Table of Contents

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 September 30, 2026

or

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

For the transition period from _____ to _____

Commission File Number: 000-23186

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

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

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

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

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

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

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

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

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

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

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

As of October 30, 2026, the registrant had 257,299,269 shares of common stock outstanding.

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 (Unaudited):	1
Condensed Consolidated Balance Sheets as of September 30, 2026 and December 31, 2025	1
Condensed Consolidated Statements of Comprehensive Income (Loss) for the Three and Nine Months Ended September 30, 2026 and 2025	2
Condensed Consolidated Statements of Cash Flows for the Nine Months Ended September 30, 2026 and 2025	3
Condensed Consolidated Statements of Stockholders' Deficit for the Three and Nine Months Ended September 30, 2026 and 2025	5
Notes to Condensed Consolidated Financial Statements	7
Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations	36
Item 3. Quantitative and Qualitative Disclosures About Market Risk	51
Item 4. Controls and Procedures	52
Part II. Other Information	53
Item 1. Legal Proceedings	53
Item 1A. Risk Factors	53
Item 5. Other Information	84
Item 6. Exhibits	85
Signatures	86

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

Cautionary Note Regarding Forward-Looking Statements

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

- the preclinical development, clinical development, commercialization, or post-marketing studies of our products and product candidates, including ORLADEYO® (berotralstat), navenibart, BCX17725, and our plans and anticipated timing regarding the same;
- our acquisition, development, and commercialization of best-in-class and first-in-class medicines;
- the timing and success of our commercialization of ORLADEYO in the United States and elsewhere and expectations regarding the commercial market for ORLADEYO, including the launch of the ORLADEYO oral pellet formulation;
- our transition to our new sole source specialty pharmacy for ORLADEYO shipments to patients, including the effect of the new specialty pharmacy's inventory levels on our quarterly product revenue;
- 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, including the implementation and estimated timing of completion of the Birmingham Closure Plan (as defined below), our expectations regarding our strategic shift to prioritize external innovation, and the anticipated role and potential benefits of our Scientific Advisory Board;
- 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 and license agreements;
- our and our subsidiary guarantors' ability to satisfy obligations under and to comply with covenants set forth in connection with the Blackstone Loan Agreement (as defined below);
- the scope of protection we are able to establish and maintain for intellectual property rights covering our products, product candidates, and technology;
- our ability to operate our business without infringing the intellectual property rights of others;
- estimates of our revenues, expenses, capital requirements, annual cash utilization, and our needs for additional capital or financing;
- the timing or likelihood of regulatory filings, regulatory agreements, deferrals, approvals, and other decisions;
- our ability to manage our liquidity needs to fund our operations or repay our recourse debt obligations;
- our financial performance;
- statements and projections regarding financial goals, including profitability or positive cash flow;

- competitive companies, technologies, and our industry; and
- the Merger (as defined below) including, but not limited to, our expectations regarding the cost, benefits and expected synergies of the transaction.

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

Risk Factor Summary

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

- We may not achieve sustained profitability, and we may need to raise additional capital in the future. If we are unable to raise capital or obtain financing if and when needed, we may need to adjust our operations.
- The anticipated benefits and synergies of the Merger may not be realized, and risks associated with Astria's (as defined below) business, including the navenibart program, could adversely affect our business and financial results.
- Our success depends in part upon our ability to manage our product candidate pipeline, advance our product candidates through the various stages of development, especially through the clinical trial process, and to receive and maintain regulatory approvals for the commercial sale of our product candidates. The development process and related regulatory processes are complex and uncertain, may be lengthy and expensive, and require, among other things, an indication that our products and product candidates are safe and effective. For example, applicable regulatory agencies could refuse to approve, or impose restrictions or warnings on, our product candidates, require us to conduct additional studies or adopt study designs that differ from our planned development strategies, suspend or terminate our clinical trials, withdraw approval for our products, or take other actions that could materially impact the cost, timing, and success of our planned development and commercialization strategies.
- We rely heavily upon third parties, including development partners, contractors, contract research organizations, and third-party suppliers, manufacturers, specialty pharmacies, and distributors, for many important stages of our product candidate development and in the commercialization of our products. 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 the U.S. Food and Drug Administration or comparable foreign regulatory authorities approve generic versions or biosimilars of any of our products that receive marketing approval, the sales of our products could be adversely affected.
- The commercial viability of any approved product could be compromised if the product is less effective than expected, causes undesirable side effects that either were not previously identified or were worse than expected, or fails to achieve market acceptance by physicians, patients, third-party payors, health authorities, and others.
- There can be no assurance that our or our partners' commercialization efforts, methods, and strategies for our products or technologies will succeed, and our future revenue generation is uncertain.

- We face intense competition, and if we are unable to compete effectively, the demand for our products may be reduced. In addition, developments by others may render our products, product candidates, or technologies obsolete or noncompetitive.
- We are subject to various laws and regulations related to our products and product candidates, and if we or our employees, consultants, or partners do not comply with these laws and regulations, we could face substantial penalties, and our reputation could be harmed. In addition, we and our partners may be subject to new legislation, regulatory proposals, and healthcare payor initiatives that may increase our costs of compliance and adversely affect our or our partners' ability to market our products or develop our product candidates.
- If we fail to adequately protect or enforce our intellectual property rights, the value of those rights would diminish. Legal proceedings to protect or enforce our patents, the patents of our partners, or our other intellectual property rights could be expensive, time-consuming, and unsuccessful. If we fail to secure the rights to patents of others, this could adversely affect our business.
- We face an inherent risk of liability in the event that the use or misuse of our products or product candidates results in personal injury or death, and our product liability insurance coverage may be insufficient.
- If we fail to reach milestones or to make annual minimum payments or otherwise breach our obligations under our license agreements, our licensors may terminate our agreements with them and/or seek additional remedies.
- The Blackstone Loan Agreement (as defined below) contains conditions and restrictions that limit our flexibility in operating 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 non-U.S. governmental laws and regulations and other obligations related to privacy, data protection, and information security could harm our business.
- If our facilities, or the facilities of our third-party vendors, incur damage or power is lost for a significant length of time, our operations will be disrupted, which will adversely affect our business.
- Cyber incidents and related disruptions in our or our third-party vendors' information technology systems, as well as challenges with properly managing or using artificial intelligence, could adversely affect our business.
- Our ability to maintain global brand uniformity for ORLADEYO may be impacted by the sale of our European ORLADEYO business.
- Our business, operations, clinical development or commercialization plans and timelines, and access to capital could be adversely affected by unpredictable and unstable market and economic conditions.
- If we fail to retain our existing key personnel or advisors, or fail to attract and retain additional key personnel or advisors, the development of our product candidates, the commercialization of our products, and the related growth of our business may be delayed or stopped.
- Future acquisitions, strategic investments, partnerships, alliances, or divestitures could fail to meet our expectations and/or adversely affect our operating results and financial condition.
- Our existing principal stockholders hold a substantial amount of our common stock and may be able to influence significant corporate decisions, which may conflict with the interests of other stockholders.
- Our stock price has been, and is likely to continue to be, highly volatile, which could cause the value of an investment in our common stock to decline significantly.
- If we fail to maintain effective internal control over financial reporting, we may not be able to produce accurate and timely financial statements, which may adversely affect investor confidence in us and our financial reporting, adversely affect our business and operating results and negatively impact the trading price of our common stock.

- Natural disasters, epidemic or pandemic disease outbreaks, trade wars, armed conflicts, political unrest, or other events could disrupt our business or operations, or those of our development partners, manufacturers, regulators, or third parties with whom we conduct business now or in the future.
- We are subject to legal proceedings, which could harm our reputation or result in other losses or unexpected expenditure of time and resources.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

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

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 96,971	$ 89,736
Restricted cash	518	201
Short-term investments	278,027	185,011
Trade receivables	121,786	106,818
Inventory, net	13,740	5,398
Prepaid expenses and other current assets	20,109	17,182
Total current assets	531,151	404,346
Long-term inventory, net	31,271	23,990
Property and equipment, net	1,662	8,783
Long-term investments	—	61,164
Right of use assets, net	5,820	10,203
Other assets	20,194	5,672
Non-current assets held for sale	972	—
Total assets	$ 591,070	$ 514,158
Liabilities and Stockholders' Deficit		
Current liabilities:		
Accounts payable	$ 14,579	$ 15,826
Accrued expenses	110,689	126,413
Share-based liability	12,838	13,743
Operating lease liabilities	2,421	317
Finance lease liabilities	1,023	1,312
Royalty financing obligations	43,745	38,455
Deferred revenue	10,535	—
Total current liabilities	195,830	196,066
Operating lease liabilities	6,840	8,571
Finance lease liabilities	623	1,441
Royalty financing obligations	377,938	427,233
Secured term loan	395,609	—
Deferred revenue	11,614	—
Total liabilities	988,454	633,311
Stockholders' deficit:		
Preferred stock, $0.01 par value; shares authorized - 5,000; no shares issued and outstanding at September 30, 2026 and December 31, 2025	—	—
Common stock, $0.01 par value; shares authorized - 450,000; shares issued and outstanding – 256,118 at September 30, 2026 and 213,060 at December 31, 2025	2,561	2,131
Additional paid-in capital	1,712,342	1,384,857
Accumulated other comprehensive (loss) income	(607)	38
Accumulated deficit	(2,111,680)	(1,506,179)
Total stockholders' deficit	(397,384)	(119,153)
Total liabilities and stockholders' deficit	$ 591,070	$ 514,158

See accompanying notes to condensed consolidated financial statements.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
Revenues:				
Product sales, net	$ 172,295	$ 157,735	$ 481,209	$ 463,952
License and other revenues	7,997	1,660	73,746	4,330
Total revenues	180,292	159,395	554,955	468,282
Expenses:				
Cost of product sales	1,574	2,187	10,791	9,553
Acquired in-process research and development	—	—	697,761	—
Research and development	58,213	44,603	170,501	125,259
Selling, general and administrative	67,175	83,014	225,705	252,866
Total operating expenses	126,962	129,804	1,104,758	387,678
Income (loss) from operations	53,330	29,591	(549,803)	80,604
Other income (expense):				
Interest income	3,073	2,238	7,763	7,778
Interest expense	(21,185)	(19,661)	(62,676)	(64,737)
Foreign currency gains (losses), net	22	35	(176)	(27)
Loss on extinguishment of debt	—	(2,740)	—	(6,911)
Other income, net	3,345	2,667	2,143	2,667
Total other expense, net	(14,745)	(17,461)	(52,946)	(61,230)
Income (loss) before income taxes	38,585	12,130	(602,749)	19,374
Income tax expense (benefit)	669	(769)	2,752	1,358
Net income (loss)	$ 37,916	$ 12,899	$ (605,501)	$ 18,016
Other comprehensive (loss) income:				
Foreign currency translation adjustment	16	(523)	(73)	545
Unrealized (loss) gain on available for sale investments	(95)	17	(572)	(326)
Total other comprehensive (loss) income	(79)	(506)	(645)	219
Net comprehensive income (loss)	$ 37,837	$ 12,393	$ (606,146)	$ 18,235
Net income (loss) per common share: basic	$ 0.15	$ 0.06	$ (2.41)	$ 0.09
Weighted average shares of common stock outstanding: basic	255,634	210,176	250,840	209,531
Net income (loss) per common share: diluted	$ 0.14	$ 0.06	$ (2.41)	$ 0.08
Weighted average shares of common stock outstanding: diluted	267,621	219,885	250,840	218,349

See accompanying notes to condensed consolidated financial statements.

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

	Nine Months Ended September 30,	
	2026	**2025**
Cash flows from operating activities:		
Net (loss) income	$ (605,501)	$ 18,016
Adjustments to reconcile net (loss) income to net cash provided by operating activities:		
Depreciation	1,218	995
Acquired in-process research and development expense	697,761	—
Amortization of assembled workforce	600	—
Inventory obsolescence	443	1,056
Stock-based compensation expense	44,963	61,273
Mark-to-market adjustment on share-based liability	345	—
Payment of Pharmakon paid-in-kind interest	—	(9,153)
Non-cash interest expense on royalty financing obligations	38,669	39,839
Amortization of debt issuance costs on term loans	561	1,387
Amortization of discount on investments, net	(1,978)	(3,240)
Loss on extinguishment of debt	—	6,911
Loss on disposal of property and equipment	93	—
Impairment of long-lived assets	9,538	—
Gain on sale of non-financial asset	—	(550)
Changes in operating assets and liabilities:		
Increase in trade receivables	(12,991)	(21,312)
Increase in inventory	(16,067)	(1,124)
Decrease (increase) in prepaid expenses and other assets	2,041	(5,890)
Decrease in royalty financing obligations	(45,647)	(55,240)
(Decrease) increase in accounts payable and accrued expenses	(45,674)	22,441
Increase in deferred revenue	7,160	—
Net cash provided by operating activities	75,534	55,409
Cash flows from investing activities:		
Acquisitions of property and equipment	(845)	(1,666)
Proceeds from disposals of property and equipment	67	—
Purchases of investments	(275,844)	(136,737)
Sales and maturities of investments	308,431	207,647
Acquisition of Astria Therapeutics, Inc., net of cash acquired	(489,480)	—
Sale of non-financial asset	—	550
Net cash (used in) provided by investing activities	(457,671)	69,794
Cash flows from financing activities:		
Net proceeds from common stock issued under stock-based compensation plans	29,921	8,458
Withholding taxes paid on stock-based awards	(6,254)	(2,845)
Common stock issued to directors in lieu of cash retainer	126	36
Proceeds from the Term Loans under the Blackstone Loan Agreement	400,000	—
Payment of debt issuance costs on the Term Loans under the Blackstone Loan Agreement	(4,952)	—
Repayment of Pharmakon term loan principal	—	(115,847)
Prepayment premium and fees on Pharmakon term loan	—	(3,802)
Principal payments on royalty financing obligations	(28,517)	(16,406)

Principal payments on finance lease liabilities	(1,001)	(1,565)
Net cash provided by (used in) financing activities	389,323	(131,971)
Effect of exchange rates on cash, cash equivalents and restricted cash	(101)	1,342
Increase (decrease) in cash, cash equivalents and restricted cash, including cash classified within current assets held for sale	7,085	(5,426)
Less: net increase in cash and cash equivalents classified within current assets held for sale	—	(14,840)
Net increase (decrease) in cash, cash equivalents, and restricted cash	7,085	(20,266)
Cash, cash equivalents and restricted cash:		
Beginning of period	91,337	106,323
End of period	$ 98,422	$ 86,057
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 96,971	$ 84,078
Restricted cash	518	579
Restricted cash in other assets	933	1,400
Total cash, cash equivalents and restricted cash	$ 98,422	$ 86,057
Supplemental cash flow disclosure:		
Issuance of common stock for the acquisition of Astria Therapeutics, Inc.	$ 251,655	$ —
Cash paid for interest	$ 22,789	$ 22,789
Cash paid for taxes	$ 3,525	$ 2,220
Capitalized software costs included in accrued expenses and accounts payable	$ 526	$ 380
Taxes withheld on stock-based awards included in accrued expenses	$ 422	$ 151

See accompanying notes to condensed consolidated financial statements.

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

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount				
Balance at December 31, 2025	**213,060**	**$ 2,131**	**$ 1,384,857**	**$ 38**	**$ (1,506,179)**	**$ (119,153)**
Net loss	—	—	—	—	(721,812)	(721,812)
Other comprehensive loss	—	—	—	(406)	—	(406)
Exercise of stock options	3,279	33	18,333	—	—	18,366
Vesting of restricted stock units	190	2	(2)	—	—	—
Employee stock purchase plan sales	199	1	1,112	—	—	1,113
Issuance of shares to directors in lieu of cash retainer	4	—	33	—	—	33
Stock-based compensation expense	—	—	16,027	—	—	16,027
Remeasurement of share-based liability	—	—	(637)	—	—	(637)
Settlement of share-based liability	—	—	971	—	—	971
Issuance of common stock for the acquisition of Astria Therapeutics, Inc.	37,282	373	251,282	—	—	251,655
Balance at March 31, 2026	**254,014**	**$ 2,540**	**$ 1,671,976**	**$ (368)**	**$ (2,227,991)**	**$ (553,843)**
Net income	—	—	—	—	78,395	78,395
Other comprehensive loss	—	—	—	(160)	—	(160)
Exercise of stock options	1,042	11	6,201	—	—	6,212
Vesting of restricted stock units	205	2	(2)	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(1)	—	(9)	—	—	(9)
Issuance of shares to directors in lieu of cash retainer	5	—	44	—	—	44
Stock-based compensation expense	—	—	14,692	—	—	14,692
Remeasurement of share-based liability	—	—	(43)	—	—	(43)
Settlement of share-based liability	—	—	423	—	—	423
Balance at June 30, 2026	**255,265**	**$ 2,553**	**$ 1,693,282**	**$ (528)**	**$ (2,149,596)**	**$ (454,289)**
Net income	—	—	—	—	37,916	37,916
Other comprehensive loss	—	—	—	(79)	—	(79)
Exercise of stock options	537	5	3,402	—	—	3,407
Vesting of restricted stock units	164	2	(2)	—	—	—
Shares withheld for taxes for vesting of restricted stock units	—	—	(4)	—	—	(4)
Employee stock purchase plan sales	148	1	835	—	—	836
Issuance of shares to directors in lieu of cash retainer	4	—	49	—	—	49
Stock-based compensation expense	—	—	14,244	—	—	14,244
Remeasurement of share-based liability	—	—	536	—	—	536
Balance at September 30, 2026	**256,118**	**$ 2,561**	**$ 1,712,342**	**$ (607)**	**$ (2,111,680)**	**$ (397,384)**

See accompanying notes to condensed consolidated financial statements.

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

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount				
Balance at December 31, 2024	**208,543**	**$ 2,085**	**$ 1,291,100**	**$ 921**	**$ (1,770,040)**	**$ (475,934)**
Net income	—	—	—	—	32	32
Other comprehensive income	—	—	—	211	—	211
Exercise of stock options	286	3	1,226	—	—	1,229
Vesting of restricted stock units	184	2	(2)	—	—	—
Shares withheld for taxes for vesting of restricted stock units	(8)	—	(56)	—	—	(56)
Employee stock purchase plan sales	202	2	1,215	—	—	1,217
Issuance of shares to directors in lieu of cash retainer	1	—	6	—	—	6
Stock-based compensation expense	—	—	21,368	—	—	21,368
Balance at March 31, 2025	**209,208**	**$ 2,092**	**$ 1,314,857**	**$ 1,132**	**$ (1,770,008)**	**$ (451,927)**
Net income	—	—	—	—	5,085	5,085
Other comprehensive income	—	—	—	514	—	514
Exercise of stock options	560	6	3,415	—	—	3,421
Vesting of restricted stock units	136	1	(1)	—	—	—
Issuance of shares to directors in lieu of cash retainer	1	—	9	—	—	9
Stock-based compensation expense	—	—	21,304	—	—	21,304
Balance at June 30, 2025	**209,905**	**$ 2,099**	**$ 1,339,584**	**$ 1,646**	**$ (1,764,923)**	**$ (421,594)**
Net income	—	—	—	—	12,899	12,899
Other comprehensive loss	—	—	—	(506)	—	(506)
Exercise of stock options, net	300	3	1,563	—	—	1,566
Vesting of restricted stock units	147	1	(1)	—	—	—
Employee stock purchase plan sales	167	2	1,079	—	—	1,081
Issuance of shares to directors in lieu of cash retainer	3	—	21	—	—	21
Stock-based compensation expense	—	—	18,644	—	—	18,644
Balance at September 30, 2025	**210,522**	**$ 2,105**	**$ 1,360,890**	**$ 1,140**	**$ (1,752,024)**	**$ (387,889)**

See accompanying notes to condensed consolidated financial statements.

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

Note 1 — Significant Accounting Policies and Concentrations of Risk

The Company

BioCryst Pharmaceuticals, Inc. (the "Company") is a global biotechnology company focused on developing and commercializing medicines for hereditary angioedema ("HAE") and other rare diseases, driven by the Company's deep commitment to improving the lives of people living with these conditions. The Company has built an established commercial infrastructure supporting ORLADEYO®, an oral, once-daily therapy discovered and developed internally for the prevention of HAE attacks, and is advancing a pipeline of potential first-in-class or best-in-class oral small-molecule and injectable protein therapeutics for a range of rare diseases. The Company was founded in 1986 and incorporated in Delaware in 1991, and its headquarters is located in Durham, North Carolina.

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

Based on the Company's expectations for revenue and operating expenses, the Company believes its financial resources available at September 30, 2026 will be sufficient to fund its operations for at least the next 12 months. The Company may, in the future, issue securities, including common stock, preferred stock, depositary shares, purchase contracts, warrants, debt securities and units, through private placement transactions or registered public offerings. The Company's future liquidity needs, and ability to address those needs, will largely be determined by the success of its products and product candidates; the success of its business development efforts; the timing, scope and magnitude of its research and development and commercial expenses; and key regulatory and operational developments.

Basis of Presentation

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

The condensed consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries, including Astria Therapeutics, Inc., which was acquired by the Company on January 23, 2026 (see "*Note 2—Acquisition of Astria Therapeutics, Inc.*"). All intercompany transactions and balances among the consolidated entities have been eliminated from the condensed consolidated financial statements. The Company operates and manages its business as one reportable and operating segment (see "*Note 14—Segment Information*").

These condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements for the fiscal year ended December 31, 2025 and the notes thereto included in the Company's 2025 Annual Report on Form 10-K as filed with the Securities and Exchange Commission (the "SEC") on February 26, 2026. Interim operating results are not necessarily indicative of operating results for the full fiscal year. The condensed consolidated balance sheet as of December 31, 2025 was derived from the audited consolidated financial statements included in the Company's most recent Annual Report on Form 10-K.

Sale of European ORLADEYO Business

On June 27, 2025, the Company entered into a stock purchase agreement (the "Stock Purchase Agreement") with BioCryst Ireland Limited ("BioCryst Ireland") and Neopharmed Gentili S.p.A., ("Neopharmed"). On October 1, 2025, the Company sold to Neopharmed all of its equity interests in BioCryst Ireland, which, together with its subsidiaries, holds certain assets, rights, and employees related to the European ORLADEYO business. The Company and BioCryst Ireland also amended and restated their existing intellectual property licence agreement, pursuant to which the Company is entitled to receive quarterly royalty payments from BioCryst Ireland equal to amounts owed under its Royalty Purchase Agreements with RPI and OMERS for the sale of ORLADEYO products in the Territory, as defined in the Stock Purchase Agreement (see "*Note 7—Royalty Financing Obligations").* The condensed consolidated financial statements for the three and nine months ended September 30, 2025 include the accounts of BioCryst Ireland, which was a wholly owned subsidiary of the Company prior to October 1, 2025. As of September 30, 2025, the assets and liabilities of BioCryst Ireland were classified as a disposal group held for sale in the Company's condensed consolidated balance sheet.

