UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

Form 10-Q

(Mark One)

x 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

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

For the transition period from _____ to _____

Commission File Number**: 001-34079**

Opus Genetics, Inc.

(Exact name of Registrant as specified in its charter)

Delaware	**11-3516358**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
8 Davis Drive, Suite 220 Durham, NC	**27713**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (984) 884-6030

N/A
(Former name or former address, if changed since last report)

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

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	**IRD**	**The Nasdaq Stock Market LLC**

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

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

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

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

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

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

The number of outstanding shares of the registrant's common stock as of November 6, 2026 was 96,654,336.

OPUS GENETICS, INC.
FORM 10-Q
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PART I – FINANCIAL INFORMATION

Item 1. Financial Statements

Opus Genetics, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share amounts and par value)

	As of	
	September 30, 2026	**December 31, 2025**
Assets	(Unaudited)	
Current assets:		
Cash and cash equivalents	$ 88,974	$ 45,091
Accounts receivable	1,005	1,995
Contract assets and unbilled receivables (Note 12)	628	1,170
Prepaids and other current assets	4,249	1,788
Total current assets	94,856	50,044
Property and equipment, net	339	199
Restricted cash	100	—
Total assets	$ 95,295	$ 50,243
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,727	$ 3,293
Accrued expenses	6,105	4,488
Total current liabilities	9,832	7,781
Long-term debt	34,530	—
Warrant liabilities	11,151	25,985
Funding agreement, related party	1,351	1,129
Total liabilities	56,864	34,895
Commitments and contingencies (Note 4)		
Stockholders' equity:		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of September 30, 2026 and December 31, 2025; no shares issued and outstanding at September 30, 2026 and December 31, 2025.	—	—
Common stock, par value $0.0001; 250,000,000 and 125,000,000 shares authorized as of September 30, 2026 and December 31, 2025, respectively; 95,799,574 and 69,894,507 shares issued and outstanding at September 30, 2026 and December 31, 2025, respectively.	10	7
Additional paid-in capital	346,032	203,930
Accumulated other comprehensive income	1,106	—
Accumulated deficit	(308,717)	(188,589)
Total stockholders' equity	38,431	15,348
Total liabilities and stockholders' equity	$ 95,295	$ 50,243

See accompanying notes.

Opus Genetics, Inc.
Condensed Consolidated Statements of
Comprehensive Loss
(in thousands, except share and per share amounts)
(Unaudited)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2026	2025	2026	2025
License and collaborations revenue	$ 1,137	$ 3,079	$ 4,049	$ 10,331
Operating expenses:				
Research and development	14,494	6,409	36,278	20,384
General and administrative	5,890	4,981	17,862	17,093
Total operating expenses	20,384	11,390	54,140	37,477
Loss from operations	(19,247)	(8,311)	(50,091)	(27,146)
Fair value change in instruments measured at fair value	(28,610)	(9,525)	(72,800)	(5,803)
Financing costs	—	—	(1,113)	(1,337)
Other income, net	1,362	382	3,876	1,218
Loss before income taxes	(46,495)	(17,454)	(120,128)	(33,068)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(46,495)	(17,454)	(120,128)	(33,068)
Change in fair value of the Notes attributable to instrument specific credit risk	1,106	—	1,106	—
Comprehensive loss	$ (45,389)	$ (17,454)	$ (119,022)	$ (33,068)
Net loss per share:				
Basic and diluted	$ (0.46)	$ (0.25)	$ (1.27)	$ (0.59)
Number of shares used in per share calculations:				
Basic and diluted	101,841,608	70,636,887	94,793,509	56,100,689

See accompanying notes.

