

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

FORM 10-Q

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

For the quarterly period ended June 30, 2022

or

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

For the transition period from _____ to _____

Commission File Number 000-23186

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

Delaware
(State or other jurisdiction of
incorporation or organization)

62-1413174
(I.R.S. Employer
Identification No.)

4505 Emperor Blvd., Suite 200
Durham, North Carolina
(Address of principal executive offices)

27703
(Zip Code)

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

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

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

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

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

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

Large accelerated filer	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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

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

The number of shares of Common Stock, par value \$0.01, of the Registrant outstanding as of July 29, 2022 was 185,945,016.

BIOCRYST PHARMACEUTICALS, INC.

INDEX

	Page No.
Cautionary Note Regarding Forward-Looking Statements	i
Risk Factor Summary	ii
Part I. Financial Information	1
Item 1. Financial Statements:	1
Consolidated Balance Sheets - June 30, 2022 and December 31, 2021	1
Consolidated Statements of Comprehensive Loss - Three and Six Months Ended June 30, 2022 and 2021	2
Consolidated Statements of Cash Flows - Six Months Ended June 30, 2022 and 2021	3
Consolidated Statements of Stockholders' Deficit - Three and Six Months Ended June 30, 2022 and 2021	4
Notes to Consolidated Financial Statements	5
Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations	27
Item 3. Quantitative and Qualitative Disclosures About Market Risk	39
Item 4. Controls and Procedures	40
Part II. Other Information	42
Item 1A. Risk Factors	42
Item 6. Exhibits	69
Signatures	71
EX-10.3	
EX-10.4	
EX-10.5	
EX-31.1	
EX-31.2	
EX-32.1	
EX-32.2	

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), BCX9930, BCX9250, peramivir, galidesivir, and early-stage discovery programs, and our plans regarding the same;
- 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;
- the potential funding from our contracts with the Biomedical Advanced Research and Development Authority within the U.S. Department of Health and Human Services (“BARDA/HHS”) and the National Institute of Allergy and Infectious Diseases within the HHS (“NIAID/HHS”) for the development of galidesivir;
- 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 our Credit 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; 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 when needed, we may need to adjust our operations.
- Our success depends upon our ability to advance our product candidates through the various stages of development, especially through the clinical trial process, to receive and maintain regulatory approval for the commercial sale of our product candidates, and to successfully commercialize any approved products. 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, or take other actions that could materially impact the cost, timing, and success of our planned development 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 were not previously identified, 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 expect to continue expanding our development and regulatory capabilities and 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 and develop our products and product candidates, obtain collaborators, and raise capital.
- If we fail to adequately protect or enforce our intellectual property rights or secure rights to patents of others, 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.
- 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.
- We face risks related to our government-funded programs and are subject to various U.S. Government contract requirements, which 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.
- 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 seek additional remedies.
- Our Credit 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 Credit 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 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.
- Natural disasters, epidemic or pandemic disease outbreaks, trade wars, armed conflicts, political unrest, or other events could disrupt our business or operations or those of our development partners, manufacturers, regulators, or third parties with whom we conduct business now or in the future.
- We are subject to legal proceedings, which could harm our reputation or result in other losses or unexpected expenditure of time and resources.

- 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.
CONSOLIDATED BALANCE SHEETS
June 30, 2022 and December 31, 2021
(In thousands, except per share data)

	2022 (Unaudited)	2021 (Note 1)
Assets		
Cash and cash equivalents	\$ 272,571	\$ 504,389
Restricted cash	1,448	3,345
Investments	144,912	3,212
Trade receivables	41,491	29,413
Inventory	23,378	15,791
Prepaid expenses and other current assets	11,053	9,986
Total current assets	494,853	566,136
Property and equipment, net	8,702	8,714
Long-term investments	—	6,829
Other assets	6,983	6,472
Total assets	<u>\$ 510,538</u>	<u>\$ 588,151</u>
Liabilities and Stockholders' Deficit		
Accounts payable	\$ 12,024	\$ 27,808
Accrued expenses	79,711	72,670
Deferred revenue	1,521	1,421
Lease financing obligation	2,134	1,819
Total current liabilities	95,390	103,718
Lease financing obligation	6,198	5,962
Royalty financing obligations	477,666	449,375
Secured term loan	144,516	136,082
Stockholders' equity:		
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 - 185,876 as of June 30, 2022 and 184,350 as of December 31, 2021	1,859	1,843
Additional paid-in capital	1,125,453	1,098,498
Accumulated other comprehensive income	15	177
Accumulated deficit	(1,340,559)	(1,207,504)
Total stockholders' deficit	(213,232)	(106,986)
Total liabilities and stockholders' deficit	<u>\$ 510,538</u>	<u>\$ 588,151</u>

See accompanying notes to consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
Three and Six Months Ended June 30, 2022 and 2021
(In thousands, except per share data-Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Revenues				
Product sales, net	\$ 64,888	\$ 33,430	\$ 114,434	\$ 51,301
Royalty revenue	540	128	887	(769)
Milestone revenue	—	15,000	—	15,000
Collaborative and other research and development	104	1,401	134	3,486
Total revenues	65,532	49,959	115,455	69,018
Expenses				
Cost of product sales	246	297	482	6,220
Research and development	61,990	52,873	127,350	95,308
Selling, general and administrative	38,017	26,325	72,299	48,439
Royalty	1	46	3	10
Total operating expenses	100,254	79,541	200,134	149,977
Loss from operations	(34,722)	(29,582)	(84,679)	(80,959)
Interest and other income	609	13	663	39
Interest expense	(24,022)	(13,495)	(47,859)	(26,399)
Foreign currency gains (losses), net	132	(134)	(45)	(163)
Loss before income taxes	(58,003)	(43,198)	(131,920)	(107,482)
Income tax expense	856	—	1,135	—
Net loss	\$ (58,859)	\$ (43,198)	\$ (133,055)	\$ (107,482)
Foreign currency translation adjustment	104	(92)	182	86
Unrealized loss on available for sale investments	(275)	(3)	(344)	(2)
Comprehensive loss	\$ (59,030)	\$ (43,293)	\$ (133,217)	\$ (107,398)
Basic and diluted net loss per common share	\$ (0.32)	\$ (0.24)	\$ (0.72)	\$ (0.60)
Weighted average shares outstanding	185,605	178,127	185,253	177,737

See accompanying notes to consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
Six Months Ended June 30, 2022 and 2021
(In thousands-Unaudited)

	Six Months Ended June 30,	
	2022	2021
Cash flows from operating activities:		
Net loss	\$ (133,055)	\$ (107,482)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	652	377
Stock-based compensation expense	19,468	13,111
Non-cash interest expense on royalty financing obligations and secured term loan and amortization of debt issuance costs	36,725	22,664
Amortization of premium/discount on investments	(88)	34
Changes in operating assets and liabilities:		
Receivables	(12,343)	(18,976)
Inventory	(7,589)	(2,809)
Prepaid expenses and other assets	(1,050)	(34)
Accounts payable and accrued expenses	(8,463)	2,743
Interest payable	—	3,547
Deferred revenue	186	403
Net cash used in operating activities	(105,557)	(86,422)
Cash flows from investing activities:		
Acquisitions of property and equipment	(650)	(912)
Purchase of investments	(139,260)	—
Sales and maturities of investments	4,000	20,089
Net cash (used in) provided by investing activities	(135,910)	19,177
Cash flows from financing activities:		
Net proceeds from common stock issued under stock-based compensation plans	7,503	7,474
Net cash provided by financing activities	7,503	7,474
Effect of exchange rate on cash, cash equivalents, and restricted cash	249	85
Decrease in cash, cash equivalents and restricted cash	(233,715)	(59,686)
Cash, cash equivalents and restricted cash at beginning of period	507,734	274,348
Cash, cash equivalents and restricted cash at end of period	\$ 274,019	\$ 214,662

See accompanying notes to consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' DEFICIT
Three and Six Months Ended June 30, 2022 and 2021
(In thousands, except per share amounts-Unaudited)

	Common Stock	Additional Paid-In Capital	Accumulated Other Comprehensive 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 options, 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 income	—	—	(171)	—	(171)
Exercise of warrants, 253 shares	3	—	—	—	3
Exercise of stock options, 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)

	Common Stock	Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Deficit
Balance at December 31, 2020	\$ 1,769	\$ 1,002,408	\$ 3	\$ (1,023,442)	\$ (19,262)
Net loss	—	—	—	(64,284)	(64,284)
Other comprehensive income	—	—	179	—	179
Employee stock purchase plan sales, 193 shares, net	2	721	—	—	723
Exercise of stock options, 593 shares, net	6	2,171	—	—	2,177
Stock-based compensation expense	—	5,479	—	—	5,479
Balance at March 31, 2021	\$ 1,777	\$ 1,010,779	\$ 182	\$ (1,087,726)	\$ (74,988)
Net loss	—	—	—	(43,198)	(43,198)
Other comprehensive income	—	—	(95)	—	(95)
Exercise of stock options, 1,056 shares, net	10	4,564	—	—	4,574
Stock-based compensation expense	—	7,632	—	—	7,632
Balance at June 30, 2021	\$ 1,787	\$ 1,022,975	\$ 87	\$ (1,130,924)	\$ (106,075)

See accompanying notes to consolidated financial statements.

BIOCRYST PHARMACEUTICALS, INC.
NOTES TO 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 commercial-stage biotechnology company that discovers novel, oral, small-molecule medicines. The Company focuses on the treatment of rare diseases in which significant unmet medical needs exist and an enzyme plays the key role in the biological pathway of the disease. 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, 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 June 30, 2022 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 2022 expenses will exceed its 2022 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 be largely 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) securing or increasing U.S. Government funding of its programs, including obtaining procurement contracts; (2) out-licensing rights to certain of its products or product candidates, pursuant to which the Company would receive cash milestone payments; (3) raising additional capital through equity or debt financings or from other sources, including royalty or other monetization transactions; (4) obtaining additional product candidate regulatory approvals, which would generate revenue, milestone payments and cash flow; (5) reducing spending on one or more research and development programs, including by discontinuing development; and/or (6) restructuring operations to change its overhead structure. The Company may issue securities, including common stock, preferred stock, depository 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 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 consolidated financial statements.

The Company’s 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 consolidated financial position, results of operations, and cash flows. There were no adjustments other than normal recurring adjustments.

These financial statements should be read in conjunction with the financial statements for the year ended December 31, 2021 and the notes thereto included in the Company’s 2021 Annual Report on Form 10-K. Interim operating

results are not necessarily indicative of operating results for the full year. The balance sheet as of December 31, 2021 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. The most significant estimates in the Company's consolidated financial statements relate to the valuation of stock options, the ORLADEYO and Factor D inhibitors royalty financing obligations 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

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 product under contracts negotiated between them and the Company's specialty pharmacy. The specialty pharmacy, in turn, charges back to the Company the difference between the price 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 Research and Development Arrangements and Royalties

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 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 the 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

Restricted cash of \$24 and \$1,924 as of June 30, 2022 and December 31, 2021, respectively, reflects royalty revenue paid by Shionogi designated for interest on the Pharma Notes (defined in Note 6). Additionally, restricted cash of \$1,424 and \$1,421 as of June 30, 2022 and December 31, 2021, respectively, reflects collateral 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. Realized gains and losses are reflected in interest and other income in the 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. At June 30, 2022, the Company believes that the cost of its investments is recoverable in all material respects.

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 the HHS, and royalty receivables from the Company's partners, including Shionogi, 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 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 labelling, including raw material, active product ingredient, and the drug product. Finished goods include packaged and labelled 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.

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 reliant 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 generally 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. 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. As of June 30, 2022 and December 31, 2021, 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 charged to expense 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. 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, such as Albert Einstein College of Medicine of Yeshiva University (“AECOM”), Industrial Research, Ltd. (“IRL”), and the University of Alabama at Birmingham (“UAB”), 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 sub-license 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.

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.

Advertising expenses related to ORLADEYO were \$4,095 and \$8,079 for the three and six months ended June 30, 2022, respectively, and \$1,800 and \$3,204 for the three and six months ended June 30, 2021, respectively.

The Company seeks patent protection on all internally developed processes and products. All patent related costs are expensed to selling, general and administrative expenses when incurred as recoverability of such expenditures is uncertain.

Leases

The Company leases certain assets under operating leases, which primarily consisted of real estate leases, laboratory equipment leases and office equipment leases as of June 30, 2022. 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 operating 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 Consolidated Balance Sheet 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 operating leases; therefore, the Company has no corresponding liability recorded on its 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 Consolidated Statements of Comprehensive Loss based on their fair values. The fair value of stock option awards is estimated using the Black-Scholes option pricing model. The fair value of restricted stock unit awards is based on the grant date closing price of the common stock. Stock-based compensation cost is recognized as expense on a straight-line basis over the requisite service period of the award. In addition, the Company has outstanding performance-based stock options for which no compensation expense is recognized until "performance" is deemed to have occurred.

Interest Expense and Deferred Financing Costs

Interest expense for the three and six months ended June 30, 2022 was \$24,022 and \$47,859, respectively, and \$13,495 and \$26,399 for the three and six months ended June 30, 2021, respectively, and primarily relates to the royalty financing obligations (Note 6), the secured term loan borrowing from the Credit Agreement (Note 7) and the PhaRMA Notes (Note 6). Costs directly associated with the borrowings have been capitalized and are netted against the corresponding debt liabilities on the 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 \$(172) and \$(339) for the three and six months ended June 30, 2022, respectively, and \$(154) and \$(211) for the three and six months ended June 30, 2021, respectively.

In December 2021, the PhaRMA Notes and associated accrued interest payable was written-off into other income as a debt extinguishment (Refer to "Note 6—Royalty Monetizations—RAPIACTA—Non-Recourse Notes Payable – Debt Extinguishment" for additional information regarding the debt extinguishment).

Interest Expense and Royalty Financing Obligations

The royalty financing obligations are eligible to be repaid based on royalties from net sales of ORLADEYO, BCX9930, and another earlier stage Factor D inhibitor. 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.

Beginning in fiscal year 2021, the Company began accruing U.S. state income taxes and foreign income taxes as a result of increased sales of ORLADEYO and increased nexus in multiple states and foreign jurisdictions where historically the Company had no presence.

