionogi & Co., Ltd.

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. The Company developed peramivir under a license from the University of Alabama at Birmingham ("UAB") and will owe sublicense payments to UAB on any future milestone payments and/or royalties received by the Company from Shionogi. 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.

Green Cross Corporation

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.

Government Collaborations

The Company has previously entered into contracts with the U.S. Government, including the procurement contract with HHS for up to 50,000 doses of RAPIVAB over a five-year period to supply the Strategic National Stockpile for use in a public health emergency and contracts with NIAID/HHS and BARDA/HHS for the development of galidesivir. As of September 30, 2023, the Company has delivered a total of 49,980 RAPIVAB doses of the 50,000 RAPIVAB doses available under the procurement contract, effectively completing the contract with HHS, and all of the Company's government funding for galidesivir has expired.

Note 12 — Subsequent Events

On October 23, 2023, certain entities affiliated with Baker Bros. Advisors LP (the "Baker Entities") net exercised the remaining balance of the pre-funded warrants held by such Baker Entities that were issued on November 21, 2019. Additionally, certain of the Baker Entities net exercised all of the pre-funded warrants that were issued on June 1, 2020. The exercises resulted in the issuance of 14,997 common shares. Following the exercises, there are no outstanding warrants.

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

Under the Clearside Agreement, Clearside will receive a $5,000 upfront license fee from the Company. 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.

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. 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 complement-mediated 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 protein therapeutics to target difficult-to-treat rare diseases. In addition to these discovery and development efforts, our business strategy includes the efficient commercialization of these drugs in the United States and certain other regions upon regulatory approval. By focusing 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, once-daily therapy discovered and developed by us for the prevention of hereditary angioedema ("HAE") attacks. ORLADEYO is approved in the United States and multiple global markets for the prevention of HAE attacks in adults and pediatric patients 12 years and older.

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 more than two and a half years of commercialization experience with ORLADEYO, we anticipate the global commercial market for ORLADEYO has the potential to reach a global peak of $1 billion in annual net ORLADEYO revenues. We expect at least 70 to 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 nine months ended September 30, 2023 is discussed under "Results of Operations" in this MD&A. Revenue from sales of ORLADEYO in future periods is subject to uncertainties and will depend on several factors, including the success of our and our partners' commercialization efforts in the United States and elsewhere, the number of new patients switching to ORLADEYO, patient retention and demand, the number of physicians prescribing ORLADEYO, the rate of monthly prescriptions, reimbursement from third-party and government payors, the number of patients receiving free product, the conversion of patients from our clinical trials and early access programs to commercial customers, our pricing strategy, and market trends. We are continuing to monitor and analyze this data as we continue to commercialize ORLADEYO.

Complement Program. The goal of our overall complement program is to advance several first-in-class and/or best-in-class compounds across multiple pathways in the complement system to treat many complement-mediated diseases. These compounds include BCX10013, a potential once-daily oral medicine, which targets the alternative pathway of complement. In addition, we are pursuing oral medicines directed at other targets across the classical, lectin, and terminal pathways of the complement system. For more information on these additional therapies, see "Recent Developments" below in this MD&A.

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. Peramivir injection is also approved in Canada (RAPIVAB), Australia (RAPIVAB), Japan (RAPIACTA), Taiwan (RAPIACTA), and Korea (PERAMIFLU).

Recently Announced Program Updates. On November 3, 2023, we held an R&D Day to describe our drug discovery process and introduce additional therapies from our pipeline. See “Recent Developments” below in this MD&A for information about these additional therapies.

Revenues and Expenses

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

Our operating expenses are also difficult to predict and depend on several factors, including research and development expenses, drug manufacturing, clinical research activities, the ongoing requirements of our development programs, the costs of commercialization, the availability of capital and direction from regulatory agencies, which are difficult to predict, and the factors discussed in the “Risk Factors” section in Part II, Item 1A of this report. Management may be able to control the timing and level of research and development and selling, general and administrative expenses, but many of these expenditures will occur irrespective of our actions due to contractually committed activities and/or payments.

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

Critical Accounting Policies and Estimates

The accompanying discussion and analysis of our financial condition and results of operations are based upon our condensed consolidated financial statements and the related disclosures, which have been prepared in accordance with accounting principles generally accepted in the United States (“U.S. GAAP”). The preparation of these condensed consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues, expenses and related disclosure of contingent assets and liabilities. We evaluate our estimates, judgments and the policies underlying these estimates on a periodic basis, as situations change, and regularly discuss financial events, policies, and issues with members of our audit committee and our independent registered public accounting firm. In particular, we routinely evaluate our estimates and policies regarding revenue recognition, administration, inventory and manufacturing, taxes, stock-based compensation, research and development, consulting and other expenses and any associated liabilities. Our estimates are based on historical experience and on various other assumptions that are believed to be reasonable under the circumstances. The results of our estimates form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. See “Critical Accounting Estimates” at the end of this MD&A for a description of accounting policies that we believe are the most critical to aid you in fully understanding and evaluating our reported financial results and that affect the more significant judgments and estimates that we use in the preparation of our financial statements.

Recent Developments

ORLADEYO® (berotralstat)

On July 19, 2023, we announced that we have entered into a collaboration with Er-Kim Pharmaceuticals to commercialize ORLADEYO in Turkey.

On November 2, 2023, we announced that Austria has approved the reimbursement of ORLADEYO for the targeted prophylaxis of HAE in patients 12 years of age or older.

On November 3, 2023, we announced that we expect to submit a U.S. supplemental new drug application for the pediatric use of ORLADEYO in 2025. The ongoing APeX-P clinical trial is assessing an oral granule formulation of ORLADEYO in pediatric HAE patients who are 2 to <12 years of age.

Complement-Mediated Diseases

BCX10013

On August 3, 2023, we announced that we have begun opening clinical trial sites for a dose-ranging trial in patients with paroxysmal nocturnal hemoglobinuria (PNH) and expect to begin patient enrollment (in countries without other approved therapies) by the end of the year. The trial is designed to identify a safe, effective, once-daily dose that we can advance into a pivotal program in renal complement-mediated diseases.

On October 26, 2023, we announced the enrollment of the first patient in a proof-of-concept clinical trial evaluating BCX10013. The goal of this proof-of-concept trial is to understand the preliminary efficacy and safety profile of once-daily dosing with BCX10013. If the results in patients living with PNH confirm its potential for a best-in-class profile, we plan to advance into a pivotal program in patients living with renal complement-mediated diseases. On November 3, 2023, we announced that we expect to report data from our ongoing proof-of-concept trial in 2024.

On November 3, 2023, we presented data at our R&D Day from the recently completed 160 mg cohort of our multiple ascending dose healthy volunteer trial, which highlights the strength and duration of alternative pathway suppression achieved at this dose level, supporting once-daily clinical dosing.

Oral C5 Inhibitor

On November 3, 2023, we announced that 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 with generalized myasthenia gravis (gMG) to switch from infused therapy and address their disease earlier in the treatment paradigm. gMG is a chronic autoimmune, neuromuscular disease that causes muscle weakness that worsens after periods of activity. We also announced that we expect to complete lead optimization in 2024 and begin clinical trials in 2025.

Bifunctional Complement Inhibitor

On November 3, 2023, we announced that we are developing a bifunctional complement inhibitor anti-C2 monoclonal antibody that also inhibits the alternative pathway. This investigational candidate could be a first-in-class combined inhibitor of the classical, lectin and alternative pathways of the complement system to treat complex renal complement-mediated diseases like IgAN and lupus nephritis, which are influenced by multiple complement pathways. We also announced that we expect to select a lead molecule in 2024 and begin clinical trials in 2025.

Oral C2 Inhibitor

On November 3, 2023, we announced that we are developing a classical and lectin pathway complement inhibitor to treat autoimmune hemolytic anemias, including cold agglutinin disease (CAD) and warm autoimmune hemolytic anemia (wAIHA). The limited approved options for treating diseases like CAD and wAIHA are injectable or infused. 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. Inhibiting C2 could decrease red cell destruction (hemolysis) in autoimmune hemolytic anemias by blocking the classical and lectin pathways. We expect to select a lead molecule in 2025.

Netherton Syndrome

On November 3, 2023, we announced that we expect to begin clinical trials of BCX17725 in 2024. BCX17725 is a potent and selective investigational fusion protein KLK5 inhibitor designed to provide best-in-class, potentially disease-modifying treatment for people with Netherton syndrome. Netherton syndrome is a rare, lifelong genetic disorder that often presents in neonates or infancy. The disease is caused by the deficiency of a natural inhibitor (SPINK5) of KLK5, a serine protease responsible for regulating skin shedding. Patients may have red, scaly and inflamed skin and susceptibility to recurrent immune reactions. Netherton syndrome can be life threatening, especially during infancy when patients are vulnerable to dehydration and recurrent infections. Currently, there is no approved treatment for Netherton syndrome.

Avoralstat

On November 3, 2023, we announced that we entered into a license agreement (the “Clearside Agreement”) with Clearside Biomedical, Inc. (“Clearside”), enabling us to develop our investigational plasma kallikrein inhibitor, avoralstat, with Clearside’s SCS Microinjector® to deliver avoralstat directly to the back of the eye through the suprachoroidal space to treat patients with diabetic macular edema (DME). DME is the most common cause of vision loss in individuals with

diabetes, and at least one-third of patients have persistent DME despite anti-vascular endothelial growth factor therapies, which are administered via monthly injection. Avoralstat, which was previously studied in an oral formulation in a phase 3 trial in patients with HAE, has high potency and low solubility, two characteristics we believe are important to achieving potential efficacy with reduced dosing frequency in the eye for DME patients.

Under the Clearside Agreement, Clearside will receive a $5.0 million upfront license fee from us. Clearside is eligible to receive up to an additional $30.0 million in clinical and regulatory milestone payments, and up to a total of $47.5 million in three post-approval sales-based milestone payments as annual global net sales progress to $2.0 billion. We will pay Clearside tiered mid-single digit royalties on annual global net product sales, at three tiers, including a top tier of >$1.5 billion.

Results of Operations (three months ended September 30, 2023 compared to the three months ended September 30, 2022)

For the three months ended September 30, 2023, total revenues were $86.7 million as compared to $75.8 million for the three months ended September 30, 2022. The increase was primarily due to ORLADEYO net revenue, including royalties, of $85.7 million, an increase of $19.7 million. The increase in ORLADEYO net revenue was partially offset by a decrease in other net revenues of $8.8 million, primarily due to a RAPIVAB stockpiling sale to the U.S. Department of Health and Human Services ("HHS") in the three months ended September 30, 2022.

Cost of product sales for the three months ended September 30, 2023 and 2022 was $1.1 million and $3.5 million, respectively. The decrease in cost of product sales was primarily associated with the peramivir product sales to our partners and the RAPIVAB stockpiling sale to HHS for the three months ended September 30, 2022, partially offset by an increase in ORLADEYO sales in the current year period.