Use of Estimates

The preparation of the condensed consolidated 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. Actual results could differ from those estimates.

Assets Held for Sale

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

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

Revenue Recognition

Pursuant to ASC Topic 606, *Revenue from Contracts with Customers* ("Topic 606"), the Company recognizes revenue when control of promised goods or services transfers to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The Company applies the five-step model under Topic 606 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.

Product Sales, Net

The Company's sources of product sales are sales of ORLADEYO and peramivir (RAPIVAB/RAPIACTA/PERAMIFLU). The Company generally ships ORLADEYO directly to patients through a single specialty pharmacy in the United States and through specialty distributors outside of the United States, which are considered its customers. The Company generally sells peramivir to its licensing partners, hospitals, and, in 2025, to the U.S. Department of Health and Human Services.

For ORLADEYO in the United States, the Company recognizes revenue when control of the product transfers to its specialty pharmacy customer, which obtains title to and maintains inventory of the product prior to dispensing it to patients. For peramivir and ORLADEYO outside of the United States, the Company recognizes revenue when the customer obtains control of the product, which generally occurs upon delivery.

Revenues from product sales are recorded at the net selling price (transaction price), which includes estimates of variable consideration, including government and managed care rebates, chargebacks, co-payment and patient assistance, quick starts, and product returns. Variable consideration is estimated using the expected value method and is included in

net product revenues only to the extent that it is probable that a significant reversal of cumulative revenue recognized will not occur. Estimates are reassessed each reporting period, and adjustments are recognized in the period identified.

Government and Managed Care Rebates. The Company estimates rebates payable to third-party payors, including managed care organizations and government programs, and records such amounts as reductions of gross product revenues at the time revenue is recognized, with corresponding liabilities recorded in accrued expenses. Rebate estimates are based on contractual terms, applicable program requirements, and product distribution information obtained from the Company's specialty pharmacy regarding payor mix.

Chargebacks. Chargebacks represent the Company's estimated obligations arising from contractual commitments to provide products to government agencies at prices lower than those charged to the Company's specialty pharmacy. The specialty pharmacy submits chargeback claims for the difference between the price paid to the Company and the applicable government-mandated price. The Company estimates chargebacks and records such amounts as reductions of gross product revenues and trade receivables at the time revenue is recognized.

Co-payment assistance programs. Patients who have commercial insurance and meet certain eligibility requirements may receive co-payment assistance. The Company estimates co-payment assistance amounts based on program terms and utilization and records such amounts as reductions of product revenue in the same period in which the related revenue is recognized, with a corresponding reduction to the prepaid co-payment assistance asset.

Patient assistance program. The Company offers a patient assistance program that provides free drug product to eligible patients who lack adequate prescription drug coverage or cannot afford treatment. Based on utilization reports provided by the specialty pharmacy, the Company records a reduction of gross product revenue for the full value of product dispensed under the program.

Quick start program. The Company offers a quick start program that provides free drug product to commercial customers while insurance approval, prior authorization, or reimbursement review is pending. The program is available for up to 90 days while the patient secures coverage. Based on utilization reports provided by the specialty pharmacy, the Company records a reduction of gross product revenue for the full value of product dispensed under the program.

Product returns. The Company generally does not accept returns from end customers and will accept returns from its specialty pharmacy only in limited circumstances including product defects, recalls, shipment damage, and certain other specified situations. The Company estimates expected returns and records a reserve at the time product revenue is recognized. Based on historical experience and the limited nature of allowable returns, the reserve has not been material.

License and Collaborative and Other Revenues

The Company has collaboration and license agreements with a number of third parties. The terms of the agreements typically include one or more of the following: upfront license fees; development, regulatory and sales-based milestone payments; and royalties on net sales of licensed products. For agreements with multiple performance obligations, revenue is allocated to each performance obligation based on its relative standalone selling price. The Company uses judgment to identify performance obligations and determine whether variable consideration should be included in the transaction price.

Upfront license fees. Arrangements that include upfront payments may require deferral of revenue recognition to a future period until obligations under such arrangements are fulfilled. If the license to the Company’s intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenue from upfront license fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. For agreements with multiple performance obligations, the Company develops assumptions that require judgment to determine the standalone selling price for each performance obligation, the determination of the transaction price, and the application of the constraints. The Company re-evaluates the transaction price at each reporting period and adjusts the estimate as changes in circumstances occur. The Company determines whether the performance obligations are satisfied over time or at a point in time and, if over time, determines the appropriate method of measuring progress for purposes of recognizing revenue.

Development, regulatory or commercial milestone payments. At the inception of each arrangement that includes payments based on the achievement of certain development, regulatory and commercial events, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company’s or the customer’s control are not considered probable until the milestone is achieved. Each subsequent reporting period, the

Company re-evaluates the probability of achieving such development and regulatory milestones and any related constraint, and if necessary, adjusts the Company's estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis and recognized as revenue during the period of adjustment.

Sales-based milestone payments and royalties. For arrangements that include sales-based royalties, including milestone payments based on the volume of sales, the Company determines whether the license is deemed to be the predominant item to which the royalties or sales-based milestones relate and if such is the case, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Cash and Cash Equivalents

The Company considers cash equivalents to be highly liquid investments with maturities of three months or less from the date of purchase.

Restricted Cash

Total restricted cash was $1,451 and $1,601 as of September 30, 2026 and December 31, 2025, respectively, and primarily consisted of $933 and $1,400 as of September 30, 2026 and December 31, 2025, respectively, for a letter of credit the Company is required to maintain associated with its Birmingham lease, which is reflected within other assets in the Condensed Consolidated Balance Sheets.

Investments

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

The Company classifies all of its investments as available-for-sale, which are reported at fair value at each condensed consolidated balance sheet date. The Company evaluates its securities in an unrealized loss position at the end of each reporting period to determine whether a credit loss exists. Credit-related impairments are recognized through an allowance for credit losses in the Condensed Consolidated Balance Sheets with a corresponding charge to earnings. Unrealized losses not attributable to credit-related factors and unrealized gains are recognized in accumulated other comprehensive (loss) income, net of applicable taxes.

Realized gains and losses are reflected in other expense, net in the Condensed Consolidated Statements of Comprehensive Income (Loss) and are determined using the specific identification method with transactions recorded on a settlement date basis. Investments with original maturities at date of purchase beyond three months and which mature at or less than 12 months from the condensed consolidated balance sheet date are classified as current. Investments with a maturity beyond 12 months from the condensed consolidated balance sheet date are classified as long-term.

Fair Value Measurements

Assets and liabilities recorded at fair value on a recurring basis in the Condensed Consolidated Balance Sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the

measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs.

Assets measured at fair value on a recurring basis include cash equivalents and investments (see "*Note 5—Fair Value Measurements and Investments*"). Liabilities measured at fair value on a recurring basis include liability classified stock option and restricted stock unit awards (see "*Note 11—Stock-Based Compensation*"). The carrying amounts reflected in the Condensed Consolidated Balance Sheets for cash, trade receivables, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Trade Receivables

The Company's trade receivables represent amounts due from its customers and partners for product sales and from its license and collaboration agreements. Trade receivables are generally stated at the invoiced amount with standard payment terms that require payment within 30 to 90 days and do not bear interest.

The Company provides reserves against trade receivables for estimated losses that may result from a customer's inability to pay. Trade receivables are evaluated to determine if any 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 allowance.

Inventory

The Company values its inventory at the lower of cost or estimated net realizable value and classifies inventory as short-term or long-term based on whether it is expected to be consumed or sold within 12 months of the condensed consolidated balance sheet date. The Company uses an actual cost method and determines the cost of its inventory on a first-in, first-out basis. Raw materials and work-in-process include all inventory costs prior to packaging and labeling, including raw material, active product ingredient, and the drug product. Finished goods include packaged and labeled products.

The Company's inventory is subject to expiration dating. At each condensed consolidated balance sheet date, the Company evaluates inventory for excess, obsolete, short-dated, unmarketable, or otherwise impaired items and records valuation reserves as necessary. Inventory is also subject to strict quality control and monitoring that is performed throughout the manufacturing process, including release of work-in-process to finished goods. If certain batches or units of product do not meet quality specifications, the Company records a write-down of any potential unmarketable inventory to its estimated net realizable value with the resulting charge recorded as cost of product sales in the Condensed Consolidated Statements of Comprehensive Income (Loss).

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

Property and Equipment

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

The Company periodically reviews its property and equipment for impairment when events or changes in circumstances indicate that the carrying amount of such assets may not be recoverable. Determination of recoverability is based on an estimate of undiscounted future cash flows resulting from the use of the asset and its eventual disposition. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the assets, the assets are written down to their estimated fair values. Property and equipment to be disposed of are reported at the lower of carrying amount or fair value less cost to sell.

Accrued Expenses

The Company enters into contractual agreements with third-party vendors who provide research and development, manufacturing, distribution, and other services in the ordinary course of business. Some of these contracts are subject to milestone-based invoicing and involve services completed over extended periods. The Company records liabilities under these contractual commitments when obligations are incurred, regardless of the invoice timing. Accrued expenses are estimated as of each condensed consolidated balance sheet date based on the facts and circumstances known at that time, which may include assumptions such as expected patient enrollment, site activation and estimated project duration. The Company accrues expenses for clinical trial activities based on the estimates of services received pursuant to contracts with multiple research institutions and clinical research organizations ("CROs") that conduct and manage clinical trials on the Company's behalf.

The Company periodically confirms the accuracy of its estimates with the service providers and adjusts if necessary. Examples of estimated accrued expenses include (i) fees paid to CROs in connection with preclinical and toxicology studies and clinical trials; (ii) fees paid to investigative sites in connection with clinical trials; (iii) fees paid to contract manufacturers in connection with the production of the Company's raw materials, drug substance, drug products, and product candidates; and (iv) professional fees. If the Company underestimates or overestimates the level of these costs, actual expenses could differ from such estimates.

Cost of Product Sales

Cost of product sales includes the cost of inventory related to our product revenue during the respective period, including costs associated with the acquisition and manufacture of such inventory. Cost of product sales also includes inventory write-downs and excess or obsolete inventory charges, as well as costs directly associated with the production of the Company's commercial products.

Research and Development Expenses

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

Direct expenses consist of compensation for research and development personnel and costs of outside parties to conduct laboratory studies, develop manufacturing processes and manufacture the product candidate, conduct and manage clinical trials, as well as other costs related to the Company's clinical and preclinical studies. Additionally, direct expenses consist of those costs necessary to discontinue and close out a development program, including termination fees and other commitments. Indirect expenses consist of lab supplies and services, facility expenses, depreciation of development equipment and an allocation of general and administrative overhead costs that support the Company's research and development efforts.

Selling, General and Administrative Expenses

Selling, general and administrative expenses represent costs incurred to commercialize the Company's products and support its operations. Sales and marketing expenses include personnel-related costs, activities supporting the commercialization and distribution of marketed products, market research, marketing, medical affairs, market access, and advertising costs.

General and administrative expenses include personnel-related costs and other expenses associated with quality, finance, human resources, information technology, legal, compliance, and other administrative functions, including transaction-related costs. All patent related costs are expensed to general and administrative expenses when incurred as recoverability of such expenditures is uncertain.

Leases

The Company leases certain assets under operating and finance leases, which consist of real estate leases, laboratory equipment leases and office equipment leases. The Company determines whether a contract is, or contains, a lease at

inception. The Company accounts for lease obligations in accordance with ASC Topic 842, *Leases* ("Topic 842"), which requires a lessee to recognize a right of use asset and a lease liability in its condensed consolidated balance sheet for most leases. For leases with an initial lease term of twelve months or less, the Company recognizes lease expense on a straight-line basis over the lease term. The Company does not separate non-lease components from lease components and instead accounts for them as a single lease component.

Certain of the Company's leases provide for renewal options, which can vary by lease. The right of use asset and lease liabilities in the Condensed Consolidated Balance Sheets represent payments over the lease term, which include renewal options for certain real estate leases that the Company is reasonably certain to exercise. Certain operating leases include rent escalation provisions, which the Company recognizes as expense on a straight-line basis.

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

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

The Company periodically reviews its right of use assets 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 right of use assets, the assets are written down to their estimated fair values.

Stock-Based Compensation

All share-based payments, including grants of stock option awards and restricted stock unit awards, are recognized in the Condensed Consolidated Statements of Comprehensive Income (Loss) based on their fair values. Stock-based compensation cost is estimated at the grant date based on the fair value of the award and is recognized as expense on a straight-line basis over the requisite service period of the award. Determining the appropriate fair value model and the related assumptions for the model requires judgment, including estimating the stock price volatility and the expected term. The Company utilizes the Black-Scholes option-pricing model or binomial lattice model to value its stock option awards. The Company reduces stock-based compensation expense for estimated forfeitures. The estimation of share-based payment awards that will ultimately vest requires judgment, and to the extent actual results or updated estimates differ from the Company's current estimates, such amounts will be recorded as a cumulative adjustment in the period estimates are revised. Actual results, and future changes in estimates, may differ substantially from the Company's current estimates.

Debt

Costs directly associated with term loan borrowings are capitalized and netted against the corresponding debt liabilities in the Condensed Consolidated Balance Sheets. These costs are amortized to interest expense over the terms of the corresponding borrowings using the effective interest rate method.

Royalty Financing Obligations

Royalty financing obligations are repaid through royalties based on net sales of ORLADEYO. Interest expense is recognized using the effective interest rate method over the estimated period during which each of the related liabilities will be paid. This requires the Company to estimate the total future royalty payments expected to be generated from product sales over the life of each agreement. The Company reassesses the expected royalty payments each reporting period and accounts for any changes through adjustments to the effective interest rates on a prospective basis. The assumptions used in determining the expected repayment terms of the debt and amortization periods of the issuance costs require that the Company make estimates that could impact the carrying values of each of the liabilities, as well as the periods over which associated issuance costs will be amortized. Significant changes in forecasted net sales could materially impact each of the liability balances, interest expense and the time periods for repayment.

Income Taxes

The asset and 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.

Deferred tax assets are reduced by a valuation allowance when, based on the weight of available evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. In assessing the realizability of deferred tax assets, the Company considers all available positive and negative evidence, including historical operating results, forecasts of future taxable income, the reversal of taxable temporary differences and available tax planning strategies.

The Company accounts for uncertain tax positions in accordance with U.S. GAAP and recognizes the financial statement effects of a tax position only when it is more likely than not that the position will be sustained upon examination by the relevant taxing authority. The Company adjusts the amount of the liability to reflect any subsequent changes in the relevant facts and circumstances surrounding the uncertain tax positions. The Company recognizes interest and penalties related to income tax matters in income tax expense.

Foreign research and development costs are capitalized and amortized over a 15-year period in accordance with Section 174 of the Internal Revenue Code of 1986, as amended ("IRC"). The amortization period begins with the midpoint of any taxable year that IRC Section 174 costs are first incurred, regardless of whether the expenditures were made prior to or after July 1, and runs until the midpoint of year sixteen.

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

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law, making permanent certain provisions of the Tax Cuts and Jobs Act, including permanent 100% bonus depreciation, expensing of domestic research costs, and amendments to the business interest expense limitation. In accordance with ASC Topic 740, *Income Taxes,* the Company recognized the effects of the new tax law in the period of enactment. As the Company maintains a full valuation allowance on its U.S. federal deferred tax assets, the legislation did not have a material impact on its condensed consolidated financial statements**.**

Foreign Currency

The functional currency of each of the Company's foreign subsidiaries is primarily the local currency of the country in which the subsidiary operates. The Company's asset and liability accounts are translated at the exchange rate in effect as of the condensed consolidated balance sheet date. Revenue and expense accounts are translated at the average exchange rate for the period. Adjustments resulting from the translation of the financial statements of the Company's foreign subsidiaries into U.S. dollars are accumulated as a separate component of stockholders' deficit within accumulated other comprehensive (loss) income. Gains or losses resulting from transactions denominated in foreign currencies are included in foreign currency gains (losses), net, within the Condensed Consolidated Statement of Comprehensive Income (Loss).

Net Income (Loss) Per Share

Basic net income (loss) per share is calculated using the weighted average number of common shares outstanding during the period. Diluted net income (loss) per share includes the effect of potentially dilutive common shares outstanding during the period, as determined using the treasury stock method. Potentially dilutive common shares include shares that the Company could be obligated to issue from its outstanding stock-based compensation awards. In periods of net loss, all potential common shares would be anti-dilutive and therefore are excluded from the calculation of diluted net income (loss) per share.

Accumulated Other Comprehensive (Loss) Income

Accumulated other comprehensive (loss) 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' deficit. Realized gain and loss amounts on available-for-sale investments are reclassified from accumulated other comprehensive (loss) income and recorded as other expense, net in the Condensed Consolidated Statements of Comprehensive Income (Loss). There were no realized gains or losses reclassified out of accumulated other comprehensive (loss) income for the three and nine months ended September 30, 2026 and 2025.

Significant Customers and Other Risks

Significant Customers

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

ORLADEYO is generally distributed through an arrangement with a single specialty pharmacy in the United States. The specialty pharmacy subsequently sells ORLADEYO to its customers (pharmacy benefit managers, insurance companies, government programs and group purchasing organizations) who dispense product to patients. During the three months ended September 30, 2026, the Company transitioned to a new sole source specialty pharmacy for ORLADEYO shipments to patients in the United States. Under the new arrangement, the new specialty pharmacy purchases and holds inventory of ORLADEYO prior to dispensing to patients, whereas under the previous arrangement, the previous specialty pharmacy did not hold inventory prior to dispensing.

For the three and nine months ended September 30, 2025, ORLADEYO and peramivir were distributed through the same specialty pharmacy in the United States, which accounted for approximately 89% and 87% of total product sales for the three and nine months ended September 30, 2025, respectively. For the three and nine months ended September 30, 2026, ORLADEYO was distributed through two specialty pharmacies as a result of the transition to the new sole source specialty pharmacy for ORLADEYO shipments to patients in the United States in the third quarter of 2026. The previous specialty pharmacy accounted for approximately 53% and 80% of total product sales for the three and nine months ended September 30, 2026, respectively, and the new specialty pharmacy accounted for approximately 43% and 16% of total product sales for the three and nine months ended September 30, 2026, respectively.

Risks from Third-Party Manufacturing and Distribution Concentration

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

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

Credit Risk

Financial instruments that potentially subject the Company to concentrations of credit risk consist of cash and cash equivalents, investments, and trade receivables. The Company maintains its cash and cash equivalents with high-credit quality financial institutions in the United States. Such amounts may exceed federally-insured limits. 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 trade receivables from sales of ORLADEYO are primarily due from its previous specialty pharmacy and new specialty pharmacy, resulting in a concentration of credit risk. As of September 30, 2026, the previous and new specialty pharmacies accounted for 24% and 68% of trade receivables, net, respectively. As of December 31, 2025, the previous specialty pharmacy accounted for 84% of trade receivables, net.

Recently Adopted Accounting Pronouncements

In September 2025, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2025-06, *Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): Targeted Improvements to the Accounting for Internal-Use Software*, which clarifies and modernizes the accounting for costs related to internal-use software, eliminating references to project stages and clarifying the threshold entities apply to begin capitalizing costs. ASU 2025-06 is effective for fiscal years beginning after December 15, 2027, and interim periods within those annual reporting periods, with early adoption permitted. The Company adopted ASU 2025-06 as of January 1, 2026. The adoption

of this standard did not have a material effect on the Company's Condensed Consolidated Balance Sheet, Condensed Consolidated Statement of Comprehensive Income (Loss), or Condensed Consolidated Statement of Cash Flows.

New Accounting Pronouncements Not Yet Adopted

In November 2024, the FASB issued ASU 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*, which requires public entities, on an annual and interim basis, to provide disaggregated disclosure of certain income statement expenses into specified categories within the footnotes to the financial statements. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027, with early adoption permitted. The Company expects adoption of ASU 2024-03 will result in additional disclosures and is currently evaluating the impact that this ASU will have on its disclosures. The Company does not expect the adoption of ASU 2024-03 to have a material effect on its Condensed Consolidated Balance Sheet, Condensed Consolidated Statement of Comprehensive Income (Loss), or Condensed Consolidated Statement of Cash Flows.

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

Note 2 — Acquisition of Astria Therapeutics, Inc.

On October 14, 2025, the Company, Axel Merger Sub, Inc., a Delaware corporation and a wholly owned subsidiary of the Company ("Merger Sub"), and Astria Therapeutics, Inc., a Delaware corporation ("Astria"), entered into an Agreement and Plan of Merger (the "Merger Agreement"). Pursuant to the Merger Agreement, on January 23, 2026 (the "Closing Date"), the Company completed the acquisition of all of the outstanding equity of Astria and Merger Sub merged with and into Astria, with Astria surviving as a wholly owned subsidiary of the Company (the "Merger").

Under the terms of the Merger Agreement, at the effective time of the Merger (the "Effective Time"), each share of Astria common stock, par value $0.001 per share, issued and outstanding immediately prior to the Effective Time was converted into the right to receive (i) 0.59 of a share of the Company's common stock, par value $0.01 per share (and, if applicable, cash in lieu of fractional shares), and (ii) $8.55 in cash, without interest, subject to certain adjustments and applicable withholding taxes. Holders of Astria's Series X Convertible Preferred Stock, warrants, and certain options were also entitled to certain consideration, as further set forth in the Merger Agreement.

The Company accounted for the Merger as an asset acquisition in accordance with ASC Topic 805, *Business Combinations,* as substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable asset, the in-process research and development asset related to navenibart (the "IPR&D – navenibart" asset). Accordingly, the total cost of the asset acquisition, including direct transaction costs incurred by the Company in connection with the Merger, was allocated to the assets acquired and liabilities assumed on a relative fair value basis.

Consideration Transferred and Purchase Price Allocation

The following is a summary of the total consideration transferred and allocation of consideration transferred to the assets acquired, liabilities assumed, and IPR&D – navenibart in connection with the Merger:

Equity consideration[1]	$	251,655
Cash consideration		608,633
Direct transaction costs		14,025
Total purchase price	$	874,313
Assets acquired and liabilities assumed:		
Cash and cash equivalents	$	130,588
Short-term investments		63,033
Trade receivables		1,980
Prepaid expenses and other current assets		5,547
Property and equipment		642
Other assets		14,417
Right of use asset		4,198
IPR&D - navenibart		705,267
Assembled workforce		600
Accounts payable		(22,627)
Accrued expenses		(10,146)
Operating lease liabilities, current		(1,249)
Deferred revenue, current		(4,598)
Operating lease liabilities, net of current portion		(2,949)
Deferred revenue, net of current portion		(10,390)
Net assets acquired	$	874,313

[1] Consists of the issuance of 37,282 shares of the Company's common stock multiplied by the Company's common stock closing price of $6.75 per share on January 22, 2026.