Opus Genetics, Inc.
Condensed Consolidated Statements of Changes in
Convertible Preferred Stock and Stockholders' Equity (Deficit)
(in thousands, except share amounts)
(Unaudited)

	Series B preferred stock		Common stock		Additional paid–in capital	Accumulated other comprehensive income	Accumulated deficit	Total stockholders' equity
	Shares	Amount	Shares	Amount				
Balance at December 31, 2025	—	$ —	69,894,507	$ 7	$ 203,930	$ —	$ (188,589)	$ 15,348
Issuance of Series B preferred stock, net of issuance costs of $264	7,374,632	24,736	—	—	—	—	—	—
Issuance of common stock in connection with at-the-market program, net of issuance costs of $68	—	—	1,000,000	—	2,182	—	—	2,182
Stock-based compensation		—	118,465	—	1,464	—	—	1,464
Share repurchases for the payment of employee taxes	—	—	(12,653)	—	(33)	—	—	(33)
Exercise of stock options	—	—	226,115	—	738	—	—	738
Vesting of restricted stock units	—	—	176,038	—	—	—	—	—
Net and comprehensive loss	—	—	—	—	—	—	(65,535)	(65,535)
Balance at March 31, 2026	7,374,632	$ 24,736	71,402,472	$ 7	$ 208,281	$ —	$ (254,124)	$ (45,836)
Conversion of Series B preferred stock to common stock	(7,374,632)	(24,736)	7,374,632	1	24,735	—	—	24,736
Issuance of common stock in connection with stock purchase and conversion agreement, net of issuance costs of $159	—	—	1,116,070	—	4,841	—	—	4,841
Exercise of warrants	—	—	1,750,126	—	9,480	—	—	9,480
Exercise of stock options	—	—	177,173	—	484	—	—	484
Stock-based compensation	—	—	—	—	1,407	—	—	1,407
Vesting of restricted stock units	—	—	827,473	—	—	—	—	—
Net and comprehensive loss	—	—	—	—	—	—	(8,098)	(8,098)
Balance at June 30, 2026	—	$ —	82,647,946	$ 8	$ 249,228	$ —	$ (262,222)	$ (12,986)
Exercise of warrants	—	—	12,523,751	1	94,232	—	—	94,233
Exercise of stock options	—	—	194,431	—	372	—	—	372
Stock-based compensation	—	—	—	—	1,590	—	—	1,590
Vesting of restricted stock units	—	—	233,446	1	—	—	—	1
Issuance of common stock in connection with stock subscription agreement	—	—	200,000	—	610	—	—	610
Change in fair value of the Notes attributable to instrument specific credit risk	—	—	—	—	—	1,106	—	1,106
Net loss	—	—	—	—	—	—	(46,495)	(46,495)
Balance at September 30, 2026	—	$ —	95,799,574	$ 10	$ 346,032	$ 1,106	$ (308,717)	$ 38,431

	Series A preferred stock		Common stock		Additional paid–in capital	Accumulated deficit	Total stockholders' equity
	Shares	Amount	Shares	Amount			
Balance at December 31, 2024	14,145.374	$ 18,843	31,574,657	$ 3	$ 145,719	$ (138,998)	$ 6,724
Issuance of common stock and pre-funded warrants in connection with the March 2025 offering and private placement	—	—	13,396,207	1	5,979	—	5,980
Issuance of common stock in connection with at-the-market program	—	—	352,953	1	408	—	409
Issuance costs	—	—	—	—	(728)	—	(728)
Stock-based compensation		—	84,243	—	913	—	913
Share repurchases for the payment of employee taxes	—	—	(31,913)	—	(36)	—	(36)
Exercise of stock options	—	—	5,000	—	5	—	5
Vesting of restricted stock units	—	—	102,676	—	—	—	—
Net and comprehensive loss	—	—	—	—	—	(8,194)	(8,194)
Balance at March 31, 2025	14,145.374	$ 18,843	45,483,823	$ 5	$ 152,260	$ (147,192)	$ 5,073
Conversion of preferred stock	(14,145.374)	(18,843)	14,145,374	1	18,842	—	18,843
Stock-based compensation	—	—	278,858	—	896	—	896
Issuance cost, net credit	—	—	—	—	81	—	81
Net and comprehensive loss	—	—	—	—	—	(7,420)	(7,420)
Balance at June 30, 2025	—	$ —	59,908,055	$ 6	$ 172,079	$ (154,612)	$ 17,473
Issuance of common stock in connection with private placement and at-the-market program	—	—	4,611,041	—	5,217	—	5,217
Issuance costs	—	—	—	—	(107)	—	(107)
Stock-based compensation	—	—	—	—	816	—	816
Exercise of stock options	—	—	25,000	—	22	—	22
Net and comprehensive loss	—	—	—	—	—	(17,454)	(17,454)
Balance at September 30, 2025	—	$ —	64,544,096	$ 6	$ 178,027	$ (172,066)	$ 5,967

See accompanying notes.