Net Loss Per Share

Net loss per share is based upon the weighted average number of common shares outstanding during the period. Diluted 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 were anti-dilutive. The calculation of diluted earnings per share does not include 23,090 and 25,877 shares of potential common stock for the three and six months ended June 30, 2022, respectively, and 27,419 and 25,871 shares of potential common stock for the three and six months ended June 30, 2021, respectively, as their impact would be anti-dilutive.

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 Consolidated Statements of Comprehensive Loss. There were no realized gains or losses reclassified out of accumulated other comprehensive income for the six months ended June 30, 2022. For the six months ended June 30, 2021, realized gains of \$1 were reclassified out of accumulated other comprehensive loss.

Significant Customers and Other Risks

Significant Customers

The Company's primary sources of revenue and cash flow are the sales of ORLADEYO to a specialty pharmacy, the reimbursement of galidesivir (formerly BCX4430) development expenses earned under cost-plus-fixed-fee contracts with BARDA/HHS and NIAID/HHS and 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. 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 relies on BARDA/HHS and NIAID/HHS to reimburse predominantly all of the development costs for its galidesivir program and stockpiling sales of RAPIVAB to HHS. Accordingly, reimbursement of these expenses represents a significant portion of the Company's collaborative and other research and development revenues. Additionally, HHS is the primary customer for RABIVAB. The completion or termination of the NIAID/HHS and BARDA/HHS galidesivir contracts or the reduction or stoppage of purchases of RAPIVAB by HHS could adversely impact the Company's business, results of operations and financial condition.

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 single source manufacturers 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 in the manufacture or distribution of any product could adversely impact the commercial revenue and future procurement stockpiling of the Company's 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 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 six months ended June 30, 2022, 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, 2021, 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 June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Product sales, net:				
ORLADEYO	\$ 64,711	\$ 28,374	\$ 114,097	\$ 39,312
RAPIVAB	177	4,622	337	4,735
Peramivir	—	434	—	7,254
Total product sales, net	64,888	33,430	114,434	51,301
Royalty revenue	540	128	887	(769)
Milestone revenue	—	15,000	—	15,000
Collaborative and other research and development revenues:				
U.S. Department of Health and Human Services	104	1,401	134	3,486
Total collaborative and other research and development revenues	104	1,401	134	3,486
Total revenues	\$ 65,532	\$ 49,959	\$ 115,455	\$ 69,018

Note 3 — Investments

The following tables summarize the fair value of the Company's investments by type. The estimated fair values of the Company's fixed income investments are classified as Level 2 in the fair value hierarchy as defined in U.S. GAAP. 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 which utilize Level 2 inputs.

	June 30, 2022				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its agencies	\$ 137,222	\$ 135	\$ 2	\$ (393)	\$ 136,966
Corporate debt securities	6,299	47	—	(38)	6,308
Certificates of deposit	1,644	8	—	(14)	1,638
Total investments	\$ 145,165	\$ 190	\$ 2	\$ (445)	\$ 144,912

	December 31, 2021				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its agencies	\$ 4,043	\$ 17	\$ —	\$ (7)	\$ 4,053
Corporate debt securities	4,294	40	—	(5)	4,329
Certificates of deposit	1,652	8	—	(1)	1,659
Total investments	\$ 9,989	\$ 65	\$ —	\$ (13)	\$ 10,041

The following table summarizes the scheduled maturity for the Company's investments at June 30, 2022 and December 31, 2021:

	June 30, 2022	December 31, 2021
Maturing in one year or less	\$ 144,912	\$ 3,212
Maturing after one year through two years	—	6,829
Total investments	\$ 144,912	\$ 10,041

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 June 30, 2022 and December 31, 2021, receivables related to sales of ORLADEYO were \$39,587 and \$27,384, respectively. At June 30, 2022 and December 31, 2021, receivables related to sales of RAPIVAB were \$61 and \$49, respectively. No reserve or allowance amounts were recorded as of June 30, 2022 and December 31, 2021, respectively.

Collaborations

Receivables from collaborations were as follows (in thousands):

	June 30, 2022		
	Billed	Unbilled	Total
U.S. Department of Health and Human Services	\$ —	\$ 1,303	\$ 1,303
Royalty receivables from partners	540	—	540
Total receivables	\$ 540	\$ 1,303	\$ 1,843

	December 31, 2021		
	Billed	Unbilled	Total
U.S. Department of Health and Human Services	\$ 5	\$ 1,670	\$ 1,675
Royalty receivables from partners	305	—	305
Total receivables	\$ 310	\$ 1,670	\$ 1,980

As of both June 30, 2022 and December 31, 2021, the Company maintained a reserve of \$701 related to royalties associated with Green Cross.

Note 5 — Inventory

At June 30, 2022 and December 31, 2021, 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):

	June 30, 2022	December 31, 2021
Raw materials	\$ 5,276	\$ 5,658
Work-in-process	15,616	9,669
Finished goods	2,739	709
Total inventory	\$ 23,631	\$ 16,036
Reserves	(253)	(245)
Total inventory, net	\$ 23,378	\$ 15,791

Note 6 — Royalty Monetizations**RAPIACTA***Overview*

On March 9, 2011, the Company completed a \$30,000 financing transaction to monetize certain future royalty and milestone payments under the Company's agreement with Shionogi (the "Shionogi Agreement"), pursuant to which Shionogi licensed from the Company the rights to market RAPIACTA in Japan and Taiwan. The Company received net proceeds of \$22,691 from the transaction after transaction costs of \$4,309 and the establishment of a \$3,000 interest reserve account by JPR Royalty Sub LLC, a wholly-owned subsidiary of the Company ("Royalty Sub"), available to help cover interest shortfalls in the future. All of the interest reserve account has been fully utilized with the September 2012 interest payment.

As part of the transaction, the Company entered into a purchase and sale agreement dated as of March 9, 2011 with Royalty Sub, whereby the Company transferred to Royalty Sub, among other things, (i) its rights to receive certain royalty and milestone payments from Shionogi arising under the Shionogi Agreement, and (ii) the right to receive payments under a Japanese yen/US dollar foreign currency hedge arrangement (as further described below, the "Currency Hedge Agreement") put into place by the Company in connection with the transaction. Royalty payments are paid by Shionogi in Japanese yen, and any milestone payments will be paid in U.S. dollars. The Company's collaboration with Shionogi was not impacted as a result of this transaction.

Non-Recourse Notes Payable

On March 9, 2011, Royalty Sub completed a private placement to institutional investors of \$30,000 in aggregate principal amount of its PhaRMA Senior Secured 14.0% Notes due on December 1, 2020 (the "PhaRMA Notes"). The PhaRMA Notes were issued by Royalty Sub under an Indenture, dated as of March 9, 2011 (the "Indenture"), by and between Royalty Sub and U.S. Bank National Association, as Trustee. Principal and interest on the PhaRMA Notes are payable from, and are secured by, the rights to royalty and milestone payments under the Shionogi Agreement transferred by the Company to Royalty Sub and payments, if any, made to Royalty Sub under the Currency Hedge Agreement. The PhaRMA Notes bear interest at 14% per annum, payable annually in arrears on September 1st of each year. The Company remains entitled to receive any royalties and milestone payments related to sales of peramivir by Shionogi following repayment of the PhaRMA Notes.

Royalty Sub's obligations to pay principal and interest on the PhaRMA Notes are obligations solely of Royalty Sub and are without recourse to any other person, including the Company, except to the extent of the Company's pledge of its equity interests in Royalty Sub in support of the PhaRMA Notes. The Company may, but is not obligated to, make capital contributions to a capital account that may be used to redeem, or on up to one occasion pay any interest shortfall on, the PhaRMA Notes.

In September 2014, Royalty Sub was unable to pay the accrued interest obligation due September 3, 2013. Under the terms of the Indenture, Royalty Sub's inability to pay the full amount of interest payable in September 2013 by the next succeeding payment date for the PhaRMA Notes, which was September 1, 2014, constituted an event of default. Accordingly, the PhaRMA Notes and related accrued interest have been classified as current liabilities on the December 31, 2014 balance sheet and thereafter. As a result of the event of default under the PhaRMA Notes, the holders of the PhaRMA Notes may foreclose on the collateral securing the PhaRMA Notes and the equity interest in Royalty Sub and exercise other remedies available to them under the Indenture in respect of the PhaRMA Notes. In such event, the Company may not realize the benefit of future royalty payments that might otherwise accrue to it following repayment of the PhaRMA Notes and it might otherwise be adversely affected. Due to the non-recourse nature of the PhaRMA Notes, in the event of any potential foreclosure, the primary impact to the Company would be the loss of future royalty payments, if any, from Shionogi and legal costs associated with retiring the PhaRMA Notes. 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 \$30,000, together with accrued and unpaid interest of \$20,614, was due in full.

Non-Recourse Notes Payable – Debt Extinguishment

During 2021, the royalty-bearing patents associated with RAPIACTA in Japan expired. Accordingly, the Company evaluated the current circumstances of the PhaRMA Notes, including (i) their non-recourse nature relative to the Company, (ii) the current state of default since September 1, 2014 and the legal maturity on December 1, 2020 and (iii) the loss of patent protection relative to RAPIACTA in Japan, upon which any significant repayment of the PhaRMA Notes is

predicated. As a result, the Company determined that it was no longer the financial obligor, and as a result, the principal balance of \$30,000 and associated accrued interest payable balance of \$25,838 were written off, resulting in a gain on extinguishment recorded in other income (expense) for the year ended December 31, 2021.

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. In addition, RPI will be entitled to receive 1.0% of global net sales, if any, of BCX9930.

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 BCX9930 and another earlier stage Factor D inhibitor in an amount equal to: (i) 3.0% of worldwide aggregate annual net sales up to \$1.5 billion and (ii) 2.0% of worldwide aggregate annual net sales between \$1.5 billion and \$3.0 billion. No royalty payments are payable on annual net sales above \$3.0 billion. 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, commencing with the calendar quarter beginning October 1, 2023, OMERS will be 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) (the “Regime A Royalty Rate”). If annual Direct Sales for calendar year 2023 reach a specified amount set forth in the OMERS Royalty Purchase Agreement, then for each calendar quarter beginning on or after January 1, 2024, OMERS will be entitled to receive the Regime A Royalty Rate. If annual Direct Sales for calendar year 2023 are less than the specified amount, 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) (the “Regime B Royalty Rate”).

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 will be 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 either 142.5% or 155.0% of the \$150,000 purchase price, depending on sales levels in calendar year 2023.

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 is permitted to be incurred under the terms of the Company’s Credit Agreement with Athyrrium Opportunities III Co-Invest 1 LP. Refer to Note 7 for further details on the Credit Agreement. The restrictions 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 consolidated balance sheet. 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 its 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 June 30, 2022, the Company adjusted its forecasts related to its Factor D program. Accordingly, this 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 27.3% to 25.5%, and the effective interest rate related to the 2021 RPI Royalty Purchase Agreement decreased from 16.5% to 12.1%.

The following table shows the activity within the Royalty financing obligations account (in thousands) as well as the effective interest rate as of June 30, 2022:

	2020 RPI Royalty Agreement	2021 RPI Royalty Agreement	OMERS Royalty Agreement	Total
Balance as of December 31, 2021	\$ 147,224	\$ 153,377	\$ 148,774	\$ 449,375
Deferred financing costs	—	(34)	—	(34)
Non-cash Interest expense on Royalty financing obligations	10,165	6,327	3,157	19,649
Royalty revenues paid and payable	(4,385)	(380)	—	(4,765)
Balance as of March 31, 2022	\$ 153,004	\$ 159,290	\$ 151,931	\$ 464,225
Non-cash Interest expense on Royalty financing obligations	10,462	6,020	3,224	19,706
Royalty revenues paid and payable	(5,764)	(501)	—	(6,265)
Balance as of June 30, 2022	\$ 157,702	\$ 164,809	\$ 155,155	\$ 477,666
Effective interest rate	25.5 %	12.1 %	8.5 %	

Deferred issuance costs pursuant to the Royalty financing obligations, which consist primarily of advisory and legal fees, totaled \$8,531 as of June 30, 2022. 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. Refer to Note 9 for further details on the common stock sale premium.

Note 7 — Debt

Credit Agreement

On December 7, 2020, the Company entered into a \$200,000 Credit Agreement (the "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 are guarantors to the Credit Agreement. The Credit Agreement provides 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 loan" on the Company's balance sheet as of June 30, 2022. 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 Credit Agreement also provides 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 "Term Loans"). Both the Term B Loan and the Term C Loan were funded on July 29, 2022 in the aggregate principal amount of \$75,000. Refer to Note 12 for further details on the funding of the Term B Loan and the Term C Loan. The maturity date of the Credit Agreement is December 7, 2025.

On November 19, 2021, the Company entered into an amendment to the Credit Agreement (i) to 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) to require the Company to pay to Athyrium, for the account of the lenders, a make-whole premium plus certain fees set forth in the Credit Agreement in the event that the Company did not draw the Term B Loan or the Term C Loan, as applicable, by the end of the applicable period available to draw the Term B Loan or the Term C Loan, subject to certain exceptions set forth in the Credit Agreement; and (iii) to require the Company to pay to Athyrium, for the account of the lenders, a make-whole premium plus certain fees set forth in the Credit Agreement in the event that the Company either (x) terminated the commitments in respect of the Term B Loan or the Term C Loan, as applicable, on or prior to the end of the applicable period available to

draw the Term B Loan or the Term C Loan, or (y) prepays or repays, or is required to prepay or repay, voluntarily or pursuant to mandatory prepayment obligation under the 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 Term Loans, in each case, subject to certain exceptions set forth in the Credit Agreement.

The Credit Agreement provides for quarterly interest-only payments until the maturity date, with the unpaid principal amount of the outstanding Term Loans due and payable on the maturity date. For each of the first eight full fiscal quarters following December 7, 2020, the Company has the option to make the applicable interest payment in-kind (a "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 Term Loans will bear interest at a rate equal to the three-month LIBOR rate, which shall be no less than 1.75% and no more than 3.50% ("LIBOR"), plus 8.25%, or for each interest period in which a PIK Interest Payment is made, LIBOR plus 10.25%.

The three-month LIBOR was 2.3125% as of June 28, 2022, the LIBOR measurement date for the three-month interest period beginning July 1, 2022. Accordingly, this LIBOR rate plus 10.25% will be used to record interest expense for the three-month interest period beginning July 1, 2022.