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

	Three Months Ended September 30,	
	2023	**2022**
R&D expenses by program:		
Factor D program	$ 18,245	$ 27,031
Berotralstat	10,758	11,638
FOP	265	9,114
Peramivir	260	130
Galidesivir	241	253
Other research, preclinical and development costs	17,110	4,574
Total R&D expenses	**$ 46,879**	**$ 52,740**

R&D expenses decreased to $46.9 million for the three months ended September 30, 2023 from $52.7 million for the three months ended September 30, 2022, primarily due to reduced R&D investment following the discontinuation of the BCX9930 and BCX9250 programs announced in December and November 2022, respectively. In the table above, the BCX9930 program R&D expenses are within the Factor D program classification and the BCX9250 program R&D expenses are within the FOP classification. These reductions were partially offset by increased spending on other research, preclinical and development costs due to reallocating our R&D investment from the discontinuation of the BCX9930 and BCX9250 programs into our newly identified targets.

Selling, general and administrative ("SG&A") expenses for the three months ended September 30, 2023 were $50.6 million compared to $36.9 million for the three months ended September 30, 2022. The increase was primarily due to increased investment to expand and enhance the U.S. commercial team and expanded international operations.

Interest expense for the three months ended September 30, 2023 was $27.3 million compared to $24.8 million for the three months ended September 30, 2022. The increase in interest expense was primarily associated with the interest accrued on the larger Tranche A Loan of $300.0 million under the Pharmakon Loan Agreement (as defined below), which we entered into on April 17, 2023. The Pharmakon Loan Agreement provides for an initial term loan, the Tranche A Loan,

in the principal amount of $300.0 million, which was funded on April 17, 2023 and was primarily used to repay the outstanding Athyrium Credit Agreement (as defined below) principal balance, outstanding interest accrued on the Athyrium term loans and associated prepayment and final payment fees totaling $263.0 million.

Interest expense for the three months ended September 30, 2023 included $17.3 million of non-cash interest expense due to the amortization of interest associated with the royalty financing obligations and $9.9 million of interest expense, including the amortization of the deferred financing associated with the borrowings under the Pharmakon Loan Agreement. Interest expense for the three months ended September 30, 2022 included $18.5 million of non-cash interest expense due to the amortization of interest associated with the royalty financing obligations and $6.3 million of interest expense, net of deferred financing amortization, associated with the borrowings under the Athyrium Credit Agreement.

For the three months ended September 30, 2023, other income of $3.4 million was comprised primarily of interest income of $4.2 million and net foreign currency losses of $0.7 million. Other income of $1.2 million for the three months ended September 30, 2022 was comprised of interest income of $1.8 million and net foreign currency losses of $0.5 million.

Results of Operations (nine months ended September 30, 2023 compared to the nine months ended September 30, 2022)

For the nine months ended September 30, 2023, total revenues were $238.0 million as compared to $191.3 million for the nine months ended September 30, 2022. The increase was primarily due to ORLADEYO net revenue, including royalties, of $235.1 million, an increase of $54.2 million. The increase in ORLADEYO revenue was partially offset by a decrease in other net revenues of $7.5 million, primarily due to a RAPIVAB stockpiling sale to HHS in the nine months ended September 30, 2022.

Cost of product sales for the nine months ended September 30, 2023 and 2022 was $2.9 million and $4.0 million, respectively. The decrease in cost of product sales was primarily associated with the peramivir product sales to our partners and the RAPIVAB stockpiling sale to HHS during the nine months ended September 30, 2022, partially offset by an increase in ORLADEYO sales in the current year period.

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

	Nine Months Ended September 30,	
	2023	**2022**
R&D expenses by program:		
Factor D program	$ 70,064	$ 118,400
Berotralstat	29,931	26,810
FOP	1,068	17,710
Galidesivir	739	719
Peramivir	506	1,266
Other research, preclinical and development costs	44,206	15,185
Total R&D expenses	**$ 146,514**	**$ 180,090**

R&D expenses decreased to $146.5 million for the nine months ended September 30, 2023 from $180.1 million for the nine months ended September 30, 2022, primarily due to reduced R&D investment following the discontinuation of the BCX9930 and BCX9250 programs announced in December and November 2022, respectively. In the table above, the BCX9930 program R&D expenses are within the Factor D program classification and the BCX9250 program R&D expenses are within the FOP classification. These reductions were partially offset by increased spending on berotralstat development programs and other research, preclinical and development costs due to reallocating our R&D investment from the discontinuation of the BCX9930 and BCX9250 programs into our newly identified targets.

SG&A expenses for the nine months ended September 30, 2023 were $149.5 million compared to $109.2 million for the nine months ended September 30, 2022. The increase was primarily due to increased investment to expand and enhance the U.S. commercial team and expanded international operations.

Interest expense for the nine months ended September 30, 2023 was $83.7 million compared to $72.6 million for the nine months ended September 30, 2022. The increase in interest expense was primarily associated with the interest accrued on the larger Tranche A Loan of $300.0 million under the Pharmakon Loan Agreement. Additionally, the increase in interest expense was due to the additional aggregate borrowing of $75.0 million of the Term B Loan and Term C Loan under the Athyrium Credit Agreement, which were funded on July 29, 2022.

Interest expense for the nine months ended September 30, 2023 included $56.1 million of non-cash interest expense due to the amortization of interest associated with the royalty financing obligations, $17.8 million of interest expense, including the amortization of the deferred financing associated with the borrowings under the Pharmakon Loan Agreement and $9.5 million of interest expense, net of deferred financing amortization, associated with the borrowings under the Athyrium Credit Agreement. Interest expense for the nine months ended September 30, 2022 included $57.8 million of non-cash interest expense due to the amortization of interest associated with the royalty financing obligations and $14.7 million of interest expense, net of deferred financing amortization, associated with the borrowings under the Athyrium Credit Agreement.

For the nine months ended September 30, 2023, other expense of $18.4 million was comprised primarily of a loss on extinguishment of debt of $29.0 million on the repayment of the term loans under the Athyrium Credit Agreement, partially offset by interest income of $11.3 million and net foreign currency losses of $0.7 million. Other income of $1.8 million for the nine months ended September 30, 2022 was comprised of interest income of $2.4 million, partially offset by net foreign currency losses of $0.6 million.

Liquidity and Capital Resources

Our operations have principally been funded through public offerings and private placements of equity securities; our credit facilities; revenues from ORLADEYO; royalty monetization transactions; and cash from collaborative and other research and development agreements, including U.S. Government contracts. In addition to the above, we have previously received funding from other sources, including other collaborative and other research and development agreements, government grants, equipment lease financing, facility leases, 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 intend to use the remaining net proceeds of approximately $25.8 million for other general corporate purposes. The Pharmakon Loan Agreement also provides for three additional term loan tranches in principal amounts of $50.0 million each, which we may request, at our option, on or prior to September 30, 2024. The maturity date of the Pharmakon Loan Agreement is April 17, 2028. See “Note 7—Debt—Pharmakon Loan Agreement” in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information about our obligations under the Pharmakon Loan Agreement.

In 2020 and 2021, we entered into the Royalty Purchase Agreements (as defined in “Note 6—Royalty Monetizations—ORLADEYO and Factor D Inhibitors” 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. We are required to make payments to OMERS commencing with the calendar quarter beginning October 1, 2023. See “Note 6—Royalty Monetizations—ORLADEYO and Factor D Inhibitors” in the Notes to Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information about these financing transactions.

As of September 30, 2023, we had net working capital of $411.7 million, an increase of approximately $0.7 million from $411.0 million at December 31, 2022. The increase in working capital was primarily the result of net proceeds of $25.8 million received following the funding of the Tranche A Loan of $300.0 million under the Pharmakon Loan Agreement, substantially offset by normal operating expenses associated with the development of our product candidates

and commercialization of ORLADEYO. Our principal sources of liquidity at September 30, 2023 were approximately $150.9 million in cash and cash equivalents and approximately $246.7 million in investments considered available-for-sale.

We intend to contain costs and cash flow requirements by closely managing our third-party costs and headcount, leasing scientific equipment and facilities, contracting with other parties to conduct certain research and development projects, and using consultants. We expect to 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. 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.

We plan to finance our needs principally from the following:

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

As our commercialization activities and research and development programs continue to advance, our costs will increase. 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 will increase our expenses. Our expenses, revenues and cash utilization rate could vary significantly depending on many factors, including our ability to raise additional capital, 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 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. The impact of the ongoing COVID-19 pandemic on one or more of the foregoing factors could negatively affect our revenues, expenses, and cash utilization rate.

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. However, we have sustained operating losses for the majority of our corporate history and expect that our 2023 expenses will exceed our 2023 revenues. We expect to continue to incur operating losses and negative cash flows until revenues reach a level sufficient to support ongoing operations. Our liquidity needs will largely be determined by the success of operations in regard to the successful commercialization of our products and the future progression of our product candidates. We regularly 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) raising additional capital through equity or debt financings or from other sources, including 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 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:

- market acceptance of approved products and successful commercialization of such products by either us or our partners;
- our ability 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, governmental agencies, distributors 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 new drug application ("NDA") filings;
- competitive and technological advances;
- the time and costs involved in obtaining regulatory approvals;
- post-approval commitments for ORLADEYO, RAPIVAB, 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 as 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.

The restrictive 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 obligations outstanding under the Pharmakon Loan Agreement. These covenants limit our ability 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. A breach of any of these covenants could result in an event of default under the Pharmakon Loan Agreement. As of September 30, 2023, we were in compliance with the covenants under the Pharmakon Loan Agreement.

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 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, sales of peramivir to our licensing partners and sales of RAPIVAB to HHS under our procurement contract. In the United States, we ship ORLADEYO directly to patients through a single specialty pharmacy, which is considered our customer. In the European Union, United Kingdom and elsewhere, we sell ORLADEYO to specialty distributors as well as hospitals and pharmacies, which collectively are considered our customers.

We recognize revenue for sales when our customers obtain control of the product, which generally occurs upon delivery. For ORLADEYO, we classify payments to our specialty pharmacy customer for certain services provided by our customer as selling, general and administrative expenses to the extent such services provided are determined to be distinct from the sale of our product.

Net revenue from sales of ORLADEYO is recorded at net selling price (transaction price), which includes estimates of variable consideration for which reserves are established for (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 are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable or as a current liability. Overall, these reserves reflect our best estimates of the amount of consideration to which we are entitled based on the terms of the applicable contract. The amount of variable consideration included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from estimates, we adjust these estimates, 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. These reserves are recorded in the same period in which the revenue is 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, (iii) a range of possible outcomes that are probability-weighted for the estimated payor mix, and (iv) product distribution information obtained from our specialty pharmacy.