Opus Genetics, Inc.
Condensed Consolidated Statements of Cash Flows
(in thousands)
(Unaudited)

	For the Nine Months Ended September 30,	
	2026	**2025**
Operating activities		
Net loss	$ (120,128)	$ (33,068)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	4,461	2,625
Depreciation	49	40
Fair value change in instruments measured at fair value, net of cash interest paid of $651	72,149	5,803
Non-cash interest, related party	222	68
Non-cash share issuances	610	—
Issuance costs related to financing activities	1,113	1,337
Unrealized loss from short-term investments	—	2
Change in assets and liabilities:		
Accounts receivable	990	647
Contract assets and unbilled receivables	542	845
Prepaids and other current assets	(772)	(300)
Accounts payable	434	(797)
Accrued expenses	1,617	(2,622)
Net cash used in operating activities	(38,713)	(25,420)
Investing activities		
Purchase of fixed assets	(189)	—
Net cash used in investing activities	(189)	—
Financing activities		
Proceeds from note purchase agreement	35,000	—
Proceeds from stock purchase and conversion agreement	5,000	—
Proceeds from issuance of Series B preferred stock	25,000	—
Proceeds from issuance of common stock and pre-funded warrants in connection with the March 2025 offering and March 2025 private placement	—	5,980
Proceeds from issuance of warrants in connection with the March 2025 offering and March 2025 private placement	—	15,520
Proceeds from issuance of common stock in connection with the at-the-market program, private placement and purchase agreement	2,250	5,626
Issuance costs attributed to equity instruments	(491)	(2,203)
Issuance costs attributed to note purchase agreement	(1,113)	—
Proceeds from funding agreement, related party	—	1,000
Exercise of warrants	15,677	—
Exercise of stock options	1,595	27
Share repurchases for the payment of employee taxes	(33)	(36)
Net cash provided by financing activities	82,885	25,914
Net increase in cash, cash equivalents and restricted cash	43,983	494
Cash, cash equivalents and restricted cash at beginning of period	45,091	30,321
Cash, cash equivalents and restricted cash at end of period	$ 89,074	$ 30,815
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 651	$ —
Supplemental non-cash financing transactions:		
Conversion of preferred stock instruments	$ 24,736	$ 18,843
Reclassification of warrant liability upon exercise	$ 86,347	$ —
Paid-in-kind interest on outstanding Notes	$ 651	$ —
Warrant exercises unpaid as of quarter end	$ 1,689	$ —
Unpaid issuance costs	$ —	$ 112

See accompanying notes.

Notes to Condensed Consolidated Financial Statements

1. Company Description and Summary of Significant Accounting Policies

Nature of Business and Basis of Presentation

Opus Genetics, Inc. (the "Company" or "Opus"), a Delaware corporation formerly known as Ocuphire Pharma, Inc., is a clinical-stage biopharmaceutical company developing gene therapies to restore vision and prevent blindness in patients with inherited retinal diseases ("IRDs"). The Company also has a small molecule therapy for other ophthalmic disorders. The Company's headquarters is located in Durham, North Carolina.

The Company's pipeline is centered on adeno-associated virus ("AAV") based gene therapy programs addressing genetically defined forms of IRD, including OPGx-LCA5, which is currently being evaluated for the treatment of Leber congenital amaurosis caused by mutations in the LCA5 gene, and OPGx-BEST1, which is currently being evaluated for retinal diseases associated with mutations in the BEST1 gene. In addition, the Company is advancing other gene therapy programs, including RDH12, MERTK, RHO, CNGB1 and NMNAT1, and research activities targeting additional monogenic retinal disorders.