Subject to certain exceptions, the Company is required to make mandatory prepayments of the 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, subject to certain exceptions. The Company may make voluntary prepayments in whole or in part. Prepayments are 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 Term Loan borrowing date, the amount, if any, by which (a) the sum of (1) 102.00% of the principal amount of the Term Loan being prepaid plus (2) the present value of all interest that would have accrued on the principal amount of the Term Loan being prepaid through and including the second anniversary of the date of the borrowing of such Term Loan, plus 0.50%, exceeds (b) the principal amount of the Term Loan being prepaid; (ii) with respect to any prepayment made between the second and third anniversaries of the applicable Term Loan borrowing date, 2.00% of the principal amount of the Term Loan being prepaid; (iii) with respect to any prepayment made between the third and fourth anniversaries of the applicable Term Loan borrowing date, 1.00% of the principal amount of the Term Loan being prepaid; and (iv) with respect to any prepayment made after the fourth anniversary of the applicable Term Loan borrowing date, 0.00% of the principal amount of the Term Loan being prepaid. Upon the prepayment or repayment, including at maturity, of all or any of the Term Loans, the Company is obligated to pay an exit fee in an amount equal to 2.00% of the principal amount of the Term Loans prepaid or repaid. In addition, each Term Loan is subject to a 1.00% commitment fee at its respective borrowing date.

The Credit 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, grant liens, make investments, incur additional indebtedness, engage in mergers, acquisitions, and similar transactions, dispose of assets, license certain property, distribute dividends, make certain restricted payments, change the nature of the Company's business, engage in transactions with affiliates and insiders, prepay other indebtedness, or engage in sale and leaseback transactions, subject to certain exceptions. Additionally, as of the last day of each fiscal quarter (a "Test Date"), beginning with the first Test Date occurring immediately after the Term C Loan is drawn, if applicable, the Company may not permit consolidated net revenues from ORLADEYO sales in the United States for the four-fiscal quarter period ending on such Test Date to be less than the specified amounts set forth in the Credit Agreement (collectively, the "Revenue Tests"). If the Company fails to satisfy the Revenue Tests as of any Test Date, it will have a one-time right (the "Cure Right") to repay in full the entire amount of the Term C Loan outstanding at such time together with all accrued and unpaid interest thereon plus the prepayment premium, exit fee, and any other fees or amounts payable under the Credit Agreement at such time. In addition, the Credit Agreement contains a minimum liquidity covenant requiring the Company to maintain at all times, as applicable, at least \$15,000 of unrestricted cash and cash equivalents if only the Term A Loan has been drawn; at least \$20,000 of unrestricted cash and cash equivalents if the Term B Loan has been drawn but the Term C Loan has not been drawn; and at least \$15,000 (or, if the Cure Right has been exercised, \$20,000) of unrestricted cash and cash equivalents if the Term C Loan has been drawn, subject to certain exceptions.

A failure to comply with the covenants in the Credit Agreement could permit the Lenders under the Credit Agreement to declare the outstanding principal as well as accrued interest and fees, to be immediately due and payable.

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

As of June 30, 2022, the Company had borrowings of \$125,000 under the Credit Agreement. Quarterly interest payments under the Credit Agreement for the six months ended June 30, 2022 and 2021 totaled \$8,774 and \$7,745, respectively, and have been designated and accounted for as PIK Interest Payments and added to the outstanding principal balance of the borrowing. As of June 30, 2022, borrowings, including the PIK Interest Payments, totaled \$150,839. The principal balance of the borrowing, including PIK amounts, are accruing interest at a rate of 12.17%. 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.

As of June 30, 2022, deferred debt fees and issuance costs totaled \$8,822 and are being amortized as interest expense on an effective interest rate method over the term of the Term A Loan. When utilizing the effective interest method, in periods in which PIK interest is designated and those amounts are added to the outstanding principal balance of the borrowing, the amortization of the deferred debt fees and issuance costs is accretive. Deferred financing amortization of \$(339) and \$(211), was recognized for the six months ended June 30, 2022 and 2021, respectively.

The Credit Agreement contains two provisions that, if deemed probable, would create the recognition of an embedded feature; however, at this time, the Company does not believe either provision is probable.

Note 8 — Lease Obligations

The Company leases certain assets under operating leases, which primarily consisted of real estate leases, laboratory equipment leases and office equipment leases as of June 30, 2022. Renewal options for the Company's leases range from 1 to 5 years in length and begin from 2023 through 2026.

Aggregate lease expense under operating leases was as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Aggregate lease expense	\$ 626	\$ 461	\$ 1,220	\$ 913

Other supplemental information related to leases was as follows:

	As of June 30, 2022	As of December 31, 2021
Weighted average remaining lease term	8.5 years	9.2 years
Weighted average discount rate	10.9%	11.2%

All of the Company's leases qualify as operating leases. The following table summarizes the presentation in the consolidated balance sheets of the Company's operating leases:

	Balance Sheet Location	As of June 30, 2022	As of December 31, 2021
Assets:			
Operating lease assets, net	<i>Other Assets</i>	\$ 6,983	\$ 6,472
Liabilities:			
Current operating lease liabilities	<i>Lease financing obligation – current liabilities</i>	\$ 2,134	\$ 1,819
Non-current operating lease liabilities	<i>Lease financing obligation – long-term liabilities</i>	6,198	5,962
Total operating lease liabilities		\$ 8,332	\$ 7,781

Operating lease assets are recorded net of accumulated amortization of \$3,440 and \$2,626 as of June 30, 2022 and December 31, 2021, respectively.

Cash paid for amounts included in the measurement of lease liabilities was \$607 and \$1,181 for the three and six months ended June 30, 2022, respectively, and \$379 and \$819 for the three and six months ended June 30, 2021, respectively.

Maturities of operating lease liabilities as of June 30, 2022, are as follows (in thousands):

2022 (remaining)	\$	1,265
2023		2,266
2024		1,717
2025		1,373
2026		617
Thereafter		6,734
Total lease payments		13,972
Less imputed interest		(5,640)
Total	\$	8,332

Note 9 — Stockholders' Equity

Sales of Common Stock

On April 24, 2020, the Company filed a shelf registration statement on Form S-3 with the SEC. This shelf registration statement became effective on May 14, 2020 and allows the Company to sell up to \$500,000 in 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 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 3,846,154 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 represents a premium of \$1.11 over the closing price of \$11.89 of the Company's common stock on November 17, 2021, the last trading day prior to the execution of the Common Stock Purchase Agreement. 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.

Note 10 — Stock-Based Compensation

As of June 30, 2022, 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 18, 2022 and approved by the Company's stockholders on June 7, 2022. The Inducement Plan was most recently amended and restated by the Board of Directors on July 23, 2021. The ESPP was most recently amended and restated on April 1, 2021 and approved by the Company's stockholders on May 25, 2021.

Stock-based compensation expense of \$19,468 (\$15,867 of expense related to the Incentive Plan, \$2,965 of expense related to the Inducement Plan, and \$636 of expense related to the ESPP) was recognized during the six months ended June 30, 2022, while \$13,111 (\$11,073 of expense related to the Incentive Plan, \$1,274 of expense related to the Inducement Plan and \$764 of expense related to the ESPP) was recognized during the six months ended June 30, 2021.

There was approximately \$104,406 of total unrecognized compensation expense related to non-vested stock option and restricted stock unit awards granted by the Company as of June 30, 2022. As of June 30, 2022, the Company expected to recognize that expense as follows: \$20,395 during the remainder of 2022, \$34,794 in 2023, \$30,343 in 2024, \$18,002 in

2025 and \$872 in 2026. In addition, the Company has outstanding performance-based stock options and restricted stock unit awards for which no compensation expense is recognized until “performance” has occurred and the award vests.

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. Commencing March 1, 2011, 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 June 30, 2022, 85% of these grants have vested.

In January 2022, the Company issued 221 performance-based restricted stock unit awards. Contingent upon successful achievement of specific commercial or operational objectives in 2022, the awards become 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.

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:

	Awards Available	Options Outstanding	Weighted Average Exercise Price
Balance December 31, 2021	3,651	28,685	\$ 7.90
Plan amendment	8,000	—	—
Restricted stock unit awards granted	(663)	—	—
Restricted stock unit awards cancelled	52	—	—
Stock option awards granted	(776)	776	11.10
Stock option awards exercised	—	(1,086)	5.72
Stock option awards cancelled	472	(472)	10.67
Balance June 30, 2022	10,736	27,903	\$ 8.03

For stock option awards granted under the Incentive Plan during the first six months of 2022 and 2021, 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 six months of 2022 and 2021 was \$7.78 and \$7.84, 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 units 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 six months of 2022 and 2021 was \$13.25 and \$10.17, 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:

	Awards Available	Options Outstanding	Weighted Average Exercise Price
Balance December 31, 2021	900	4,408	\$ 6.20
Restricted stock unit awards granted	(203)	—	—
Restricted stock unit awards cancelled	2	—	—
Stock option awards granted	(697)	697	14.75
Stock option awards exercised	—	(55)	3.12
Stock option awards cancelled	130	(130)	8.18
Balance June 30, 2022	<u>132</u>	<u>4,920</u>	<u>\$ 7.43</u>

For stock option awards granted under the Inducement Plan during the first six months of 2022 and 2021, 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 six months of 2022 and 2021 was \$10.28 and \$5.86, 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 units 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 six months of 2022 was \$13.88. The fair value of the restricted stock unit awards is amortized to expense over the vesting periods using a straight-line expense attribution method. No restricted stock unit awards were granted under the Inducement Plan during the first six months of 2021.

The following table summarizes the key assumptions used by the Company to value the stock option awards granted under all plans during the first six months of 2022 and 2021, respectively. The expected life is based on the average of the assumption that all outstanding stock option awards will be exercised at full vesting and the assumption that all outstanding stock option awards will 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. 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 Plans**

	2022	2021
Expected Life in Years	5.5	5.5
Expected Volatility	84.1 %	84.2 %
Expected Dividend Yield	0.0 %	0.0 %
Risk-Free Interest Rate	2.5 %	0.8 %

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 5,937 shares remain available for purchase as of June 30, 2022. 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 3 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 six months ended June 30, 2022, and 2021, the Company issued 115 and 193 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

National Institute of Allergy and Infectious Diseases. In September 2013, NIAID/HHS contracted with the Company for the development of galidesivir as a treatment for Marburg virus disease and subsequently, Yellow Fever and Ebola virus disease. On September 15, 2021, the Company entered into an amendment to pay for certain additional costs, including additional manufacturing development costs and overhead, and to change the total value of the contract, as amended, to \$47,315 from \$45,931. This is the commencement of closing out the contract. All options under the contract have been awarded.

In August 2020, NIAID/HHS awarded the Company a contract, with potential aggregate funding of up to \$43,908 if all contract options are exercised, to manufacture and evaluate the safety, efficacy and tolerability of galidesivir. NIAID/HHS made an initial award of \$6,326 to the Company under this contract.

Biomedical Advanced Research and Development Authority. In March 2015, BARDA/HHS awarded the Company a contract for the continued development of galidesivir as a potential treatment for diseases caused by RNA pathogens, including filoviruses. This BARDA/HHS contract includes a base contract of \$16,265 to support galidesivir drug manufacturing, as well as \$22,855 in additional development options that can be exercised by the government, bringing the potential value of the contract to \$39,120. As of June 30, 2022, a total of \$20,574 has been awarded under exercised options within this contract. The most recent development option was completed as of June 30, 2022.

The contracts with NIAID/HHS and BARDA/HHS are cost-plus-fixed-fee contracts. The Company is entitled to receive reimbursement for all costs incurred in accordance with the contract provisions that are related to the development of galidesivir plus a fixed fee, or profit. BARDA/HHS and NIAID/HHS will make periodic assessments of progress, and the continuation of the contracts is based on the Company’s performance, the timeliness and quality of deliverables, and other factors. The government has rights under certain contract clauses to terminate these contracts. These contracts are terminable by the government at any time for breach or without cause.

U.S. Department of Health and Human Services. In September 2018, HHS awarded the Company a \$34,660 contract for the procurement of up to 50,000 doses of RAPIVAB (peramivir injection) over a five-year period. As of June 30, 2022, the Company has delivered a total of 29,980 RAPIVAB doses of the 50,000 RAPIVAB doses available under the contract with HHS for a total of \$20,782.

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.

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 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.

Albert Einstein College of Medicine of Yeshiva University and Industrial Research, Ltd. In June 2000, the Company licensed a series of potent inhibitors of Purine Nucleoside Phosphorylase from AECOM and IRL (together, the "Licensors"). The lead product candidate from this collaboration is forodesine. The Company has obtained worldwide exclusive rights to develop and ultimately distribute this, or any other, product candidate that might arise from research on these inhibitors. The Company has the option to expand the agreement to include other inventions in the field made by the investigators or employees of the Licensors. Under this agreement, as amended and restated, the Company has agreed to use commercially reasonable efforts to develop these drugs, to pay certain milestone payments for each licensed product (which range in the aggregate from \$1,400 to almost \$4,000 per indication) for future development and single digit royalties on net sales of any resulting product made by the Company, and to share a portion of future payments received from other third-party partners, if any. In addition, the Company has agreed to pay annual license fees, which can range from \$150 to \$500, that are creditable against actual royalties and other payments due to the Licensors. The Licensors have also granted the Company an exclusive worldwide license of galidesivir for any antiviral use.

The University of Alabama at Birmingham. The Company currently has agreements with UAB for influenza neuraminidase and complement inhibitors. Under the terms of these agreements, UAB performed specific research for the Company in return for research payments and license fees. UAB has granted the Company certain rights to any discoveries in these areas resulting from research developed by UAB or jointly developed by UAB with the Company. The Company has agreed to pay single digit royalties on sales of any resulting product and to share in future payments received from other third-party partners. The Company has completed the research under the UAB agreements. These two agreements each have an initial 25-year term, are automatically renewable for five-year terms throughout the life of the last patent and are terminable by the Company upon three months' notice and by UAB under certain circumstances. Upon termination, both parties shall cease using the other parties' proprietary and confidential information and materials, the parties shall jointly own joint inventions and UAB shall resume full ownership of all UAB licensed products. There is currently no activity between the Company and UAB on these agreements, but when the Company licenses this technology, such as in the case of the Shionogi and Green Cross collaborations, or commercializes products related to these programs, the Company will owe sublicense fees or royalties on amounts received.