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 products under contracts negotiated between them and our specialty pharmacy. The specialty pharmacy, in turn, charges back to us the difference between the price 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 from us. We estimate chargebacks and adjust gross product revenues and accounts receivable based on the estimates 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 are able to 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. 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, as well as research and development agreements with certain government entities. 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.

Milestone payments are recognized as licensing revenue upon the achievement of specified milestones if (i) the milestone is substantive in nature and the achievement of the milestone was not probable at the inception of the agreement and (ii) we have a right to payment. Any milestone payments received prior to satisfying these revenue recognition criteria are recorded as deferred revenue.

Reimbursements received for direct out-of-pocket expenses related to research and development costs are recorded as revenue in the Condensed Consolidated Statements of Comprehensive Loss rather than as a reduction in expenses. Under our contracts with BARDA/HHS and NIAID/HHS, revenue is recognized as reimbursable direct and indirect costs are incurred.

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 inventories primarily relate to ORLADEYO. Additionally, our inventory includes RAPIVAB and peramivir.

We value our inventories at the lower of cost or estimated net realizable value. We determine the cost of our inventories, which includes amounts related to materials, labor, manufacturing overhead and shipping and handling costs 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.

Our inventories are subject to expiration dating. We regularly evaluate the carrying value of our inventories and provide valuation reserves for any estimated obsolete, short-dated or unmarketable inventories. 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. During the nine months ended September 30, 2023, we evaluated our inventory levels and associated expiration dating relative to the latest sales forecasts for ORLADEYO, RAPIVAB, and peramivir and estimated those inventories at risk of obsolescence. Accordingly, we recorded an increase to the inventory valuation reserve of $0.2 million for a total reserve of $1.3 million as of September 30, 2023.

We expense costs related to the production of inventories as research and development expenses in the period incurred until such time it is believed that future economic benefit is expected to be recognized, which generally is upon receipt of regulatory approval. Upon regulatory approval, we capitalize subsequent costs related to the production of inventories.

Accrued Expenses

We enter 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. We record liabilities under these contractual commitments when we determine 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 our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on the facts and circumstances, which can include assumptions such as expected patient enrollment, site activation and estimated project duration. We periodically confirm the accuracy of our estimates with the service providers and make 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 our raw materials, drug substance, drug products, and product candidates; and (iv) professional fees.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and CROs that conduct and manage clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort 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. As of September 30, 2023 and December 31, 2022, the carrying value of accrued expenses approximates their fair value due to their short-term settlement.

Research and Development Expenses

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. 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. Research and development expenses include, among other items, personnel costs, including salaries and benefits, manufacturing costs, clinical, regulatory, and toxicology services performed by 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. Costs for studies performed by CROs are accrued by us over the service periods specified in the contracts, and estimates are adjusted, if required, based upon our ongoing review of the level of services actually performed.

Additionally, we have license agreements with third parties which require fees related to sublicense agreements or maintenance fees. We expense sublicense payments as incurred unless they are related to revenues that have been deferred, in which case the expenses are deferred and recognized over the related revenue recognition period. We expense maintenance payments as incurred.

Deferred collaboration expenses represent sublicense payments paid to our academic partners upon receipt of consideration from various commercial partners, and other consideration paid to our academic partners for modification to existing license agreements. These deferred expenses would not have been incurred without receipt of such payments or modifications from our commercial partners and are being expensed in proportion to the related revenue being recognized. We believe that this accounting treatment appropriately matches expenses with the associated revenue.

We group our R&D expenses into two major categories: direct external expenses and indirect expenses. Direct expenses consist of compensation for R&D 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 work on non-active product candidates and our discovery research efforts.

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 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 life of an award, 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 vesting periods. The estimation of share-based payment awards that will ultimately vest requires judgment, and to the extent actual results or updated estimates differ from our current estimates, such amounts will be recorded as a cumulative adjustment in the period estimates are revised. In addition, we have outstanding performance-based stock options and restricted stock units for which no compensation expense is recognized until “performance” is deemed to have occurred. Significant management judgment is also required in determining estimates of future stock price volatility and forfeitures to be used in the valuation of the options. 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 and BCX10013. 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.

We account for uncertain tax positions in accordance with U.S. GAAP. 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 taxable income in each of the jurisdictions in which we operate and the period over which our deferred tax assets will be recoverable.

Beginning in fiscal year 2021, we 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 we had no presence.

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- or 15-year period, depending on the location of the activities performed. 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 five for activities conducted in the United States or year 15 in the case of development conducted on foreign soil.

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 is 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. As of September 30, 2023, interest was accrued at an effective rate of 13.24% on the $300.0 million borrowing under the Pharmakon Loan Agreement.

We invest in marketable securities in accordance with our investment policy. The primary objectives of our investment policy are to preserve capital, maintain proper liquidity to meet operating needs and maximize yields. 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 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. We also had other transactions denominated in foreign currencies during the nine months ended September 30, 2023, primarily related to operations in Europe, contract manufacturing and ex-U.S. clinical trial activities, and we expect to continue to do so. Our royalties from Torii are derived from Torii's sales of ORLADEYO in Japan. Those sales are denominated in Japanese yen and converted into U.S. dollars for purposes of determining the royalty owed to us. Our limited foreign currency exposure relative to our European operations is to fluctuations in the Euro, British Pound, Swiss Franc, Danish Krone, and Swedish Krona. Additionally, we have initiated operations in Canada and have foreign currency exposure 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 nine months ended September 30, 2023; however, we may do so in the future.

Inflation Risk

Inflation generally impacts us by potentially increasing our operating expenses, including 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 have established disclosure controls and procedures designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms and is accumulated and communicated to management, including the principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

Our management, with participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act as of September 30, 2023. Based upon this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of September 30, 2023, our disclosure controls and procedures were not effective due to the material weaknesses in internal control over financial reporting described below.

Specifically, management has identified the following material weaknesses:

1. We have experienced staffing issues and do not have sufficient resources in our accounting function, which restricts our ability to perform requisite reviews and approval of manual journal entries posted to the general ledger.

2. We have experienced staffing issues and do not have sufficient resources in our accounting function, which restricts our ability to consistently execute review procedures over general ledger account reconciliations and other control activities over financial statement close processes.

Planned Remediation:

We have already commenced the following actions designed to improve our internal control over financial reporting to remediate these material weaknesses:

- hiring, developing and retaining incremental personnel with appropriate accounting and internal controls expertise;
- engaging a third-party specialist to assist management with improving the Company's internal controls;
- reviewing and updating (as appropriate) the organizational design of the controllership function;
- reviewing and updating (as appropriate) our methodologies, policies and procedures designed to ensure adequate internal control over financial reporting, including underlying information technology and business process controls; and
- reviewing and updating (as appropriate) training programs on relevant internal control over financial reporting matters.

Management will continue to implement measures to remediate these material weaknesses, such that these controls are designed, implemented, and operating effectively.

We are currently working to improve our internal processes and implement enhanced controls, as discussed above, to address the material weaknesses in our internal control over financial reporting. These material weaknesses will not be considered remediated until the applicable remediated controls are operating for a sufficient period and management has concluded, through testing, that these controls are operating effectively. This is a high priority for the Company, and management is working promptly to address these issues.

Despite the existence of these material weaknesses, we believe the condensed consolidated financial statements included in the period covered by this Quarterly Report on Form 10-Q 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.

PART II. OTHER INFORMATION

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.

Risks Relating to Our Business

Risks Relating to COVID-19

Our business, operations, clinical development or commercialization plans and timelines, and access to capital could be adversely affected by the effects of the ongoing COVID-19 pandemic on us or on third parties with whom we conduct business, including without limitation our development partners, manufacturers, CROs, and others, as well as on the regulatory and government agencies with whom we work.

The global COVID-19 pandemic continues to affect the United States and global economies, and 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. For example, government orders and evolving business policies and procedures have impacted and may continue to impact, among other things: (1) our personnel and those of third parties on whom we rely, including our development partners, manufacturers, CROs, and others; (2) the conduct of our current and future clinical trials and commercial interactions; and (3) the operations of the U.S. Food and Drug Administration ("FDA"), European Medicines Agency ("EMA"), Japanese Pharmaceuticals and Medical Devices Agency ("PMDA"), and other health and governmental authorities, which could result in delays of reviews and approvals, including as we continue to expand internationally and bring ORLADEYO to additional global markets.

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 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 as a result of the COVID-19 pandemic or other health epidemics. 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. Although we carefully manage our relationships with our suppliers and vendors, there can be no assurance that we will not encounter challenges or delays in the future or that these challenges or delays will not have an adverse impact on our business, financial condition and prospects.

In addition, our clinical trials have been and may continue to be affected by the COVID-19 pandemic. For example, the acceleration of COVID-19 slowed the startup of the inadequate C5 responder cohorts in our complement oral Factor D program and, as a result, delayed the reporting of related data in 2020. Clinical site initiation and patient enrollment may be delayed due to prioritization of hospital resources toward the COVID-19 pandemic or concerns among patients about participating in clinical trials during a pandemic. Some patients may have difficulty following certain aspects of clinical trial protocols if quarantines impede patient movement or interrupt healthcare services. Similarly, our inability to successfully recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19 or experience additional restrictions by their institutions, city, or state could adversely impact our clinical trial operations.

If global health concerns prevent the FDA, EMA, PMDA or other regulatory authorities from conducting their inspections, reviews, or other regulatory activities, it could significantly impact the ability of such authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business and clinical development and commercialization plans and timelines.

Where possible and practical, we continue to provide work-from-home flexibility for our employees, which could negatively impact productivity, disrupt our business and delay our clinical programs and timelines. We cannot accurately predict the impact on operations of any return-to-the-office plan on our business or on third parties with whom we conduct business. Our business may be negatively impacted in the event that large numbers of employees or key employees do not comply with any applicable protocols. These and similar, and perhaps more severe, disruptions to our operations could negatively impact our business, operating results and financial condition.

The spread of COVID-19, which has caused a broad impact globally, could also materially affect our access to capital. While the future economic impact brought by, and the duration of, COVID-19 may be difficult to assess or predict, the pandemic could result in further significant disruption of global financial markets, reducing our ability to access the equity or debt capital markets or obtain other sources of capital, 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.

The global pandemic continues to evolve, with the ultimate impact of the COVID-19 pandemic or a similar health epidemic being uncertain and subject to change. These effects could be material, and we will continue to monitor the COVID-19 situation closely. We do not yet know the full extent and magnitude of the impacts that COVID-19 has had or will have on our business, the healthcare system, the pharmaceutical industry, or the global economy. In addition, the COVID-19 pandemic could have the effect of heightening many of the other risks described below.

Financial and Liquidity Risks

We have incurred losses since our inception, expect to continue to incur such losses, and may never be profitable.