Apart from gene therapies, the Company's pipeline also includes Phentolamine Ophthalmic Solution 0.75%, a non-selective alpha-1 and alpha-2 adrenergic antagonist to reduce pupil size.

In November 2022, the Company entered into a license and collaboration agreement (as amended, the "Viatris License Agreement") with Viatris, Inc. ("Viatris"), pursuant to which it granted Viatris an exclusive license to develop, manufacture, import, export and commercialize its refractive product candidate Phentolamine Ophthalmic Solution 0.75% ("PS"). PS is a once-daily eye drop formulation of phentolamine mesylate designed to reduce pupil diameter and improve visual acuity. PS was approved by the U.S. Food and Drug Administration ("FDA") for the treatment of pharmacologically induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents, or a combination thereof under the brand name RYZUMVI® in September 2023 and was launched commercially in April 2024. In October 2026, the FDA approved PS for the treatment of presbyopia in adults (see Note 16 – Subsequent Events). The Company is also developing PS for the treatment of decreased vision under mesopic (low-light) conditions following keratorefractive surgery, pursuant to a Special Protocol Assessment agreement with the FDA.

Basis of Presentation

The accompanying condensed consolidated financial statements have been prepared by the Company, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC") and include the accounts of the Company and its subsidiaries. All intercompany transactions and balances have been eliminated in consolidation. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. generally accepted accounting principles ("GAAP") have been condensed or omitted pursuant to such rules and regulations. The Company's fiscal year begins on January 1 and ends on December 31.

The December 31, 2025 condensed consolidated balance sheet was derived from audited financial statements and may not include all disclosures required by GAAP; however, the Company believes that the disclosures are adequate to make the information presented not misleading. The accompanying condensed consolidated financial statements should be read in conjunction with the audited financial statements and related notes thereto for the year ended December 31, 2025 included in the Company's Annual Report on Form 10-K filed with the SEC on March 12, 2026.

In the opinion of management, all adjustments, consisting of only normal recurring adjustments that are necessary to present fairly the financial position, results of operations, and cash flows for the interim periods, have been made. The results of operations for the interim periods are not necessarily indicative of the operating results for the full fiscal year or any future periods.

Liquidity

Since its inception, the Company has devoted substantially all of its resources to drug development and clinical trials.

As of September 30, 2026, the Company had $89.0 million in cash and cash equivalents. The Company believes its current available cash and cash equivalents will be sufficient to fund its operations for at least the next 12 months from the date of issuance of these financial statements.

Notes to Condensed Consolidated Financial Statements

In the future, the Company may need to raise additional funds until it is able to generate sufficient revenues to fund its development activities. The Company's future operating activities, coupled with its plans to raise capital or issue debt financing, may provide additional liquidity in the future; however, these actions are not solely within the control of the Company and the Company is unable to predict the outcome of these actions to generate the liquidity ultimately required.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the condensed consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

Segment Information

Operating segments are components of an enterprise for which separate financial information is available and are evaluated regularly by the Company's chief operating decision maker (the "CODM") in deciding how to allocate resources and assess performance. The Company's CODM is its Chief Executive Officer, who views the Company's operations and manages its business in one operating segment, which is the business of development of products related to vision performance and health. Accordingly, the condensed consolidated financial statements and accompanying notes contained herein include the measure of profit or loss, categories of expenses and other financial information that is evaluated by the Company's Chief Executive Officer.

Summary of Significant Accounting Policies

The Company's significant accounting policies are described in Note 1, *"Company Description and Summary of Significant Accounting Policies*" to the consolidated financial statements in its Annual Report on Form 10-K for the year ended December 31, 2025, that was filed with the SEC on March 12, 2026. Since the date of those financial statements, there were no material changes to the significant accounting policies, except as discussed below.