Note 12 — Subsequent Event

Having achieved all required revenue-based milestones, the Company exercised its option to draw upon the additional funding available under the 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 a 1% commitment fee, resulting in net proceeds to the Company of \$74,250. The Term B Loan and the Term C Loan are subject to the same provisions under the Credit Agreement, including the maturity date of December 7, 2025.

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 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 commercial-stage biotechnology company that discovers novel, oral, small-molecule medicines. We focus on oral treatments for rare diseases in which significant unmet medical needs exist and an enzyme plays the key role in the biological pathway of the disease. We integrate 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. 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 built out our U.S. commercial infrastructure in 2020 to support the launch of ORLADEYO in the United States and are continuing to build our commercial infrastructure to support additional launches in Europe and elsewhere. Based on proprietary analyses of HAE prevalence and market research studies with HAE patients, physicians, and payors in the United States and Europe, and our first full year of experience with the ORLADEYO launch in 2021, we anticipate the commercial market for ORLADEYO has the potential to reach no less than \$250 million in net ORLADEYO revenues in 2022 and 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 commercialization efforts, methods, and strategies for our products or technologies will succeed, and our future revenue generation is uncertain” in Part II, Item 1A of this report for further discussion of these risks.

Revenue from sales of ORLADEYO for the three and six months ended June 30, 2022 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 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.

BCX9930. BCX9930 is a novel, oral, potent, and selective small molecule inhibitor of Factor D discovered by us and in clinical development for the treatment of complement-mediated diseases. Based on the safety and proof-of-concept data generated in patients with paroxysmal nocturnal hemoglobinuria (“PNH”), the program has advanced to pivotal studies of oral BCX9930 in PNH and to a proof-of-concept trial of oral BCX9930 in three renal complement-mediated diseases, C3 glomerulopathy (“C3G”), IgA nephropathy (“IgAN”), and primary membranous nephropathy (“PMN”). We are also actively evaluating potential clinical development opportunities across a broad set of additional indications. The goal of the program is to develop oral BCX9930 as a monotherapy for complement-mediated diseases. Refer to the “Recent Developments” section below for updates on the BCX9930 program, including with respect to our plans to resume enrollment of new patients in clinical trials with BCX9930 following our announcement on August 4, 2022 that the FDA has lifted the partial clinical hold on the program. Refer also to “Risk Factors—Risks Relating to Drug Development and Commercialization—Our success depends upon our ability to advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive regulatory approval for the commercial sale of our

product candidates” in Part II, Item 1A of this report for a discussion of certain risks and uncertainties associated with our BCX9930 program. There can be no assurance that our development plan for the program will succeed.

Galidesivir. Galidesivir is a broad-spectrum antiviral (“BSAV”) that has been shown to be active against more than 20 RNA viruses in nine different families. The objective of our BSAV program is to develop galidesivir as a broad-spectrum therapeutic for viruses that pose a threat to national health and security. The primary focus of the program is treatment of hemorrhagic fever viruses. The galidesivir program has been substantially funded with federal funds from the National Institute of Allergy and Infectious Diseases within the U.S. Department of Health and Human Services (“NIAID/HHS”) and by the Biomedical Advanced Research and Development Authority within the HHS (“BARDA/HHS”).

BCX9250. The goal of our activin receptor-like kinase-2 inhibitor program is to discover and develop orally administered kinase inhibitor drug candidates that are able to slow or prevent the irregular formation of bone outside the normal skeleton, also known as heterotopic ossification (“HO”), that affects patients with fibrodysplasia ossificans progressiva (“FOP”). In a phase 1 clinical trial in healthy volunteers, BCX9250 was safe and well tolerated at all doses studied, with linear and dose-proportional exposure supporting once-daily dosing.

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).

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, the resources dedicated to our products and product candidates by us and our collaborative partners, and ongoing discussions with government agencies regarding contract awards for development and procurement, 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 (and whether these expenses are reimbursable under government contracts), 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 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 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 Policies” at the end of this “Management’s Discussion and Analysis of Financial Condition and Results of Operations” 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

COVID-19

The outbreak of COVID-19 has severely impacted global economic activity and caused significant volatility in financial markets. To date, our financial condition, results of operations, and liquidity have not been materially impacted by the direct effects of the COVID-19 pandemic. Please refer to “Risk Factors—Risks Relating to Our Business—Risks Relating to COVID-19” in Part II, Item 1A of this report for a discussion of COVID-19 risks as they relate to our business. We are continuing to monitor developments with respect to the COVID-19 pandemic and to make adjustments as needed to assist in protecting the safety of our employees and communities while continuing our business activities. Our remote working arrangements continue to be flexible where it is both practical and possible for the business. Business-related travel has resumed, and we will continue to monitor developments with respect to COVID-19 going forward. To date, implementation of specific COVID-19 measures has not required material expenditures or significantly impacted our ability to operate our business or our internal control over financial reporting and disclosure controls and procedures. We continue to monitor the potential impacts of COVID-19 on our operations and those of our partners, suppliers, customers, and regulators.

ORLADEYO® (berotralstat)

On June 6, 2022, we announced that Health Canada has approved ORLADEYO for the routine prevention of recurrent HAE attacks in patients 12 years and older in Canada.

On June 7, 2022, we announced that Swissmedic has granted marketing authorization for ORLADEYO for the routine prevention of recurrent HAE attacks in patients 12 years and older in Switzerland.

On June 9, 2022, we announced that the Company has entered into an exclusive collaboration with Pint Pharma GmbH to register and promote ORLADEYO in the pan-Latin America region. Under the terms of our agreement, Pint Pharma will be responsible for obtaining and maintaining all marketing authorizations and for commercializing ORLADEYO in the region.

On July 1, 2022, we announced new long-term efficacy and safety data from the APeX-2 and APeX-S clinical trials evaluating ORLADEYO for the prophylactic treatment of HAE showing sustained reductions in attack rates and improvement in quality of life among patients living with HAE, as well as improved management of symptoms after switching to ORLADEYO from an injectable long-term prophylactic treatment.

On August 4, 2022, we announced that pricing for ORLADEYO was finalized in Germany, France, and Switzerland in the second quarter of 2022.

Complement-Mediated Diseases

On April 8, 2022, we announced that we had voluntarily paused enrollment in clinical trials with BCX9930 while we investigated observed elevations in serum creatinine seen in some patients at the 500 mg twice-daily dose. The FDA subsequently placed the program on a partial clinical hold. Patients already enrolled in the trials who were receiving clinical benefit from BCX9930 treatment, and had no other available treatment options, were able to continue on therapy.

On August 4, 2022, we announced that the FDA has lifted its partial clinical hold on the BCX9930 program. We will resume enrollment in global clinical trials under revised protocols at a reduced dose of 400 mg twice-daily of BCX9930. This includes the REDEEM-1 and REDEEM-2 pivotal trials in patients with PNH and the RENEW proof-of-concept trial in patients with C3G, IgAN, and PMN. Clinical evidence and recent laboratory studies have informed our hypothesis that crystals form in the kidneys of some patients. We believe that lowering the dose to 400 mg and ensuring adequate hydration will dilute the concentration of drug in the urine below the threshold where crystals can form. Our goal is to find a safe and effective dose for BCX9930. We expect this can be accomplished in a reasonable timeframe after resuming enrollment, in a relatively small number of patients given the rate and timing of the serum creatinine rises in patients prior to the enrollment pause. If successful, we plan to invest more significantly in BCX9930 to tap the full potential of reaching many patients suffering from a number of alternative pathway diseases, and, if not successful, we will stop investment in BCX9930 and move on to other molecules in the pipeline.

Fibrodysplasia Ossificans Progressiva

On April 27, 2022, we announced that the European Medicines Agency (“EMA”) has granted access to the Priority Medicines (“PRIME”) scheme for BCX9250. PRIME is a program launched by the EMA to enhance support for the development of medicines that target an unmet medical need. This voluntary program is based on enhanced interaction and early dialogue with developers of promising medicines and is designed to optimize development plans and speed up evaluation so these medicines can potentially reach patients earlier. According to the EMA, developers of medicines that are eligible for PRIME can expect additional opportunities for scientific advice and be eligible for accelerated assessment at the time of application for a marketing authorization. On August 4, 2022, we announced that the EMA also has recently granted Orphan Drug designation for BCX9250.

On June 8, 2022, we announced that the FDA has granted Fast Track designation for BCX9250 for the prevention of HO in patients with FOP. According to the FDA, the purpose of the Fast Track designation is to get important new drugs to patients earlier by facilitating the development, and expediting the review, of drugs to treat serious conditions and fill an unmet medical need. Companies that receive this designation are eligible for benefits including more frequent meetings with and written communication from the FDA, eligibility for accelerated approval and priority review, if relevant criteria are met, and rolling review of the new drug application (“NDA”).

Results of Operations (three months ended June 30, 2022 compared to the three months ended June 30, 2021)

For the three months ended June 30, 2022, total revenues were \$65.5 million as compared to \$50.0 million for the three months ended June 30, 2021. The increase was primarily due to ORLADEYO net revenue, including royalties, of \$65.2 million, an increase of \$36.7 million. This increase was partially offset by a decrease in RAPIVAB revenue of \$4.4 million primarily due to a stockpiling sale to HHS of \$4.6 million in the prior year as well as a decrease in peramivir revenue of \$0.4 million on a sale to our partner in the prior year. Additionally, a \$15.0 million milestone payment related to the NHI approval of ORLADEYO in Japan was recognized in the prior year. Contract revenue for the three months ended June 30, 2022 decreased \$1.3 million compared to the same period in the prior year as existing contracts continue to close out.

Cost of product sales for the three months ended June 30, 2022 and 2021 was \$0.2 million and \$0.3 million, respectively. The cost of product sales in 2021 was primarily associated with the peramivir product sales to our partners whereas the cost of product sales in 2022 relates primarily to sales of ORLADEYO.

Research and development (“R&D”) expenses increased to \$62.0 million for the three months ended June 30, 2022 from \$52.9 million for the three months ended June 30, 2021, primarily due to increased investment in the development of our Factor D program, including BCX9930, our FOP program, as well as other research, preclinical and development costs.

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.

	Three Months Ended June 30,	
	2022	2021
R&D expenses by program:		
Factor D program	\$ 38,722	\$ 31,362
Berotrastat	6,246	9,729
FOP	5,023	654
Galidesivir	390	2,823
Peramivir	156	303
Other research, preclinical and development costs	11,453	8,002
Total R&D expenses	\$ 61,990	\$ 52,873

Selling, general and administrative (“SG&A”) expenses for the three months ended June 30, 2022 were \$38.0 million compared to \$26.3 million in the three months ended June 30, 2021. The increase was primarily due to increased investment to support the commercial launch of ORLADEYO and the expansion of international operations.

Interest expense for the three months ended June 30, 2022 was \$24.0 million compared to \$13.5 million for the three months ended June 30, 2021. The increase in interest expense was primarily associated with the sale of certain royalty payments under the 2021 RPI Royalty Purchase Agreement and the OMERS Royalty Purchase Agreement, which were entered into in November 2021. The nature of the royalty sales requires that we recognize a liability (the “Royalty Financing Obligations”) for the future sale of royalties under these agreements. These liabilities are amortized using the effective interest rate method, resulting in the recognition of non-cash interest expense over the estimated term of the Royalty Purchase Agreements (as defined in “Note 6—Royalty Monetizations—ORLADEYO and Factor D Inhibitors” in the Notes to Consolidated Financial Statements in Part I, Item 1 of this report).

Interest expense for the three months ended June 30, 2022 included \$19.7 million of non-cash interest expense due to the amortization of interest associated with the Royalty Financing Obligations and \$4.3 million of interest expense, net of deferred financing amortization, associated with the Term A Loan under the Credit Agreement. Interest expense for the three months ended June 30, 2021 included \$8.1 million of non-cash interest expense due to the amortization of interest associated with the Royalty Financing Obligation under the 2020 RPI Royalty Purchase Agreement and \$3.8 million of interest expense, net of deferred financing amortization, associated with the Term A Loan under the Credit Agreement. Additionally, we recognized \$1.6 million in interest expense on the non-recourse Pharma Notes issued in March 2011, which were written-off in December 2021.

For the three months ended June 30, 2022, other income of \$0.7 million was comprised of interest income of \$0.6 million and net foreign currency gains of \$0.1 million. Other expense for the three months ended June 30, 2021 was primarily related to net foreign currency losses of \$0.1 million.

Results of Operations (six months ended June 30, 2022 compared to the six months ended June 30, 2021)

For the six months ended June 30, 2022, total revenues were \$115.5 million as compared to \$69.0 million for the six months ended June 30, 2021. The increase was primarily due to ORLADEYO net revenue, including royalties, of \$114.9 million, an increase of \$75.5 million. Additionally, other royalties increased \$0.9 million for the six months ended June 30, 2022 as compared to the six months ended June 30, 2021. These increases were partially offset by a decrease in peramivir revenue of \$7.3 million on sales to our partners in the prior year as well as a decrease in RAPIVAB revenue of \$4.4 million primarily due to a stockpiling sale to HHS of \$4.6 million in the prior year. Additionally, a \$15.0 million milestone payment related to the NHI approval of ORLADEYO in Japan was recognized in the prior year. Contract revenue for the six months ended June 30, 2022 decreased \$3.4 million compared to the same period in the prior year as existing contracts continue to close out.

Cost of product sales for the six months ended June 30, 2022 and 2021 was \$0.5 million and \$6.2 million, respectively. The cost of product sales in 2021 was primarily associated with the peramivir product sales to our partners whereas the cost of product sales in 2022 relates primarily to sales of ORLADEYO.

R&D expenses increased to \$127.4 million for the six months ended June 30, 2022 from \$95.3 million for the six months ended June 30, 2021, primarily due to increased investment in the development of our Factor D program, including BCX9930, our FOP program, as well as other research, preclinical and development costs.

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.

	Six Months Ended June 30,	
	2022	2021
R&D expenses by program:		
Factor D program	\$ 80,691	\$ 57,331
Berotrastat	13,689	17,651
FOP	7,692	1,021
Galidesivir	920	3,919
Peramivir	521	554
Other research, preclinical and development costs	23,837	14,832
Total R&D expenses	\$ 127,350	\$ 95,308

SG&A expenses for the six months ended June 30, 2022 were \$72.3 million compared to \$48.4 million in the six months ended June 30, 2021. The increase was primarily due to increased investment to support the commercial launch of ORLADEYO and the expansion of international operations.