The entire amount allocated to the IPR&D – navenibart asset was expensed in accordance with ASC Topic 730, *Research and Development*. Of the total value ascribed to the IPR&D – navenibart asset, $7,506 related to direct transaction costs incurred prior to the transaction close and were recorded in selling, general and administrative expense in the Consolidated Statement of Comprehensive Income (Loss) for the year ended December 31, 2025. The remaining value ascribed to the IPR&D – navenibart asset of $697,761 was recognized in acquired in-process research and development in the Condensed Consolidated Statement of Comprehensive Income (Loss) for the nine months ended September 30, 2026. The amount allocated to the assembled workforce was amortized over two months to reflect the estimated benefit derived from that asset which resulted in amortization expense of $600 being recognized in research and development expense in the Condensed Consolidated Statement of Comprehensive Income (Loss) for the nine months ended September 30, 2026.

Astria Stock Options

In connection with the Merger, the vesting of outstanding in-the-money Astria stock options was accelerated and the options were cancelled in exchange for a cash payment equal to the product of the difference between $13.00 and the option's exercise price and the number of shares underlying the option, for total consideration of $43,403. Options with an exercise price equal to or exceeding $13.00 were cancelled for no consideration. The total consideration was allocated between the portion attributable to pre-combination service, not to exceed the fair value of the underlying options, and the portion attributable to post-combination service. The amount attributable to pre-combination service was measured based on the fair value of the options immediately prior to cash settlement, totaling $14,274, and was included in the total purchase price as cash consideration. The remaining $29,129 was attributable to post-combination service, of which $10,709 was recognized in research and development expense and $18,420 was recognized in selling, general and

administrative expense in the Condensed Consolidated Statement of Comprehensive Income (Loss) for the nine months ended September 30, 2026.

Separation Costs

Concurrent with the Closing Date, the Company entered into separation agreements with certain Astria employees. The Company recognized $11,927 of costs associated with the separation agreements during the nine months ended September 30, 2026, of which $3,954 was recognized in research and development expense and $7,973 was recognized in selling, general and administrative expense in the Condensed Consolidated Statement of Comprehensive Income (Loss) for the nine months ended September 30, 2026. The following table summarizes the accrued liability activity recorded in connection with the separation agreements for the nine months ended September 30, 2026:

Balance at December 31, 2025	$ —
Workforce reduction expense recorded during the three months ended March 31, 2026	11,927
Amounts paid during the three months ended March 31, 2026	(2,871)
Balance at March 31, 2026	$ 9,056
Amounts paid during the three months ended June 30, 2026	(3,900)
Balance at June 30, 2026	$ 5,156
Amounts paid during the three months ended September 30, 2026	(2,670)
Balance at September 30, 2026	$ 2,486

The Company does not expect to incur any additional significant costs related to the separation agreements. The remaining unpaid costs are expected to be disbursed by September 30, 2027.

Note 3 — Birmingham Closure

On June 29, 2026, the Company announced its plan to discontinue its internal discovery programs and close its Discovery Center of Excellence facility (the "Birmingham facility") in Birmingham, Alabama (the "Birmingham Closure Plan"). The Company recognized the following one-time expenses in connection with the Birmingham Closure Plan during the three and nine months ended September 30, 2026:

	Three and Nine Months Ended September 30, 2026
Employee termination benefits and severance[(1)]	$ 7,058
Property and equipment impairment	1,526
Leasehold improvement impairment	4,733
Right of use asset impairment	3,279
Total	$ 16,596

[(1)] Includes stock-based compensation expense for certain impacted employees.

The Company expects activities related to the Birmingham Closure Plan to be substantially completed by December 31, 2026, except for ongoing obligations under the Birmingham facility lease.

Separation Costs

In connection with the Birmingham Closure Plan, the Company entered into separation agreements with certain employees. The Company notified all impacted employees in August 2026, and the majority of the impacted employees have planned termination dates in 2026, with certain employees exiting in the first quarter of 2027. The Company recognized $6,061 of costs associated with the separation agreements during the three and nine months ended September 30, 2026, of which $5,150 was recognized in research and development expenses and $911 was recognized in selling, general and administrative expenses in the Condensed Consolidated Statements of Comprehensive Income (Loss). As of September 30, 2026, $1,457 of the costs were paid and the remaining $4,604 is included within accrued expenses in the Condensed Consolidated Balance Sheet.

In addition, the Company recognized incremental stock-based compensation expense of $997 during the three and nine months ended September 30, 2026 related to modifications of equity awards for certain impacted employees.

The following table summarizes the accrued liability activity recorded in connection with the separation agreements for the three months ended September 30, 2026:

Balance at June 30, 2026	$ —
Separation costs recorded during the three months ended September 30, 2026	6,061
Amounts paid during the three months ended September 30, 2026	(1,457)
Balance at September 30, 2026	$ 4,604

The remaining unpaid costs are expected to be disbursed by September 30, 2027.

Property and Equipment

In connection with the Birmingham Closure Plan, the Company entered into agreements to sell the owned property and equipment associated with the Birmingham facility. As of September 30, 2026, the related disposal group met the criteria for classification as held for sale under Topic 360. Accordingly, an impairment charge of $1,526 was recognized in research and development expense in the Condensed Consolidated Statements of Comprehensive Income (Loss) during the three and nine months ended September 30, 2026 to reduce the carrying value of the disposal group to its fair value less costs to sell. As of September 30, 2026, the property and equipment disposal group was classified as held for sale in the Condensed Consolidated Balance Sheet.

Right of Use Assets

The Company has lease arrangements associated with its Birmingham facility and certain equipment. As part of the Birmingham Closure Plan, the Company committed to a plan to vacate the Birmingham facility and cease use of the leased equipment. As a result of the decision to vacate the facility, the Company was no longer reasonably certain to exercise the optional extension periods under the facility lease. In accordance with Topic 842, the Company reassessed the facility lease term to exclude the optional extension periods and remeasured the related lease liability using the revised lease term and discount rate, resulting in a $2,827 reduction to both the right of use asset and lease liability as of September 30, 2026.

Following the lease remeasurement, the planned actions were also determined to be indicators of impairment under Topic 360. Accordingly, the Company evaluated the facility asset group, which included the facility right of use asset and related leasehold improvements, for recoverability and concluded that its carrying amount was not recoverable. The carrying amounts of the assets were therefore reduced to their estimated fair values of zero. The Company also evaluated the right of use assets associated with the leased equipment for impairment and reduced their carrying amounts to their estimated fair values of zero. As a result, the Company recognized impairment charges of $3,279 related to right of use assets and $4,733 related to leasehold improvements in research and development expense in the Condensed Consolidated Statements of Comprehensive Income (Loss) during the three and nine months ended September 30, 2026.

Note 4 — Revenue

The Company recorded the following revenues for the three and nine months ended September 30, 2026 and 2025:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
ORLADEYO	$ 174,833	$ 159,077	$ 481,382	$ 450,157
License and other revenues	5,459	318	73,573	18,125
Total revenues	$ 180,292	$ 159,395	$ 554,955	$ 468,282

ORLADEYO revenue includes product sales and royalties. License and other revenues includes revenue recognized related to the Kaken License Agreement and Neopharmed License Agreement (see "*Note 12—Collaborative and Other*

Relationships"), quarterly royalty payments from BioCryst Ireland Limited (see "*Note 7—Royalty Financing Obligations"*), and product sales and royalty revenue related to peramivir.

Revenues from customers in the U.S. represented approximately 96% of ORLADEYO revenues for the three and nine months ended September 30, 2026. Revenues from customers in the U.S. represented approximately 89% of ORLADEYO revenues for the three and nine months ended September 30, 2025.

Note 5 — Fair Value Measurements and Investments

Fair Value Measurements

Fair value is an exit price, representing the amount that would be received from the sale of an asset or paid to transfer a liability in an orderly transaction between market participants. Fair value is determined based on observable and unobservable inputs. Observable inputs reflect readily obtainable data from independent sources, while unobservable inputs reflect certain market assumptions. As a basis for considering such assumptions, U.S. GAAP establishes a three-tier value hierarchy, which prioritizes the inputs used to develop the assumptions and for measuring fair value as follows:

- Level 1 — observable inputs such as quoted prices in active markets for identical assets;
- Level 2 — inputs other than the quoted prices in active markets that are observable either directly or indirectly; and
- Level 3 — unobservable inputs for which there is little or no market data, which requires the Company to develop its own assumptions.

This hierarchy requires the Company to use observable market data, when available, and to minimize the use of unobservable inputs when determining fair value.

Assets measured at fair value on a recurring basis were as follows:

	September 30, 2026			
	Quoted Price in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Cash equivalents:				
Money market funds	$ 55,108	$ —	$ —	$ 55,108
Total cash equivalents	55,108	—	—	55,108
Available-for-sale investments:				
Obligations of U.S. Government and its Agencies	—	278,027	—	278,027
Total available-for-sale investments	—	278,027	—	278,027
Total assets measured at fair value	$ 55,108	$ 278,027	$ —	$ 333,135

	December 31, 2025			
	Quoted Price in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Cash equivalents:				
Money market funds	$ 35,800	$ —	$ —	$ 35,800
Total cash equivalents	35,800	—	—	35,800
Available-for-sale investments:				
Obligations of U.S. Government and its Agencies	—	246,175	—	246,175
Total available-for-sale investments	—	246,175	—	246,175
Total assets measured at fair value	$ 35,800	$ 246,175	$ —	$ 281,975

Investments

The following tables summarize the fair value of the Company's investments by type:

	September 30, 2026				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its Agencies	$ 277,654	$ 695	$ 7	$ (329)	$ 278,027
Total investments	$ 277,654	$ 695	$ 7	$ (329)	$ 278,027

	December 31, 2025				
	Amortized Cost	Accrued Interest	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Obligations of U.S. Government and its Agencies	$ 244,455	$ 1,491	$ 234	$ (5)	$ 246,175
Total investments	$ 244,455	$ 1,491	$ 234	$ (5)	$ 246,175

The Company's investments consist of fixed income securities whose 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.

As of September 30, 2026, the Company had 30 securities with a total estimated fair value of $243,583 in an unrealized loss position. The unrealized losses were primarily attributable to changes in market interest rates and not to deterioration in the credit quality of the issuers. The Company does not have an intent to sell these investments, and it is more likely than not that the investments will be held until recovery of their amortized cost basis. As such, no allowance was recognized.

The following table summarizes the scheduled maturity for the Company's investments at September 30, 2026 and December 31, 2025:

	September 30, 2026	December 31, 2025
Maturing in one year or less	$ 278,027	$ 185,011
Maturing after one year through two years	—	61,164
Total investments	$ 278,027	$ 246,175

Note 6 — Inventory

At September 30, 2026 and December 31, 2025, the Company's inventory related to ORLADEYO and peramivir consisted of the following:

	September 30, 2026	December 31, 2025
Raw materials	$ 10,712	$ 9,997
Work-in-process	29,986	12,891
Finished goods	5,490	7,275
Total inventory	46,188	30,163
Reserves	(1,177)	(775)
Total inventory, net	$ 45,011	$ 29,388

Note 7 — Royalty Financing Obligations

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

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

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

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

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

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

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

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

Under the 2020 RPI Royalty Purchase Agreement, the Company is required to make royalty payments of amounts owed to RPI each calendar quarter following the first commercial sale of ORLADEYO in any country. Under the 2021 RPI Royalty Purchase Agreement, the Company is required to make payments to RPI in respect of net sales or sublicense revenue in each calendar quarter from and after October 1, 2021. Under the OMERS Royalty Purchase Agreement, the Company is required to make payments to OMERS in respect of net sales or sublicense revenue in each calendar quarter from and after October 1, 2023. OMERS will no longer be entitled to receive any payments on the date in which aggregate payments actually received by OMERS equals 155.0% of the $150,000 purchase price. As of September 30, 2026, cumulative royalties paid and payable under the OMERS Royalty Purchase Agreement were $128,160.

Under the Royalty Purchase Agreements, the Company has agreed to specified affirmative and negative covenants, including covenants regarding periodic reporting of information by the Company to RPI and OMERS, third-party audits of royalties paid under the Royalty Purchase Agreements, and restrictions on the ability of the Company or any of its subsidiaries to incur indebtedness other than certain royalty sales and as was permitted to be incurred under the terms of the Pharmakon Loan Agreement (as defined in Note 8 herein) through its payoff and termination on October 8, 2025 or, subsequent to that date, the Blackstone Loan Agreement (as defined in Note 8 herein), as applicable. See "*Note 8—Debt*" for further details on the Pharmakon Loan Agreement and Blackstone Loan Agreement. The restrictions under the Royalty Purchase Agreements on the ability of the Company or any of its subsidiaries to incur indebtedness are eliminated after the achievement of certain specified milestones in the Royalty Purchase Agreements.

In connection with the Stock Purchase Agreement, RPI and OMERS provided their written consent to the consummation of the sale of the Company's European ORLADEYO business. Concurrent with the closing of the transaction on October 1, 2025, Neopharmed paid a $15,000 royalty release fee to RPI on behalf of the Company and the Company paid a $500 royalty release fee to OMERS. The payments were accounted for as a debt modification and the royalty release fees, totaling $15,500, were capitalized as a reduction to royalty financing obligations and are being amortized as interest expense over the terms of the arrangements using the effective interest rate method. In accordance with the Stock Purchase Agreement, the Company receives quarterly royalty payments from BioCryst Ireland equal to amounts owed under its Royalty Purchase Agreements with RPI and OMERS for the sale of ORLADEYO products in the Territory. During the three and nine months ended September 30, 2026, the Company recognized $1,322 and $7,211, respectively, in license and other revenues in the Condensed Consolidated Statements of Comprehensive Income (Loss) related to the quarterly royalty payments from BioCryst Ireland.

The cash consideration received pursuant to the Royalty Purchase Agreements was recorded as royalty financing obligations in the Company's Condensed Consolidated Balance Sheets. Deferred financing costs, which consisted primarily of advisory and legal fees, were capitalized as a reduction to royalty financing obligations and are amortized using the effective interest method over the terms of the arrangements. At inception, the royalty financing obligations were measured at fair value based on the Company's estimates of future royalties expected to be paid to the counterparties over the terms of the arrangements. The Company subsequently records the obligations using the effective interest method. As of September 30, 2026 and December 31, 2025, the carrying values of the royalty financing obligations approximated their fair values and were measured based on the Company's current estimates of future payments to RPI and OMERS over the lives of the arrangements, which are considered Level 3 inputs. The Company utilizes the prospective method to account for changes in estimated future royalty payments. Under the prospective method, revised estimates of future cash flows result in the determination of new effective interest rates that equate the present value of the revised estimated remaining

cash flows with the carrying amounts of the royalty financing obligations. The revised effective interest rate is used prospectively to recognize interest expense for the remaining periods.

The Company periodically assesses the amount and timing of expected royalty payments based on internal projections of future net product sales, which are based on key assumptions, including paid patients and price. If projected royalty payments for the next twelve months exceed interest accretion for the same period, the excess is classified in the royalty financing obligations current liability in the Company's Condensed Consolidated Balance Sheets.

The following table shows the royalty financing obligations activity for the three and nine months ended September 30, 2026, as well as the effective interest rate as of September 30, 2026:

	2020 RPI Royalty Agreement	2021 RPI Royalty Agreement	OMERS Royalty Agreement	Total
Balance as of December 31, 2025	$ 169,559	$ 172,671	$ 123,458	$ 465,688
Non-cash interest expense on royalty financing obligations	10,111	—	3,183	13,294
Royalty revenues payable	(14,132)	(1,220)	(16,132)	(31,484)
Balance as of March 31, 2026	$ 165,538	$ 171,451	$ 110,509	$ 447,498
Non-cash interest expense on royalty financing obligations	10,159	—	2,877	13,036
Royalty revenues payable	(15,148)	(1,309)	(17,288)	(33,745)
Balance as of June 30, 2026	$ 160,549	$ 170,142	$ 96,098	$ 426,789
Non-cash interest expense on royalty financing obligations	9,843	—	2,496	12,339
Royalty revenues payable	(6,810)	(3,169)	(7,466)	(17,445)
Balance as of September 30, 2026	$ 163,582	$ 166,973	$ 91,128	$ 421,683
Effective interest rate	24.0 %	— %	10.3 %	

Cash paid for interest on the royalty financing obligations was $18,859 and $45,647 for the three and nine months ended September 30, 2026, respectively. Cash paid for interest on the royalty financing obligations was $18,319 and $55,240 for the three and nine months ended September 30, 2025, respectively.

Note 8 — Debt

Blackstone Loan Agreement

On January 23, 2026, the Company entered into a Loan Agreement (the "Blackstone Loan Agreement") with Blackstone Alternative Credit Advisors LP and Blackstone Life Sciences Advisors L.L.C. (together, "Blackstone"), the guarantors and lenders from time to time party thereto, and Wilmington Trust, National Association, as agent, pursuant to which the Company borrowed $400,000 in aggregate principal amount of term loans (the "Term Loans"). The Term Loans were funded on January 23, 2026 (the "Term Loans Closing Date"), and the proceeds were used to fund the cash portion of the consideration for the acquisition of Astria (see "*Note 2—Acquisition of Astria Therapeutics, Inc.*"). Subject to the mutual agreement between the Company, Blackstone and the lenders, the Company may request additional term loans of up to $150,000 in the aggregate.

The Term Loans mature on January 23, 2031 (the "Maturity Date") and require quarterly interest-only payments until the Maturity Date, with outstanding principal due at maturity. Interest accrues at a rate equal to the three-month Secured Overnight Financing Rate ("SOFR"), subject to a floor of 1.75%, plus 4.50% per annum.

Prior to the second anniversary of the Term Loans Closing Date, and provided that no event of default is ongoing, the Company may elect to pay a portion of the applicable interest payment in-kind (a "Blackstone PIK Interest Payment") by capitalizing as principal up to 200 basis points of interest accrued during the applicable interest period, subject to an incremental margin increase of 0.50% per annum on the capitalized portion.

The Company is required to make a mandatory prepayment of the Term Loans (i) upon the occurrence of a change of control, (ii) upon the incurrence of certain non-permitted indebtedness, (iii) upon the occurrence of certain asset sales (subject to certain exceptions), or (iv) upon the occurrence of certain events of loss related to the assets of the Company or its subsidiaries (subject to certain exceptions). The Company may make voluntary prepayments in whole or in part in an aggregate principal amount of $5,000 or any whole multiple of $1,000 in excess thereof. Prepayments are subject to a prepayment premium that declines over time.

The Blackstone Loan Agreement contains representations and warranties and affirmative and negative covenants customary for financings of this type (including a minimum liquidity covenant), as well as customary events of default. A failure to comply with the covenants in the Blackstone Loan Agreement, or an occurrence of any other event of default, could permit the lenders under the Blackstone Loan Agreement to declare the borrowings thereunder, together with accrued interest and fees, and any applicable prepayment premium, to be immediately due and payable.

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

As of September 30, 2026, the Company had total borrowings of $400,000 under the Blackstone Loan Agreement. Interest expense accrued on the Term Loans for the three and nine months ended September 30, 2026 totaled $8,419 and $22,884, respectively, of which $22,789 was paid during the nine months ended September 30, 2026, and $95 was included in accrued expenses in the Condensed Consolidated Balance Sheet as of September 30, 2026.

Debt fees and issuance costs incurred for the Term Loans under the Blackstone Loan Agreement totaled $4,952 and are being amortized as interest expense using the effective interest rate method over the remaining term of the Term Loans. Deferred financing amortization of $209 and $561 was recognized for the three and nine months ended September 30, 2026, respectively.

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

Pharmakon Loan Agreement

On April 17, 2023, the Company entered into a $450,000 Loan Agreement (the "Pharmakon Loan Agreement") with BioPharma Credit Investments V (Master) LP and BPCR Limited Partnership, as lenders, and BioPharma Credit PLC, as collateral agent for the lenders. Certain of the Company's wholly owned subsidiaries were guarantors to the Pharmakon Loan Agreement. The Pharmakon Loan Agreement provided for an initial term loan in the principal amount of $300,000 (the "Tranche A Loan") funded on April 17, 2023 (the "Tranche A Closing Date"). The Company used a portion of the proceeds from the Tranche A Loan to repay the $241,787 of outstanding indebtedness (principal and interest due as of April 17, 2023) under the then-existing credit facility with Athyrium Opportunities III Co-Invest 1 LP (the "Athyrium Credit Agreement") and to pay associated transaction costs and fees, and used the remaining net proceeds of $25,805 for other general corporate purposes.

The Pharmakon Loan Agreement provided for quarterly interest-only payments until the maturity date of April 17, 2028, with the unpaid principal amount of the outstanding Tranche A Loan due and payable on the maturity date. During the first 18 months following the Tranche A Closing Date, the Company had the option to make a portion of the applicable interest payment on the Tranche A Loan in-kind (a "Pharmakon PIK Interest Payment") by capitalizing as principal up to 50% of the amount of interest accrued on the Tranche A Loan during the applicable interest period. The Tranche A Loan bore interest at a rate equal to the three-month SOFR, which could be no less than 1.75%, plus 7.00%, per annum or, for each interest period in which a Pharmakon PIK Interest Payment was made, with respect to the Tranche A Loan, SOFR plus 7.25%, per annum.

Interest expense on the Tranche A Loan for the three and nine months ended September 30, 2025 was $6,110 and $22,789, respectively, all of which was paid at the end of each quarterly period. Deferred financing amortization for the three and nine months ended September 30, 2025 was $384 and $1,387, respectively.

On April 18, 2025, the Company made a $75,000 partial prepayment on the outstanding principal amount under the Tranche A Loan. In conjunction with the partial prepayment, the Company incurred a $2,250 prepayment premium and paid $424 of interest accrued through the payment date. Additionally, unamortized deferred financing costs of $1,921 associated with the Tranche A Loan were written-off at the time of repayment. Collectively, the prepayment and

unamortized deferred financing costs totaled $4,171 and were reflected as a one-time loss on extinguishment of debt in the Condensed Consolidated Statements of Comprehensive Income (Loss) for the nine months ended September 30, 2025.

On July 24, 2025, the Company made a $50,000 partial prepayment on the outstanding principal amount under the Tranche A Loan. In conjunction with the partial prepayment, the Company incurred a $1,500 prepayment premium and fees of $52 and paid $376 of interest accrued through the payment date. Additionally, unamortized deferred financing costs of $1,188 associated with the Tranche A Loan were written-off at the time of repayment. Collectively, the prepayment and unamortized deferred financing costs totaled $2,740 and are reflected as a one-time loss on extinguishment of debt on the Condensed Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2025.

The Company paid off in full the remainder of the outstanding principal balance on the Tranche A Loan of $198,704 as of September 30, 2025 in a final prepayment in the fourth quarter of 2025.