Fair Value Option Election

The Company has elected to account for its senior secured notes (the "Notes") under the fair value option in accordance with ASC 825, *Financial Instruments*. The Notes were issued pursuant to the senior secured note purchase agreement (as amended from time to time, the "Note Purchase Agreement"), dated as of April 2, 2026, by and among the Company and certain of its subsidiaries as guarantors and OPCM SA LLC, as purchaser agent ("Purchaser Agent"), and certain purchasers party thereto (the "Purchasers") (see Note 7 – Debt) and are financial liabilities that contain certain embedded features that otherwise may have required separate evaluation and potential bifurcation under ASC 815, *Derivatives and Hedging*. Under the fair value option, the Notes were initially recognized at fair value on the issuance date and are subsequently remeasured at fair value at each reporting date. Changes in fair value are recognized in earnings, except for changes attributable to instrument-specific credit risk, which are recognized in other comprehensive income.

Recent Accounting Pronouncements

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. This ASU is intended to improve the disclosures related to expenses and provide investors more detailed information about certain types of expenses. This ASU is effective for annual periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027, with early adoption permitted. The Company is currently evaluating the potential impact that this new standard will have on its consolidated financial statements and related disclosures.

2. Cash, cash equivalents and restricted cash

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the condensed consolidated balance sheets that sum to the total of the same such amounts shown in the condensed consolidated statements of cash flows (in thousands):

Notes to Condensed Consolidated Financial Statements

	As of	
	September 30, 2026	**December 31, 2025**
Cash and cash equivalents	$ 88,974	$ 45,091
Restricted cash	100	—
Total cash, cash equivalents and restricted cash	**$ 89,074**	**$ 45,091**

Amounts included in restricted cash as of September 30, 2026 represent cash held as collateral for outstanding letters of credit provided as security deposit for the Company's credit card facility.

3. Fair Value Measurements

The Company follows accounting guidance that emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Fair value is defined as "the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date." Fair value measurements are defined on a three-level hierarchy:

- Level 1 inputs: Unadjusted quoted prices for identical assets or liabilities in active markets;
- Level 2 inputs: Quoted prices for similar assets and liabilities in active markets, quoted prices in markets that are not active, or inputs which are observable, whether directly or indirectly, for substantially the full term of the asset or liability; and
- Level 3 inputs: Unobservable inputs in which there is little or no market data available, which requires management to develop its own assumptions in pricing the asset or liability.

As of September 30, 2026 and December 31, 2025, the fair values of cash and cash equivalents, accounts receivable, contract assets and unbilled receivables, prepaid and other assets, accounts payable and accrued expenses approximated their carrying values because of the short-term nature of these assets or liabilities. The value of the long-term funding agreement, related party line item was determined on an amortized cost basis, based on an effective rate of interest.

The fair values of the financial liabilities associated with the March 2025 Warrants and, prior to their exercise, the March 2025 Private Placement Warrants (as defined below) are estimated using Monte Carlo simulation models that incorporate Level 3 inputs, including the Company's stock price, expected volatility, expected term (including the estimated probability and timing of the call features) and risk-free interest rates (see Note 9 – Financings).

The fair value of the financial liabilities associated with the Notes pursuant to the Note Purchase Agreement represents the present value of estimated future payments, including interest, principal, and Repayment Amount (as defined in the Note Purchase Agreement; see Note 7 – Debt). The fair value measurement is based on significant Level 3 unobservable inputs such as the probability and timing of the Repayment Amount and the discount rate. The Company determined the fair value of the Notes utilizing a discounted cash flow model of estimated future payments including interest, principal, and Repayment Amount utilizing a discount rate calculated as the term-matched risk-free rate plus credit spread. At April 21, 2026, the Notes issuance date, the Company utilized a discount rate of 8.9% and determined the fair value of the Notes to be $35.0 million. At September 30, 2026, the Company utilized a discount rate of 10.8%. The fair value of the Notes at September 30, 2026 was $34.5 million**,** which differed from the contractual principal amount of $35.7 million, inclusive of paid-in-kind interest, by $(1.2) million. Significant increases or decreases in any of these inputs in isolation could result in a significantly lower or higher fair value measurement.

There were no transfers between fair value hierarchy levels during the three and nine months ended September 30, 2026 and 2025.