Interest expense for the six months ended June 30, 2022 was \$47.9 million compared to \$26.4 million for the six months ended June 30, 2021. The increase in interest expense was primarily associated with the sale of certain royalty payments under the 2021 RPI Royalty Purchase Agreement and the OMERS Royalty Purchase Agreement, which were entered into in November 2021. The nature of the royalty sales requires that we recognize the Royalty Financing Obligations for the future sale of royalties under these agreements. These liabilities are amortized using the effective interest rate method, resulting in the recognition of non-cash interest expense over the estimated term of the Royalty Purchase Agreements.

Interest expense for the six months ended June 30, 2022 included \$39.4 million of non-cash interest expense due to the amortization of interest associated with the Royalty Financing Obligations and \$8.4 million of interest expense, net of deferred financing amortization, associated with the Term A Loan under the Credit Agreement. Interest expense for the six months ended June 30, 2021 included \$15.6 million of non-cash interest expense due to the amortization of interest associated with the Royalty Financing Obligation under the 2020 RPI Royalty Purchase Agreement and \$7.5 million of interest expense, net of deferred financing amortization, associated with the Term A Loan under the Credit Agreement. Additionally, we recognized \$3.2 million in interest expense on the non-recourse Pharma Notes issued in March 2011, which were written-off in December 2021.

For the six months ended June 30, 2022, other income of \$0.6 million was comprised of interest income of \$0.7 million, partially offset by net foreign currency losses of \$0.1 million. Other expense for the six months ended June 30, 2021 was primarily related to net foreign currency losses of \$0.1 million.

Liquidity and Capital Resources

Our operations have principally been funded through public offerings and private placements of equity securities; cash from collaborative and other research and development agreements, including U.S. Government contracts for RAPIVAB and galidesivir; to a lesser extent, the Pharma Notes financing; our credit facilities; and more recently, the Royalty Sales and revenues from ORLADEYO. To date, we have been awarded a BARDA/HHS RAPIVAB development contract totaling \$234.8 million, which expired on June 30, 2014; a NIAID/HHS galidesivir development contract totaling \$47.3 million, which is in the process of being closed out; a second NIAID/HHS galidesivir development contract with a potential value totaling \$43.9 million, which is ongoing; and a BARDA/HHS galidesivir development contract with a potential value totaling \$39.1 million, which is also ongoing. The total amount of funding obligated under awarded options under the active NIAID/HHS and BARDA/HHS galidesivir contracts is \$53.6 million and \$20.6 million, respectively. 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.

Our Credit Agreement with Athyrium provides for three term loans. We received the proceeds from the \$125.0 million Term A Loan in December 2020. The Term B Loan and the Term C Loan were both funded in the respective principal amounts of \$25.0 million and \$50.0 million on July 29, 2022. The maturity date of the Credit Agreement is December 7, 2025. See “Note 7—Debt—Credit Agreement” in the Notes to Consolidated Financial Statements in Part I, Item 1 of this report for additional information about the Credit Agreement.

As of June 30, 2022, we had net working capital of \$399.5 million, a decrease of approximately \$62.9 million from \$462.4 million at December 31, 2021. The decrease in working capital was primarily due to normal operating expenses associated with the development of our product candidates and commercialization of ORLADEYO. Our principal sources of liquidity at June 30, 2022 were approximately \$272.6 million in cash and cash equivalents and approximately \$144.9 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 in both the short-term and long-term principally from the following:

- lease, royalty, or loan financing and future public or private equity and/or debt financing;
- our existing capital resources and interest earned on that capital;
- revenues from product sales;
- payments under existing, and executing new, contracts with the U.S. Government; 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 and timing of funding we receive from existing U.S. Government contracts for galidesivir, the amount of funding or assistance, if any, we receive from new U.S. Government contracts or other 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 expenses, revenues, 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 2022 expenses will exceed our 2022 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 be largely determined by the success of operations in regard to the successful commercialization of our products and the future progression of our product candidates. We regularly evaluate other opportunities to fund future operations, including: (1) securing or increasing U.S. Government funding of our programs, including obtaining procurement contracts; (2) out-licensing rights to certain of our products or product candidates, pursuant to which we would receive cash milestone payments; (3) raising additional capital through equity or debt financings or from other sources, including royalty or other monetization transactions; (4) obtaining additional product candidate regulatory approvals, which would generate revenue, milestone payments and cash flow; (5) reducing spending on one or more research and development programs, including by discontinuing development; and/or (6) restructuring operations to change our overhead structure. 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 in both the short-term and long-term 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 perform under our government contracts and to receive reimbursement and stockpiling procurement contracts;
- the magnitude of work under our government contracts;
- the progress and magnitude of our research, drug discovery and development programs;
- changes in existing collaborative relationships or government contracts;
- our ability to establish additional collaborative relationships with academic institutions, biotechnology or pharmaceutical companies and governmental agencies or other third parties;
- the extent to which our partners, including governmental agencies, will share in the costs associated with the development of our programs or run the development programs themselves;
- our ability to negotiate favorable development and marketing strategic alliances for certain products and product candidates;

- any decision to build or expand internal development and commercial capabilities;
- the scope and results of preclinical studies and clinical trials to identify and develop product candidates;
- our ability to engage sites and enroll subjects in our clinical trials;
- the scope of manufacturing of our products to support our commercial operations and of our product candidates to support our preclinical research and clinical trials;
- increases in personnel and related costs to support the development and commercialization of our products and product candidates;
- the scope of manufacturing of our drug substance and product candidates required for future NDA filings;
- competitive and technological advances;
- the time and costs involved in obtaining regulatory approvals;
- post-approval commitments for ORLADEYO, 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 expect that we will be required to raise additional capital to complete the development and commercialization of our current 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, whether through additional sales of equity or debt securities, collaborative or other arrangements with corporate partners or from other sources, including governmental agencies in general and existing government contracts specifically, may not be available when needed 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 the currently outstanding common stock, could have the effect of diluting or adversely affecting the holdings or rights of our existing stockholders. In addition, collaborative arrangements may require us to transfer certain material rights to such corporate partners. Insufficient funds may require us to delay, scale back or eliminate certain of our research and development programs. Our future working capital requirements, including the need for additional working capital, will be largely determined by the advancement of our portfolio of product candidates and the commercialization of ORLADEYO, as well as the rate of reimbursement by U.S. Government agencies of our galidesivir expenses and any future decisions regarding the future of the RAPIVAB and galidesivir programs, including those relating to stockpiling procurement. 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.

The restrictive covenants contained in our Credit Agreement could cause us to be unable to pursue business opportunities that we or our stockholders may consider beneficial without the lender's permission or without repaying all obligations outstanding under the Credit Agreement. These covenants limit our ability to, among other things, grant certain types of liens on our assets; make certain investments; incur or assume certain debt; engage in certain mergers, acquisitions, and similar transactions; dispose of assets; license certain property; distribute dividends; make certain restricted payments; change the nature of our business; engage in transactions with affiliates and insiders; prepay other indebtedness; or engage in sale and leaseback transactions. A breach of any of these covenants could result in an event of default under the Credit Agreement. As of June 30, 2022, we were in compliance with the covenants under the Credit Agreement.

Financial Outlook for 2022

Based on the strength of the ORLADEYO launch through the first half of 2022, and the continued steady growth from new patient demand we expect for the remainder of the year, we expect full year 2022 net ORLADEYO revenue to be between \$255 million and \$265 million.

Based on the reduced spending on the BCX9930 program in the first half of the year, and lower than projected spending on the program for the remainder of the year, we now expect operating expenses for full year 2022, not including non-cash stock compensation, to be between \$390 million and \$400 million.

Critical Accounting Policies

We have established various accounting policies that govern the application of U.S. GAAP, which were utilized in the preparation of our 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 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 the Company 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 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. 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 purchase price with 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 Research and Development Arrangements and Royalties

We recognize revenue when we satisfy a performance obligation by transferring promised goods or services to a customer. Revenue is measured at the transaction price that is based on the amount of consideration that we expect to receive in exchange for transferring the promised goods or services to the customer. The transaction price includes estimates of variable consideration to the extent it is probable that a significant reversal of revenue recognized will not occur.

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 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 includes all inventory costs prior to packaging and labeling, including raw material, active product ingredient, and 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.

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. This accrual process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed and estimating the level of service performed and the associated cost 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 based on the facts and circumstances known to us. 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.

Contract Balances

The timing of revenue recognition, billings and cash collections results in billed accounts receivable, unbilled receivables (contract assets) and deferred revenue and billings in excess of revenue recognized (contract liabilities) on the Consolidated Balance Sheets.

Contract assets. Our long-term contracts are billed as work progresses in accordance with the contract terms and conditions, either at periodic intervals or upon achievement of certain milestones. Often this results in billing occurring subsequent to revenue recognition, resulting in contract assets. Contract assets are generally classified as current assets in the Consolidated Balance Sheets.

Contract liabilities. We often receive cash payments from customers in advance of our performance, resulting in contract liabilities. These contract liabilities are classified as either current or long-term in the Consolidated Balance Sheets based on the timing of when we expect to recognize the revenue.

Contract Costs

We may incur direct and indirect costs associated with obtaining a contract. Incremental contract costs that we expect to recover are capitalized and amortized over the expected term of the contract. Non-incremental contract costs and costs that we expect to recover are expensed as incurred.

Research and Development Expenses

Our research and development costs are charged to expense when incurred. 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. 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, such as Albert Einstein College of Medicine of Yeshiva University (“AECOM”), Industrial Research, Ltd. (“IRL”), and the University of Alabama (“UAB”), 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 sub-license payments paid to our academic partners upon receipt of consideration from various commercial partners, and other consideration 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. 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 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 for which no compensation expense is recognized until “performance” has 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, BCX9930, and another earlier stage Factor D inhibitor. 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.

Tax

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 U.S. state income taxes and foreign income taxes as a result of increased sales of ORLADEYO and increased nexus in multiple states and foreign jurisdictions where historically we had no presence.

Recent Accounting Pronouncements

“Note 1—Significant Accounting Policies and Concentrations of Risk” in the Notes to 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 Credit Agreement. The Term Loans under the Credit Agreement bears interest each quarter at a rate equal to the three-month LIBOR rate, which is capped to be no less than 1.75% and no more than 3.50% (“LIBOR”), plus 8.25% or, for each quarterly interest period in which a PIK Interest Payment is made, LIBOR plus 10.25%. Accordingly, increases in interest rates could increase the associated interest payments that we are required to make on the Term Loans. As of June 30, 2022, interest was accrued at 12.17% on the \$125.0 million Term A Loan under the Credit Agreement.

The three-month LIBOR was 2.3125% as of June 28, 2022, the LIBOR measurement date for the three-month interest period beginning July 1, 2022. Accordingly, this LIBOR rate plus 10.25% will be used to record interest expense for the three-month interest period beginning July 1, 2022.

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, and as a result, we have not experienced significant foreign currency transaction gains and losses to date. 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 six months ended June 30, 2022, 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.

We do not anticipate that foreign currency transaction gains or losses will be significant at our current level of operations. However, transaction gains or losses may become significant in the future as we continue to expand our operations internationally. We have not engaged in foreign currency hedging during the six months ended June 30, 2022; 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 maintain a set of disclosure controls and procedures that are designed to ensure that information relating to the Company required to be disclosed in our periodic filings under the Exchange Act is recorded, processed, summarized and reported in a timely manner under the Exchange Act. We carried out an evaluation, under the supervision and with the participation of management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures. Based upon that evaluation, the Chief Executive Officer and Chief Financial Officer concluded that, as of June 30, 2022, the Company's disclosure controls and procedures are effective to ensure that information required to be disclosed by the Company in the reports filed or submitted by it under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and include controls and procedures designed to ensure that information required to be disclosed by the Company in

such reports is accumulated and communicated to the Company's management, including the Chief Executive Officer and Chief Financial Officer of the Company, as appropriate to allow timely decisions regarding required disclosure.

There have been no changes in our internal control over financial reporting that occurred during the quarter ended June 30, 2022 that have materially affected, or are reasonably likely to materially affect, the Company's internal control over financial reporting.

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 (such as Torii), manufacturers, CROs, and others; (2) the conduct of our current and future clinical trials and commercial interactions; and (3) the operations of the FDA, EMA, Japanese Pharmaceuticals and Medical Devices Agency (“PMDA”), and other health and governmental authorities, which could result in delays of reviews and approvals.

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 delays or challenges 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 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.

Although our business operations under the ongoing COVID-19 pandemic continue to evolve, 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. In addition, we are a government contractor, and as such, we are subject to the federal COVID-19 safety protocols, including the vaccine mandate. We cannot accurately predict the impact on operations of our return-to-the-office plan, nor of the federal COVID-19 safety protocols 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 these protocols. These and similar, and

perhaps more severe, disruptions in 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 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 resulting from the spread of COVID-19 and related responses could materially affect our business and the value of our common stock.

The global pandemic of COVID-19 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, on the healthcare system, or on 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 approval, 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 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, BCX9930, and another earlier stage Factor D inhibitor 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 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 2022 expenses will exceed our 2022 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.

Our liquidity needs will be largely determined by the success of operations in regard to the commercialization of our products 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 through equity and/or debt financings, and commercializing our approved products. We regularly evaluate other opportunities to fund operations including: (1) securing or increasing U.S. Government funding of our programs, including obtaining additional and delivering on procurement contracts; (2) out-licensing rights to certain of our products or product candidates, pursuant to which we would receive cash milestone payments and/or royalties; (3) raising additional capital through equity or debt financings or from other sources, including royalty or other monetization transactions; (4) obtaining additional product candidate regulatory approvals, which would generate revenue, milestone payments and cash flow; (5) reducing spending on research and development programs, including by discontinuing and suspending development; and/or (6) restructuring operations to change our overhead structure.

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, 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 advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive regulatory approval for the commercial sale of our product candidates.