Since our inception, we have not achieved sustained profitability. We expect to incur additional losses for the foreseeable future, and our losses could increase as our research and development efforts and commercial activities progress. We expect that such losses will fluctuate from quarter to quarter and that losses and fluctuations may be substantial. To become profitable, 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. It could take longer than expected before we receive, or we may never receive, significant revenue from any current or future license agreements or significant revenues directly from product sales. 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 and BCX10013 under the Royalty Purchase Agreements, may reduce the profitability of such products.

Because of the numerous risks and uncertainties associated with developing our product candidates, launching new products, and their potential for commercialization, we are unable to predict the extent of any future losses. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. If we are unable to achieve and sustain profitability, 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 as and when needed, we may need to adjust our operations.

We have sustained operating losses for the majority of our corporate history and expect that our 2023 expenses will exceed our 2023 revenues. We expect to continue to incur operating losses and negative cash flows unless and until revenues reach a level sufficient to support ongoing operations.

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 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. 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 as 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, and the progression of our product candidates in the future. Our plans for managing

our liquidity needs primarily include controlling the timing and spending on our research and development programs, raising additional funds as discussed herein, 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, when needed. If we are unable to obtain sufficient additional capital as and when needed, we may be forced to adjust or curtail our operations; delay, reduce, or stop ongoing clinical trials or commercialization efforts; cease operations altogether; or file for bankruptcy.

Risks Relating to Drug Development and Commercialization

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

The success of our business depends upon our ability to manage our product candidate pipeline, including through expanding the pipeline, as appropriate, through our internal identification and discovery of product candidates or otherwise in-licensing or acquiring products or product candidates and integrating them into our business effectively and efficiently; advancing our product candidates through the various stages of development; and receiving regulatory approvals for the commercial sale of our product candidates. Identifying, selecting, and in-licensing or acquiring products or product candidates requires substantial expense and technical and financial expertise, and if we are unable to effectively manage our pipeline and integrate viable products or product candidates into our business on acceptable terms, or at all, our business and drug development efforts would 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. We 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 pre-determined 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 our complement program (inclusive of BCX10013) and our other rare disease product candidates (including the additional therapies in our pipeline described in “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Overview—Recent Developments” in Part I, Item 2 of this report), could result in delays in or modifications to our trials or require the performance of additional unplanned trials. For example, dose-related observations in an ongoing BCX10013 nonclinical study reported earlier this year delayed the clinical program. 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, such as we experienced with BCX9930 in 2022 prior to discontinuing its development later that year.

In addition, the development plans for our product candidates, including our clinical trials (inclusive of BCX10013), may not be adequately designed or executed, which could negatively affect the outcome and analysis of study results. Because of the cost and duration of clinical trials, we have decided in the past, and may in the future decide, to discontinue

development of product candidates for various reasons, including, but not limited to, that they are unlikely to show favorable results in clinical trials, unlikely to help advance a product to the point of a meaningful collaboration, or unlikely to have reasonable commercial potential.

Undesirable or inconclusive data in our preclinical studies and clinical trials or side effects in humans could result in the FDA or foreign regulatory authorities (including, e.g., the EMA, the Ministry of Health, Labor and Welfare ("MHLW") in Japan or the United Kingdom's Medicines and Healthcare 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 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 the ongoing COVID-19 pandemic 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, called enzyme targets;
- 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 enzyme targets or inhibitors from academic institutions or from other biotechnology companies on acceptable terms, or at all, our drug development efforts would 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 as 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 are likely to increase. Our current and planned discovery, development, approval, and commercialization efforts will 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 market acceptance of our products, including ORLADEYO; our ability to raise additional capital as and when needed; 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 as and when needed, or at all, may be limited and may greatly depend upon our 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) for our complement program (including BCX10013) for diseases of the complement system and other rare disease product candidates (including the additional therapies in our pipeline described in "Management's Discussion and Analysis of Financial Condition and Results of Operations—Overview—Recent Developments" 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 COVID-19, rising inflation, increased interest rates, or disruption or instability in the banking industry, may restrict our future flexibility to raise capital as 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 as 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 COVID-19, rising inflation, increased interest rates, disruption or instability in the banking industry, a potential U.S. Government shutdown, or the conflicts in Ukraine and Israel. Any such instability may impact these parties' ability to fulfill contractual obligations to us, or it might limit or place burdensome conditions upon future transactions with us. Also, it is possible that suppliers may be negatively impacted. Any such unfavorable outcomes in our current programs or unfavorable economic conditions have in the past and could again place severe downward pressure on the price of our common stock and may decrease opportunities to raise capital in the capital or credit markets, and further could reduce the return available on invested corporate cash, which, if severe and sustained, could have a material and adverse impact on our results of operations and cash flows and limit our ability to continue development and commercialization of our products and product candidates.

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

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

The process of preparing for and obtaining regulatory approval in any jurisdiction may be lengthy and expensive, and approval is never certain. Because of the risks and uncertainties inherent to the development process, including risks and uncertainties related to the impact of COVID-19, 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.

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 on rare diseases, which may create additional risks and challenges.

Because we focus 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. 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.

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

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

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

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

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

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

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

- we or our partners may seek to renegotiate or terminate our relationships due to unsatisfactory commercial, regulatory or clinical results, including post-approval clinical commitments, a change in business strategy, a change of control or other reasons;
- our contracts for collaborative arrangements may expire;

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

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

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

We have an agreement with Torii for the development and commercialization of ORLADEYO in Japan. Our ability to realize the expected benefits of this collaboration, including with respect to the receipt or amounts of royalty payments, is subject to a number of risks, including that the commercial potential of ORLADEYO may not meet our current expectations, we or Torii may fail to comply with our respective obligations under the Torii Agreement, and third parties may fail to perform their obligations to us on a timely basis or at all.

The Torii Agreement provides that we are entitled to receive tiered royalty payments, the amounts of which will depend upon the amount of annual net sales of ORLADEYO in Japan during each calendar year and other factors. We currently remain responsible for regulatory activities with respect to ORLADEYO in Japan, and we continue to use third parties to satisfy many of our obligations under the Torii Agreement, including, but not limited to, our regulatory and other responsibilities in Japan. If our interactions, or those of our third-party agents, are unsuccessful, we could fail to meet our obligations under the Torii Agreement, which could negatively impact the commercial success and the partnership, impact the economic benefit expected, or require additional development of ORLADEYO.

Torii has sole control over, and decision-making authority with respect to, commercialization activities for ORLADEYO for the prevention of HAE attacks in Japan, subject to oversight from a joint steering committee. Therefore, our receipt, and the amounts, of any royalty payments under the Torii Agreement are dependent upon Torii's successful performance of such commercialization activities. In addition, competitive products and variations in patient demand, prescription levels, reimbursement determinations or other factors may limit the commercial potential of ORLADEYO in Japan, which could materially reduce the amount of any royalties we are entitled to receive under the Torii Agreement.

Under the Torii Agreement, we are responsible for supplying Torii with its required amounts of ORLADEYO for commercial sale. If, due to the failure of our third-party contract manufacturers to produce sufficient drug product, we fail to supply to Torii the required amounts of ORLADEYO, then Torii's ability to successfully commercialize ORLADEYO in Japan could be materially impaired, and we may receive less royalty income under the Torii Agreement, or none at all.

Any of the foregoing risks could materially adversely impact our ability to perform our obligations under the Torii Agreement, which could reduce the economic benefits of the Torii Agreement to us and impair or result in the termination of our collaboration with Torii.

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 the COVID-19 pandemic on us or our partners.

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

Even if we are able to successfully commercialize our existing products, or to develop new commercially viable products, certain obligations we have to third parties, including, without limitation, our obligations to pay royalties on certain revenues from ORLADEYO and BCX10013 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 inventories, 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 nonrenewal of a critical 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, particularly associated with ORLADEYO, BCX10013, peramivir and our early-stage compounds.

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 inventories 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.

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 and acceptance for 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. We subsequently received regulatory approvals for ORLADEYO in multiple markets. In addition, we are performing research on or developing products for the treatment of several other rare diseases, including 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, such as BCX10013, 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 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 physician 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. If we fail to comply with 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 the 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.

Advertising and promotion are subject to stringent FDA rules and oversight, and as an NDA holder, we may be held responsible for any advertising and promotion that is not in compliance with the 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 which 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 penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, 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.

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, develop our product candidates, obtain collaborators and raise capital.

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 critical 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 the CMS. The IRA includes several provisions that will impact our business to varying degrees, including provisions that reduce the out-of-pocket spending cap for Medicare Part D beneficiaries to $2,000 starting in 2025; impose new manufacturer financial liability on all drugs in Medicare Part D; allow the U.S. Government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for drug prices that increase faster than inflation; and delay the rebate rule that would require pass through of pharmacy benefit manager rebates to beneficiaries. Further, under the IRA, orphan drugs are exempted

from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication is for that disease or condition. If a product receives multiple rare disease designations or has multiple approved indications, it may not qualify for the orphan drug exemption. We cannot be sure whether additional legislation or rulemaking 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 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 effect of the IRA on our business and the healthcare industry in general is not yet known.

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 are subject to data security and privacy 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 are subject to legal obligations related to privacy and data protection. Compliance with U.S. and international 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, which gives California residents expanded rights to access and require deletion of their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. 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.

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. 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. 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, 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.

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 therapeutics product candidates are targeting.

Product Liability Risks

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

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

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and face even greater risks upon commercialization by us of our products or product candidates. We have product liability insurance covering our clinical trials. Clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance or increase our existing coverage at a reasonable cost to protect us against losses that could have a material adverse effect on our business. An individual may bring a product liability claim against us if one of our products or product candidates causes, or is claimed to have caused, an

injury or is found to be unsuitable for consumer use. Any product liability claim brought against us, with or without merit, could result in:

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

Risks Relating to Contractual Arrangements

We face risks related to our government-funded programs and are subject to various U.S. Government contract requirements, which may create a disadvantage and additional risks to us.

We have contracts with BARDA/HHS and 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 these government agencies, we are 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 government funding for galidesivir expired in 2022, we still face risks related to our U.S. Government 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 rely on U.S. Government contracts. These risks include the ability of the U.S. Government to unilaterally:

- terminate or reduce the scope of our contract with or without cause;
- interpret relevant regulations (federal acquisition regulation clauses);
- require performance under circumstances which may not be favorable to us;
- require an in-process review where the U.S. Government will review the project and its options under the contract;
- control the timing and amount of funding, which impacts the development progress of our programs; and
- audit and object to our contract-related costs and fees, including allocated indirect costs.

Upon termination or expiration of a contract, the U.S. Government may dispute wind-down and termination costs and may question prior expenses under the contract and deny payment of those expenses. Should we choose to challenge the U.S. Government for denying certain payments under a contract, such a challenge could subject us to substantial additional expenses which we may or may not recover.