Note 9 — Lease Obligations

The Company leases certain assets under operating leases, which primarily consist of real estate leases, and finance leases, which generally consist of laboratory equipment leases and office equipment leases. The Company's real estate agreements expire at various times between 2028 and 2033 and its Durham lease includes a five-year renewal option.

Lease expense under operating and finance leases was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating lease expense	$ 753	$ 526	$ 2,240	$ 1,596
Finance lease expense:				
Amortization of right of use assets	$ 302	$ 547	$ 1,045	$ 1,639
Interest on lease liabilities	45	84	164	268
Total finance lease expense	$ 347	$ 631	$ 1,209	$ 1,907

Other supplemental information related to leases was as follows:

	September 30, 2026	December 31, 2025
Weighted average remaining lease term:		
Operating leases	4.0 years	9.4 years
Finance leases	1.8 years	2.3 years
Weighted average discount rate:		
Operating leases	8.3 %	10.7 %
Finance leases	10.1 %	9.8 %

Maturities of lease liabilities as of September 30, 2026 are as follows:

	Operating Leases	Finance Leases
2026 (remaining)	$ 791	$ 325
2027	3,102	991
2028	3,037	458
2029	1,538	26
2030	1,092	—
Thereafter	1,491	—
Total lease payments	11,051	1,800
Less imputed interest	(1,790)	(154)
Total	$ 9,261	$ 1,646

Supplemental cash flow information related to leases was as follows:

	Nine Months Ended September 30,	
	2026	2025
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows for finance leases	$ 164	$ 268
Operating cash flows for operating leases	$ 1,875	$ 1,347
Operating lease assets obtained in exchange for operating lease liabilities	$ 4,198	$ 366
Finance lease assets obtained in exchange for finance lease liabilities	$ —	$ 870
Non-cash (decrease) increase to operating lease assets due to remeasurement of operating lease liabilities	$ (2,827)	$ 786
Non-cash decrease to finance lease assets due to remeasurement of finance lease liabilities	$ (175)	$ —

Note 10 — Stockholders' Equity

Issuance of Common Stock

On November 20, 2025, the Company filed with the SEC, and amended on December 15, 2025, a registration statement on Form S-4. This registration statement was declared effective by the SEC on December 18, 2025 and registered the Company's offer of up to 45,000 shares of the Company's common stock in connection with the Merger (as defined in "*Note 2—Acquisition of Astria Therapeutics, Inc."* herein).

On January 23, 2026, the Company issued 37,282 shares of common stock to Astria's equity holders in connection with the Merger (see "*Note 2—Acquisition of Astria Therapeutics, Inc."*).

Shares Reserved for Future Issuance of Common Stock

The Company had reserved shares of common stock for issuance as follows:

	September 30, 2026	December 31, 2025
Shares reserved for exercises of outstanding stock options	36,434	43,344
Shares reserved for vesting of restricted stock units	12,172	12,166
Shares reserved for future issuance under the Stock Incentive Plan	15,785	6,852
Shares reserved for future issuance under the Inducement Equity Incentive Plan	1,001	1,460
Shares reserved for future issuance under the Employee Stock Purchase Plan	4,327	4,674
Total shares reserved for future issuance	69,719	68,496

Note 11 — Stock-Based Compensation

As of September 30, 2026, 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 20, 2026 and approved by the Company's stockholders on June 11, 2026. The Inducement Plan was most recently amended and restated by the Company's Board of Directors on October 26, 2023. The ESPP was most recently amended and restated by the Company's Board of Directors on July 7, 2023.

The Company recorded the following stock-based compensation expense:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Incentive Plan	$ 12,797	$ 16,232	$ 40,421	$ 54,841
Inducement Plan	1,248	2,179	4,049	5,852
ESPP	199	233	493	623
Total stock-based compensation costs	$ 14,244	$ 18,644	$ 44,963	$ 61,316
Capitalized stock-based compensation costs	—	(43)	—	(43)
Total stock-based compensation costs included in operating expenses	$ 14,244	$ 18,601	$ 44,963	$ 61,273

Extension of Exercise Period

In December 2025, the Company extended the post-termination exercise period of certain vested stock option awards at the time of retirement for certain individuals to the original expiration date of the option awards. The Company considered this to be a modification of existing awards under ASC Topic 718, *Compensation - Stock Compensation* ("Topic 718") and determined the incremental fair value associated with the modification using a binomial lattice model. In connection with the modification, the Company recognized incremental stock-based compensation expense of $1,891 and $8,323 during the three and nine months ended September 30, 2026, respectively.

Sale of the European ORLADEYO Business

In connection with the sale of the European ORLADEYO business on October 1, 2025, the Company modified certain outstanding stock option awards and restricted stock unit awards held by employees who transferred to Neopharmed. Under the original terms of the Company's Incentive Plan and Inducement Plan, unvested awards would have been forfeited upon the employees' termination with the Company at closing. As part of the transaction, the Company approved modifications that (i) allowed previously unvested awards to continue to vest based on continued service to Neopharmed after closing and (ii) extended the post-termination exercise period for certain vested stock option awards.

The modified awards vest, and for certain vested stock options remain exercisable, only if the employees remain employed by Neopharmed for specified periods following the closing of the transaction. Because the vesting of the

modified awards depends on service to Neopharmed, the awards contain an "other" condition under Topic 718 and are therefore classified as liability awards until the required service to Neopharmed is provided.

The Company accounted for the continued vesting of unvested awards as a Type III modification under Topic 718 and measured the awards at their modification-date fair value. The extension of the post-termination exercise period for certain vested stock option awards was accounted for as a Type I modification under Topic 718, with the incremental fair value measured on the modification date.

The liability for the modified awards is measured at fair value at each reporting period. The options are valued using a Black-Scholes option pricing model which incorporates significant unobservable inputs. This remeasurement resulted in the recognition of a gain of $2,986 and loss of $345 in other income, net in the Consolidated Statements of Comprehensive Income (Loss) for the three and nine months ended September 30, 2026, respectively, and a $536 increase and $144 decrease to additional paid-in capital during the three and nine months ended September 30, 2026, respectively.

The following is a reconciliation of the beginning and ending balances of recurring fair value measurements recognized in the Condensed Consolidated Balance Sheets using Level 3 inputs:

Balance at December 31, 2025	$	13,743
Remeasurement of share-based liability		3,444
Options exercised		(971)
Balance at March 31, 2026	$	16,216
Remeasurement of share-based liability		567
Options exercised		(423)
Balance at June 30, 2026	$	16,360
Remeasurement of share-based liability		(3,522)
Balance at September 30, 2026	$	12,838

Stock Incentive Plan

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

	Shares (in thousands)	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2025	38,664	$ 7.83		
Granted	1,015	9.01		
Exercised	(3,734)	5.93		$ 11,617
Cancelled or Forfeited	(2,882)	10.11		
Outstanding at September 30, 2026	33,063	$ 7.89	5.74	$ 36,712
Exercisable at September 30, 2026	20,968	$ 8.07	4.51	$ 24,347

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

	Shares (in thousands)	Weighted Average Grant Date Fair Value
Unvested at December 31, 2025	11,141	$ 7.40
Granted	796	8.60
Vested	(339)	9.30
Forfeited	(875)	7.20
Unvested at September 30, 2026	10,723	$ 7.47

For restricted stock unit awards granted under the Incentive Plan, the fair value of the awards is determined based on the market value of the Company's shares on the grant date. The weighted average grant date fair value of these awards granted during the nine months ended September 30, 2026 and 2025 was $8.60 and $9.17, 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 following table summarizes stock option activity under the Inducement Plan:

	Shares (in thousands)	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2025	4,680	$ 8.33		
Granted	353	7.37		
Exercised	(1,124)	5.21		$ 4,299
Cancelled or Forfeited	(538)	12.64		
Outstanding at September 30, 2026	3,371	$ 8.59	5.51	$ 5,566
Exercisable at September 30, 2026	2,337	$ 8.96	4.76	$ 4,411

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

	Shares (in thousands)	Weighted Average Grant Date Fair Value
Unvested at December 31, 2025	1,025	$ 8.17
Granted	810	7.96
Vested	(220)	9.24
Forfeited	(166)	7.70
Unvested at September 30, 2026	1,449	$ 7.94

For restricted stock unit awards granted under the Inducement Plan, the fair value of the awards is determined based on the market value of the Company's shares on the grant date. The weighted average grant date fair value of these awards granted during the nine months ended September 30, 2026 and 2025 was $7.96 and $8.47, 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.

Note 12 — Collaborative and Other Relationships

Navenibart

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

On August 6, 2025, Astria, which became a wholly owned subsidiary of the Company on January 23, 2026 (see "*Note 2—Acquisition of Astria Therapeutics, Inc.*"), entered into a license agreement with Kaken (the "Kaken License Agreement"), granting Kaken an exclusive license to develop, package, and commercialize navenibart (the "Navenibart Licensed Product in Japan") for the prevention of HAE attacks in Japan. The Kaken License Agreement included an upfront payment of $16,000, potential commercialization and sales milestone payments of $16,000, and tiered royalties ranging from 15% to 30% of annual net sales of the Navenibart Licensed Product in Japan. Under the terms of the Kaken License Agreement, Kaken will also provide support for the Phase 3 trial in Japan, is responsible for regulatory submissions in Japan, and will provide reimbursement for a portion of the costs associated with the navenibart Phase 3 program.

In accordance with Topic 606, two performance obligations were identified under the Kaken License Agreement: (i) the performance of research and development services and (ii) the delivery of the license. The Company re-evaluates the transaction price at the end of each reporting period and adjusts the estimate as changes in circumstances occur. As of September 30, 2026, the estimated transaction price was determined to be approximately $18,321, consisting of fixed consideration of $16,000 related to the upfront payment and variable consideration of $2,321 related to the estimated reimbursement of research and development services. Variable consideration is estimated each reporting period using the expected value method based on the most current forecasts. Potential milestone payments and sales-based royalties were excluded from the transaction price as they were constrained and will be recognized if and when the related milestones or sales occur.

As of September 30, 2026, the Company allocated $15,921 of the transaction price to the research and development services and the remaining $2,400 was allocated to delivery of the license. The fixed consideration was allocated to the performance obligations based on their relative standalone selling prices. The variable consideration was allocated to the performance obligation to which it is determined to be related, which is the reimbursement of research and development services to be incurred.

Revenue allocated to the research and development services is recognized over time using an input method based on costs incurred. Revenue allocated to the license will be recognized at the point in time at which control of the Navenibart Licensed Product in Japan is transferred to Kaken. During the three and nine months ended September 30, 2026, the Company recognized $1,635 and $4,089 of revenue related to the Kaken License Agreement, respectively, in license and other revenues in the Condensed Consolidated Statements of Comprehensive Income (Loss). As of September 30, 2026, the deferred revenue related to the upfront payment and cost-sharing reimbursements was $10,105, of which $4,117 is classified as current and $5,988 is classified as non-current in the Condensed Consolidated Balance Sheet.

Neopharmed

On May 4, 2026, the Company announced that it entered into a license agreement with an Irish affiliate of Neopharmed (the "Neopharmed License Agreement") granting Neopharmed exclusive rights to commercialize navenibart for the prevention of HAE attacks in specified territories in Europe (the "Navenibart Licensed Product in Europe"). The Neopharmed License Agreement included an upfront payment of $70,000, potential regulatory and sales milestone payments of $275,000, and tiered royalties ranging from 18% to 30% of annual net sales of the Navenibart Licensed Product in Europe.

In accordance with Topic 606, two performance obligations were identified under the Neopharmed License Agreement: (i) the delivery of the license and (ii) the performance of research and development services. The Company re-evaluates the transaction price at the end of each reporting period and adjusts the estimate as changes in circumstances occur. As of September 30, 2026, the estimated transaction price was determined to be $70,000, consisting of the upfront payment. Potential milestone payments and sales-based royalties were excluded from the transaction price as they were fully constrained and will be recognized if and when the related milestones or sales occur.

The Company allocated $54,513 of the transaction price to the delivery of the license and the remaining $15,487 was allocated to the research and development services. The transaction price was allocated to the performance obligations based on their relative standalone selling prices.

Revenue allocated to the license was recognized upon execution of the Neopharmed License Agreement, when control of the Navenibart Licensed Product in Europe transferred to Neopharmed. Revenue allocated to the research and development services is recognized over time using an input method based on costs incurred. During the three months ended September 30, 2026, the Company recognized $2,228 of revenue related to the performance of research and development services. During the nine months ended September 30, 2026, the Company recognized $57,957 of revenue, comprised of $54,513 related to the delivery of the license and $3,444 related to the performance of research and development services. Revenue recognized under the Neopharmed License Agreement is reflected in license and other revenues in the Condensed Consolidated Statements of Comprehensive Income (Loss). As of September 30, 2026, the deferred revenue related to the upfront payment was $12,044, of which $6,418 is classified as current and $5,626 is classified as non-current in the Condensed Consolidated Balance Sheet.

Note 13 — Workforce Reduction

2026 Workforce Reduction

In September 2026, the Company notified employees impacted by a workforce reduction. The majority of the impacted employees had termination dates in October 2026, with certain employees having termination dates in the first quarter of 2027.

The Company recognized $3,374 of costs related to this workforce reduction during the three and nine months ended September 30, 2026, of which $665 was recognized in research and development expenses and $2,709 was recognized in selling, general and administrative expenses in the Condensed Consolidated Statements of Comprehensive Income (Loss). The following table summarizes the accrued liability activity recorded in connection with the workforce reduction for the three months ended September 30, 2026:

Balance at June 30, 2026	$	—
Workforce reduction expense recorded during the three months ended September 30, 2026		3,374
Amounts paid during the three months ended September 30, 2026		—
Balance at September 30, 2026	$	3,374

The Company does not expect to incur any additional significant costs related to this workforce reduction. The remaining unpaid costs are expected to be disbursed by September 30, 2027.

2025 Workforce Reduction

In December 2025, the Company notified employees impacted by a workforce reduction. The majority of the impacted employees had termination dates in December 2025, and certain employees had termination dates in the first quarter of 2026.

The Company recognized $6,314 of costs related to the workforce reduction during the year ended December 31, 2025. As of September 30, 2026, all of the costs were paid and the Company does not expect to incur any additional significant costs related to this workforce reduction.

The following table summarizes the accrued liability activity recorded in connection with the workforce reduction for the nine months ended September 30, 2026:

Balance at December 31, 2025	$	5,478
Amounts paid during the three months ended March 31, 2026		(4,882)
Balance at March 31, 2026	$	596
Amounts paid during the three months ended June 30, 2026		(532)
Balance at June 30, 2026	$	64
Amounts paid during the three months ended September 30, 2026		(64)
Balance at September 30, 2026	$	—

Note 14 — Segment Information

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

The CODM assesses performance and decides how to allocate resources based on consolidated net income (loss). This measure is used to monitor budget versus actual results to evaluate the performance of the segment. The CODM uses consolidated cash, cash equivalents and investments as the measure of segment assets. As of September 30, 2026 and December 31, 2025, the Company's cash, cash equivalents, and investments were $374,998 and $335,911, respectively.

The following table illustrates information about segment revenues, significant segment expenses, and segment net income (loss) for the three and nine months ended September 30, 2026 and 2025:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenues	$ 180,292	$ 159,395	$ 554,955	$ 468,282
Less[1]:				
Cost of product sales	1,574	2,187	10,791	9,553
Acquired in-process research and development	—	—	697,761	—
Research and development (excluding stock-based compensation)				
Navenibart	17,995	—	45,064	—
BCX17725	3,772	7,574	10,919	13,346
Berotralstat	683	2,575	4,090	9,214
Avoralstat	(5)	1,800	1,239	7,584
Research, discovery and preclinical programs	147	5,493	8,332	12,095
Compensation and related personnel costs	13,777	13,313	52,258	38,025
Other non-program specific and indirect costs	16,079	7,329	30,542	20,621
Sales and marketing (excluding stock-based compensation)	37,726	44,114	114,626	137,373
General and administrative (excluding stock-based compensation)	20,970	26,818	84,173	78,594
Stock-based compensation	14,244	18,601	44,963	61,273
Interest income	(3,073)	(2,238)	(7,763)	(7,778)
Interest expense	21,185	19,661	62,676	64,737
Foreign currency (gains) losses, net	(22)	(35)	176	27
Loss on extinguishment of debt	—	2,740	—	6,911
Other income, net	(3,345)	(2,667)	(2,143)	(2,667)
Income tax expense (benefit)	669	(769)	2,752	1,358
Consolidated net income (loss)	**$ 37,916**	**$ 12,899**	**$ (605,501)**	**$ 18,016**

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

All material long-lived assets of the Company reside in the U.S. For geographic information about the Company's product revenues, see "*Note 4— Revenue*".

Note 15 — Commitments and Contingencies

Abbreviated New Drug Application

In January 2025, the Company received a Paragraph IV notice of certification (the "First Notice Letter") from Annora Pharma Private Limited ("Annora") regarding U.S. Patent Nos. 10,662,160; 11,117,867; and 11,618,733. In January 2026, the Company received an additional Paragraph IV notice of certification (the "Second Notice Letter" and, together with the First Notice Letter, the "Notice Letters") from Annora regarding U.S. Patent No. 12,344,585. The Notice Letters advise that Annora has submitted an Abbreviated New Drug Application ("ANDA") to the U.S. Food and Drug Administration ("FDA") seeking approval to manufacture, use or sell a generic version of ORLADEYO in the United States prior to the expiration of four patents listed in the FDA's Orange Book: U.S. Patent Nos. 10,662,160; 11,117,867; 11,618,733; and 12,344,585 (the "Challenged Patents"). The Notice Letters allege that the Challenged Patents, which expire in May 2040, are invalid, unenforceable and/or will not be infringed by the commercial manufacture, use or sale of the generic product described in Annora's ANDA. The Notice Letters do not challenge the following six ORLADEYO Orange Book patents that expire in 2035: U.S. Patent Nos. 10,125,102; 10,329,260; 10,689,346; 11,230,530; 11,708,333; and 12,116,346.

On March 10, 2025 (as supplemented by the First Amended Complaint filed in December 2025), the Company filed a patent infringement lawsuit in the United States District Court for the District of Delaware against Annora, Hetero Labs Limited, Hetero USA, Inc., and Camber Pharmaceuticals, Inc. (collectively, the "Defendants"), asserting infringement of the Challenged Patents arising from Annora's ANDA filing with the FDA. The Company is seeking, among other remedies, equitable relief enjoining the Defendants from infringing the Challenged Patents, as well as an order that the effective date of any FDA approval of the ANDA would be a date no earlier than the expiration of the Challenged Patents (including any regulatory extensions). While the Company intends to vigorously defend its intellectual property rights protecting ORLADEYO, this matter is in the early stages of litigation and has been stayed until 2031, and no assessment can be made as to the likely outcome of this matter or whether it will be material to the Company. Accordingly, an estimate of the potential loss, or range of loss, if any, to the Company relating to this matter is not possible at this time.

Other Arrangements

The Company has collaborative, licensing, and research and development services agreements in place for which it could be obligated to make payments upon certain developmental, regulatory, or commercial milestones, and future royalty payments, which would be reflected in the financial statements upon the occurrence of such contingent events. Except as disclosed in "*Note 12—Collaborative and Other Relationships*", there have been no changes to material arrangements during the nine months ended September 30, 2026 as compared to those disclosed in "*Note 16—Collaborative and Other Relationships*" to the audited consolidated financial statements in the Annual Report on Form 10-K for the year ended December 31, 2025.

Note 16 — Net Income (Loss) Per Share

Basic and diluted net income (loss) per share for the three and nine months ended September 30, 2026 and 2025 were calculated as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Numerator:				
Net income (loss)	$ 37,916	$ 12,899	$ (605,501)	$ 18,016
Denominator:				
Weighted average shares of common stock outstanding: basic	255,634	210,176	250,840	209,531
Net income (loss) per common share: basic	$ 0.15	$ 0.06	$ (2.41)	$ 0.09
Effect of dilutive securities:				
Stock options to purchase common stock	5,652	5,023	—	5,261
Unvested restricted stock unit awards	6,302	4,663	—	3,532
Shares issuable under the employee stock purchase plan	33	23	—	25
Dilutive potential common shares	11,987	9,709	—	8,818
Weighted average shares of common stock outstanding: diluted	267,621	219,885	250,840	218,349
Net income (loss) per common share: diluted	$ 0.14	$ 0.06	$ (2.41)	$ 0.08

For the three months ended September 30, 2026 and three and nine months ended September 30, 2025, the dilutive effect of outstanding stock options, restricted stock unit awards, and shares issuable under the employee stock purchase plan was calculated using the treasury method, whereby all such awards are assumed to be exercised at the beginning of the period. The hypothetical proceeds from such exercises, including the average unrecognized stock compensation expense for outstanding stock options, restricted stock units and shares issuable under the employee stock purchase plan, were assumed to be used to purchase outstanding common stock at the average price during the period. The net share impact of dilutive securities was added to the weighted average basic common shares outstanding to calculate weighted average diluted shares outstanding. The following table summarizes potential shares of common stock that were excluded from the computation of diluted net income (loss) per share as they were anti-dilutive for the three months ended September 30, 2026 and three and nine months ended September 30, 2025:

	As of September 30,	
	2026	2025
Outstanding stock options	16,519	21,818
Unvested restricted stock unit awards	64	373
Total	16,583	22,191

For the nine months ended September 30, 2026, during which the Company recorded a net loss, all potentially dilutive securities, including outstanding stock options and restricted stock unit awards, were excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share, and thus they are considered “anti-dilutive.” For this period, the weighted average number of shares of common stock outstanding used to calculate both basic and diluted net loss per share of common stock is the same.

Note 17 — Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the condensed consolidated financial statements to provide additional evidence for certain estimates or to identify matters that require additional disclosure. The Company has concluded that no subsequent events have occurred that require disclosure.

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

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

Overview

We are a global biotechnology company focused on developing and commercializing medicines for hereditary angioedema ("HAE") and other rare diseases, driven by our deep commitment to improving the lives of people living with these conditions. We have built a robust commercial infrastructure to support the successful commercialization of ORLADEYO, an oral, once-daily therapy discovered and developed internally for the prevention of HAE attacks. Our business strategy includes leveraging this established commercial platform to successfully commercialize a pipeline of potential first-in-class or best-in-class oral small-molecule and injectable protein therapeutics targeting a range of rare diseases. In June 2026, we announced that we would discontinue our internal discovery programs and close our Discovery Center of Excellence facility in Birmingham, Alabama (the "Birmingham Closure Plan"). We are focusing on identifying and advancing high-value opportunities through external innovation, rigorous scientific evaluation, and disciplined capital allocation. By utilizing our existing commercial capabilities and focusing on rare disease markets, we believe that we can most effectively optimize our costs and strategically allocate resources to support long-term, sustainable growth.