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, 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, and 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. Failure to achieve any of these endpoints in any of our programs, including our Factor D program (inclusive of BCX9930), BCX9250, galidesivir, and our other rare disease product candidates could result in delays in or modifications to our trials or require the performance of additional unplanned trials. If any of our product candidates is associated with adverse events or undesirable side effects or has properties that are unexpected, we may need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit 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 and delays in the development plans for our product candidates, significant unexpected costs, or the termination of programs. For example, in April 2022, we voluntarily paused enrollment of our BCX9930 clinical trials due to observed elevations in serum creatinine seen in some patients. The FDA subsequently placed the program on a partial clinical hold. On August 4, 2022, we announced that the FDA has lifted its partial clinical hold with respect to BCX9930 and we plan to resume enrollment under revised protocols which include a number of additional requirements and restrictions (e.g., a dose reduction, recommended hydration, and increased monitoring). The elevated serum creatinine signal and revisions to the BCX9930 development program create additional risk to the successful development of BCX9930, and the timelines for the BCX9930 program are uncertain. Specifically, the revised BCX9930 studies may not address the elevated serum creatinine issue, we may not be able to execute the BCX9930 clinical studies in a timely fashion or at all, we may not be able to recruit enough patients for the clinical studies, our hypothesis may not be correct or our data may be inconclusive, the lower dose of BCX9930 may not be effective, and the additional requirements and restrictions that have been imposed, or that may be imposed in the future, by regulatory authorities and others may impede development. In addition, the development plans for our product candidates, including our clinical trials (including for BCX9930 and BCX9250), 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 may decide to discontinue development of product candidates for various reasons, including, but not limited to, 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 pre-clinical 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 successfully complete the clinical development process 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 protocol 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 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 pre-clinical 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 or 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, may not receive regulatory approval for the product candidates, in which case we would be unable to generate any revenues from product sales or licensing arrangements, or 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, 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 in connection with our clinical trials, 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 are 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 obtain regulatory approval for our product candidates, including BCX9930, BCX9250, and galidesivir; our ability to maintain regulatory approval for, successfully commercialize, and achieve market acceptance of our products, including ORLADEYO; our ability to raise additional capital; the amount of funding we receive from partnerships with third parties for the development and commercialization of our products and product candidates (including our collaborations with Torii, BARDA/HHS, and NIAID/HHS); the commercial success of our products achieved by our partners; the amount or profitability of any orders for peramivir or galidesivir by any government agency or other party; 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 development programs, and commercialize our current products and product candidates, we will be required to raise additional capital. In addition to seeking strategic partnerships, transactions and government funding, we may access the equity or debt markets, incur additional borrowings, 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, including corporate partners such as Torii and governmental agencies such as BARDA/HHS or NIAID/HHS, or from other sources, may not be available when needed 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 our Credit Agreement. In addition, collaborative arrangements may require us to transfer certain material rights to such 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.

Our ability to raise additional capital 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 BCX9250 for the treatment of FOP, our Factor D program (including BCX9930) for diseases of the complement system, our broad-spectrum antiviral program, including galidesivir, and other

rare disease product candidates, 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, may restrict our future flexibility to raise capital when such needs arise. Furthermore, we have exposure to many different industries, financing partners and counterparties, including commercial banks, investment banks and partners (which include investors, licensing partners, and the U.S. Government), 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, and the conflict in Ukraine. 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 could 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 governmental approval for our product candidates or maintain governmental approval 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 approval 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 advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive regulatory approval for the commercial sale of our product candidates," we or our partners have experienced, and may again in the future experience, any number of unfavorable outcomes during or as a result of pre-clinical 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. These requirements are wide ranging and govern, among other things:

- adverse drug experience reporting regulations;
- product promotion;
- product manufacturing, including good manufacturing practice requirements; and
- product changes or modifications.

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. For instance, although BCX9930 for PNH has received Fast Track and Orphan Drug designations from the FDA and BCX9250 has received Fast Track designation from the FDA and has been granted Orphan Drug designation and access to PRIME by the EMA, we may not experience a faster development, review or approval process compared to the conventional process in the relevant jurisdictions. We may not be able to obtain or maintain these designations for BCX9930, BCX9250, or other 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 were not previously identified, or fails to achieve market acceptance within the medical community.

If, after obtaining regulatory approval of a product, we or others discover that it is less effective than previously believed or causes undesirable side effects that were not previously identified, 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 even 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 include 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 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:

- our partners may seek to renegotiate or terminate their relationships with us 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 partner;
- 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 may terminate the Torii Agreement under certain limited circumstances, including upon one year's written notice after the sixth anniversary of the first commercial sale of ORLADEYO in Japan. If the Torii Agreement is terminated in connection with these provisions, we will no longer be entitled to receive any milestone or royalty payments thereunder, which could have a material adverse impact on our business and results of operations.

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 materially 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 and 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 successfully complete clinical trials, or satisfy post-marketing commitments, sufficient to obtain and keep 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 would depend 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 would 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 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, BCX9930, and another earlier stage Factor D inhibitor under the Royalty Purchase Agreements may reduce the profitability of such products.

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

We expect to continue experiencing significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, sales, marketing, and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. For example, we expanded our internal commercial team in 2020 in preparation for the commercial launch of ORLADEYO, and we have continued to add additional headcount throughout 2021 and the first half of 2022. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit, train, and retain additional 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 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, which may be the only vendor we have engaged for a particular product 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, BCX9930, BCX9250, galidesivir, 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, acts of terrorism or war, equipment malfunctions, or raw material shortages. 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 inventories and sales, serious adverse events, and/or product complaints, our business, including our commercial launch 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, is not effectively managed, the continuance of our commercial launch 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 procedure, 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 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.

Commercialization 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 strategy may not be effective;
- 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 or 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 12 years and older, in December 2020. We subsequently received regulatory approvals for ORLADEYO in multiple global 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 and FOP, as well as developing broad spectrum antivirals for use as medical countermeasures. 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. There are licensed therapies for HAE (including Berinert®, Haegarda®, Cinryze®, Kalbitor®, Takhzyro®, Firazyr® (icatibant), generic icatibant, and Ruconest®), therapies for certain complement-mediated diseases such as PNH, aHUS, myasthenia gravis, and neuromyelitis optica spectrum disorder (Soliris®, Ultomiris®, and Empaveli®), products for the prevention or treatment of influenza (seasonal flu vaccines, Tamiflu® (oseltamivir), generic oseltamivir, Relenza®, and Inavir®, favipiravir, and Xofluza®), and a number of additional products in clinical development in these therapeutic areas and for the treatment of FOP. In addition, various government entities throughout the world may offer incentives, grants and contracts to encourage additional investment into preventative and therapeutic agents against viruses such as influenza, coronavirus, Ebola, and others, 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 BCX9930, BCX9250, and galidesivir, 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. Anti-kickback laws generally prohibit a manufacturer from soliciting, offering, receiving, or paying any remuneration to generate business, including the purchase or prescription of a particular drug. Although the specific provisions of these laws vary, their scope is generally broad and there may be no regulations, guidance or court decisions that clarify how the laws apply to particular industry practices. False claims laws prohibit anyone from knowingly and willingly presenting, or causing to be presented, for payment to third party payors (including Medicare and Medicaid) claims for reimbursement or services that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services. The sunshine provisions apply to manufacturers with products reimbursed under certain government programs and require those manufacturers to disclose annually to the federal government certain payments made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as, ownership and investment interests held by physicians (as defined above) and their immediate family members. State laws may also require disclosure of pharmaceutical pricing information and marketing expenditures. 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. In particular, the claims in all promotional materials and activities must be consistent with the FDA approvals for approved products and must be appropriately substantiated and fairly balanced with information on the safety risks and limitations of the products. We are also required to engage in appropriate truthful, non-misleading, and non-promotional scientific exchange concerning our products, and 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, other divisions of the 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 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 and consultants 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 and consultants, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign 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 foreign 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 and develop our products and product candidates, obtain collaborators and raise capital.

The Patient Protection and Affordable Care Act ("PPACA") made extensive changes to the delivery of healthcare in the United States. The PPACA included numerous provisions that affect pharmaceutical companies, some of which became effective immediately and others of which have taken effect over the past several years. For example, the PPACA expanded healthcare coverage to the uninsured through private health insurance reforms and an expansion of Medicaid. The PPACA also imposed substantial costs on pharmaceutical manufacturers, such as an increase in liability for rebates paid to Medicaid, new drug discounts that must be offered to certain enrollees in the Medicare prescription drug benefit, an annual fee imposed on all manufacturers of brand prescription drugs in the United States, and an expansion of an existing program requiring pharmaceutical discounts to certain types of hospitals and federally subsidized clinics. The PPACA also contains cost containment measures that could reduce reimbursement levels for healthcare items and services generally, including pharmaceuticals. It also required reporting and public disclosure of payments and other transfers of value provided by pharmaceutical companies to physicians and teaching hospitals.

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 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, which has resulted in several Congressional inquiries and proposed bills designed to, among other things, reform government program reimbursement methodologies and establish certain government negotiated drug prices. Individual states in the United States have been increasingly active in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. 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 "Risks Relating to Our Business—Risks Relating to International Operations—Our actual or perceived failure to comply with European governmental regulations and other legal obligations related to privacy, data protection and information security could harm our business" in this section for additional discussion of international 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 or secure rights to patents of others, the value of those rights would diminish.

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.

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 or, if we are found to be in violation, could result in contract termination. If the U.S. Government terminates any of its contracts with us for its convenience, or if we default by failing to perform in accordance with the contract schedule and terms, this could have an adverse impact on the programs associated with such funding and could also adversely impact our cash flows and operations.

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.

The U.S. Government may terminate its contracts with us either for its convenience or if we default by failing to perform in accordance with the contract schedule and terms. Termination for convenience provisions generally enable us to recover only our costs incurred or committed, and settlement expenses and profit on the work completed prior to termination. Termination does not permit these recoveries under default provisions. In the event of termination or upon 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. Further, if the U.S. Government terminates its contracts with us for its convenience, or if we default by failing to perform in accordance with the contract schedule and terms, this could have an adverse impact on our cash flows and operations.

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. 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 active 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 and could impact our cash flows under the contracts prospectively. 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 termination of our contracts, 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.

In September 2018, we entered into a contract with the U.S. Government for the procurement of up to 50,000 doses of RAPIVAB (peramivir injection) over a five-year period. In addition, we are working with NIAID/HHS to further develop galidesivir. There can be no assurance that we or our manufacturers will be able to fully meet the demand for such antivirals with respect to these or 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. There can be no assurance that galidesivir will be approved for use in any 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 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 or seek other available remedies. As a result, our development of the respective product candidate or commercialization of the product would cease.

Royalties and milestone payments, if any, from Shionogi under the Shionogi Agreement are required to be used by Royalty Sub to satisfy its obligations under its PhaRMA Notes, and generally will not be available to us for other purposes unless and until Royalty Sub has repaid in full its obligations under the PhaRMA Notes.

In March 2011, our wholly-owned subsidiary, Royalty Sub, issued \$30.0 million in aggregate principal amount of PhaRMA Notes. The PhaRMA Notes are secured principally by (i) certain royalty and milestone payments under 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. As a result of the continuing event of default, 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.

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 in full the outstanding principal amount of the PhaRMA Notes, together with any accrued and unpaid interest, at the December 1, 2020 final maturity date constituted an additional event of default under the PhaRMA Notes. 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. 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 6—Royalty Monetizations—RAPIACTA—Non-Recourse Notes Payable—Debt Extinguishment” in the Notes to Consolidated Financial Statements in Part I, Item 1 of this report for additional information about the write-off.

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.

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. 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 in full the outstanding principal amount of the PhaRMA Notes, together with any accrued and unpaid interest, at the December 1, 2020 final maturity date constituted an additional event of default under the PhaRMA Notes. 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. 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 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 6—Royalty Monetizations—RAPIACTA—Non-Recourse Notes Payable—Debt Extinguishment” in the Notes to Consolidated Financial Statements in Part I, Item 1 of this report for additional information about the write-off.

We have incurred significant indebtedness, which could adversely affect our business. Additionally, our Credit 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.

As of June 30, 2022, we had an outstanding principal balance under our Credit Agreement of \$150.8 million, inclusive of the quarterly PIK Interest Payments. The Term B Loan and the Term C Loan under the Credit Agreement were subsequently funded on July 29, 2022 in the aggregate principal amount of \$75.0 million. We will be required to pay to Athyrium, for the account of the lenders, a make-whole premium plus certain fees set forth in the Credit 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 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 Term Loans under the Credit Agreement, in each case, subject to certain exceptions set forth in the Credit 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 our Credit Agreement are at variable rates;

- 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, our Credit 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, grant certain types of liens on our assets; make certain investments; incur or assume certain debt; engage in certain mergers, acquisitions, and similar transactions; dispose of assets; license certain property; distribute dividends; make certain restricted payments; change the nature of our business; engage in transactions with affiliates and insiders; prepay other indebtedness; or engage in sale and leaseback transactions.

The Credit Agreement also contains certain financial covenants, including a minimum liquidity covenant that requires us to maintain at all times, as applicable, at least \$15.0 million of unrestricted cash and cash equivalents if only the Term A Loan has been drawn; at least \$20.0 million of unrestricted cash and cash equivalents if the Term B Loan has been drawn but the Term C Loan has not been drawn; and at least \$15.0 million (or, in certain circumstances, \$20.0 million) of unrestricted cash and cash equivalents if the Term C Loan has been drawn, subject to certain exceptions. In addition, as a result of drawing upon the Term C Loan, we are required to achieve certain minimum targets for consolidated net revenues from ORLADEYO sales in the United States.

The covenants contained in the Credit 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 Credit Agreement.

A breach of any of these covenants could result in an event of default under the Credit Agreement. An event of default will also occur if, among other things, we fail to pay amounts due under the Credit Agreement, we fail to repay certain other indebtedness having an aggregate principal amount in excess of one percent of our borrowings under the Credit Agreement, 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 Credit Agreement occurs, we experience a change of control, certain negative regulatory events occur, including without limitation the loss of a required permit or a recall of a product, or we fail to make required payments under our Royalty Purchase Agreements. In the case of a continuing event of default under the Credit Agreement, the lenders under the Credit 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 Credit 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 Credit 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. 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;
- natural disasters and political and economic instability, including wars (e.g., the conflict in Ukraine), 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 future international expansion of operations and, consequently, 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.

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. For example, the GDPR imposes strict requirements on controllers and processors of personal data, including special protections for “special category data,” which includes health, biometric and genetic information of data subjects located in the EEA and, through incorporation in national legislation, the United Kingdom. Further, the GDPR provides a broad right for EU member states to create supplemental national laws, for example relating to the processing of health, genetic and biometric data, which could further limit our ability to use and share such data or could cause our costs to increase and harm our business and financial condition. The GDPR and similar national legislation grant individuals the opportunity to object to the processing of their personal information, allows them to request deletion of personal information in certain circumstances, and provides the individual with an express right to seek legal remedies in the event the individual believes his or her rights have been violated. Further, the GDPR and similar national legislation impose strict rules on the transfer of personal data out of the EEA, the United Kingdom, Switzerland, and other countries to the United States or other regions that have not been deemed to offer “adequate” privacy protections.