In addition, as a U.S. Government contractor, we are required to comply with applicable laws, regulations and standards relating to our accounting practices and are subject to periodic audits and reviews, including a final financial audit. As part of any such audit or review, the U.S. Government may review the adequacy of, and our compliance with, our internal control systems and policies, including those relating to our purchasing, property, estimating, compensation and management information systems. Audits under the BARDA/HHS and NIAID/HHS galidesivir contracts may occur at the election of the U.S. Government and have been concluded through fiscal 2019; all subsequent fiscal years are still open and auditable. Based on the results of its audits, the U.S. Government may adjust our contract-related costs and fees, including allocated indirect costs. This adjustment could impact the amount of revenues reported on a historic basis. In addition, in the event BARDA/HHS or NIAID/HHS determines that certain costs and fees were unallowable or determines that the allocated indirect cost rate was higher than the actual indirect cost rate, BARDA/HHS or NIAID/HHS would be entitled to recoup any overpayment from us as a result. In addition, if an audit or review uncovers any improper or illegal activity, we may be subject to civil and criminal penalties and administrative sanctions, including forfeiture of profits, suspension of payments, fines and suspension or prohibition from doing business with the U.S. Government. We could also suffer serious harm to our reputation if allegations of impropriety were made against us. In addition, under U.S. Government purchasing regulations, some of our costs may not be reimbursable or allowed under our contracts. Further, 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.

There are risks related to the potential government use or sale of our antivirals.

Government use or sale, in emergency situations or otherwise, of our antivirals (including peramivir for the treatment of influenza) may result in risks to us or our collaborative partners. There can be no assurance that government use of our antivirals (whether as indicated or outside of their current indications) will prove to be generally safe, well-tolerated and effective. Any government sale or use (on an emergency basis or otherwise) of our antivirals in any country may create liabilities for us or our partners.

There can be no assurance that we or our manufacturers will be able to fully meet the demand for our antivirals with respect to any future arrangements. Further, we may not receive a favorable purchase price for future orders, if any, of our antivirals by governmental entities. Our competitors may develop products that could compete with or replace any antivirals selected for government sale or use. We may face competition in markets where we have no existing intellectual property protection or are unable to successfully enforce our intellectual property rights.

There can be no assurance that the non-U.S. partnerships that we have entered into for peramivir will result in any order for peramivir in those countries or that peramivir will be approved for any use or will achieve market approval in additional countries. In the event that any emergency use or market approval is granted in any country, there can be no assurance that any government order or commercialization of the applicable product or product candidate in such countries will be substantial or will be profitable to us.

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 (the “Shionogi Agreement”), 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. Payments, if any, from Shionogi to us on non-governmental sales under the Shionogi Agreement will generally not be available to us for other purposes unless and until Royalty Sub has repaid in full its obligations under the PhaRMA Notes. Accordingly, these funds have been and will continue to be required to be dedicated to Royalty Sub’s debt service and not available to us for product development or other purposes. 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.

We wrote off the balance due under the PhaRMA Notes to other income as a debt extinguishment as of December 31, 2021. See "Note 8—Royalty Monetizations—RAPIACTA—Non-Recourse Notes Payable−Debt Extinguishment" in the Notes to Consolidated Financial Statements in Part II, Item 8 of our most recent Annual Report on Form 10-K for additional information about the write-off.

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 and closed on an initial term loan thereunder in the principal amount of $300.0 million. Under the new 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 repay, or are required to prepay or repay, 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 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.

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, 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 (e.g., the conflicts in Ukraine and Israel), terrorism, political unrest, results of certain elections and votes, actual or threatened public health emergencies and outbreak of disease (e.g., the ongoing 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, the Canadian Dollar, and Japanese yen. 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.

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, 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 the GDPR or related national data protection laws, which may deviate from the GDPR, may result in significant fines of up to 4% of global revenues, or €20.0 million, whichever is greater, and in addition to such fines, our failure to comply with the requirements of GDPR or similar national legislation may 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 audit.

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 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.

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

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 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. Duplicate copies of most critical data are secured off-site. 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.

A significant disruption in our or our third-party vendors' information technology systems or a cybersecurity breach 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. We currently store most of our preclinical research data, our clinical data and our manufacturing data at our facilities. While we do store duplicate copies of most of our clinical data offsite 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. In addition, we have outsourced significant parts of our information technology and business infrastructure to third-party providers, and we currently use these providers to perform business critical information technology and business services for us. We are therefore vulnerable to cybersecurity attacks and incidents on the associated networks and systems, whether they are managed by us directly or by the third parties with whom we contract, and we have experienced, and may in the future experience, such cybersecurity threats and attacks.

Like other companies in our industry, our networks and infrastructure, as well as those of our third-party providers, may be vulnerable to cyber-attacks or intrusions, including by computer hackers, foreign governments, foreign companies, or competitors, or may be breached by employee error, malfeasance or other disruption. Cyber-attacks could also include the use of artificial intelligence ("AI") and machine learning to launch more automated, targeted and coordinated attacks on targets. We are vulnerable to the risks of 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 critical 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.

Any compromise of our data security could also result in a violation of applicable privacy and other laws, significant legal 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. 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. Similarly, the increasing use of AI and machine learning technology in the biopharmaceutical industry presents new risks and challenges. 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.

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 50% 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 September 30, 2023, the 52-week range of the market price of our stock was from $6.62 to $14.50 per share. The following factors, in addition to other risk factors described in this section, may have, and in some cases have had, a significant impact on the market price of our common stock:

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

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

We have identified material weaknesses in our internal control over financial reporting. These material weaknesses could divert management’s attention and adversely affect our ability 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 may negatively impact the trading price of our common stock.

Our management has identified material weaknesses in our internal control over financial reporting, as described in "Controls and Procedures" in Part I, Item 4 of this report. As further described in that section, we are implementing measures, and will continue to implement measures, to remediate the material weaknesses identified by management and to improve our internal control over financial reporting such that these controls are designed, implemented, and operating effectively. These material weaknesses will not be considered remediated until the applicable remediated controls are operating for a sufficient period of time and management has concluded, through testing, that these controls are operating effectively. Remediating the material weaknesses could take longer than expected and divert management's attention away from other areas of the business.

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. Although we believe the condensed 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. Failure to remedy any material weakness in our internal control over financial reporting could also restrict our future access to the capital markets.

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 October 31, 2023, there were 204,809,380 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 October 31, 2023, there were 33,450,125 stock options and restricted stock units outstanding and 11,994,917 shares available for issuance under our Amended and Restated Stock Incentive Plan, 6,326,168 stock options and restricted stock units outstanding and 102,469 shares available for issuance under our Amended and Restated Inducement Equity Incentive Plan, and 5,454,406 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.

In March 2017, we entered into a Registration Rights Agreement with entities affiliated with Baker Bros. Advisors LP (the "Baker Entities") to provide that, if requested, we will register the shares of our common stock beneficially owned by the Baker Entities for resale under the Securities Act of 1933, as amended (the "Securities Act"). Our registration obligations pursuant to the Registration Rights Agreement cover all shares then held or thereafter acquired by the Baker Entities, for up to ten years, and include our obligation to facilitate certain underwritten public offerings of our common stock by the Baker Entities in the future. On May 10, 2017, we filed a registration statement on Form S-3 with respect to 11,710,951 shares of common stock held by the Baker Entities. Subsequently, on November 21, 2019, certain of the Baker Entities acquired pre-funded warrants to purchase 11,764,706 shares of our common stock at a price of $1.69 per warrant. In addition, on June 1, 2020, we issued to certain of the Baker Entities pre-funded warrants to purchase 3,511,111 shares of our common stock at a price of $4.49 per warrant. All such warrants have since been exercised at an exercise price of $0.01 per share. If the Baker Entities, by exercising their registration rights or otherwise, sell a large number of our shares, or the market perceives that the Baker Entities intend to sell a large number of our shares, this could adversely affect the market price of our common stock.

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 Bylaws 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 Bylaws 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 Bylaws 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, our Certificate of Incorporation or Amended and Restated Bylaws, 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 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 ongoing COVID-19 pandemic), trade wars, armed conflict, political unrest, government shutdowns, 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—General Risk Factors—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 armed conflicts in Ukraine and Israel, 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.

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 rising inflation, increased interest rates, disruption or instability in the banking industry, the effects of the ongoing COVID-19 pandemic, foreign exchange rate fluctuations, a potential U.S. Government shutdown, and the conflicts in Ukraine and Israel. 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 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, COVID-19 pandemic, and the conflicts in Ukraine and Israel 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 “Risk Factors” section.

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.

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

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

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

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

Item 5. Other Information

During the three months ended September 30, 2023, no director or officer of the Company adopted or terminated a “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits

Number	Description
3.1	Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed December 22, 2006.
3.2	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed July 24, 2007.
3.3	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed May 7, 2014.
3.4	Certificate of Elimination of the Series B Junior Participating Preferred Stock. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed May 13, 2020.
3.5	Certificate of Amendment to the Third Restated Certificate of Incorporation. 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 Registrant effective October 29, 2008. Incorporated by reference to Exhibit 3.2 to the Company's Form 8-K filed November 4, 2008.
3.7	Amendment to Amended and Restated By-Laws of BioCryst Pharmaceuticals, Inc., dated January 21, 2018. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed January 22, 2018.
10.1*	BioCryst Pharmaceuticals, Inc. Employee Stock Purchase Plan (as amended and restated as of July 7, 2023). Incorporated by reference to Exhibit 10.3 to the Company's Form 10-Q filed August 7, 2023.
(10.2)*	BioCryst Pharmaceuticals, Inc. Inducement Equity Incentive Plan (as amended and restated as of October 26, 2023).
(31.1)	Certification of the Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
(31.2)	Certification of the Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
(32.1)	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
(32.2)	Certification 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 nine months ended September 30, 2023, formatted in Inline XBRL: (i) Condensed Consolidated Balance Sheets, (ii) Condensed Consolidated Statements of Comprehensive Loss, (iii) Condensed Consolidated Statements of Cash Flows, and (iv) Notes to Condensed Consolidated Financial Statements, tagged as blocks of text and including detailed tags.
(104)	Cover Page Interactive Data File – The cover page from this Quarterly Report on Form 10-Q for the quarter ended September 30, 2023 is formatted in Inline XBRL (contained in Exhibit 101).
( )	Filed or furnished herewith.
*	Management contract.

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 8th day of November, 2023.

BIOCRYST PHARMACEUTICALS, INC.