Products and Product Candidates

ORLADEYO® (berotralstat)

ORLADEYO is an oral, once-daily therapy discovered and developed by us for the prevention of HAE attacks. A capsule formulation of ORLADEYO is approved in the United States and other global markets for the prevention of HAE attacks in adults and pediatric patients 12 years and older. In December 2025, the U.S. Food and Drug Administration ("FDA") approved an oral pellet formulation of once-daily ORLADEYO for prophylactic therapy in pediatric patients with HAE aged 2 to <12 years, and in October 2026, the FDA approved the 150 mg oral pellet formulation of once-daily ORLADEYO for prophylactic therapy in adults and adolescents aged 12 years and older. Additionally, in August 2026, Japan's Ministry of Health, Labor and Welfare granted marketing approval for ORLADEYO for prophylactic therapy in pediatric patients with HAE aged 2 to <12 years.

Revenue from sales of ORLADEYO for the three and nine months ended September 30, 2026 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, but not limited to, the success of our and our partners' commercialization efforts in the United States and elsewhere, the number of new patients switching to ORLADEYO, patient retention and demand, the number of physicians prescribing ORLADEYO, the rate of monthly prescriptions, reimbursement from third-party and government payors, the number of patients receiving free product, our pricing strategy, competition and market trends. We monitor and analyze this data on an ongoing basis as we continue to commercialize ORLADEYO and adjust our forecasts accordingly.

Navenibart (STAR-0215)

On January 23, 2026, we completed the previously announced merger (the "Merger") with Astria Therapeutics, Inc. ("Astria"). Pursuant to the Merger, we acquired Astria's lead product candidate navenibart, an injectable monoclonal antibody designed to inhibit plasma kallikrein for the prevention of HAE attacks. Navenibart is currently in Phase 3 clinical development, and the FDA has granted Fast Track and Orphan Drug designations to navenibart for the treatment of HAE. The goal for navenibart is to develop an injectable prophylactic therapy with potential best-in-class dosing every 3- or 6-months, which could offer significant improvements over existing injectable options and address key unmet needs in the HAE patient community.

BCX17725 (Netherton syndrome)

BCX17725 is a potent and selective investigational protein therapeutic KLK5 inhibitor, currently in Phase 1 clinical development, and designed to provide best-in-class, potentially disease-modifying, treatment for people with Netherton syndrome. Netherton syndrome is a serious, rare, lifelong genetic disorder causing disruption of the skin barrier with premature separation of the skin layers, chronic inflammation and vulnerability to serious infections, caused by lack of

normal function of a natural inhibitor of KLK5. People with Netherton syndrome often have itchy, red, scaly, inflamed skin, fragile hair, and are more likely to develop severe food allergies, asthma and eczema. Netherton syndrome can be life-threatening, especially during infancy when patients are vulnerable to dehydration and recurrent infections. Currently, there are no approved treatments that target the underlying cause of Netherton syndrome. BCX17725 is designed to replace missing functions of the natural KLK5 inhibitor, which could restore the normal skin barrier and result in improved skin function, including protection from severe inflammatory and infectious complications of the disease.

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, and the resources dedicated to our products and product candidates by us and our collaborative partners, as well as entering into or modifying licensing agreements for our product candidates. Furthermore, revenues related to our collaborative development activities are dependent upon the progress toward, and the achievement of, developmental milestones by us or our collaborative partners. In addition, as noted below under "Results of Operations" in this MD&A, our new specialty pharmacy established its initial inventory position in the three months ended September 30, 2026, and the related purchases contributed favorably to revenue during the period.

Our operating expenses are also difficult to predict and depend primarily on research and development activities, including clinical research activities, and the ongoing requirements of our development programs, as well as the costs of commercialization, drug manufacturing, direction from regulatory agencies 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. In June 2026, we announced the Birmingham Closure Plan, and we recognized certain related one-time expenses during the three months ended September 30, 2026. See "*Note 3—Birmingham Closure*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional details on the Birmingham Closure Plan and related expenses.

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

Recent Developments

ORLADEYO (berotralstat)

On August 5, 2026, we announced that initial product shipments of ORLADEYO oral pellets to pediatric patients began the week of August 3, 2026, marking a new paradigm in the treatment of HAE in pediatrics. In addition, to support the growing scale of ORLADEYO across both adults and pediatrics, we announced that we engaged a new commercial pharmacy partner for ORLADEYO to serve as our sole source specialty pharmacy for ORLADEYO shipments to patients in the United States beginning in the third quarter of 2026.

On August 25, 2026, we announced that Japan's Ministry of Health, Labor and Welfare granted marketing approval for ORLADEYO for prophylactic therapy in pediatric patients with HAE aged 2 to <12 years.

On October 21, 2026, we announced that the FDA approved the supplemental new drug application for use of the 150 mg oral pellet formulation of once-daily ORLADEYO for prophylactic therapy in adults and adolescents aged 12 years and older.

Scientific Advisory Board

On October 5, 2026, we announced the formation of a Scientific Advisory Board ("SAB") comprising seven accomplished scientific and industry leaders with deep expertise across rare disease research and drug development. The SAB will provide independent scientific and strategic guidance as we aim to advance our research and development strategy and build a high value novel drug pipeline.

Results of Operations for the three and nine months ended September 30, 2026 compared to the three and nine months ended September 30, 2025

Revenues

The following table summarizes our revenues for the three months ended September 30, 2026 and 2025:

	Three Months Ended September 30,	
	2026	**2025**
ORLADEYO	$ 174,833	$ 145,265
European ORLADEYO business	—	13,812
Total ORLADEYO	174,833	159,077
License and other revenues	5,459	318
Total revenues	$ 180,292	$ 159,395

The $20.9 million increase in total revenues for the three months ended September 30, 2026 compared to the three months ended September 30, 2025 was primarily driven by:

- $29.6 million increase in ORLADEYO revenue, excluding revenues associated with our European ORLADEYO business, primarily due to the transition to a new sole source specialty pharmacy for ORLADEYO shipments to patients in the United States in the third quarter of 2026, as well as growth in the underlying U.S. business (reflecting an increase in net price and higher volume of oral pellets for pediatric patients, partially offset by lower capsule shipments). Unlike the previous specialty pharmacy arrangement, the new specialty pharmacy takes title to and maintains inventory of ORLADEYO prior to dispensing to patients and is required to maintain 7-21 days of inventory on hand. In connection with the transition, the new specialty pharmacy established its initial inventory position in the third quarter of 2026, and the related purchases contributed favorably to revenues during the period in an amount in the upper teens of millions of dollars. As of September 30, 2026, inventory levels at the new specialty pharmacy were at the lower end of the required range; and

- $5.1 million increase in license and other revenues primarily attributable to the recognition of $2.2 million of revenue related to the Neopharmed License Agreement and $1.6 million of revenue related to the Kaken License Agreement (see "*Note 12—Collaborative and Other Relationships*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information on these agreements), and $1.3 million in quarterly royalty payments from BioCryst Ireland Limited; partially offset by:

- $13.8 million decrease in revenues associated with our European ORLADEYO business due to the sale of our European ORLADEYO business to Neopharmed Gentili S.p.A. ("Neopharmed") on October 1, 2025.

The following table summarizes our revenues for the nine months ended September 30, 2026 and 2025:

	Nine Months Ended September 30,	
	2026	2025
ORLADEYO	$ 481,382	$ 411,499
European ORLADEYO business	—	38,658
Total ORLADEYO	481,382	450,157
License and other revenues	73,573	18,125
Total revenues	$ 554,955	$ 468,282

The $86.7 million increase in total revenues for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025 was primarily driven by the following:

- $69.9 million increase in ORLADEYO revenue, excluding revenues associated with our European ORLADEYO business, primarily due to the transition to a new sole source specialty pharmacy for ORLADEYO shipments to patients in the United States in the third quarter of 2026, as well as growth in the underlying U.S. business (reflecting an increase in net price and higher volume of capsules and oral pellets for pediatric patients). Unlike the previous specialty pharmacy arrangement, the new specialty pharmacy takes title to and maintains inventory of ORLADEYO prior to dispensing to patients and is required to maintain 7-21 days of inventory on hand. In connection with the transition, the new specialty pharmacy established its initial inventory position in the third quarter of 2026, and the related purchases contributed favorably to revenues during the period in an amount in the upper teens of millions of dollars. As of September 30, 2026, inventory levels at the new specialty pharmacy were at the lower end of the required range; and
- $55.4 million increase in license and other revenues primarily attributable to:
  - $58.0 million increase due to the recognition of revenue related to the Neopharmed License Agreement;
  - $7.2 million increase due to revenue related to quarterly royalty payments from BioCryst Ireland Limited; and
  - $4.1 million increase due to the recognition of revenue related to the Kaken License Agreement; partially offset by:
  - $13.2 million decrease in direct sales of peramivir primarily due to lower direct sales of peramivir to the U.S. Department of Health and Human Services (“HHS”) following the expiration of the initial 12-month base ordering period in September 2025, after which no additional ordering periods were exercised; partially offset by:
- $38.7 million decrease in revenues associated with our European ORLADEYO business due to the sale of our European ORLADEYO business to Neopharmed on October 1, 2025.

Cost of product sales

The following table summarizes our cost of product sales for the three months ended September 30, 2026 and 2025:

	Three Months Ended September 30,	
	2026	2025
Cost of product sales - ORLADEYO	$ 1,558	$ 1,462
Cost of product sales - peramivir	16	5
European ORLADEYO business	—	720
Total cost of product sales	$ 1,574	$ 2,187

The $0.6 million decrease in cost of product sales for the three months ended September 30, 2026 compared to the three months ended September 30, 2025 was primarily driven by a decrease in cost of product sales associated with our European ORLADEYO business due to the sale of our European ORLADEYO business to Neopharmed on October 1, 2025.

The following table summarizes our cost of product sales for the nine months ended September 30, 2026 and 2025:

	Nine Months Ended September 30,	
	2026	2025
Cost of product sales - ORLADEYO	$ 7,991	$ 4,099
Cost of product sales - peramivir	2,800	2,989
European ORLADEYO business	—	2,465
Total cost of product sales	$ 10,791	$ 9,553

The $1.2 million increase in cost of product sales for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025 was primarily due to an increase in ORLADEYO sales to our partners, partially offset by a decrease in cost of product sales associated with our European ORLADEYO business due to the sale of our European ORLADEYO business to Neopharmed on October 1, 2025.

Acquired in-process research and development expense

Acquired in-process research and development expense was $697.8 million for the nine months ended September 30, 2026 related to the non-cash charge recognized for the value assigned to the navenibart in-process research and development asset acquired as part of the Merger. See "*Note 2—Acquisition of Astria Therapeutics, Inc.*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information on the Merger.

Research and development expenses

Research and development expenses include all costs incurred to discover, develop and advance our product candidates and related technologies. These costs include direct costs, such as compensation for research and development personnel and costs paid to third-parties for laboratory studies, process development and manufacturing of product candidates, and the conduct and management of clinical trials and other clinical and preclinical activities. Additionally, direct expenses include those costs necessary to discontinue and close out a development program, including termination fees and other commitments. Research and development expenses also include indirect costs, such as lab supplies and services, facility costs, depreciation of lab equipment and other overhead of our research and development activities. Research and development expenses vary based on the number of programs in development and the stage of development of each program. Later stage clinical programs generally require higher spending than earlier stage programs due to the longer length of time of the clinical trials and the higher patient enrollment.

We do not maintain or evaluate internal research and development costs on a program-by-program basis as certain costs may benefit multiple programs. We do not allocate non-program specific external costs or internal costs to programs.

The following table summarizes our research and development expenses for the three months ended September 30, 2026 and 2025:

	Three Months Ended September 30,	
	2026	2025
Navenibart	$ 17,995	$ —
BCX17725	3,772	7,574
Berotralstat	683	2,574
Avoralstat	(5)	1,800
Research, discovery and preclinical programs	147	5,491
Compensation and related personnel costs	13,777	13,218
Stock-based compensation	5,765	6,519
Other non-program specific and indirect costs	16,079	7,331
European ORLADEYO business (excluding stock-based compensation)	—	96
Total research and development expenses	$ 58,213	$ 44,603

The increase in research and development expenses for the three months ended September 30, 2026 compared to three months ended September 30, 2025 was primarily driven by the following:

- $18.0 million increase in navenibart due to the acquisition of the Phase 3 product candidate in connection with the Merger; and
- $8.7 million increase in other non-program specific and indirect costs primarily due to the recognition of $9.5 million of impairment expense related to the Birmingham Closure Plan (see "*Note 3—Birmingham Closure*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report).

These increases were partially offset by the following:

- $5.3 million decrease in research, discovery and preclinical programs due to the discontinuation of various programs as a result of the Birmingham Closure Plan;
- $3.8 million decrease in BCX17725 primarily due to a decrease in drug manufacturing activities;
- $1.9 million decrease in berotralstat primarily attributed to a decrease in manufacturing and other costs incurred to support FDA approval in pediatric patients in 2025; and
- $1.8 million decrease in avoralstat due to the discontinuation of the program in the first quarter of 2026.

The following table summarizes our research and development expenses for the nine months ended September 30, 2026 and 2025:

	Nine Months Ended September 30,	
	2026	2025
Navenibart	$ 45,064	$ —
BCX17725	10,919	13,346
Berotralstat	4,090	8,237
Avoralstat	1,239	7,584
Research, discovery and preclinical programs	8,332	12,095
Compensation and related personnel costs	52,258	37,464
Stock-based compensation	18,057	24,374
Other non-program specific and indirect costs	30,542	20,620
European ORLADEYO business (excluding stock-based compensation)	—	1,539
Total research and development expenses	$ 170,501	$ 125,259

The increase in research and development expenses for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025 was primarily driven by the following:

- $45.1 million increase in navenibart due to the acquisition of the Phase 3 product candidate in connection with the Merger;
- $14.8 million increase in compensation and related personnel costs primarily due to $10.7 million associated with the portion of the Astria stock option payout attributable to post-combination service in connection with the Merger, and a $6.4 million increase in severance costs driven by employee termination and separation costs related to the Birmingham Closure Plan and Astria related severance costs, partially offset by a $2.5 million decrease in personnel costs due to lower headcount; and
- $9.9 million increase in other non-program specific and indirect costs primarily due to the recognition of $9.5 million of impairment expense related to the Birmingham Closure Plan.

These increases were partially offset by the following:

- $6.3 million decrease in avoralstat due to the discontinuation of the program in the first quarter of 2026;
- $6.3 million decrease in stock-based compensation expense primarily due to research and development related turnover and prior period acceleration of expense pursuant to the BioCryst Pharmaceuticals, Inc. Equity Award Retirement Policy, partially offset by a modification to extend the post-termination exercise period of certain vested stock option awards at the time of retirement for certain individuals to the original expiration date, resulting in $6.8 million of incremental expense in 2026, and $1.0 million equity modifications in connection with employee terminations related to the Birmingham Closure Plan;
- $4.1 million decrease in berotralstat primarily attributed to a decrease in manufacturing and other costs to support FDA approval in pediatric patients in 2025;
- $3.8 million decrease in research, discovery and preclinical programs due to the discontinuation of various programs as a result of the Birmingham Closure Plan;
- $2.4 million decrease in BCX17725 primarily due to a decrease in drug manufacturing activities; and
- $1.5 million decrease in research and development expenses associated with our European ORLADEYO business (excluding stock-based compensation) due to the sale of our European ORLADEYO business to Neopharmed on October 1, 2025.

Selling, general, and administrative expenses

Selling, general and administrative expenses represent costs incurred to commercialize our products and support our operations. Sales and marketing expenses include personnel-related costs, activities supporting the commercialization and distribution of marketed products, market research, marketing, medical affairs, market access, and advertising costs. General and administrative expenses include personnel-related costs and other expenses associated with quality, finance, human resources, information technology, legal, compliance, and other administrative functions, including transaction-related costs.

The following table summarizes our selling, general, and administrative expenses for the three months ended September 30, 2026 and 2025:

	Three Months Ended September 30,	
	2026	**2025**
Sales and marketing (excluding stock-based compensation)	$ 37,726	$ 34,977
General and administrative (excluding stock-based compensation)	20,970	23,835
European ORLADEYO business (excluding stock-based compensation)	—	12,120
Stock-based compensation	8,479	12,082
Total selling, general, and administrative expenses	$ 67,175	$ 83,014

The increase in sales and marketing expenses (excluding stock-based compensation) for the three months ended September 30, 2026 compared to the three months ended September 30, 2025 was primarily driven by $3.0 million of expense associated with the transition to a new sole source specialty pharmacy for ORLADEYO shipments to patients in the United States in the third quarter of 2026 and $1.7 million of cash separation costs related to the September 2026 workforce reduction (see "*Note 13—Workforce Reduction*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report); partially offset by a $1.2 million decrease in manufacturing support related costs.

The decrease in general and administrative expenses (excluding stock-based compensation) for the three months ended September 30, 2026 compared to the three months ended September 30, 2025 was primarily driven by a decrease in transaction-related costs, partially offset by $1.9 million of cash separation costs related to the Birmingham Closure Plan and the September 2026 workforce reduction.

Expenses associated with our European ORLADEYO business (excluding stock-based compensation) were $12.1 million for the three months ended September 30, 2025. There were no corresponding expenses for the three months ended September 30, 2026 due to the sale of our European ORLADEYO business to Neopharmed on October 1, 2025.

The decrease in stock-based compensation expense for the three months ended September 30, 2026 compared to the three months ended September 30, 2025 was primarily attributed to employee turnover, prior period acceleration of expense pursuant to the BioCryst Pharmaceuticals, Inc. Equity Award Retirement Policy, and prior period adjustments for estimated forfeitures.

The following table summarizes our selling, general, and administrative expenses for the nine months ended September 30, 2026 and 2025:

	Nine Months Ended September 30,	
	2026	**2025**
Sales and marketing (excluding stock-based compensation)	$ 114,626	$ 106,878
General and administrative (excluding stock-based compensation)	84,173	70,505
European ORLADEYO business (excluding stock-based compensation)	—	38,584
Stock-based compensation	26,906	36,899
Total selling, general, and administrative expenses	$ 225,705	$ 252,866

The increase in sales and marketing expenses (excluding stock-based compensation) for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025 was primarily driven by the following:

- $3.7 million of expense associated with the portion of the Astria stock option payout attributable to post-combination service in connection with the Merger;
- $3.8 million increase in severance costs primarily driven by Astria related severance costs and cash separation costs related to the September 2026 workforce reduction; and
- $3.0 million of expense associated with the transition to a new sole source specialty pharmacy for ORLADEYO shipments to patients in the United States in the third quarter of 2026.

These increases were partially offset by $3.3 million decrease in manufacturing support related costs.

The increase in general and administrative expenses (excluding stock-based compensation) for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025 was primarily driven by the following:

- $14.7 million of expense associated with the portion of the Astria stock option payout attributable to post-combination service in connection with the Merger;
- $6.5 million increase in severance costs driven by $6.3 million of Astria related severance costs in connection with the Merger; and
- $6.5 million increase due to incremental employee, facility, and other costs associated with Astria.

These increases were partially offset by:

- $8.5 million of transaction-related costs incurred in 2025 associated with the sale of our European ORLADEYO business to Neopharmed on October 1, 2025;
- $4.6 million decrease in personnel related costs due to lower headcount; and
- $1.5 million of transaction-related costs incurred in 2025 in connection with the Merger.

Expenses associated with our European ORLADEYO business (excluding stock-based compensation) were $38.6 million for the nine months ended September 30, 2025. There were no corresponding expenses for the nine months ended September 30, 2026 due to the sale of our European ORLADEYO business to Neopharmed on October 1, 2025.

The decrease in stock-based compensation expense for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025 was primarily attributed to employee turnover, prior period acceleration of expense pursuant to the BioCryst Pharmaceuticals, Inc. Equity Award Retirement Policy, and prior period adjustments for estimated forfeitures.

Other income (expense)

The following tables summarize our other income (expense) for the three and nine months ended September 30, 2026 and 2025, respectively:

	Three Months Ended September 30,	
	2026	2025
Interest income	$ 3,073	$ 2,238
Interest expense	(21,185)	(19,661)
Foreign currency gains, net	22	35
Loss on extinguishment of debt	—	(2,740)
Other income, net	3,345	2,667
Total other expense, net	$ (14,745)	$ (17,461)

	Nine Months Ended September 30,	
	2026	2025
Interest income	$ 7,763	$ 7,778
Interest expense	(62,676)	(64,737)
Foreign currency losses, net	(176)	(27)
Loss on extinguishment of debt	—	(6,911)
Other income, net	2,143	2,667
Total other expense, net	$ (52,946)	$ (61,230)

Interest expense was primarily comprised of non-cash interest expense due to the amortization of interest associated with our royalty financing obligations and interest expense associated with the borrowings under the Blackstone Loan Agreement (as defined below) for the three and nine months ended September 30, 2026 and Pharmakon Loan Agreement (as defined in "*Note 8—Debt*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report) for the three and nine months ended September 30, 2025. The decrease in interest expense for the nine months ended September 30, 2026 was primarily due to a lower effective interest rate under the Blackstone Loan Agreement as compared to the Pharmakon Loan Agreement.

In April 2025 and July 2025, we made $75.0 million and $50.0 million partial prepayments, respectively, on the outstanding principal amount under the Pharmakon term loan resulting in a one-time loss on extinguishment of debt of $2.7 million and $6.9 million for the three and nine months ended September 30, 2025, respectively. We paid off the remainder of the outstanding principal balance of $198.7 million as of September 30, 2025 in two separate prepayments in the third and fourth quarters of 2025.

Income tax expense

For the three months ended September 30, 2026, income tax expense was $0.7 million compared to an income tax benefit of $0.8 million for the three months ended September 30, 2025. The increase in income tax expense was primarily due to the tax benefit recognized in the third quarter of 2025 resulting from the enactment of the One Big Beautiful Bill Act ("OBBBA") during the three months ended September 30, 2025 which allowed additional taxable deductions for domestic research and development expenditures.

For the nine months ended September 30, 2026, income tax expense was $2.8 million compared to $1.4 million for the nine months ended September 30, 2025. The increase in income tax expense was primarily driven by the increase in U.S. and foreign taxable income for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025.

Liquidity and Capital Resources

As of September 30, 2026, our principal sources of liquidity consisted of cash, cash equivalents and available-for-sale investments totaling $375.0 million, cash generated from ORLADEYO sales, the potential for additional term loans

under the Blackstone Loan Agreement (as defined below), proceeds from licensing and collaborative agreements, and royalty financing arrangements. In addition to the above, we have previously received funding from other sources, including public offerings and private placements of equity securities, cash from research and development agreements, including U.S. Government contracts, government grants, research grants, and interest income on our investments.