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 are subject to the supervision of local data protection authorities in those jurisdictions where we undertake clinical trials. 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.

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 United Kingdom 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 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.

Like other companies in our industry, our networks and infrastructure 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. 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.

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 close to 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 June 30, 2022, the 52-week range of the market price of our stock was from \$7.61 to \$19.99 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;
- developments and announcements regarding new and virulent strains of influenza;
- we 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;
- 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 or capital commitments;
- 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.

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

Future sales of our common stock by us or our current stockholders into the public market could cause the market price of our stock to fall. As of July 29, 2022, there were 185,945,016 shares of our common stock outstanding. We may from time to time issue securities in relation to a license arrangement, collaboration, merger or acquisition. We may also sell, for our own account, shares of common stock or other equity securities, from time to time at prices and on terms to be determined at the time of sale.

As of July 29, 2022, there were 29,999,408 stock options and restricted stock units outstanding and 11,077,032 shares available for issuance under our Amended and Restated Stock Incentive Plan, 5,071,076 stock options and restricted stock units outstanding and 162,132 shares available for issuance under our Amended and Restated Inducement Equity Incentive Plan, and 5,792,623 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 and stock awards have been 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 1993, 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, of which warrants to purchase 11,511,472 shares of our common stock remain outstanding. 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. Each warrant has an exercise price of \$0.01 per share. If the Baker Entities, by exercising their underwriting 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 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 or 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, including natural disasters, epidemic or pandemic disease outbreaks (such as the ongoing COVID-19 pandemic), trade wars, armed conflicts, political unrest or other events could disrupt our business or operations or those of our development partners (such as Torii), 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 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 "Risk Factors—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, clinical research organizations ("CROs"), and others, as well as on the regulatory and government agencies with whom we work." In addition, other events, such as the armed conflict between Russia and Ukraine, could adversely impact our business. Furthermore, the conflict could lead to sanctions, embargoes, supply shortages, regional instability, geopolitical shifts, cyberattacks, other retaliatory actions, and adverse effects on macroeconomic conditions, currency exchange rates, and financial markets, which could adversely impact our operations and financial results, as well as those of third parties with whom we conduct business.

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

From time to time, we may be involved in disputes, including, without limitation, disputes with our employees, collaborative partners, and third-party vendors. We may be called upon to initiate legal proceedings or to defend ourselves in such legal proceedings relating to our relationships with these parties, our decisions and actions or omissions with respect thereto, and our business. In addition, if our stock price is volatile, we may become involved in securities class action lawsuits in the future. Due to the inherent uncertainties in legal proceedings, we cannot accurately predict the ultimate outcome of any such proceedings. An unfavorable outcome in any such proceedings 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. This risk has been heightened in the current environment as a result of the ongoing COVID-19 pandemic. 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 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 Bylaws 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. Stock Incentive Plan (as amended and restated as of April 18, 2022). Incorporated by reference to Exhibit 10.1 to the Company's Form 8-K filed June 7, 2022.
10.2*	BioCryst Pharmaceuticals, Inc. Non-Employee Director Compensation Policy, effective April 18, 2022. Incorporated by reference to Exhibit 10.2 to the Company's Form 10-Q filed May 9, 2022.
(10.3)*	Form of Notice of Grant of Non-Employee Director Stock Option and Stock Option Agreement under the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan.
(10.4)*	Form of Notice of Grant of Non-Employee Director Restricted Stock Unit Award and Restricted Stock Unit Agreement under the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan.
(10.5)	Amendment #5 to the Agreement between BioCryst Pharmaceuticals, Inc. and the National Institute of Allergy and Infectious Diseases, dated June 29, 2022.
(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 six months ended June 30, 2022, formatted in Inline XBRL: (i) Consolidated Balance Sheets, (ii) Consolidated Statements of Comprehensive Loss, (iii) Consolidated Statements of Cash Flows, and (iv) Notes to 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 June 30, 2022 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 5th day of August, 2022.

BIOCRYSST 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)

/s/ Michael L. Jones

Michael L. Jones

Executive Director, Finance and Principal Accounting Officer

(Principal Accounting Officer)

BIOCRYST PHARMACEUTICALS, INC.

NOTICE OF GRANT OF NON-EMPLOYEE DIRECTOR STOCK OPTION

Notice is hereby given of the following stock option grant (the "Option") to purchase shares of the Common Stock of BioCryst Pharmaceuticals, Inc. (the "Company") pursuant to the Director Grant Program in effect under the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan (the "Plan"):

Optionee:	_____
Grant Date:	_____
Option Price:	_____
Number of Optioned Shares:	_____
Expiration Date:	_____
Type of Option:	Non-Qualified Stock Option

Exercise Schedule: The Option will become exercisable on the twelve (12)-month anniversary of the Grant Date. The Option shall not become exercisable following the Optionee's cessation of Service.

Optionee understands that the Option is granted subject to and in accordance with the express terms and conditions of the Plan and the BioCryst Pharmaceuticals, Inc. Non-Employee Director Compensation Policy (the "Policy"), and Optionee agrees to be bound by and conform to the terms and conditions of the Plan, the Plan Prospectus, this Notice of Grant of Non-Employee Director Stock Option and its accompanying Non-Employee Director Stock Option Agreement, and the Policy. Optionee acknowledges that copies of the Plan, the Plan Prospectus, the Non-Employee Director Stock Option Agreement, and the Policy have been made available to Optionee.

No Guarantee of Board Service: Nothing in the Non-Employee Director Stock Option Agreement, the Plan, or the Policy shall confer upon the Optionee the right to continue in the Service of the Company for any period of specific duration or interfere with or otherwise restrict in any way the rights of the Company or the stockholders to remove Optionee from the Board in accordance with applicable law.

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

OPTIONEE

Signature

BIOCRYST PHARMACEUTICALS, INC.

Jon P. Stonehouse
President & CEO

**BIOCRYST PHARMACEUTICALS, INC.
STOCK INCENTIVE PLAN**

NON-EMPLOYEE DIRECTOR STOCK OPTION AGREEMENT

WITNESSETH:

RECITALS

- A. The Company's Board of Directors (the "Board") has approved a director grant program under the Company's Stock Incentive Plan (the "Plan") pursuant to which the non-employee members of the Board will receive option grants under the Plan.
- B. Optionee is a non-employee member of the Board, and this Agreement is executed pursuant to, and is intended to carry out the purposes of, the Plan in connection with the grant of an option to purchase shares of the Company's Common Stock under the Plan.
- C. The granted option is intended to be a non-statutory option which does not meet the requirements of Section 422 of the Internal Revenue Code and is designed to provide Optionee with a meaningful incentive to serve as a member of the Board.

NOW, THEREFORE, it is hereby agreed as follows:

1. **Grant of Option.** Subject to and upon the terms and conditions set forth in this Agreement, the Company hereby grants to Optionee, as of the grant date (the "Grant Date") specified in the accompanying Notice of Grant of Non-Employee Director Stock Option (the "Grant Notice"), a stock option to purchase up to that number of shares of the Company's Common Stock (the "Optioned Shares") specified in the Grant Notice. The Optioned Shares shall be purchasable from time to time during the option term at the option price per share (the "Option Price") specified in the Grant Notice.
2. **Option Term.** This option grant shall expire at the close of business on the Expiration Date specified in the Grant Notice (which shall be ten (10) years measured from the Grant Date), unless sooner terminated in accordance with this Agreement.
3. **Limited Transferability.** During the lifetime of the Optionee, this option (together with its tandem stock appreciation right) shall be exercisable only by the Optionee and shall not be assignable or transferable by the Optionee except for a transfer by will or by the laws of descent and distribution following the Optionee's death. Notwithstanding the foregoing, this option may, in connection with the Optionee's estate plan, be assigned in whole or in part during the Optionee's lifetime either as (i) as a gift to one or more members of Optionee's immediate family, to a trust in which Optionee and/or one or more such family members hold more than fifty percent (50%) of the beneficial interest or an entity in which more than fifty percent (50%) of the voting interests are owned by Optionee and/or one or more such family members, or (ii) pursuant to a domestic relations order. The assigned portion shall be exercisable only by the person or persons who acquire a proprietary interest in the option pursuant to such assignment. The terms applicable to the assigned portion shall be the same as those in effect for this option immediately prior to such assignment and shall be set forth in such documents issued to the assignee as the Plan Administrator may deem appropriate.
4. **Exercisability.** This option shall become exercisable for the Optioned Shares in installments as is specified in the Grant Notice. As the option becomes exercisable for one or more installments, the installments shall accumulate and the option shall remain exercisable for the accumulated installments until the Expiration Date or the sooner termination of the option term under this Agreement.
5. **Cessation of Board Service.** Should Optionee cease to serve as a Board member for any reason while holding this option, then Optionee shall have the remainder of the ten (10) year term of this option in which to exercise such option for any or all of the Optioned Shares for which it is exercisable at the time of such cessation of Board service. This option shall immediately terminate and cease to be outstanding, at the time of such cessation of Board service, with respect to Optioned Shares for which this Option is not otherwise at that time exercisable. Upon the death of the Optionee, whether resulting in cessation of Service or occurring thereafter, the personal representative of the Optionee's estate or the person or persons to whom this Option is transferred pursuant to the Optionee's will or in accordance with the laws of descent and distribution may exercise this Option for the remainder of the ten (10) year term with respect to the Optioned Shares for which the Option was exercisable at the time of Optionee's death.

6. **Corporate Transaction/Change in Control.** In the event of a Corporate Transaction or a Change in Control, the option shall be treated as specified in Article Two of the Plan. This Agreement shall not in any way affect the right of the Company to adjust, reclassify, reorganize or otherwise make changes in its capital or business structure or to merge, consolidate, dissolve, liquidate or sell or transfer all or any part of its business or assets.

7. **Adjustment in Optioned Shares.**

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

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

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

9. **Manner of Exercising Option.**

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

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

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

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

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

(C) full payment in a combination of shares of Common Stock held for the requisite period necessary to avoid a charge to the Company's earnings and valued at Fair Market Value on the Exercise Date and cash or check drawn to the Company's order; or

(D) If the Company's outstanding Common Stock is registered under Section 12(g) of the Securities Exchange Act of 1934, as amended (the "1934 Act"), at the time this option is exercised, then payment of the Option Price may also be effected through a broker-dealer sale and remittance procedure pursuant to which Optionee shall provide irrevocable written instructions (A) 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, an amount equal to the aggregate Option Price payable for the purchased shares and (B) to the Company to deliver the certificates for the purchased shares directly to such brokerage firm in order to complete the sale.

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

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

(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 by the National Association of Securities Dealers through the Nasdaq system or any successor system. If there are no reported bid and asked prices (or closing selling price) for the Common Stock on the date in question, then the mean between the highest bid price and lowest asked price (or the closing selling price) on the last preceding date for which such quotations exist shall be determinative of Fair Market Value.

(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 such exchange on the date in question, then the Fair Market Value shall be the closing selling price on the exchange on the last preceding date for which such quotation exists.

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

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

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

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

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

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

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

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

12. **Liability of Company.**

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

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

13. **No Guarantee of Board Service.** This Agreement shall in any way be construed or interpreted so as to affect adversely or otherwise impair the right of the Company or the stockholders to remove Optionee from the Board at any time in accordance with the provisions of applicable law.

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

15. **Construction.** This Agreement and the option evidenced hereby are made and granted pursuant to the Plan and are in all respects limited by and subject to the express terms and provisions of the Plan.

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

17. **Tandem Stock Appreciation Right.** Optionee is hereby granted a tandem stock appreciation right, which entitles Optionee to surrender all or part of this option with respect to Optioned Shares for which it is then vested and exercisable, for a distribution from the Company in an amount equal to the excess of (A) the Fair Market Value (on the option surrender date) of the Optioned Shares for which the option is surrendered, over (B) the Option Price for such Optioned Shares. The distribution shall be made in shares of Common Stock valued at Fair Market Value on the option surrender date. Any portion of this option that is surrendered in accordance with this Paragraph shall cease to be outstanding and shall no longer be exercisable by Optionee.

**BIOCRYST PHARMACEUTICALS, INC.
STOCK INCENTIVE PLAN**

NOTICE OF GRANT OF NON-EMPLOYEE DIRECTOR RESTRICTED STOCK UNIT AWARD

Notice is hereby given of the following grant of restricted stock units with respect to the Company's Common Stock (such award referred to herein as the "Award") pursuant to the Director Grant Program in effect under the BioCryst Pharmaceuticals, Inc. Stock Incentive Plan (the "Plan"):

Name of Grantee: _____

Number of Underlying Shares: _____

Grant Date: _____

Vesting: _____

Grantee understands that the Award is granted subject to and in accordance with the express terms and conditions of the Plan and the BioCryst Pharmaceuticals, Inc. Non-Employee Director Compensation Policy (the "Policy"), and Grantee agrees to be bound by and conform to the terms and conditions of the Plan, the Plan Prospectus, this Notice of Grant of Non-Employee Director Restricted Stock Unit Award and its accompanying Non-Employee Director Restricted Stock Unit Agreement, and the Policy. Grantee acknowledges that copies of the Plan, the Plan Prospectus, the Non-Employee Director Restricted Stock Unit Agreement, and the Policy have been made available to Grantee.

No Guarantee of Board Service: Nothing in the Non-Employee Director Restricted Stock Unit Agreement, the Plan, or the Policy shall confer upon Grantee the right to continue in the Service of the Company for any period of specific duration or interfere with or otherwise restrict in any way the rights of the Company or the stockholders to remove Grantee from the Board in accordance with applicable law.

By my signature below, I hereby acknowledge receipt of the Award granted to me on the Grant Date specified above and issued to me pursuant to the terms and conditions of the Plan and the attached Agreement.

Agreed and Accepted:

BIOCRYST PHARMACEUTICALS, INC.

By: _____

Grantee: _____

Dated: _____

Jon P. Stonehouse
President & CEO

BIOCRYST PHARMACEUTICALS, INC.

NON-EMPLOYEE DIRECTOR RESTRICTED STOCK UNIT AGREEMENT

WITNESSETH:

RECITALS

A. The Company's Board of Directors (the "Board") has approved a director grant program under the Company's Stock Incentive Plan (the "Plan") pursuant to which the non-employee members of the Board will receive restricted stock units under the Plan.