/s/ Jon P. Stonehouse

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

/s/ Anthony Doyle

Anthony Doyle
Chief Financial Officer
(Principal Financial Officer and Interim Principal Accounting Officer)

BIOCRYST PHARMACEUTICALS, INC.
INDUCEMENT EQUITY INCENTIVE PLAN
(AS AMENDED AND RESTATED AS OF OCTOBER 26, 2023)

ARTICLE ONE
GENERAL PROVISIONS

I. PURPOSES OF THE PLAN

A. This Inducement Equity Incentive Plan (as amended and restated, the "Plan") is intended to promote the interests of BioCryst Pharmaceuticals, Inc., a Delaware corporation (the "Company"), by providing a method whereby certain equity awards may be granted to prospective employees of the Company and its subsidiaries. The Plan is not subject to the approval of the Company's stockholders and may only be used for equity incentive grants that qualify as "inducement grants" under Listing Rule 5635(c)(4) of the corporate governance rules of the Nasdaq Stock Market (the "Nasdaq Inducement Exception").

B. The Plan allows for the grant of awards with respect to a total of 9,525,500 shares of the Company's common stock, par value $0.01 per share (the "Common Stock").

C. The Plan was approved and adopted by the Company's Board of Directors (the "Board") effective on October 26, 2023 in order to increase by 1,700,000 the number of shares of Common Stock available for issuance under the Plan.

II. ADMINISTRATION OF THE PLAN

A. The Plan shall be administered by the Committee who shall be the Compensation Committee of the Board or, in the absence of a Compensation Committee, a properly constituted committee in compliance with the Nasdaq Inducement Exception (the administrator is referred to herein as the "Committee" or the "Plan Administrator"). Any power of the Committee may also be exercised by the Board, except to the extent that the grant or exercise of such authority would cause any award or transaction to become subject to (or lose an exemption under) the short-swing profit recovery provisions of Section 16 of the Securities Exchange Act of 1934, as amended (the "1934 Act"). To the extent that any permitted action taken by the Board conflicts with action taken by the Committee, the Board action shall control. The Committee may delegate any or all aspects of the day-to-day administration of the Plan to one or more officers or employees of the Company or any subsidiary or affiliate, and/or to one or more agents.

B. Subject to the express provisions of this Plan, the Committee shall be authorized and empowered to do all things that it determines to be necessary or appropriate in connection with the administration of this Plan, including, without limitation: (1) to prescribe, amend and rescind rules and regulations relating to this Plan and to define terms not otherwise defined herein; (2) to determine which persons are grantees, to which of such grantees, if any, awards shall be granted hereunder and the timing of any such awards; (3) to grant awards to grantees and determine the terms and conditions thereof, including the number of shares of Common Stock subject to awards and the exercise or purchase price of such shares and the circumstances under which awards become exercisable or vested or are forfeited or expire, which terms may but need not be conditioned upon the passage of time, continued employment, the satisfaction of performance criteria, the occurrence of certain events (including events which constitute a Change in Control (as such term is defined below)), or other factors; (4) to establish and verify the extent of satisfaction of any performance goals or other conditions applicable to the grant, issuance, exercisability, vesting and/or ability to retain any award; (5) to prescribe and

amend the terms of the agreements or other documents evidencing awards made under this Plan (which need not be identical) and the terms of or form of any document or notice required to be delivered to the Company by grantees under this Plan; (6) to determine the extent to which adjustments are required pursuant to Article One; (7) to interpret and construe this Plan, any rules and regulations under this Plan and the terms and conditions of any award granted hereunder, and to make exceptions to any such provisions for the benefit of the Company; (8) to approve corrections in the documentation or administration of any award; and (9) to make all other determinations deemed necessary or advisable for the administration of this Plan.

C. All decisions, determinations and interpretations by the Committee regarding the Plan, any rules and regulations under the Plan and the terms and conditions of or operation of any award granted hereunder, shall be final and binding on all grantees, beneficiaries, heirs, assigns or other persons holding or claiming rights under the Plan or any award. The Committee shall consider such factors as it deems relevant, in its sole and absolute discretion, to making such decisions, determinations and interpretations including, without limitation, the recommendations or advice of any officer or other employee of the Company and such attorneys, consultants and accountants as it may select.

III. ELIGIBILITY

A. The persons eligible to participate in the Plan are prospective employees of the Company and its subsidiaries. For the avoidance of doubt, grants promised to such individuals prior to their commencement of employment may be granted following such commencement to the extent permitted under the Nasdaq Inducement Exception.

B. The Plan Administrator shall, within the scope of its administrative jurisdiction under the Plan, have full power and authority to determine the eligible persons to receive grants under the Plan and, subject to the Plan, the terms of those grants.

IV. STOCK SUBJECT TO THE PLAN

A. Shares of the Company's Common Stock shall be available for issuance under the Plan and shall be drawn from either the Company's authorized but unissued shares of Common Stock or from reacquired shares of Common Stock, including shares repurchased by the Company on the open market.

B. Should an outstanding option under this Plan expire or terminate for any reason prior to exercise in full, the shares subject to the portion of the option not so exercised shall be available for subsequent option grants, direct stock issuances, or restricted stock units ("RSUs") under the Plan. Unvested shares issued under the Plan and subsequently repurchased by the Company, at the original issue price paid per share, pursuant to the Company's repurchase rights under the Plan, or shares underlying terminated RSUs, shall be added back to the number of shares of Common Stock reserved for issuance under the Plan and shall accordingly be available for reissuance through one or more subsequent option grants, direct stock issuances, or RSUs under the Plan. However, shares subject to an award under the Plan may not again be made available for issuance under the Plan if such shares are: (1) shares used to pay the exercise price of an option, (2) shares delivered to or withheld by the Company to pay the withholding taxes related to an award, or (3) shares repurchased on the open market with the proceeds of an option exercise.

C. In the event any change is made to the Common Stock issuable under the Plan by reason of any stock split, stock dividend, recapitalization, combination of shares, exchange of shares or other change affecting the outstanding Common Stock as a class without receipt of consideration, then appropriate adjustments shall be made to (1) the maximum number

and/or class of securities issuable under the Plan, (2) the number and/or class of securities and price per share in effect under each outstanding option under the Plan, and (3) the number and/or class of securities in effect under each outstanding direct stock issuance and RSU under the Plan. The purpose of such adjustments shall be to preclude the enlargement or dilution of rights and benefits under the Plan.

D. The fair market value per share of Common Stock on any relevant date under the Plan shall be determined in accordance with the following provisions:

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

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

3. If the Common Stock is at the time neither listed nor admitted to trading on any securities exchange nor traded in the over-the-counter market, then the fair market value shall be determined by the Plan Administrator after taking into account such factors as the Plan Administrator shall deem appropriate.

V. MINIMUM VESTING PERIOD

Notwithstanding any other provision of this Plan to the contrary, in no event shall any award granted pursuant to this Plan vest prior to the twelve (12)-month anniversary of the date of grant, other than in connection with the grantee's death or permanent disability or, to the extent permitted hereunder, in connection with a Change in Control (provided that this limitation shall not apply with respect to up to five percent (5%) of the shares of Common Stock available for issuance under this Plan). The minimum vesting period set forth in this Section V may not be waived or superseded by any provision in an award or other agreement.

ARTICLE TWO
DISCRETIONARY OPTION GRANT PROGRAM

I. TERMS AND CONDITIONS OF OPTIONS

Options granted pursuant to this Article Two shall be authorized by action of the Plan Administrator and shall be non-statutory options. Each option granted shall be evidenced by one or more instruments in the form approved by the Plan Administrator. Each such instrument shall, however, comply with the terms and conditions specified below.

A. **Option Price.**

1. The option price per share shall be fixed by the Plan Administrator. In no event, however, shall the option price per share be less than one hundred percent (100%) of the fair market value per share of Common Stock on the date of the option grant.

2. The option price shall become immediately due upon exercise of the option and shall, subject to the provisions of Section II of this Article Two and the instrument evidencing the grant, be payable through one of the following methods (or a combination thereof):

(i) full payment in cash or check drawn to the Company's order;

(ii) full payment in shares of Common Stock held by the optionee for the requisite period necessary to avoid a charge to the Company's earnings for financial reporting purposes and valued at fair market value on the Exercise Date (as such term is defined below);

(iii) full payment through a combination of shares of Common Stock held by the optionee for the requisite period necessary to avoid a charge to the Company's earnings for financial reporting purposes and valued at fair market value on the Exercise Date and cash or cash equivalent;

(iv) full payment through a "net settlement" procedure pursuant to which the Company shall withhold shares of Common Stock issuable in connection with the exercise of the option with a fair market value equal to the exercise price and, if elected by the optionee, all applicable Federal and State income and employment taxes required to be withheld by the Company in connection with such exercise;

(v) full payment through a broker-dealer sale and remittance procedure pursuant to which the optionee (I) shall provide irrevocable written instructions to a designated brokerage firm to effect the immediate sale of the purchased shares and remit to the Company, out of the sale proceeds available on the settlement date, sufficient funds to cover the aggregate option price payable for the purchased shares plus all applicable Federal and State income and employment taxes required to be withheld by the Company in connection with such purchase and (II) shall provide written directives to the Company to deliver the certificates for the purchased shares directly to such brokerage firm in order to complete the sale transaction; or

(vi) such other method as permitted by the Plan Administrator.

For purposes of this subparagraph 2, the Exercise Date shall be the date on which written notice of the option exercise is delivered to the Company. Except to the extent the sale and remittance procedure is utilized in connection with the exercise of the option, payment of the option price for the purchased shares must accompany such notice.

B. **Term and Exercise of Options.**

Each option granted under this Article Two shall be exercisable at such time or times, during such period, and for such number of shares as shall be determined by the Plan Administrator and set forth in the instrument evidencing the option grant. No such option, however, shall have a maximum term in excess of ten (10) years from the grant date. During the lifetime of the optionee, the option shall be exercisable only by the optionee and shall not be assignable or transferable by the optionee except for a transfer of the option by will or by the laws of descent and distribution following the optionee's death. However, the Plan Administrator shall have the discretion to provide that an option may, in connection with the optionee's estate plan, be assigned in whole or in part during the optionee's lifetime either (i) as a gift to one or more members of optionee's immediate family, to a trust in which optionee and/or one or more such family members hold more than fifty percent (50%) of the beneficial interest or an entity in which more than fifty percent (50%) of the voting interests are owned by optionee and/or one or more such family members, or (ii) pursuant to a domestic relations order. The assigned portion shall be exercisable only by the person or persons who acquire a proprietary interest in the option pursuant to such assignment. The terms applicable to the assigned portion shall be the same as those in effect for this option immediately prior to such assignment and shall be set forth in such documents issued to the assignee as the Plan Administrator may deem appropriate.

C. **Termination of Service.**

1. Except to the extent otherwise provided pursuant to an applicable award agreement, the following provisions shall govern the exercise period applicable to any options held by the optionee at the time of cessation of Service (as such term is defined below) or death.