On January 23, 2026, we entered into a Loan Agreement (the "Blackstone Loan Agreement") with Blackstone Alternative Credit Advisors LP and Blackstone Life Sciences Advisors L.L.C., (together, "Blackstone"), as the Blackstone representatives thereunder, the guarantors from time to time party thereto, the lenders from time to time party thereto, and Wilmington Trust, National Association, as agent, pursuant to which the lenders funded term loans in the aggregate principal amount of $400.0 million (the "Term Loans"). We used the proceeds from the Term Loans to pay the cash portion of the consideration required to consummate the Merger. The maturity date of the Term Loans under the Blackstone Loan Agreement is January 23, 2031. Subject to the mutual agreement between us, Blackstone and the lenders, we may request additional term loans up to an aggregate principal amount not exceeding $150.0 million. Our obligations under the Blackstone Loan Agreement are secured by a security interest in, subject to certain exceptions, substantially all of our and our subsidiaries' assets.

The Blackstone Loan Agreement also contains representations and warranties and affirmative and negative covenants customary for financings of this type (including a minimum liquidity covenant), as well as customary events of default. Certain of the customary negative covenants limit our ability and the ability of certain of our subsidiaries to, among other things, dispose of assets, engage in mergers, acquisitions and similar transactions, incur additional indebtedness, grant liens, make investments, pay dividends or make distributions or certain other restricted payments in respect of equity, prepay certain other indebtedness, enter into restrictive agreements, undertake fundamental changes or amend certain material contracts, among other customary covenants, in each case subject to certain exceptions. A failure to comply with the covenants in the Blackstone Loan Agreement, or an occurrence of any other event of default, could permit the lenders under the Blackstone Loan Agreement to declare the borrowings thereunder, together with accrued interest and fees, and any applicable yield protection premium, to be immediately due and payable. We were in compliance with the covenants in the Blackstone Loan Agreement as of September 30, 2026. See "*Note 8—Debt*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information about the Blackstone Loan Agreement.

In 2020 and 2021, we entered into the Royalty Purchase Agreements (as defined in "*Note 7—Royalty Financing Obligations*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report) with RPI 2019 Intermediate Finance Trust ("RPI") and OCM IP Healthcare Holdings Limited, an affiliate of OMERS Capital Markets ("OMERS"). Under the Royalty Purchase Agreements, RPI and OMERS are entitled to receive tiered, sales-based royalties on net product sales of ORLADEYO in the United States and certain key European markets (collectively, the "Key Territories"), and other markets where we sell ORLADEYO directly or through distributors. In addition, RPI and OMERS are entitled to receive a tiered revenue share on amounts generally received by us on account of ORLADEYO sublicense revenue or net sales by licensees outside of the Key Territories. Our required payments to OMERS commenced with the calendar quarter beginning October 1, 2023. No royalty payments are due on direct sales over $550.0 million. See "*Note 7—Royalty Financing Obligations*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information about these financing transactions.

Cash Flows

The following table summarizes our cash flows for each period presented:

	Nine Months Ended September 30,	
	2026	2025
Net cash provided by (used in):		
Operating activities	$ 75,534	$ 55,409
Investing activities	(457,671)	69,794
Financing activities	389,323	(131,971)
Effect of exchange rates on cash, cash equivalents and restricted cash	(101)	1,342
Increase (decrease) in cash, cash equivalents and restricted cash, including cash classified within current assets held for sale	7,085	(5,426)
Less: net increase in cash and cash equivalents classified within current assets held for sale	—	(14,840)
Net increase (decrease) in cash, cash equivalents, and restricted cash	$ 7,085	$ (20,266)

Operating Activities

During the nine months ended September 30, 2026, we incurred a net loss of $605.5 million, which was primarily driven by the non-cash acquired in-process research and development charge of $697.8 million related to the Merger. After adjusting for this, other non-cash cash items totaling $94.5 million, and operating assets and liabilities used of $111.2 million, net cash provided by operating activities totaled $75.5 million.

During the nine months ended September 30, 2025, we generated net income of $18.0 million. After adjusting for non-cash items totaling $98.5 million, and operating assets and liabilities used of $61.1 million, net cash provided by operating activities totaled $55.4 million.

Investing Activities

During the nine months ended September 30, 2026, net cash used in investing activities of $457.7 million primarily related to cash paid for the acquisition of Astria, net of cash acquired, of $489.5 million and $275.8 million of purchases of investment securities, partially offset by $308.4 million of sales and maturities of investment securities.

During the nine months ended September 30, 2025, net cash provided by investing activities of $69.8 million primarily related to sales and maturities of investment securities of $207.6 million, partially offset by purchases of investment securities of $136.7 million.

Financing Activities

During the nine months ended September 30, 2026, net cash provided by financing activities of $389.3 million primarily consisted of net proceeds from the Term Loans under the Blackstone Loan Agreement of $395.0 million and common stock issued under stock-based compensation plans of $29.9 million, partially offset by principal payments on royalty financing obligations of $28.5 million and withholding taxes paid on stock-based awards of $6.3 million.

During the nine months ended September 30, 2025, net cash used in financing activities of $132.0 million primarily consisted of repayment of Pharmakon term loan principal and related prepayment premium and fees totaling $119.6 million and principal payments on royalty financing obligations of $16.4 million, partially offset by net proceeds from common stock issued under stock-based compensation plans of $8.5 million.

Plan of Operation and Future Funding Requirements

We intend to contain costs and cash flow requirements by closely managing our third-party costs and headcount, leasing our facilities, and contracting with other parties to conduct certain research and development projects. We may incur additional expenses, potentially resulting in significant losses, as we continue to pursue our research and development activities, commercialize ORLADEYO, and engage in strategic business development. We may incur additional expenses related to the filing, prosecution, maintenance, defense, and enforcement of patent and other intellectual property claims and additional regulatory costs as our clinical programs advance through later stages of development or as regulatory exclusivity for our products expires. The objective of our investment policy is to ensure the safety and preservation of invested funds, as well as to maintain liquidity sufficient to meet cash flow requirements. We place our excess cash with

high credit quality financial institutions. We invest in marketable debt securities that may consist of U.S. Treasury obligations, U.S. Government agency securities, money market funds, certificates of deposit, and corporate notes and bonds in order to limit the amount of our credit exposure. We have not realized any significant losses on our investments.

Our expenses, revenues and cash utilization rate could vary significantly depending on many factors, some of which will consume significant capital resources, including:

- the progress and results of our current and proposed clinical trials for our product candidates;
- the progress made in the development, manufacturing, and regulatory approval process of our product candidates;
- the success of our commercialization efforts for, and market acceptance of, our products;
- our business development activities;
- the amount of funding or assistance, if any, we receive from new partnerships with third parties for the development and/or commercialization of our products and product candidates;
- the development progress of any collaborative agreements for our product candidates; and
- the amount and timing of funding we receive, if any, from U.S. Government contracts.

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. 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. In addition to financing our needs through our existing capital resources and interest earned on that capital, as well as revenues from product sales, we periodically evaluate other opportunities to fund future operations, including: (1) out-licensing rights to certain of our products or product candidates, pursuant to which we would receive cash milestone payments; (2) obtaining additional product candidate regulatory approvals, which would generate revenue, milestone payments and cash flow; (3) reducing spending on one or more research and development programs, including by discontinuing development; (4) restructuring operations to change our overhead structure; (5) securing U.S. Government funding of our programs, including obtaining procurement contracts; and/or (6) pursuing royalty or loan financing. We may also, in the future, issue securities, including common stock, preferred stock, depositary shares, purchase contracts, warrants, debt securities, and units, through private placement transactions or registered public offerings. Our future liquidity needs, and our ability to address those needs, will largely be determined by the success of our products and product candidates; the success of our business development efforts; the timing, scope, and magnitude of our research and development and commercial expenses; and key regulatory and operational developments.

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

- sustained market acceptance of approved products and successful commercialization of such products by either us or our partners;
- the progress and magnitude of our research and development programs;
- the success of our external innovation strategy;
- changes in existing collaborative relationships;
- our ability to establish additional collaborative relationships if and when needed;
- the extent to which our partners will share in the costs associated with the development of our programs or run the development programs themselves;
- our ability to negotiate favorable development and marketing strategic alliances for certain products and product candidates;
- any decision to build or expand our commercial capabilities;
- the scope and results of preclinical studies and clinical trials to 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;
- the scope of manufacturing of our drug substance and product candidates required for future new drug application filings;
- competitive and technological advances;
- the time and costs involved in obtaining regulatory approvals;
- post-approval commitments for any products that receive regulatory approval;
- our business development activities; and
- the costs involved in all aspects of intellectual property strategy and protection, including the costs involved in preparing, filing, prosecuting, maintaining, defending, and enforcing patent claims.

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

Critical Accounting Estimates

The preparation of these consolidated financial statements in accordance with U.S. GAAP requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues, expenses, and related disclosures for the periods presented. Some of these estimates can be subjective and complex with a significant level of estimation uncertainty, and, consequently, actual results may differ from these estimates. The judgments and assumptions used by management are based on historical experience and information available to us at the time that we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates.

While our significant accounting policies are more fully described in "*Note 1—Significant Accounting Policies and Concentrations of Risk*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our Condensed Consolidated Financial Statements.

Revenue Recognition

The application of Accounting Standards Codification ("ASC") Topic 606, *Revenue from Contracts with Customers,* substantially impacts our reported results, particularly product sales, net, which requires certain estimates in determining the transaction price.

Revenues from product sales are recorded at the net selling price (transaction price), which includes estimates of variable consideration including government and managed care rebates, chargebacks, co-payment and patient assistance, quick starts, and product returns. Variable consideration is estimated using the expected value method and is included in product sales, net only to the extent that it is probable that a significant reversal of cumulative revenue recognized will not occur. Estimates are reassessed each reporting period. If actual results vary from estimates, adjustments are recognized, which would affect net product revenue and earnings in the period identified.

The most subjective of these estimates are government and managed care rebates. We estimate rebates payable to third-party payors, including managed care organizations and government programs, and record such amounts as reductions of gross product revenues at the time revenue is recognized, with corresponding liabilities recorded in accrued expenses. Rebate estimates are based on contractual terms, applicable program requirements, and product distribution information obtained from our specialty pharmacy regarding payor mix.

Research and Development Expenses and Related Accruals

We estimate research and development expenses based on the review of contracts and purchase orders, discussions with internal personnel to identify services that have been performed on our behalf, discussions with service providers, and assumptions regarding actual work completed and the associated costs incurred when we have not yet been invoiced or otherwise notified of actual cost. We consider facts and circumstances known to us at the time, which may include assumptions regarding expected patient enrollment, site activation and estimated project duration. In expensing service fees, we estimate the time period over which services will be performed and the level of effort expended in each period. Examples of estimated accrued research and development 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.

The financial terms of our agreements may result in uneven payment flows, as payments may depend on factors such as the successful enrollment of patients and the completion of milestones. We accrue for expenses when we determine an obligation has been incurred, regardless of the timing of the invoice. We periodically confirm the accuracy of our estimates with the service providers and adjust if necessary. 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.

Royalty Financing Obligations

Under the royalty financing obligations, RPI and OMERS are entitled to receive sales-based royalties on net product sales of ORLADEYO. Interest expense is accrued using the effective interest rate method over the estimated period each of the related liabilities will be paid. This requires us to estimate the total amount of future royalty payments to be generated from product sales over the life of the agreements. We impute interest on the carrying values 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 adjustments to the effective interest rates on a prospective basis. The assumptions used in determining the expected repayment terms of the debt and amortization periods 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. Significant changes in forecasted net sales could materially impact each of the liability balances, interest expense and the time periods for repayment.

Asset Acquisitions and Acquired In-Process Research and Development

Accounting for asset acquisitions requires significant judgment, including the application of the screen test in accordance with ASC Topic 805, *Business Combinations,* and the valuation of acquired in-process research and development ("IPR&D"). Determining whether substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset requires estimates of relative fair values. During 2026, we acquired significant IPR&D related to the navenibart development program in connection with the Merger. The determination of the fair value of acquired IPR&D represents a critical accounting estimate because it requires significant management judgment and the use of unobservable inputs. Key assumptions include development timelines, probabilities of technical and regulatory success, expected future revenues, market conditions, and discount rates. In certain circumstances, we may conclude that an acquired IPR&D asset has no measurable fair value at the acquisition date based on market participant considerations. Changes in these assumptions or judgments could materially affect our accounting conclusions and amounts recognized in our condensed consolidated financial statements. See "*Note 2—Acquisition of Astria Therapeutics, Inc.*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report for additional information on the Merger.

Income Taxes

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

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

Recent Accounting Pronouncements

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

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Interest Rate Risk

We are subject to interest rate risk on our investment portfolio and borrowings under our Blackstone Loan Agreement. The Term Loans under the Blackstone Loan Agreement accrue interest each quarter at a rate equal to the three-month Secured Overnight Financing Rate ("SOFR"), which shall be no less than 1.75%, plus 4.50% per annum, plus 0.50% per annum for each quarterly interest period in which a Blackstone PIK Interest Payment (as defined in "*Note 8—Debt*" in the Notes to the Condensed Consolidated Financial Statements included in Part I, Item 1 of this report) is made. Accordingly, increases in interest rates will increase the associated interest payments that we are required to make on the Term Loans. For the three months ended September 30, 2026, interest was accrued at an effective rate of 8.88% on the Term Loans under the Blackstone Loan Agreement.

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

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

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

Foreign Currency Risk

Most of our revenues and expenses are denominated in U.S. dollars. Our royalties from Torii Pharmaceutical Co., Ltd. ("Torii") are in Japanese Yen. We also had other transactions denominated in foreign currencies during the nine months ended September 30, 2026, including contract manufacturing and ex-U.S. clinical trial activities, and we expect to continue to do so. Our limited foreign currency exposure relative to our operations is primarily to fluctuations in the Japanese Yen, Canadian Dollar, and Euro. We do not anticipate that foreign currency transaction gains or losses will be significant at our current level of operations.

Inflation Risk

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

Item 4. Controls and Procedures

We maintain a set of disclosure controls and procedures that are designed to ensure that information relating to the Company required to be disclosed in our periodic filings under the Exchange Act is recorded, processed, summarized and reported in a timely manner under the Exchange Act. We carried out an evaluation, under the supervision and with the participation of management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures. Based upon that evaluation, the Chief Executive Officer and Chief Financial Officer concluded that, as of September 30, 2026, the Company's disclosure controls and procedures are effective to ensure that information required to be disclosed by the Company in the reports filed or submitted by it under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and include controls and procedures designed to ensure that information required to be disclosed by the Company in such reports is accumulated and communicated to the Company's management, including the Chief Executive Officer and Chief Financial Officer of the Company, as appropriate to allow timely decisions regarding required disclosure.

There have been no changes in our internal control over financial reporting that occurred during the three months ended September 30, 2026 that have materially affected, or are reasonably likely to materially affect, the Company's internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

As previously disclosed, in January 2025, we received a Paragraph IV notice of certification (the "First Notice Letter") from Annora Pharma Private Limited ("Annora") regarding U.S. Patent Nos. 10,662,160; 11,117,867; and 11,618,733. In January 2026, we received an additional Paragraph IV notice of certification (the "Second Notice Letter" and together with the First Notice Letter, the "Notice Letters") from Annora regarding U.S. Patent No. 12,344,585. The Notice Letters advise that Annora has submitted an Abbreviated New Drug Application ("ANDA") to the FDA seeking approval to manufacture, use or sell a generic version of ORLADEYO in the United States prior to the expiration of four patents listed in the FDA's Orange Book: U.S. Patent Nos. 10,662,160; 11,117,867; 11,618,733; and 12,344,585 (the "Challenged Patents"). The Notice Letters allege that the Challenged Patents, which expire in May 2040, are invalid, unenforceable and/or will not be infringed by the commercial manufacture, use or sale of the generic product described in Annora's ANDA. The Notice Letters do not challenge the following six ORLADEYO Orange Book patents that expire in 2035: U.S. Patent Nos. 10,125,102; 10,329,260; 10,689,346; 11,230,530; 11,708,333; and 12,116,346.

On March 10, 2025 (as supplemented by the First Amended Complaint filed in December 2025), we filed a patent infringement lawsuit in the United States District Court for the District of Delaware against Annora, Hetero Labs Limited, Hetero USA, Inc., and Camber Pharmaceuticals, Inc. (collectively, the "Defendants"), asserting infringement of the Challenged Patents arising from Annora's ANDA filing with the FDA. We are seeking, among other remedies, equitable relief enjoining the Defendants from infringing the Challenged Patents, as well as an order that the effective date of any FDA approval of the ANDA would be a date no earlier than the expiration of the Challenged Patents (including any regulatory extensions). While we intend to vigorously defend our intellectual property rights protecting ORLADEYO, this matter is in the early stages of litigation and has been stayed until 2031, and no assessment can be made as to the likely outcome of this matter or whether it will be material to us. Accordingly, an estimate of the potential loss, or range of loss, if any, to us relating to this matter is not possible at this time.

Item 1A. Risk Factors

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

Risks Relating to Our Business

Financial and Liquidity Risks

We may not achieve sustained profitability.

Although we achieved net income on a U.S. GAAP basis for the year ended December 31, 2025 for the first time on an annual basis, we have not yet achieved sustained profitability. Our expectations as to the sustainability of our profitability may change based upon our ability to execute our commercialization goals and operational initiatives and whether or not the assumptions underlying our projected revenues and expenses are correct. Our beliefs and projections regarding the attainment of our financial goals may differ from actual results based on market factors like competition, patient and physician acceptance of our products, reimbursement levels, or on our ability to execute our operational and budget plans, including management's ability to properly forecast our capital allocation needs. To achieve sustained profitability, we, or our collaborative partners, must successfully manufacture and develop or acquire products and product candidates, receive regulatory approvals, and successfully commercialize our products and/or enter into profitable commercialization arrangements with other parties. Even if we are able to successfully commercialize our existing products, or to develop or otherwise acquire new commercially viable products, certain obligations we have to third parties, including, without limitation, our obligation to pay RPI and OMERS, as applicable, royalties on certain revenues from ORLADEYO under the Royalty Purchase Agreements (as defined in "*Note 7—Royalty Financing Obligations*" in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report), may reduce the profitability of such products.

Because of the numerous risks and uncertainties associated with developing or acquiring product candidates, launching new products, and their potential for commercialization, we are unable to predict the extent of any potential

future losses. Even though we have achieved profitability in a given reporting period, we may not be able to sustain or increase profitability on a quarterly or annual basis. For example, as a result of the accounting treatment for the Merger, we recorded a net loss on a U.S. GAAP basis for the quarter ended March 31, 2026, due to a special, non-cash charge related to the acquired in-process research and development asset for navenibart. If we are unable to achieve sustained profitability on our anticipated timeline, or at all, the market value of our common stock will likely decline.

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

We have sustained operating losses for the majority of our corporate history. Even if we achieve sustained profitability, in order to continue future operations, progress any development programs, engage in strategic business development activities and commercialize our products, we may be required to raise additional capital or obtain additional financing in the future. In addition to seeking strategic partnerships and transactions, 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 or issuances of securities, additional borrowings, collaborative arrangements with partners, or from other sources, may not be available if or when needed or in a form or on terms acceptable to us. The issuance of preferred or common stock or convertible securities, with terms and prices significantly more favorable than those of our currently outstanding common stock, could have the effect of diluting or adversely affecting the holdings or rights of our existing stockholders. Additional borrowings may subject us to more restrictive covenants than are currently applicable to us under the Blackstone Loan Agreement. In addition, collaborative arrangements may require us to transfer certain material rights to our corporate partners. Insufficient funds or lack of an acceptable partnership have in the past, and may again in the future, require us to delay, scale-back or eliminate certain of our research and development programs.

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

Our liquidity needs will largely be determined by the success of operations in regard to the commercialization of our products, particularly ORLADEYO, the progression of our product candidates, including the progress, timeline and ultimate outcome of our 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), as well as any post-approval studies for our products, and our ability to execute our budget plans. Constriction and volatility in the equity and debt markets, including as a result of the impacts of inflation, increased interest rates, disruption or instability in the banking industry, geopolitical instability, or public health emergencies such as the COVID-19 pandemic, may restrict our future flexibility to raise capital if and when such needs arise. Our current plans for managing our liquidity needs primarily include controlling the timing and spending on our research and development programs and commercializing our approved products. See "*Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources*" in Part I, Item 2 of this report for additional information about our liquidity needs, capital requirements, potential funding alternatives, and adequacy of available funds.

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

which, if severe and sustained, could have a material and adverse impact on our results of operations and cash flows and limit our ability to continue development and commercialization of our products and product candidates.

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

Risks Relating to the Merger

The anticipated benefits and synergies of the Merger may not be realized, and risks associated with Astria's business, including the navenibart program, could adversely affect our business and financial results.

The success of the Merger depends, in part, on our ability to realize the anticipated benefits, cost savings and synergies of the transaction. While we have generally completed the integration of Astria with our business, the benefits may not be realized fully or at all, may take longer to realize than expected, or may not offset transaction and integration expenses over time. An inability to realize the full extent of the anticipated benefits of the Merger could have an adverse effect on our revenues and expenses.

Although we conducted due diligence on Astria before completing the Merger, we may not yet be aware of all material risks, liabilities or challenges associated with Astria's business, including the navenibart program. Unexpected risks may arise, and previously known risks may materialize in a manner that differs from our preliminary risk assessments or assumptions. Any failure to realize the expected benefits of the Merger, any adverse effects from risks related to Astria's business, or any effect of the Merger on our financial results that is inconsistent with the expectations of financial analysts or investors could adversely affect our business and financial results.

Risks Relating to Product Development and Commercialization

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

The success of our business depends in part upon our ability to manage our product candidate pipeline, including through expanding the pipeline, as appropriate, through in-licensing or acquiring products or product candidates and integrating them into our business effectively and efficiently; advancing our product candidates through the various stages of development; and receiving regulatory approvals for the commercial sale of our product candidates. In June 2026, we announced the Birmingham Closure Plan to discontinue our internal discovery programs as part of a strategic shift to focus on identifying and advancing high-value opportunities through external innovation. As a result of this decision, we will generally not be able to expand our pipeline with internal product candidates. Identifying, selecting, and in-licensing or acquiring products or product candidates requires substantial expense and technical and financial expertise, and if we are unable to effectively manage our pipeline, expand our portfolio of product candidates through acquisitions or in-licensing, or integrate viable products or product candidates into our business on acceptable terms, or at all, our business and product development efforts could suffer.

To receive the regulatory approvals necessary for the commercial sale of our product candidates, we or our partners must demonstrate through preclinical studies and clinical trials that each product candidate is safe and effective. The development process and related regulatory process are complex and uncertain. The preclinical and clinical development of our product candidates is susceptible to the risk of failure inherent at any stage of drug or biologic development, including failure to demonstrate efficacy, or for biologics, purity and potency, and safety, failure to demonstrate adequate benefit-risk balance, failure to achieve a commercially attractive and competitive product label, failure to achieve approval in commercially attractive indications, the occurrence of adverse events that are severe or medically or commercially unacceptable, our or our partners' failure to comply with trial protocols, applicable regulatory requirements, or industry standards, or a determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or be approved in accordance with our development plans or at all. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and for some product candidates, there may not be an ideal model for preclinical testing. We also cannot guarantee that any preclinical studies and clinical trials will be conducted as planned or completed on schedule, if at all, or that the results of such trials will be sufficient to support regulatory approval for our product candidates.