B. Grantee is a non-employee member of the Board, and this Agreement is executed pursuant to, and is intended to carry out the purposes of, the Plan in connection with the grant of restricted stock units under the Plan.

NOW, THEREFORE, it is hereby agreed as follows:

The terms and conditions of the Award of restricted stock units with respect to Common Stock of the Company (the "RSUs") made to the Grantee, as set forth in the accompanying Notice of Non-Employee Director Restricted Stock Unit Award (the "Award Notice"), are as follows:

1. Issuance of RSUs.

(a) The RSUs are hereby granted and issued to the Grantee, effective as of the Grant Date set forth in the accompanying Award Notice, in consideration of the services rendered and to be rendered by the Grantee to the Company. Each RSU represents the right to receive one share of Common Stock, subject to the terms and conditions hereof. This Award is made subject to and awarded upon the terms and conditions set forth in this Agreement and the Plan.

(b) As promptly as practicable following the vesting of the RSUs pursuant to Section 2 (and in all events no later than March 15 of the year following the year of vesting (unless earlier delivery is required by Section 409A of the Code or delivery is deferred pursuant to a nonqualified deferred compensation plan in accordance with the requirements of Section 409A of the Code)), the Company shall issue, in the name of the Grantee, the number of shares of Common Stock that have vested.

(c) The Grantee agrees that the RSUs shall be subject to the forfeiture provisions set forth in Section 3 of this Agreement and the restrictions on transfer set forth in Section 4 of this Agreement.

2. General Vesting Terms; Lapsing of Restrictions.

(a) Vesting Schedule. The RSUs shall vest as set forth in the Award Notice (the date of vesting of the RSUs, the "Vesting Date").

(b) Change in Control. If a Corporate Transaction or a Change in Control occurs, Article Three of the Plan shall govern the treatment of the RSUs in connection therewith.

(c) Continuous Employment and Service. For purposes of this Agreement, Grantee shall be deemed to remain in continuous service with the Company for so long as the Grantee continues to render periodic services to the Company or any parent or subsidiary corporation, whether as an employee, a non-employee member of the Company's Board of Directors or an independent consultant or advisor. The Grantee shall be deemed to be an "employee" if the Grantee is in the employ of the Company or one or more of its parent or subsidiary corporations subject to the control and direction of the employer entity as to both the work to be performed and the manner and method of performance. For purposes of this Agreement, a corporation shall be considered to be a subsidiary corporation of the Company if it is a member of an unbroken chain of corporations beginning with the Company, provided each such corporation in the chain (other than the last corporation) owns, at the time of determination, stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain. Similarly, for purposes of this Agreement, a corporation shall be considered to be a parent corporation of the Company if it is a member of an unbroken chain ending with the Company, provided each such corporation in the chain (other than the Company) owns, at the time of determination, stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain.

3. Forfeiture of Unvested RSUs Upon Termination of Service.

In the event that the Grantee ceases to be continuously in service to the Company or one or more of its parent or subsidiary corporations for any reason or no reason, with or without cause, except as otherwise expressly provided in

Section 2 above, all of the RSUs that are unvested as of the time of such termination of service shall be forfeited immediately and automatically to the Company and no shares of Common Stock shall be issued with respect thereto, without the payment of any consideration to the Grantee, effective as of such separation from service. The Grantee shall have no further rights with respect to any RSUs that are so forfeited.

4. Restrictions on Transfer.

The Grantee shall not sell, assign, transfer, pledge, hypothecate or otherwise dispose of, by operation of law or otherwise (collectively "transfer") any RSUs, or any interest therein (but may transfer Common Stock after its issuance pursuant to Section 1(b) above). Notwithstanding the foregoing to the extent permitted by applicable law, the RSUs may be assigned in whole or part during the Grantee's lifetime pursuant to a domestic relations order; provided, however, that such RSUs shall in all cases remain subject to this Agreement (including, without limitation, the forfeiture provisions set forth in Section 3 and the restrictions on transfer set forth in this Section 4) and such permitted transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Agreement. The Company shall not be required: (i) to transfer on its books any of the RSUs (or issue shares of Common Stock with respect thereto) which have been transferred in violation of any of the provisions of this Agreement or (ii) to treat as owner of such RSUs any transferee to whom such RSUs have been transferred in violation of any of the provisions of this Agreement.

5. Rights as a Shareholder.

The Grantee shall have no rights as a shareholder with respect to the RSUs until such times as shares of Common Stock are issued in settlement thereof; provided, however, that if any dividends and distributions with respect to the shares of Common Stock underlying the RSUs are paid in cash or shares, or consist of a dividend or distribution to holders of Common Stock other than an ordinary cash dividend, the shares, cash or other property will be credited to a notional account on behalf of the Grantee subject to the same restrictions on transferability and forfeitability as the related RSUs.

6. Tax Matters.

Grantee acknowledges that Grantee is responsible for obtaining the advice of the Grantee's own tax advisors with respect to the acquisition and vesting of the RSUs and that Grantee is relying solely on such advisors and not on any statements or representations of the Company or any of its agents with respect to the tax consequences relating to the RSUs. The Grantee understands that the Grantee (and not the Company) shall be responsible for any and all of Grantee's tax liabilities that may arise in connection with the acquisition, vesting and/or settlement of the RSUs.

7. Miscellaneous.

(a) Authority of the Plan Administrator. In making any decisions or taking any actions with respect to the matters covered by this Agreement, the Plan Administrator shall have full authority and discretion, and shall be subject to all of the protections provided for in the Plan. All decisions and actions by the Plan Administrator with respect to this Agreement shall be made in the Plan Administrator's sole discretion and shall be final and binding on all.

(b) No Service Contract. The Grantee acknowledges and agrees that, notwithstanding the fact that vesting and settlement of the RSUs is contingent upon Grantee's continued service to the Company (or any parent or subsidiary corporation of the Company employing or retaining Grantee), this Agreement does not constitute an express or implied promise of continued service and nothing herein or in the Plan shall confer upon the Grantee any rights to continue in the service of the Company (or any parent or subsidiary corporation of the Company employing or retaining Grantee) for any period of time or interfere with or otherwise restrict in any way the rights of the Company or the stockholders to remove the Grantee from the Board in accordance with applicable law.

(c) Notices. Any notice required to be given or delivered to the Company under the terms of this Agreement shall be in writing and addressed to the Company in care of the Corporate Secretary at its principal corporate offices. Any notice required to be given or delivered to Grantee shall be in writing and addressed to the Grantee at the address indicated below Grantee's signature line of the Award Notice or such address as Grantee may provide for the Company to keep on file as updated from time to time. All notices shall be deemed to have been given or delivered upon personal delivery or upon deposit in the U. S. Mail, postage prepaid and properly addressed to the party to be notified.

(d) Construction; Amendment. The Grantee acknowledges that Grantee has read this Agreement, has received and read the Plan, and understands the terms and conditions of this Agreement and the Plan. This Agreement and the RSUs evidenced hereby are made and granted pursuant to the Plan and are in all respects limited by and subject to the express terms and provisions of the Plan. All decisions of the Plan Administrator with respect to any question or issue arising under the Plan or this Agreement shall be conclusive and binding on all persons having or claiming an interest in the RSUs. This Agreement may only be amended by a writing executed by the parties hereto expressly providing for

amendment of this Agreement except that the Plan Administrator may unilaterally make amendments that do not adversely affect Grantee's rights hereunder, provided timely notice of such amendments is provided to Grantee.

(e) Successors and Assigns. Except to the extent otherwise expressly provided herein, the provisions of this Agreement shall inure to the benefit of, and be binding upon, the successors, administrators, heirs, legal representatives and assigns of Grantee and the successors and assigns of the Company.

(f) Liability of the Company. If the RSUs exceed, as of the Grant Date, the number of shares of Common Stock which may without shareholder approval be issued under the Plan, then this Award shall be void with respect to such excess shares unless shareholder approval of an amendment sufficiently increasing the number of shares of Common Stock issuable under the Plan is obtained in accordance with the provisions of this Plan and all applicable laws. The inability of the Company to obtain approval from any regulatory body having authority deemed by the Company to be necessary to the lawful issuance and sale of any Common Stock pursuant to this Agreement shall relieve the Company of any liability with respect to the non-issuance or sale of the Common Stock as to which such approval shall not have been obtained. The Company, however, shall use its best efforts to obtain all such approvals.

(g) Compliance with Laws and Regulations. The award of RSUs hereunder and the settlement thereof is subject to compliance by the Company and Grantee with all applicable requirements of law relating thereto and all applicable regulations of any stock exchange or over-the-counter market on which such shares may be listed or traded at the time of such exercise and issuance. In connection with the settlement of RSUs, Grantee shall execute and deliver to the Company such representations in writing as may be requested by the Company in order for it to comply with the applicable requirements of federal and state securities laws.

(h) Capitalized Terms/Conflict. Capitalized terms not specifically defined herein have the meaning specified in the Plan. In the event of a conflict between the terms and conditions of this Agreement and the Plan, the Plan controls.

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

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

(k) Governing Law. The interpretation, performance, and enforcement of this Agreement shall be governed by the internal laws of the State of Delaware without regard to that state's conflict-of-laws rules.

AMENDMENT OF SOLICITATION/MODIFICATION OF CONTRACT		1. CONTRACT ID CODE	PAGE OF PAGES 1 6
2. AMENDMENT/MODIFICATION NO. P00004	3. EFFECTIVE DATE See Block 16C	4. REQUISITION/PURCHASE REQ. NO.	5. PROJECT NO. (If applicable)
6. ISSUED BY National Institutes of Health National Institute of Allergy and Infectious Diseases Bethesda, MD 20892-7612	CODE NIAID-ROCK	7. ADMINISTERED BY (If other than Item 6) National Institutes of Health National Institute of Allergy and Infectious Diseases Bethesda, MD 20892-7612	CODE NIAID
8. NAME AND ADDRESS OF CONTRACTOR (No., street, county, State and ZIP Code) BIOCRYST PHARMACEUTICALS, INC.:1231655 4505 EMPEROR BOULEVARD SUITE 200 DURHAM NC 277038457		(x) 9A. AMENDMENT OF SOLICITATION NO.	9B. DATED (SEE ITEM 11)
CODE MGXADJ6SCLJ5	FACILITY CODE	X 10A. MODIFICATION OF CONTRACT/ORDER NO. 75N93020C00055	10B. DATED (SEE ITEM 13) 08/31/2020

11. THIS ITEM ONLY APPLIES TO AMENDMENTS OF SOLICITATIONS

The above numbered solicitation is amended as set forth in Item 14. The hour and date specified for receipt of Offers is extended. is not extended. Offers must acknowledge receipt of this amendment prior to the hour and date specified in the solicitation or as amended, by one of the following methods: (a) By completing Items 8 and 15, and returning _____ copies of the amendment; (b) By acknowledging receipt of this amendment on each copy of the offer submitted; or (c) By separate letter or electronic communication which includes a reference to the solicitation and amendment numbers. FAILURE OF YOUR ACKNOWLEDGEMENT TO BE RECEIVED AT THE PLACE DESIGNATED FOR THE RECEIPT OF OFFERS PRIOR TO THE HOUR AND DATE SPECIFIED MAY RESULT IN REJECTION OF YOUR OFFER. If by virtue of this amendment you desire to change an offer already submitted, such change may be made by letter or electronic communication, provided each letter or electronic communication makes reference to the solicitation and this amendment, and is received prior to the opening hour and date specified.

12. ACCOUNTING AND APPROPRIATION DATA (If required)
See Schedule

13. THIS ITEM ONLY APPLIES TO MODIFICATION OF CONTRACTS/ORDERS. IT MODIFIES THE CONTRACT/ORDER NO. AS DESCRIBED IN ITEM 14.

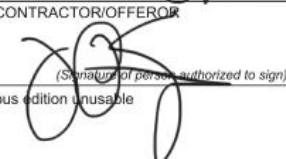
CHECK ONE	A. THIS CHANGE ORDER IS ISSUED PURSUANT TO: (Specify authority) THE CHANGES SET FORTH IN ITEM 14 ARE MADE IN THE CONTRACT ORDER NO. IN ITEM 10A.
	B. THE ABOVE NUMBERED CONTRACT/ORDER IS MODIFIED TO REFLECT THE ADMINISTRATIVE CHANGES (such as changes in paying office, appropriation data, etc.) SET FORTH IN ITEM 14, PURSUANT TO THE AUTHORITY OF FAR 43.103(b).
	C. THIS SUPPLEMENTAL AGREEMENT IS ENTERED INTO PURSUANT TO AUTHORITY OF:
X	D. OTHER (Specify type of modification and authority) Mutual Agreement of the parties

E. IMPORTANT: Contractor is not is required to sign this document and return 1 copies to the issuing office.

14. DESCRIPTION OF AMENDMENT/MODIFICATION (Organized by UCF section headings, including solicitation/contract subject matter where feasible.)

Purpose: Remove the original "Attachment 1 Statement of Work". Incorporate Attachment 1 dated June 1, 2022 to this contract. (Attached) All other terms remain the same.

Except as provided herein, all terms and conditions of the document referenced in Item 9 A or 10A, as heretofore changed, remains unchanged and in full force and effect.

15A. NAME AND TITLE OF SIGNER (Type or print) Jon P. Stonehouse CEO	16A. NAME AND TITLE OF CONTRACTING OFFICER (Type or print) JOHN E. OUTEN
15B. CONTRACTOR/OFFEROR  (Signature of person authorized to sign)	15C. DATE SIGNED 6/29/22
16B. UNITED STATES OF AMERICA John E. Outen -S (Signature of Contracting Officer)	16C. DATE SIGNED Digitally signed by John E. Outen -S Date: 2022.06.29 13:59:55 -04'00'

Previous edition unusable

CERTIFICATIONS

I, Jon P. Stonehouse, certify that:

1. I have reviewed this quarterly report on Form 10-Q of BioCryst Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 5, 2022

/s/ Jon P. Stonehouse

Jon P. Stonehouse

President and Chief Executive Officer

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: August 5, 2022

/s/ Anthony Doyle
Anthony Doyle
Chief Financial Officer

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

In connection with the Quarterly Report of BioCryst Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended June 30, 2022 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: August 5, 2022

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

In connection with the Quarterly Report of BioCryst Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended June 30, 2022 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: August 5, 2022