(i) Should the optionee cease to remain in Service for any reason other than death or permanent disability, then the period for which each outstanding option held by such optionee is to remain exercisable shall be limited to the three (3)-month period following the date of such cessation of Service. However, should optionee die during the three (3)-month period following his or her cessation of Service, the personal representative of the optionee's estate or the person or persons to whom the option is transferred pursuant to the optionee's will or in accordance with the laws of descent and distribution shall have a twelve (12)-month period following the date of the optionee's death during which to exercise such option.

(ii) In the event such Service terminates by reason of permanent disability (as defined in Section 22(e)(3) of the Internal Revenue Code), then the period for which each outstanding option held by the optionee is to remain exercisable shall be limited to the twelve (12)-month period following the date of such cessation of Service.

(iii) Should the optionee, after completing five (5) full years of Service, die while in Service, then the exercisability of each of his or her outstanding options shall automatically accelerate so that each such option shall become fully exercisable with respect to the total number of shares of Common Stock at the time subject to such option and may be exercised for all or any portion of such shares. The personal representative of the optionee's estate or the person or persons to whom the option is transferred pursuant to the optionee's will or in accordance with the laws of descent and distribution shall have a twelve (12)-month period following the date of the optionee's death during which to exercise such option.

(iv) In the event such Service terminates by reason of death prior to the optionee obtaining five (5) full years of Service, then the period for which each outstanding vested option held by the optionee at the time of death shall be exercisable by the optionee's estate or the person or persons to whom the option is transferred pursuant to the optionee's will or in accordance with the laws of descent and distribution shall be limited to the twelve (12)-month period following the date of the optionee's death.

(v) Under no circumstances, however, shall any such option be exercisable after the specified expiration date of the option term.

(vi) Each such option shall, during such limited exercise period, be exercisable for any or all of the shares for which the option is exercisable on the date of the optionee's cessation of Service. Upon the expiration of such limited exercise period or (if earlier) upon the expiration of the option term, the option shall terminate and cease to be exercisable. However, each outstanding option shall immediately terminate and cease to remain outstanding, at the time of the optionee's cessation of Service, with respect to any shares for which the option is not otherwise at that time exercisable or in which the optionee is not otherwise vested.

(vii) Should (i) the optionee's Service be terminated for misconduct (including, but not limited to, any act of dishonesty, willful misconduct, fraud or embezzlement) or (ii) the optionee make any unauthorized use or disclosure of confidential information or trade secrets of the Company or its parent or subsidiary corporations, then in any such event all outstanding options held by the optionee under this Article Two shall terminate immediately and cease to be exercisable.

2. The Plan Administrator shall have complete discretion, exercisable either at the time the option is granted or at any time while the option remains outstanding, to permit one or more options held by the optionee under this Article Two to be exercised, during the limited period of exercisability provided under subparagraph 1 above, not only with respect to the number of shares for which each such option is exercisable at the time of the optionee's cessation of Service but also with respect to one or more subsequent installments of purchasable shares for which the option would otherwise have become exercisable had such cessation of Service not occurred.

3. For purposes of the foregoing provisions of this Section I.C (and for all other purposes under the Plan):

(i) The optionee shall be deemed to remain in the Service of the Company for so long as such individual renders services on a periodic basis to the Company (or any parent or subsidiary corporation) in the capacity of an Employee (as such term is defined below), a non-employee member of the board of directors or an independent consultant or advisor, unless the agreement evidencing the applicable option grant specifically states otherwise.

(ii) The optionee shall be considered to be an Employee for so long as such individual remains in the employ of the Company or one or more of its parent or subsidiary corporations, subject to the control and direction of the employer entity not only as to the work to be performed but also as to the manner and method of performance.

D. **Stockholder Rights.**

An optionee shall have no stockholder rights with respect to any shares covered by the option until such individual shall have exercised the option and paid the option price for the purchased shares. Without limitation, an optionee shall not have any right to receive dividends with respect to unexercised options.

E. **No Repricing.**

No option may be repriced, regranted through cancellation, including cancellation in exchange for cash or other awards, or otherwise amended to reduce its option price (other than with respect to adjustments made in connection with a transaction or other change in the Company's capitalization as permitted under this Plan) without the approval of the stockholders of the Company.

II. CORPORATE TRANSACTIONS/CHANGES IN CONTROL

A. For purposes of this Section II (and for all other purposes under the Plan), a Corporate Transaction shall be deemed to occur in the event of any of the following stockholder-approved transactions:

1. a merger or consolidation in which the Company is not the surviving entity, except for a transaction the principal purpose of which is to change the State of the Company's incorporation,

2. the sale, transfer or other disposition of all or substantially all of the assets of the Company in liquidation or dissolution of the Company, or

3. any reverse merger in which the Company is the surviving entity but in which securities possessing more than fifty percent (50%) of the total combined voting power of the Company's outstanding securities are transferred to a person or persons different from the persons holding those securities immediately prior to such merger.

B. Immediately after the consummation of a Corporate Transaction, all outstanding options under this Article Two shall fully vest, terminate and cease to be outstanding, except to the extent continued or assumed (as applicable) by the Company or the successor corporation or its parent company. The Plan Administrator shall have complete discretion to provide, on such terms and conditions as it sees fit, for a cash payment to be made to any optionee on account of any option terminated in accordance with this paragraph, in an amount equal to the excess (if any) of (1) the fair market value of the shares subject to the option as of the date of the Corporate Transaction, over (2) the aggregate exercise price of the option.

C. Each outstanding option under this Article Two which is assumed in connection with a Corporate Transaction or is otherwise to continue in effect shall be appropriately adjusted, immediately after such Corporate Transaction, to apply and pertain to the number and class of securities which would have been issued to the option holder, in consummation of such Corporate Transaction, had such person exercised the option immediately prior to such Corporate Transaction. Appropriate adjustments shall also be made to the option price payable per share, provided the aggregate option price payable for such securities shall remain the same. In addition, the class and number of securities available for issuance under the Plan following the consummation of the Corporate Transaction shall be appropriately adjusted. Any such options that are so continued or assumed in connection with a Corporate Transaction shall be treated as follows: if the grantee's employment is terminated by the Company without

Cause (as such term is defined below) or the grantee resigns due to a Constructive Termination (as such term is defined below), in either case within the ninety (90)-day period preceding or the two (2)-year period following the Corporate Transaction, the exercisability of such option shall automatically accelerate, and the Company's outstanding repurchase rights under this Article Two shall immediately terminate; provided, however, that if the Company, the acquiror or successor refuses to continue (or, as applicable, assume) the option in connection with the Corporate Transaction, the exercisability of such option under this Article Two shall automatically accelerate, and the Company's outstanding repurchase rights under this Article Two shall immediately terminate upon the occurrence of such Corporate Transaction.

D. The grant of options under this Article Two shall in no way affect the right of the Company to adjust, reclassify, reorganize or otherwise change its capital or business structure or to merge, consolidate, dissolve, liquidate or sell or transfer all or any part of its business or assets.

E. In the event of a Change in Control, options shall be treated as follows: if the grantee's employment is terminated by the Company without Cause or the grantee resigns due to a Constructive Termination, in either case within the ninety (90)-day period preceding or the two (2)-year period following the Change in Control, the exercisability of such option shall automatically accelerate, and the Company's outstanding repurchase rights under this Article Two shall immediately terminate; provided, however, that if the acquiror or successor refuses to assume the option in connection with the Change in Control, the exercisability of such option under this Article Two shall automatically accelerate, and the Company's outstanding repurchase rights under this Article Two shall immediately terminate upon the occurrence of such Change in Control. In the event that the acquiror or successor refuses to assume the option in connection with the Change in Control, the Plan Administrator shall have complete discretion to provide, on such terms and conditions as it sees fit, for a cash payment to be made to any optionee on account of any option terminated in accordance with this paragraph, in an amount equal to the excess (if any) of (1) the fair market value of the shares subject to the option as of the date of the Change in Control, over (2) the aggregate exercise price of the option.

F. For purposes of this Section II (and for all other purposes under the Plan), a Change in Control shall be deemed to occur in the event:

1. 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 power of the Company's outstanding securities pursuant to a tender or exchange offer made directly to the Company's stockholders; or

2. there is a change in the composition of the Board over a period of twenty-four (24) consecutive months or less such that a majority of the Board members (rounded up to the next whole number) ceases, by reason of one or more contested elections for Board membership, to be comprised of individuals who either (i) have been Board members continuously since the beginning of such period or (ii) 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 (i) who were still in office at the time such election or nomination was approved by the Board.

G. All options accelerated in connection with a Corporate Transaction or Change in Control (either at the time of the Corporate Transaction or Change in Control or as otherwise provided in this Section II) shall remain fully exercisable until the expiration or sooner termination of the option term.

H. For purposes of this Plan:

1. "Cause" means, unless otherwise provided in the applicable award agreement, the Company's termination of the grantee's employment for any of the following reasons: (i) failure or refusal to comply in any material respect with lawful policies, standards or regulations of the 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 the grantee'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 the grantee's duties.

2. "Constructive Termination" means, unless otherwise provided in the applicable award agreement, the grantee's resignation of employment with the Company within ninety (90) days of the occurrence of any of the following: (i) a material reduction in the grantee's responsibilities; (ii) a material reduction in the grantee's base salary; or (iii) a relocation of the grantee's principal office to a location more than 50 miles from the location of the grantee's existing principal office.

III. EXTENSION OF EXERCISE PERIOD

The Plan Administrator shall have full power and authority, exercisable either at the time the option is granted or at any time while the option remains outstanding, to extend the period of time for which any option granted under this Article Two is to remain exercisable following the optionee's cessation of Service or death from the limited period in effect under Section I.C.1 of Article Two to such greater period of time as the Plan Administrator shall deem appropriate; provided, however, that in no event shall such option be exercisable after the specified expiration date of the option term.

ARTICLE THREE
STOCK ISSUANCE PROGRAM

I. STOCK ISSUANCE TERMS

Shares of Common Stock may be issued under the Stock Issuance Program through direct and immediate issuances without any intervening option grants. Each such stock issuance shall be evidenced by a Stock Issuance Agreement which complies with the terms specified below. Shares of Common Stock may also be issued under the Stock Issuance Program pursuant to RSUs, which are awards granted to eligible employees that entitle them to shares of Common Stock (or cash in lieu thereof) in the future following the satisfaction of vesting conditions imposed by the Plan Administrator.

A. **Vesting Provisions**.

1. The Plan Administrator may issue shares of Common Stock under the Stock Issuance Program which are to vest in one or more installments over the grantee's period of Service or upon attainment of specified performance objectives. Alternatively, the Plan Administrator may issue RSUs under the Stock Issuance Program which shall entitle the recipient to receive a specified number of shares of Common Stock upon the attainment of one or more Service and/or performance goals established by the

Plan Administrator. Upon the attainment of such Service and/or performance goals, fully-vested shares of Common Stock shall be issued in satisfaction of those RSUs.