Progression of our product candidates through the clinical development process is dependent upon our trials indicating that our product candidates have adequate safety and efficacy or purity and potency in the patients being treated by achieving predetermined endpoints according to the clinical trial protocols, as well as an adequate benefit-risk profile. Failure to achieve any of these endpoints or to show adequate benefit-risk profile in any of our programs has in the past, and could again in the future, result in delays in, modifications to, or discontinuations of our trials or require the performance of additional unplanned trials. If any of our product candidates is associated with adverse events or undesirable side effects or has properties that are unexpected, we may need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a benefit-risk perspective. Product candidates that initially show promise in clinical or preclinical testing have in the past, and could again in the future, later be found to be associated with or to cause undesirable or unexpected side effects that could result in substantial modifications or delays in the development plans for our product candidates, significant unexpected costs, or the termination of programs.

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

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

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

- our or our partners' ability to secure suitable clinical sites and investigators and to enroll and maintain an adequate number of patients on a timely basis or at all;
- patients that enroll in a clinical trial may not comply with the clinical trial protocols or maintain contact with investigators to provide complete data during and after treatment;
- our product candidates may not prove to be either safe or effective for our targeted indications, or at all, or may produce unfavorable or inconclusive results;
- we or our partners may decide, or be required by regulatory authorities, to pause enrollment in, suspend, or terminate clinical research for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate, noncompliance with regulatory requirements or their standards of conduct or evolving guidance, or findings of undesirable effects caused by a chemically or mechanistically similar product or product candidate;
- regulatory authorities may disagree with our or our partners' clinical trial protocols or our or their interpretation of data from preclinical studies and clinical trials;
- clinical protocols or study procedures may not be adequately designed or followed by the investigators, including ensuring that all data is accurately recorded and reported;
- formulation improvements may not work as expected, which could negatively impact commercial demand for our product candidates;
- regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we or our partners enter into agreements for clinical and commercial supplies;
- the supply or quantity of raw materials or manufactured product candidates or other materials necessary to conduct development activities may be insufficient, inadequate, or unavailable at an acceptable cost, and we or our partners may experience interruptions in supply;

- our or our partners' development plans may be delayed or changed as a result of changes in development strategy, the impact of new or different regulations, requirements, and guidelines, or other unexpected events or conditions;
- the cost of preclinical studies and clinical trials may be greater than we anticipate;
- we or our third-party contractors, including those manufacturing our product candidates or components or ingredients thereof, or conducting clinical trials or laboratory testing on our or our partners' behalf, may fail to comply with regulatory requirements and industry standards or meet contractual obligations in a timely manner or at all; and
- the impact of any global health epidemic or pandemic, such as COVID-19, on one or more of the foregoing factors.

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

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

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

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

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

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

any of the foregoing risks is realized, our business, financial condition and results of operations could be materially adversely affected.

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

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

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

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

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

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

Because we focus primarily on developing drugs as treatments for rare diseases, we may seek orphan drug, breakthrough therapy or fast track designations for our product candidates in the United States or the equivalent designations elsewhere in the world. Often, regulatory authorities have broad discretion in determining whether or not to grant such designations. We cannot guarantee that our product candidates will receive orphan drug status from the FDA or equivalent designations from other regulatory authorities. Even with an orphan drug designation for our current and potential future product candidates, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for an existing or future product candidate, that exclusivity may not effectively protect the product from competition.

In addition, we do not know if, when, or how the FDA, Congress, or future judicial challenges may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted. See "*Business—Government Regulation—FDA Regulation—Orphan Drugs*" in Part I, Item 1 of our most recent Annual Report on Form 10-K.

We also cannot guarantee that we will receive breakthrough therapy, fast track, or equivalent designations, which provide certain potential benefits such as more frequent meetings with the applicable regulatory authorities to discuss development plans, intensive guidance on efficient product development programs, and potential eligibility for rolling review or priority review. Even if we are successful in obtaining any such designations for our product candidates, such

designations may not lead to faster development or regulatory review or approval and do not increase the likelihood that our product candidates will receive marketing approval. We may not be able to obtain or maintain these designations for our product candidates that receive them, and our competitors may obtain these designations for their product candidates, which could impact our ability to develop and commercialize our products and product candidates or compete with such competitors, which may adversely impact our business, financial condition or results of operations.

Given the small number of patients who have the diseases that we are targeting, it is important to our ability to grow and sustain profitability that we continue to successfully identify patients with these rare diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our products and product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations, or claims-based market research studies, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. Additionally, the potentially addressable patient population for each of our products and product candidates may be limited or may not be amenable to treatment with our products and product candidates, and new patients may become increasingly difficult to identify or access. Further, even if we obtain significant market share for our products and product candidates, because the potential target populations are small, we may not maintain profitability or generate sufficient long-term revenue growth to sustain our business.

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

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

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

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

We may face competition from biosimilars, which may have a material adverse impact on the future commercial prospects of our biological products and product candidates.

Even if we are successful in achieving regulatory approval to commercialize a biological product candidate faster than our competitors, we may face competition from biosimilars with respect to our biological product candidates. In the United States, the Biologics Price Competition and Innovation Act ("BPCIA") was included in the Affordable Care Act ("ACA") and created an abbreviated approval pathway for biological products that are demonstrated to be "highly similar," or biosimilar, to or "interchangeable" with an FDA-approved biological product. The BPCIA prohibits the FDA from approving a biosimilar or interchangeable product that references a brand biological product until 12 years after the licensure of the reference product, but permits submission of an application for a biosimilar or interchangeable product to the FDA four years after the reference product was first licensed. The BPCIA does not prevent another company from developing a product that is highly similar to the innovative product, generating its own data, and seeking approval. The

law is complex and continues to evolve through ongoing FDA implementation and judicial interpretation. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. Modification of the BPCIA, or changes to the interpretation or implementation of the BPCIA, could have a material adverse effect on the future commercial prospects for our biological products and product candidates.

If competitors are able to obtain marketing approval for biosimilars referencing our biological products, our biological products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences which could adversely affect our business and financial results.

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

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

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

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

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

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

Currently, we have established collaborative relationships including with, among others, third-party distributors for ORLADEYO in certain markets, with Torii for ORLADEYO in Japan, with Neopharmed for the commercialization of ORLADEYO and navenibart in Europe, and with each of Shionogi & Co., Ltd. (“Shionogi”) and Green Cross Corporation for the development and commercialization of peramivir. In addition, in August 2025, Astria announced that it exclusively licensed development and commercialization rights in Japan to Kaken Pharmaceutical Co., Ltd. for navenibart. The process of establishing and implementing collaborative relationships is difficult, time-consuming and involves significant uncertainty, including:

- we or our partners may seek to renegotiate or terminate our relationships due to unsatisfactory commercial, regulatory or clinical results, including post-approval clinical commitments, a change in business strategy, a change of control or other reasons;
- our contracts for collaborative arrangements may expire;
- the possibility that expiration or termination of collaborative relationships, such as those with certain of our distribution partners, may trigger repurchase obligations of the Company for unsold product held by our partners;
- our partners may choose to pursue alternative technologies, including those of our competitors;
- we have had in the past, and in the future may have, disputes with a partner that could lead to litigation or arbitration, which could result in substantial costs and divert the attention of our management;

- we do not have day-to-day control over the activities of our partners and have limited control over their decisions;
- our ability to generate future event payments and royalties from our partners depends upon their abilities to establish the safety and efficacy of our product candidates, obtain regulatory approvals and achieve market acceptance of products developed from our product candidates;
- we or our partners may fail to properly initiate, maintain or defend our intellectual property rights, where applicable, or a party may utilize our proprietary information in such a way as to invite litigation that could jeopardize or potentially invalidate our proprietary information or expose us to potential liability;
- we or our partners may not devote sufficient capital or resources toward our products and product candidates;
- our partners may declare bankruptcy or face other financial distress that could put our partnership or collaborative arrangements at risk; 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, when or where needed, we may have to delay or discontinue further development or commercialization of one or more of our products or product candidates, undertake commercialization activities at our own expense or find alternative sources of funding. Any delay in the development or commercialization of our products and product candidates would severely affect our business, because if our product candidates do not progress through the development process or reach the market in a timely manner, or at all, or if our products do not achieve market success, we may not receive any revenues from product sales or licensing arrangements.

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

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

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

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

- we or our partners may fail to complete clinical trials successfully, or satisfy post-marketing commitments, sufficient to obtain and maintain regulatory agency marketing approval;
- many competitors are more experienced and have significantly more resources, and their products could reach the market faster, be more cost effective or have a better efficacy or tolerability profile than our products and product candidates;
- we may fail to employ a comprehensive and effective intellectual property strategy, which could result in the decreased commercial value of our Company, our products and product candidates, or royalties associated with such products;
- 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;
- revenue from product sales depends on our ability to obtain and maintain favorable pricing;
- reimbursement is constantly changing, which could greatly affect usage of our products;
- future revenue from product sales will depend on our ability to successfully complete clinical studies, obtain regulatory approvals, and manufacture, market, distribute and commercialize our future approved products;

- delays in regulatory approvals, product launches, initial shipments, or ongoing supply arising from manufacturing, formulation, or supply chain issues could impede our ability to meet commercial expectations; and
- the impact of public health emergencies or the outbreak of disease, such as the COVID-19 pandemic, on us or our partners.

In addition, future revenue from sales of ORLADEYO is subject to uncertainties and will depend on several factors, including, but not limited to, the success of our and our partners' commercialization efforts in the United States and elsewhere, the number of new patients switching to ORLADEYO, patient retention and demand, the number of physicians prescribing ORLADEYO, the rate of monthly prescriptions, reimbursement from third-party and government payors, the number of patients receiving free product, our pricing strategy, competition and market trends. Additionally, manufacturing issues relating to our products could result in delays, interruptions or recalls in commercial supply, which could adversely affect our revenue and results of operations. For example, in May 2026, we announced that we identified a manufacturing issue affecting our oral pellet formulation of ORLADEYO that delayed our initial product fulfillment. The issue was resolved, and the week of August 3, 2026, we began shipping the ORLADEYO oral pellets in the United States to pediatric patients with HAE aged 2 to <12 years.

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

We have experienced significant changes in the scope of our operations and capabilities. In addition, we may continue to change certain aspects of our business in connection with acquisitions, licenses, collaborations or other strategic business development activities. As a result, we may encounter difficulties in managing such changes, which could disrupt our operations.

We have experienced significant changes in the scope of our operations, and any future changes resulting from strategic business development opportunities may require us to further evolve our operational, managerial and financial capabilities. To manage such changes, we must continue to implement and improve our managerial, operational and financial systems and processes and recruit, train, and retain qualified personnel as needed. Due to our limited financial resources and the challenges associated with integrating and supporting new programs, technologies, personnel or business activities, we may not be able to effectively manage any future changes to our operations, implement appropriate systems and processes in a timely manner or at all, or recruit, train, and retain qualified personnel. The evolution of our operations, including as a result of acquisitions, licenses, collaborations or other strategic transactions, may lead to significant costs and may divert management and business development resources. In addition, if a commercial launch for any product or product candidate for which we establish commercial or 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 such changes could delay the execution of our business plans or disrupt our operations.

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

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

- insufficient resources being devoted in the manner necessary to satisfy our requirements within expected timeframes;
- inconsistent production yields;
- product liability claims or recalls of commercial product;
- difficulties in scaling production to commercial and validation sizes;
- interruption of the delivery of materials required for the manufacturing process;

- failure to distribute commercial supplies of our products to commercial vendors or end users in a timely manner;
- scheduling of plant time with other vendors or unexpected equipment failure;
- potential catastrophes that could strike their facilities or have an effect on infrastructure;
- potential impurities in our drug substance or products that could affect availability of product for our clinical trials or future commercialization;
- poor quality control and assurance or inadequate process controls;
- failure to provide us with accurate or timely information regarding inventory, the number of patients who are using our products, or serious adverse events and/or product complaints regarding our products;
- inability of third parties to satisfy their financial obligations to us or to others;
- potential breach of the manufacturing or distribution agreement by the third party;
- possible termination or non-renewal of an 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.

The process of manufacturing pharmaceutical products, devices and, in particular, biologics, is complex, highly regulated, and subject to multiple risks. Manufacturing biologics is highly susceptible to product loss due to contamination, equipment failure, improper installation or operation of equipment, vendor or operator error, inconsistency in yields, variability in product characteristics, difficulties in scaling the production process and use of excipients which may, among other things, impact shelf life and present concerns with process or quality controls. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects, other supply disruptions and higher costs. If microbial, viral or other contaminations are discovered at the facilities of our third-party contract manufacturers, such facilities may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical trials, result in higher costs of drug product and adversely affect our business.

Many additional factors could cause production or distribution interruptions with the manufacture and distribution of any of our products and product candidates, including human error, natural disasters, pandemics, labor disputes or shortages, acts of terrorism or war, equipment malfunctions, raw material shortages or supply chain issues. If our commercial distribution partners are not able to satisfy our requirements within the expected timeframe, or are unable to provide us with accurate or timely information and data, including with respect to inventory and sales, serious adverse events, and/or product complaints, our business, including our commercialization efforts for and sales of ORLADEYO, may be at risk. In addition, if specialty pharmacy services, including our third-party call center services, which provide patient support and financial services, prescription intake and distribution, reimbursement adjudication, and ongoing compliance support, are not effectively operated or managed, the continuance of our commercialization efforts for and sales of ORLADEYO may be delayed or compromised. On August 5, 2026, we announced that we transitioned to a new sole source specialty pharmacy provider for ORLADEYO shipments to patients. Any disruption, delay or failure associated with this transition, including interruptions in prescription processing, patient onboarding, reimbursement support or product distribution, could adversely affect our commercialization efforts and our product revenue. In addition, unlike our previous specialty pharmacy, our new specialty pharmacy purchases and holds inventory of ORLADEYO prior to dispensing it to patients. As a result, our quarterly product revenue may be affected by changes in the new specialty pharmacy's inventory levels and ordering patterns, which may not reflect underlying patient demand in the early stages of the transition and may cause our product revenue to fluctuate from period to period until their inventory position is stabilized. For example, the initial inventory build by our new specialty pharmacy contributed an amount in the upper teens of millions of dollars to our product revenue in the third quarter of 2026.

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. Additionally, if we are unable to timely establish an alternate supply from one or more third-party contract manufacturers, we could experience delays in our development efforts as we seek to locate and qualify new or additional manufacturers. For particular products, product candidates, services or particular regions where we rely on a single vendor, these and other related risks are exacerbated for us.

Our third-party manufacturers also may not meet our manufacturing requirements, which could result in a delay in approval, or rejection, of our marketing applications by the FDA or other regulatory authority. Furthermore, changes in the manufacturing process or procedures, including a change in the location where the drug is manufactured or a change of a

third-party manufacturer, may require prior review and approval in accordance with the FDA's cGMP and comparable foreign requirements. This review may be costly and time-consuming and could delay or prevent the launch of a product. The FDA or foreign regulatory authorities may at any time implement new standards, or change their interpretation and enforcement of existing standards, for manufacture, packaging or testing of products. If we or our contract manufacturers are unable to comply, we or they may be subject to regulatory action, civil actions or penalties, any of which could be costly to us and could result in a delay or shortage of product.

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

We have in the past and may from time to time in the future reassess our existing third-party vendor arrangements and determine that changes to one or more vendors are necessary, which could result in delays to development or commercialization. In addition, if we are unable to maintain current third-party relationships, or enter into new agreements with additional third parties on commercially reasonable terms, or at all, or if there is poor manufacturing or distribution performance or failure to comply with any regulatory agency on the part of any of our third-party vendors, we may not be able to complete development of, obtain timely approval of, or commercialize our products and product candidates. If we cannot rely on existing third-party vendors, including our third-party manufacturers, distributors, CROs, or specialty pharmacies, 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 and could negatively affect our business, financial results, and revenue.

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

Commercial success of our products is uncertain and is subject to all the risks and uncertainties disclosed in our other risk factors relating to product 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, including supplying sufficient product to meet commercial demand, and we may not be able to maintain or establish sufficient and acceptable commercial manufacturing, either directly or through third-party manufacturers;
- the commercial demand for and acceptance of our products by healthcare providers and by patients may not be sufficient to result in substantial product revenues to us or to our partners and may result in little to no revenue, milestone payments, or royalties to us;
- effectiveness of marketing and commercialization efforts for our products by us or our partners;
- market satisfaction with existing alternative therapies;
- perceived efficacy relative to other available therapies;
- disease prevalence;
- cost of treatment;
- our pricing and reimbursement strategy may not be effective;
- new legislative or regulatory proposals may influence our pricing and reimbursement strategy, which could impact product revenues;
- pricing and availability of imports or alternative products, including the potential for parallel importing;
- 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. Many 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, many of our competitors have greater experience than we do in conducting clinical trials and obtaining FDA and other regulatory approvals. Accordingly, our competitors may succeed in obtaining FDA or other regulatory approvals of product candidates more rapidly than we do for products that compete with our products. Companies that complete clinical trials, obtain required regulatory approvals, and commence commercial sale of their drugs before we do may achieve a significant competitive advantage, including patent and FDA exclusivity rights that would delay our ability to market products. We face, and will continue to face, competition in the commercialization of our products, licensing of potential product candidates for desirable disease targets, 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 product development technologies;
- methods of preventing or reducing the incidence of disease, including vaccines; and
- new small molecule or other classes of therapeutic agents.

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

We received FDA approval of ORLADEYO, an oral, once-daily therapy for the prevention of HAE attacks in adults and pediatric patients aged 12 years and older, in December 2020, and subsequently received regulatory approvals for ORLADEYO in other global markets. In December 2025, the FDA approved the use of an oral pellet formulation of once-daily ORLADEYO for prophylactic therapy in pediatric patients with HAE aged 2 to <12 years, and in October 2026, the FDA approved the 150 mg oral pellet formulation of once-daily ORLADEYO for prophylactic therapy in adults and adolescents aged 12 years and older. Additionally, in August 2026, Japan's Ministry of Health, Labor and Welfare granted marketing approval for ORLADEYO for prophylactic therapy in pediatric patients with HAE aged 2 to <12 years. We are also performing research on or developing products for the treatment of other rare diseases, and 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. Various government entities throughout the world may also offer incentives, grants and contracts to encourage additional investment into certain preventative and therapeutic agents, which may have the effect of further increasing the number of our competitors and/or providing advantages to certain competitors. In addition, the approval of a generic drug or biosimilar of one of our products or a product with which we compete could have a material impact on our business because it may be significantly less costly to bring to market and may be priced significantly lower than our products or the other products with which we compete. See "*Business—Competition*" in Part I, Item 1 of our most recent Annual Report on Form 10-K for further discussion of our competitors, competitive products or programs, and the competitive conditions in these and other therapeutic areas.

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

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

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

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

Legal and Regulatory Risks

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

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

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

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

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

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

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

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

If our operations with respect to our products that are subject to healthcare laws and regulations are found to be in violation of any of the healthcare fraud and abuse laws described above or in "*Business—Government Regulation*" in Part I, Item 1 of our most recent Annual Report on Form 10-K or any other governmental regulations that apply to us, we may be subject to liability and penalties, including civil and criminal penalties, damages, fines, debarment or exclusion from participating in government-funded healthcare programs such as Medicare or Medicaid, and the curtailment or restructuring of our operations. Any penalties, damages, fines, debarment, exclusion, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with all applicable fraud and abuse laws may be costly.

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

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

In June 2024, the Supreme Court overruled the *Chevron* doctrine, which had given deference to regulatory agencies' statutory interpretations of ambiguous regulations in litigation against federal government agencies, such as the FDA. The overruling of the *Chevron* doctrine may significantly increase the number of challenges brought by companies and other stakeholders against federal agencies such as the FDA and its longstanding decisions and policies, including the FDA's statutory interpretations of market exclusivities and the "substantial evidence" requirements for drug approvals, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, any of which could delay the FDA's review of regulatory submissions. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action.

Further, under the new leadership at HHS under the current administration, agency reorganization, mass layoffs due to the reduction in force initiative and other measures may impact the normal operations of the FDA as well as other federal agencies. FDA may lack adequate staff and resources to meet current review, approval, and inspection schedules, which could delay our anticipated timelines. Recent developments at the FDA include announcement of a plan to phase out animal testing for monoclonal antibodies and certain other drugs, the proposed rare disease evidence principles (RDEP) program to facilitate approval of drugs to treat rare diseases with very small patient populations with significant unmet medical need and with a known genetic defect that is the major driver of the pathophysiology, and the announcement of a new Commissioner's National Priority Voucher program for companies supporting certain U.S. national health priorities and interests. To the extent our competitors are selected for this new voucher pilot program, or are otherwise able to participate in any of these initiatives intended to accelerate drug development and application review, and obtain faster approval than us, our competitive position may be harmed. The FDA has also increased its scrutiny of foreign drug manufacturing facilities and other contractors based in China, especially with respect to the transfer of biological materials, genetic data, and other sensitive data of U.S. patients to parties located in China. It is unclear how our industry and our clinical programs will be impacted by policies and regulations implemented under the current administration and FDA

leadership, or other executive orders. There is significant uncertainty in the industry and how federal agencies like the FDA will change in the coming years under the current administration. To the extent the agency reorganization and other agency changes lead to disruptions in the FDA's operations, our correspondence and regulatory review processes with the FDA may be materially delayed.

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

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

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

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

In addition, there have been a number of other legislative and regulatory proposals aimed at changing the pharmaceutical industry. For example, legislation has been enacted in certain states and at a federal level that requires manufacturers and other entities in the drug supply chain to track and trace each prescription drug at the saleable unit level through the distribution system. The FDA has finalized and proposed regulations implementing such requirements at a federal level. Our compliance with these requirements may be deemed insufficient and we could face a material adverse effect on our business, financial condition, results of operations and growth prospects. As a result of these and other new proposals, we may determine to change our current manner of operation, provide additional benefits or change our contract arrangements, any of which could have a material adverse effect on our business, financial condition and results of operations.

Adequate coverage and reimbursement in the United States and other markets is essential to the commercial success of our approved products. Recently in the United States, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products. For example, the Inflation Reduction Act of 2022 ("IRA") implements a number of drug pricing measures intended to lower the cost of prescription drugs and related healthcare reforms, including limits on price increases and subjecting an escalating number of drugs to annual price negotiations with CMS. The IRA includes several provisions that will impact our business to varying degrees, including provisions that

reduced the out-of-pocket spending cap for Medicare Part D beneficiaries to $2,100 in 2026; impose new manufacturer financial liability on all drugs in Medicare Part D; allow the U.S. Government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for drug prices that increase faster than inflation; and delay the rebate rule that would require pass through of pharmacy benefit manager rebates to beneficiaries. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication or indications are for that disease or condition. If a product receives multiple rare disease designations or has multiple approved indications for more than one disease or condition, it may not qualify for the orphan drug exemption.

In 2025, the U.S. Government took various steps, both legislative and executive, intended to lower the cost of prescription drugs. The President issued multiple executive orders and took other steps to secure pharmaceutical manufacturers' agreements to lower certain U.S. prescription drug prices to most favored nation levels, to facilitate direct-to-patient sales of certain U.S. prescription drugs, and to secure agreements to repatriate increased ex-U.S. revenues generated as a result of U.S. Government action that increases drug prices outside the United States. On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law in the United States. The OBBBA contains a variety of provisions that could impact our business and results of operations. On January 15, 2026, the White House proposed that Congress enact the Great Healthcare Plan Act, which seeks to lower prescription drug and insurance prices. Multiple U.S. states have also enacted legislation intended to decrease the price of prescription drugs.

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

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

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

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

We also may be subject to international privacy and data protection laws, such as the General Data Protection Regulation ("GDPR") in the European Economic Area ("EEA") and similar legislation in the United Kingdom and Switzerland. See "*Business—Government Regulation—Data Privacy and Security Laws*" in Part I, Item 1 of our most recent Annual Report on Form 10-K and "*Risks Relating to Our Business—Risks Relating to International Operations—*

Our actual or perceived failure to comply with European or other international governmental laws and regulations and other legal obligations related to privacy, data protection and information security could harm our business" in this section for additional discussion of privacy laws and regulations. Failure to comply with these laws and regulations could result in government enforcement actions, private litigation, or harm to our reputation and our business.

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

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

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

Intellectual Property Risks

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

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

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

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

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights, or may design around our patent claims to produce competitive products that fall outside the scope of our patents. For example, a third party may develop a competitive drug that is similar to one or more of our products or product candidates but that has a different composition that falls outside the scope of our patent protection. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive, time-consuming, and unsuccessful.

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

In addition, as described under "*Business—Government Regulation—FDA Regulation—Abbreviated New Drug Applications for Generic Drugs*" in Part I, Item 1 of our most recent Annual Report on Form 10-K, third parties may not file an ANDA for a generic drug with the FDA until the expiration of five years following the original product approval unless the submission is accompanied by a Paragraph IV certification, in which case third parties may submit an ANDA four years following the original product approval (referred to as the "NCE-1 date"). The NCE-1 date for ORLADEYO was in December 2024. In January 2025 and January 2026, we received a Paragraph IV notice of certification from Annora advising that Annora has submitted an ANDA to the FDA seeking approval to manufacture, use or sell a generic version of ORLADEYO in the United States prior to the expiration of four patents listed in the FDA's Orange Book, which expire in May 2040. On March 10, 2025, as supplemented by the First Amended Complaint filed in December 2025, we filed a patent infringement lawsuit in the United States District Court for the District of Delaware against the Defendants (as defined in "*Legal Proceedings*" included in Part II, Item 1 of this report) asserting infringement of the challenged patents arising from Annora's ANDA filing with the FDA. For further information, see the section titled "*Legal Proceedings*" included in Part II, Item 1 of this report and "*Note 15—Commitments and Contingencies"* in the Notes to the Condensed Consolidated Financial Statements in Part I, Item 1 of this report. We intend to vigorously defend our intellectual property rights protecting ORLADEYO. Additional third parties could challenge our applicable patents, which may result in our initiation of patent infringement litigation in response to such challenge. We cannot predict how any additional third party would address our listed patents, whether we would sue on any such patents, or the outcome of any such suit. However, litigation to enforce or defend intellectual property rights is complex, costly, and involves significant commitments of management's time.

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

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

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

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

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

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

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

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

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

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

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

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

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

any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and distract management and other employees.

Product Liability Risks

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

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

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

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

Risks Relating to Contractual Arrangements

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

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

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

In March 2011, JPR Royalty Sub LLC, our wholly owned subsidiary ("Royalty Sub"), issued $30.0 million in aggregate principal amount of PhaRMA Senior Secured 14.0% Notes due on December 1, 2020 (the "PhaRMA Notes"). The PhaRMA Notes are secured principally by (i) certain royalty and milestone payments under our agreement with Shionogi, pursuant to which Shionogi licensed from us the rights to market peramivir in Japan and Taiwan and (ii) the pledge by us of our equity interest in Royalty Sub. Since September 1, 2014, payments from Shionogi have been insufficient for Royalty Sub to service its obligations under the PhaRMA Notes, resulting in a continuing event of default

with respect to the PhaRMA Notes since that time. In addition, the PhaRMA Notes had a final legal maturity date of December 1, 2020, at which time the outstanding principal amount of the PhaRMA Notes of $30.0 million, together with accrued and unpaid interest of $20.6 million, was due in full. The failure by Royalty Sub to repay these amounts at the maturity date constituted an additional event of default under the PhaRMA Notes. As Royalty Sub has been unable to service its obligations under the PhaRMA Notes and continuing events of default exist under the PhaRMA Notes, the holders of the PhaRMA Notes may be able to foreclose on the collateral securing the PhaRMA Notes and our equity interest in Royalty Sub and may exercise other remedies available to them under the indenture or other documents related to the PhaRMA Notes. In such event, we may not realize the benefit of future royalty payments, if any, that might otherwise accrue to us following repayment of the PhaRMA Notes, we may incur legal costs, and we might otherwise be adversely affected.

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

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

We have incurred significant indebtedness, which could adversely affect our business. Additionally, the Blackstone Loan Agreement contains conditions and restrictions that limit our flexibility in operating our business. We may be required to make a prepayment or repay our outstanding indebtedness earlier than we expect if a prepayment event or an event of default occurs, including a material adverse change with respect to us, which could have a material adverse effect on our business.

On January 23, 2026, we entered into the Blackstone Loan Agreement, pursuant to which the lenders funded initial term loans in the aggregate principal amount of $400.0 million. Subject to the mutual agreement between the Company, Blackstone and the lenders, we may request additional term loans up to an aggregate amount not exceeding $150.0 million. Under the Blackstone Loan Agreement, we will be required to pay to the lenders a prepayment premium or a make-whole premium, as applicable, in the event that, prior to the fourth anniversary of the closing date of the Blackstone Loan Agreement, we prepay or repay, or are required to prepay or repay, voluntarily or pursuant to a mandatory prepayment obligation under the Blackstone Loan Agreement (e.g., upon certain asset sales, a change of control of the Company and specified other events, subject to certain exceptions), all or part of the then-outstanding term loans under the Blackstone Loan Agreement, in each case, subject to certain exceptions as set forth in the Blackstone Loan Agreement.

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

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

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

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

A breach of any of these covenants could result in an event of default under the Blackstone Loan Agreement. An event of default will also occur if, among other things, we fail to pay amounts due under the Blackstone Loan Agreement, we fail to repay certain other indebtedness having an aggregate principal amount in excess of a threshold amount, an insolvency event occurs with respect to us, judgments for the payment of money in excess of a threshold amount are entered into against us, or a material impairment of our ability to perform our obligations under the Blackstone Loan Agreement occurs or certain negative regulatory events occur. In the case of a continuing event of default under the Blackstone Loan Agreement, the lenders under the Blackstone Loan Agreement could elect to declare all amounts outstanding to be immediately due and payable, proceed against the collateral in which we granted to the lenders a security interest, or otherwise exercise the rights of a secured creditor. Amounts outstanding under the Blackstone Loan Agreement are secured by a security interest in, subject to certain exceptions, substantially all of our assets. Because substantially all of our assets are pledged to secure the Blackstone Loan Agreement obligations, our ability to incur additional secured indebtedness or to sell or dispose of assets to raise capital may be impaired, which could have an adverse effect on our financial flexibility.

Risks Relating to International Operations

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

We currently conduct clinical studies and regulatory activities and have hired 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;
- the potential for parallel importing;
- complexities and difficulties in obtaining and maintaining protection for, and enforcing, our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits on our ability to penetrate international markets;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products, and exposure to foreign currency exchange rate fluctuations, which have been increasingly prevalent alongside a fluctuating U.S. dollar;
- natural disasters and political and economic instability, including wars, terrorism, political unrest, results of certain elections and votes, actual or threatened public health emergencies and outbreak of disease, epidemics or pandemics (e.g., the COVID-19 pandemic), boycotts, adoption or expansion of government trade restrictions, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance;
- regulatory and compliance risks that relate to maintaining accurate information and control over commercial operations and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, including its books and records provisions or anti-bribery provisions, and foreign laws and regulations; and
- regulatory and compliance risks relating to doing business with any entity that is subject to sanctions administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury.

Any of these factors could significantly harm our international expansion of operations and adversely affect our business and results of operations. Additionally, in some countries, such as Japan, the pricing of prescription pharmaceuticals is subject to governmental control and access. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or

pricing approval in some countries, we or our partners may be required to conduct a clinical trial that compares the cost-effectiveness of our product to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

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

We conduct operations in countries outside of the United States involving transactions in a variety of currencies other than the U.S. dollar. These transactions include, without limitation, commercial sales, contract manufacturing, and clinical trial activities. Although most of our revenues and expenses are denominated in U.S. dollars, we have foreign currency exposure to fluctuations in other foreign currencies, such as the Euro, Japanese Yen and Canadian Dollar. Changes in the value of these currencies relative to the U.S. dollar may impact our condensed consolidated operating results, including our revenues and expenses, causing fluctuations in our operating results from period to period and/or resulting in foreign currency transaction losses that adversely impact our results of operations, financial position, and cash flows. See "*Quantitative and Qualitative Disclosures about Market Risk—Foreign Currency Risk*" in Part I, Item 3 of this report for additional information about our foreign currency risk.

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

Outside the United States, an increasing number of laws and regulations may govern data privacy and security. EU member states, the United Kingdom, Switzerland and other countries have adopted data protection laws and regulations, which impose significant compliance obligations. These laws include the GDPR and similar national legislation within the EEA, the United Kingdom GDPR, Switzerland's Federal Data Protection Act, the EU Clinical Trials Regulation, and the e-Privacy Directive (2002/58/EC), as well as laws and regulations outside Europe, and are discussed in more detail in "*Business—Government Regulation—Data Privacy and Security Laws*" in Part I, Item 1 of our most recent Annual Report on Form 10-K. Failure to comply with the requirements of these laws may result in significant fines. For example, noncompliance with the GDPR or related national data protection laws, which may deviate from the GDPR, may result in significant fines of up to 4.0% of global revenues, or €20.0 million, whichever is greater.

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

Compliance with evolving laws regarding the transfer of personal data to the United States and other countries also requires increased resources and may result in increased exposure to regulatory actions, fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. We are also subject to evolving European and other privacy laws on electronic marketing and cookies.

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

litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences.

Risks Relating to Technology

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

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

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

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

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

Cyber incidents could also include the use of artificial intelligence ("AI") and machine learning to launch more automated, targeted and coordinated attacks on targets. Cyber incidents may lead to operational outages, loss of intellectual property due to industrial espionage, malware, and financial or data attacks via social engineering. These risks are heightened by the wide adoption of virtual and remote working, with sensitive data accessed by employees working in less secure, home-based environments, as well as the increasing use of generative AI by both employees and third parties. A breakdown, invasion, corruption, destruction, or interruption of information technology systems could negatively impact operations. If our systems are damaged, fail to function properly or otherwise become unavailable, we may incur substantial costs to repair or replace them, and we may experience loss of critical data and interruptions or delays in our ability to perform critical functions, which could adversely affect our business, financial condition or results of operations.

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

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

Any compromise of our data security could also result in a violation of applicable privacy and other laws, significant legal, regulatory, and financial exposure, damage to our reputation, loss or misuse of the information and a loss of confidence in our data security measures, which could harm our business. Loss or misuse of our intellectual property, clinical trial data, or commercially sensitive data could adversely impact our business. While we have implemented security measures designed to protect against security incidents and a significant portion of our data is included in regular

backups of our systems, there can be no assurance that our efforts to protect our data and information technology systems will prevent breakdowns or breaches in our systems, or those of third parties with which we do business, and any such events could adversely affect our business.

We use AI in our business, and challenges with properly managing its use could adversely affect our business.

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

Other Operational Risks

Our ability to maintain global brand uniformity for ORLADEYO may be impacted by the sale of our European ORLADEYO business.

In connection with the sale of our European ORLADEYO business, we entered into the Global Brand and Support Agreement, which provides for coordination of brand and regulatory activities regarding ORLADEYO products. While this agreement is intended to promote alignment on brand and related activities with Neopharmed, we do not have sole control over how ORLADEYO is positioned, supported or communicated in Europe, and we may not be able to maintain global brand uniformity with respect to ORLADEYO. This risk may be heightened given that ORLADEYO is indicated for a rare disease with a limited number of key opinion leaders and relatively few scientific publications or forums that reach a broad global audience. If we are unable to maintain a consistent and effective global brand presence for ORLADEYO, our ability to maximize the anticipated benefits of the sale, support future growth and realize the expected long-term value of ORLADEYO could be adversely affected.

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

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

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

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

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

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

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

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

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

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

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

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

The unexpected loss of service of our senior management and other key personnel, or our scientific advisors, might impede the achievement of our development and commercial objectives. Competition for key personnel with the experience that we require is intense and is expected to continue to increase. Our inability to attract and retain the required number of skilled and experienced management, commercial, operational and scientific personnel may harm our business because we rely upon these personnel for many important functions of our business.

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

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

attend. The risk management committee, along with the other sub-committees in the Company, identifies key risks and mitigation strategies which are reported directly to our senior management, the Audit Committee and to the full Board of Directors on a regular basis.

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

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

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

Our strategic shift to focus on external innovation, including in connection with the Birmingham Closure Plan, may increase our reliance on acquisitions, strategic investments, partnerships or other business development transactions to expand our product candidate pipeline. We may not identify suitable opportunities, complete transactions on acceptable terms or at all, or realize the anticipated operational, financial or strategic benefits of any such transactions or related initiatives on the expected timeline or at all. Additionally, actual costs of the Birmingham Closure Plan may exceed our estimates, anticipated savings may not be realized or may be offset by costs associated with external innovation, business development, acquired or licensed programs or other strategic priorities, and the closure of our Birmingham facility and related workforce reduction may result in the loss of institutional knowledge or technical expertise, adversely affect employee morale or retention, divert management attention or disrupt our operations. If we do not execute this strategy effectively, our business, financial condition, results of operations, liquidity and ability to execute our strategic priorities could be adversely affected.

In addition, we may divest or license all or a portion of certain business or product categories, which could cause a decline in revenue or profitability and may make our financial results more volatile. We may be unable to complete any such divestiture or license on terms favorable to us, within the expected timeframes, or at all, in which case we may not realize any return on our investment in these programs and may incur costs associated with their termination. For example, after evaluating potential partnering opportunities for continued development, we previously determined to terminate the avoralstat program and the STAR-0310 atopic dermatitis program acquired in the Merger to focus the pipeline on rare diseases. We may have continued financial exposure to divested or licensed businesses following the completion of any such transactions, including increased costs due to potential litigation, contingent liabilities and indemnification of the buyer or licensee related to, among other things, lawsuits, regulatory matters or tax liabilities. Such divestitures or licenses may also divert management’s attention from our core businesses and lead to potential issues with employees, customers or suppliers.

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

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

expenses, related to activist stockholder matters. Our stock price could be subject to significant fluctuation or otherwise be adversely affected by the events, risks and uncertainties of any stockholder activism.

Risks Relating to Investing in Our Common Stock

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

Some of our stockholders own greater than 5% of our outstanding common stock. Our top ten stockholders own approximately 50% of our common stock and can individually, and as a group, influence our operations based upon their concentrated ownership and may also be able to influence the outcome of matters requiring approval of the stockholders, including the election of our directors and other corporate actions.

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

The market prices for securities of biotechnology companies in general have been highly volatile and may continue to be highly volatile in the future. Moreover, our stock price has fluctuated frequently, and these fluctuations are often not related to our financial results. For the twelve months ended September 30, 2026, the 52-week range of the market price of our stock was from $6.00 to $11.22 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 or issuances 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 or changes to our operations or business strategy (such as the Birmingham Closure Plan);
- us or our partners achieving or failing to achieve development milestones;
- publicity regarding actual or potential medical results relating to products under development by us or our competitors;
- publicity regarding certain public health concerns for which we are or may be developing treatments;
- regulatory developments in both the United States and foreign countries;
- public concern as to the safety of pharmaceutical products;
- actual or anticipated fluctuations in our operating results;
- changes in financial estimates or recommendations by securities analysts and the comparison of such estimates to our actual results;
- online automated financial platforms' treatment or classification of our financial information;
- changes in our public guidance;
- changes in the structure of healthcare payment systems, including developments in price control legislation;
- announcements by us or our competitors of significant acquisitions (such as the Merger), strategic partnerships, divestitures (such as the sale of our European ORLADEYO business to Neopharmed), joint ventures, capital commitments or other monetization transactions;
- additions or departures of key personnel or members of our Board of Directors;
- purchases or sales of substantial amounts of our stock by existing stockholders, including officers or directors;
- economic and other external factors or other disasters or crises; and
- period-to-period fluctuations in our financial results.

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

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

As a public company, we are required to maintain effective internal control over financial reporting (as described in "*Controls and Procedures*" in Part I, Item 4 of this report), and effective disclosure controls and procedures. If we identify one or more material weaknesses in our internal control over financial reporting, we will not be able to assert that our internal controls and procedures are effective. A material weakness, as defined in Rule 12b-2 under the Exchange Act, is a deficiency, or combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of annual or interim financial statements will not be prevented or detected on a timely basis. In 2023, we identified and timely reported two material weaknesses in our internal control over financial reporting, which management determined to be subsequently remediated as of December 31, 2023 and September 30, 2024, respectively.

Although we believe the financial statements included in this report fairly present, in all material respects, our financial condition, results of operations and cash flows for the periods presented in conformity with U.S. GAAP, any failure to maintain effective internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If our internal control over financial reporting is not effective, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

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

Future sales of our common stock by us or our current stockholders into the public market could cause the market price of our stock to fall. As of September 30, 2026, there were 256,117,756 shares of our common stock outstanding. We may from time to time issue securities in relation to a license arrangement, collaboration, merger or acquisition (such as in connection with the Merger). 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 September 30, 2026, there were 43,786,380 stock options and restricted stock units outstanding and 15,785,435 shares available for issuance under our Amended and Restated Stock Incentive Plan, 4,819,944 stock options and restricted stock units outstanding and 1,000,929 shares available for issuance under our Amended and Restated Inducement Equity Incentive Plan, and 4,326,612 shares available for issuance under our Amended and Restated Employee Stock Purchase Plan. In addition, we could also make equity grants outside of our Amended and Restated Stock Incentive Plan or Amended and Restated Inducement Equity Incentive Plan. The shares underlying existing stock options, restricted stock units and possible future stock options, stock appreciation rights, restricted stock units and stock awards have been, or will be, registered pursuant to registration statements on Form S-8.

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

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

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

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

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

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

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

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

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

General Risk Factors

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

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

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

From time to time, we may be involved in disputes, including, without limitation, disputes with our employees, collaborative partners, and third-party vendors. We may be called upon to initiate legal proceedings or to defend ourselves in such legal proceedings relating to our relationships with these parties, our decisions and actions or omissions with respect thereto, and our business. In addition, if our stock price is volatile, we may become involved in securities class action lawsuits in the future. Due to the inherent uncertainties in legal proceedings, we cannot accurately predict the ultimate outcome of any such proceeding. An unfavorable outcome in any such proceeding could have an adverse impact

on our business, financial condition and results of operations. Any current or future dispute resolution or legal proceeding, regardless of the merits of any such proceeding, could harm our reputation and result in substantial costs and a diversion of management's attention and resources that are needed to successfully run our business.

Item 5. Other Information

Director and Officer Trading Arrangements

During the three months ended September 30, 2026, no director or officer of the Company adopted, modified or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits

Number	Description
3.1	Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed December 22, 2006.
3.2	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed July 24, 2007.
3.3	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed May 7, 2014.
3.4	Certificate of Elimination of the Series B Junior Participating Preferred Stock. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed May 13, 2020.
3.5	Certificate of Amendment to the Third Restated Certificate of Incorporation of Registrant. Incorporated by reference to Exhibit 3.2 to the Company's Form 8-K filed May 13, 2020.
3.6	Amended and Restated By-Laws of BioCryst Pharmaceuticals, Inc., effective January 16, 2024. Incorporated by reference to Exhibit 3.1 to the Company's Form 8-K filed January 18, 2024.
(31.1)	Certification of the Principal Executive Officer Pursuant to Rule 13a-14(a) and Rule 15d-14(a) of the Securities Exchange Act of 1934, as Amended, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
(31.2)	Certification of the Principal Financial Officer Pursuant to Rule 13a-14(a) and Rule 15d-14(a) of the Securities Exchange Act of 1934, as Amended, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
(32.1)*	Certification of the Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
(32.2)*	Certification of the Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
(101)	Financial statements from the Quarterly Report on Form 10-Q of BioCryst Pharmaceuticals, Inc. for the three and nine months ended September 30, 2026, formatted in Inline XBRL: (i) Condensed Consolidated Balance Sheets, (ii) Condensed Consolidated Statements of Comprehensive Income (Loss), (iii) Condensed Consolidated Statements of Cash Flows, (iv) Condensed Consolidated Statements of Stockholders' Deficit, and (v) Notes to Condensed Consolidated Financial Statements, tagged as blocks of text and including detailed tags.
(104)	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).
( )	Filed herewith.
*	The certification is being furnished solely to accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, and will not be deemed "filed" for purposes of Section 18 of the Exchange Act, except to the extent that the Company specifically incorporates it by reference.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized on this 4th day of November, 2026.

BIOCRYST PHARMACEUTICALS, INC.

By: /s/ Charles Gayer

Charles Gayer
Chief Executive Officer
(Principal Executive Officer)

By: /s/ Babar Ghias

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

Exhibit 31.1

CERTIFICATIONS

I, Charles Gayer, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 4, 2026

/s/ Charles Gayer

Charles Gayer

Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Babar Ghias, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 4, 2026

/s/ Babar Ghias

Babar Ghias

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

Exhibit 32.1

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

In connection with the Quarterly Report of BioCryst Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended September 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Charles Gayer, Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

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

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

/s/ Charles Gayer

Charles Gayer

Chief Executive Officer
(Principal Executive Officer)
Date: November 4, 2026

Exhibit 32.2

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

In connection with the Quarterly Report of BioCryst Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ended September 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Babar Ghias, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

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

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

/s/ Babar Ghias

Babar Ghias

Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)

Date: November 4, 2026