2. Any new, substituted or additional securities or other property (including money paid other than as a regular cash dividend) issued by reason of any stock dividend, stock split, recapitalization, combination of shares, exchange of shares or other change affecting the outstanding Common Stock as a class without the Company's receipt of consideration, shall be issued or set aside with respect to the shares of unvested Common Stock granted to a grantee or subject to a grantee's RSUs, subject to (i) the same vesting requirements applicable to the grantee's unvested shares of Common Stock or RSUs, and (ii) such escrow arrangements as the Plan Administrator shall deem appropriate.

3. The grantee shall have full stockholder rights with respect to any shares of Common Stock issued to the grantee under the Stock Issuance Program, whether or not the grantee's interest in those shares is vested, except that the grantee shall not have dividend rights with respect to such shares prior to the vesting of such shares. However, the Plan Administrator may provide for a grantee to receive one or more dividend equivalents with respect to such shares, entitling the grantee to all regular cash dividends payable on such shares of Common Stock, which amounts shall be (i) subject to the same vesting requirements applicable to the shares of Common Stock granted hereunder, and (ii) payable upon vesting of the shares to which such dividend equivalents relate.

4. The grantee shall not have any stockholder rights with respect to any shares of Common Stock subject to an RSU. However, the Plan Administrator may provide for a grantee to receive one or more dividend equivalents with respect to such shares, entitling the grantee to all regular cash dividends payable on the shares of Common Stock underlying the RSU, which amounts shall be (i) subject to the same vesting requirements applicable to the shares of Common Stock underlying the RSU, and (ii) payable upon issuance of the shares to which such dividend equivalents relate.

5. Should the grantee cease to remain in Service while holding one or more unvested shares of Common Stock issued under the Stock Issuance Program or should the performance objectives not be attained with respect to one or more such unvested shares of Common Stock, then those shares shall be immediately surrendered to the Company for cancellation, and the grantee shall have no further stockholder rights with respect to those shares. To the extent the surrendered shares were previously issued to the grantee for consideration paid in cash, the Company shall repay to the grantee the cash consideration paid for the surrendered shares.

6. Except as prohibited by Section V of Article One, the Plan Administrator may in its discretion waive the surrender and cancellation of one or more unvested shares of Common Stock which would otherwise occur upon the cessation of the grantee's Service or the non-attainment of the performance objectives applicable to those shares. Such waiver shall result in the immediate vesting of the grantee's interest in the shares of Common Stock as to which the waiver applies. Such waiver may be effected at any time, whether before or after the grantee's cessation of Service or the attainment or non-attainment of the applicable performance objectives.

7. Outstanding RSUs under the Stock Issuance Program shall automatically terminate, and no shares of Common Stock shall actually be issued in satisfaction of those awards, if the Service and/or performance goals established for such awards are not attained. The Plan Administrator, however, shall, except as prohibited by

Section V of Article One, have the discretionary authority to issue shares of Common Stock in satisfaction of one or more outstanding RSUs as to which the designated Service and/or performance goals are not attained. Such authority may be exercised at any time, whether before or after the grantee's cessation of Service or the attainment or non-attainment of the applicable performance objectives.

II. CORPORATE TRANSACTION/CHANGE IN CONTROL

A. Each award which is assigned in connection with (or is otherwise to continue in effect after) a Corporate Transaction shall be appropriately adjusted such that it shall apply and pertain to the number and class of securities issued to the grantee in consummation of the Corporate Transaction with respect to the shares granted to grantee under this Article Three.

B. In the event of a Change in Control, shares of restricted stock and RSUs shall be treated as follows: if the grantee's employment is terminated by the Company without Cause or the grantee resigns due to a Constructive Termination, in either case within the ninety (90)-day period preceding or the two (2)-year period following the Change in Control, the vesting of such restricted stock and RSUs shall automatically accelerate (and all of the shares of Common Stock subject to such RSUs shall be issued to grantees), and the Company's outstanding repurchase rights under this Article Three shall immediately terminate; provided, however, that if the acquiror or successor refuses to assume the shares of restricted stock or RSUs or substitute an award of equivalent value (as determined by the Committee in its discretion) in connection with the Change in Control, the vesting of such restricted stock or RSUs under this Article Three shall automatically accelerate (and all of the shares of Common Stock subject to such RSUs shall be issued to grantees). To the extent any shares of restricted stock or RSUs vest in whole or in part based on the achievement of performance criteria, the amount that shall vest in accordance with the proviso to the immediately-preceding sentence shall vest based on the higher of actual performance goal attainment through the date of the Change in Control or a prorated amount using target performance and based on the time elapsed in the performance period as of the date of the Change in Control.

III. STOCKHOLDER RIGHTS

A. Individuals who are granted shares of Common Stock pursuant to this Article Three shall be the owners of such shares for all purposes while holding such Common Stock and may exercise full voting rights with respect to those shares at all times while held by the individuals. Individuals who have been granted RSUs shall have no voting rights with respect to Common Stock underlying RSUs unless and until such Common Stock is reflected as issued and outstanding shares on the Company's stock ledger.

B. Individuals who are granted shares of Common Stock pursuant to this Article Three shall not have dividend rights with respect to such shares prior to the vesting of such shares. However, the Plan Administrator may provide for a grantee to receive one or more dividend equivalents with respect to such shares, entitling the grantee to all regular cash dividends payable on such shares of Common Stock, which amounts shall be (1) subject to the same vesting requirements applicable to the shares of Common Stock granted hereunder, and (2) payable upon vesting of the shares to which such dividend equivalents relate.

IV. SHARE ESCROW / LEGENDS

Unvested shares may, in the Plan Administrator's discretion, be held in escrow by the Company until the grantee's interest in such shares vests or may be issued directly to the grantee with restrictive legends on the certificates evidencing those unvested shares.

ARTICLE FOUR
MISCELLANEOUS

I. AMENDMENT OF THE PLAN

The Board shall have complete and exclusive power and authority to amend or modify the Plan in any or all respects whatsoever. However, no such amendment or modification shall materially adversely affect any award previously granted under the Plan without the consent of the holder of such award.

II. TAX WITHHOLDING

A. The Company's obligation to deliver shares upon the exercise of stock options or upon the grant or vesting of direct stock issuances or RSUs under the Plan shall be subject to the satisfaction of all applicable Federal, State, non-U.S. and local income and employment tax withholding requirements.

B. Holders of outstanding options or stock issuances and RSUs under the Plan may elect to have the Company withhold, from the shares of Common Stock otherwise issuable upon the exercise or vesting of such awards, a whole number of such shares with an aggregate fair market value equal to the minimum amount necessary to satisfy the Federal, State and local income and employment tax withholdings (the "Taxes") incurred in connection with the acquisition or vesting of such shares. In lieu of such direct withholding, one or more grantees may also be granted the right to deliver whole shares of Common Stock to the Company in satisfaction of such Taxes. Any withheld or delivered shares shall be valued at their fair market value on the applicable determination date for such Taxes.

III. EFFECTIVE DATE AND TERM OF PLAN

The Plan shall be effective on the date specified in the Board resolution adopting the Plan.

IV. USE OF PROCEEDS

Any cash proceeds received by the Company from the sale of shares pursuant to options or stock issuances granted under the Plan shall be used for general corporate purposes.

V. REGULATORY APPROVALS

A. The implementation of the Plan, the granting of any option hereunder, and the issuance of stock (1) upon the exercise or surrender of any option or (2) under the Stock Issuance Program shall be subject to the procurement by the Company of all approvals and permits required by regulatory authorities having jurisdiction over the Plan, the options granted under it and the stock issued pursuant to it.

B. No shares of Common Stock or other assets shall be issued or delivered under the Plan unless and until there shall have been compliance with all applicable requirements of Federal and state securities laws, including (to the extent required) the filing and effectiveness of the Form S-8 registration statement for the shares of Common Stock issuable under the Plan, and all applicable listing requirements of any stock exchange (or the Nasdaq Global Select Market or any successor system, if applicable) on which the Common Stock is then trading.

VI. NO EMPLOYMENT/SERVICE RIGHTS

Neither the action of the Company in establishing or restating the Plan, nor any action taken by the Plan Administrator hereunder, nor any provision of the Plan shall be construed so as to grant any individual the right to remain in the employ or service of the Company (or any parent or subsidiary corporation) for any period of specific duration, and the Company (or any parent or subsidiary corporation retaining the services of such individual) may terminate such individual's employment or service at any time and for any reason, with or without cause.

VII. MISCELLANEOUS PROVISIONS

A. Except to the extent otherwise expressly provided in the Plan, the right to acquire Common Stock or other awards under the Plan may not be assigned, encumbered or otherwise transferred by any grantee.

B. Awards issued under the Plan shall be subject to any clawback policy of the Company as in effect from time-to-time.

C. The provisions of the Plan relating to the exercise of options and the issuance and/or vesting of shares shall be governed by the laws of the State of Delaware without resort to that state's conflict-of-laws provisions, as such laws are applied to contracts entered into and performed in such State.

D. The Plan is intended to be an unfunded plan. Grantees are and shall at all times be general creditors of the Company with respect to their awards. If the Committee or the Company chooses to set aside funds in a trust or otherwise for the payment of awards under the Plan, such funds shall at all times be subject to the claims of the creditors of the Company in the event of its bankruptcy or insolvency.

E. **Awards to Non-U.S. Employees.** The Committee shall have the power and authority to determine which subsidiary corporations shall be covered by this Plan and which employees outside the United States shall be eligible to participate in the Plan. The Committee may adopt, amend, or rescind rules, procedures, or sub-plans relating to the operation and administration of the Plan to accommodate the specific requirements of local laws, procedures, and practices. Without limiting the generality of the foregoing, the Committee is specifically authorized to adopt rules, procedures, and sub-plans with provisions that limit or modify rights on death, disability, or retirement or on termination of employment; available methods of exercise or settlement of an award; payment of income, social insurance contributions and payroll taxes; the withholding procedures and handling of any stock certificates or other indicia of ownership which vary with local requirements. The Committee may also adopt rules, procedures or sub-plans applicable to particular subsidiary corporations or locations.

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: November 8, 2023

/s/ Jon P. Stonehouse
Jon P. Stonehouse
President and Chief Executive Officer

Exhibit 31.2

CERTIFICATIONS

I, Anthony Doyle, 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: November 8, 2023

/s/ Anthony Doyle
Anthony Doyle
Chief Financial Officer

Exhibit 32.1

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

In connection with the Quarterly Report of BioCryst Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended September 30, 2023 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) of the Securities Exchange Act of 1934; and

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

/s/ Jon P. Stonehouse
Jon P. Stonehouse
President and Chief Executive Officer
Date: November 8, 2023

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 September 30, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Anthony Doyle, 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) of the Securities Exchange Act of 1934; and

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

/s/ Anthony Doyle
Anthony Doyle
Chief Financial Officer
Date: November 8, 2023