Notes to Condensed Consolidated Financial Statements

The fair value of financial instruments measured on a recurring basis is as follows (in thousands):

	As of September 30, 2026			
Description	**Total**	**Level 1**	**Level 2**	**Level 3**
Financial liabilities:				
Warrant liabilities	$ 11,151	$ —	$ —	$ 11,151
Notes	$ 34,530	$ —	$ —	$ 34,530
Total financial liabilities at fair value	$ 45,681	$ —	$ —	$ 45,681

	As of December 31, 2025			
Description	**Total**	**Level 1**	**Level 2**	**Level 3**
Financial liabilities:				
Warrant liabilities	$ 25,985	$ —	$ —	$ 25,985
Notes	$ —	$ —	$ —	$ —
Total financial liabilities at fair value	$ 25,985	$ —	$ —	$ 25,985

The following table provides a roll-forward of the warrant financial liability measured at fair value on a recurring basis using unobservable Level 3 inputs for the nine months ended September 30, 2026 and 2025 (in thousands):

	Nine Months Ended September 30,	
	2026	**2025**
Warrant liabilities		
Balance as of beginning of period	$ 25,985	$ —
Issuance of March 2025 Warrants and March 2025 Private Placement Warrants	—	15,520
Change in fair value	71,513	5,805
Warrant exercises	(86,347)	—
Balance as of end of period	$ 11,151	$ 21,325

The following table provides a roll-forward of the Notes financial liability measured at fair value on a recurring basis using unobservable Level 3 inputs for the period from April 21, 2026 through September 30, 2026 (in thousands):

Fair value at April 21, 2026 (issuance)	**$ 35,000**
Change in fair value reported in net loss	1,287
Change in fair value reported in other comprehensive income	(1,106)
Interest payments	(651)
Fair value at September 30, 2026	**$ 34,530**

There were no financial instruments measured on a non-recurring basis for any of the periods presented.

4. Commitments and Contingencies

Apexian Sublicense Agreement

On January 21, 2020, the Company entered into a sublicense agreement with Apexian Pharmaceuticals, Inc. (“Apexian”), pursuant to which the Company obtained exclusive worldwide patent and other intellectual property rights. In exchange for the patent and other intellectual property rights, the Company agreed to certain milestone payments and royalty payments on future sales (see Note 11 – Apexian Sublicense Agreement). The sublicense agreement was terminated effective July 10, 2026, and the Company has no further milestone or royalty payment obligations thereunder.

Notes to Condensed Consolidated Financial Statements

University of Pennsylvania LCA5/RDH12 License Agreement

On June 15, 2022, Opus entered into an amended and restated license agreement (the "LCA5/RDH12 Agreement") with the Trustees of the University of Pennsylvania ("Penn") pursuant to which it was granted an exclusive, royalty-bearing license to certain patents and a non-exclusive license to certain information relating to products directed towards treatment or correction of mutation of the LCA5 or RDH12 genes. In return for these rights, the Company is obligated to make certain development, regulatory and commercial milestone payments up to a maximum potential aggregate amount of $2.6 million and royalty payments on future net sales of such products. Until the Company is required to pay royalties under the LCA5/RDH12 Agreement, the Company must pay a *de minimis* annual license maintenance fee to Penn. The Company is also obligated to make payments on any sublicense income, with such percentage depending on the stage of product development. As of September 30, 2026, the Company determined that none of the future obligations under the LCA5/RDH12 Agreement were probable, and accordingly, no liabilities were recorded related to the agreement.

Iveric Asset Purchase Agreement – BEST1 and RHO Programs

On December 23, 2022, Opus entered into an asset purchase agreement (the "Iveric Agreement") with a subsidiary of Iveric Bio, Inc. ("Iveric") pursuant to which the Company acquired certain assets, including the BEST1 License (as defined below), relating to the BEST1 and RHO products. In return for these rights, the Company is obligated to make payments to Iveric upon the achievement of specified development, regulatory and commercial milestones, the maximum potential aggregate amount of such payments being $111.7 million. As of September 30, 2026, the Company determined that none of the future obligations under the Iveric Agreement were probable, and accordingly, no liabilities were recorded related to the Iveric Agreement.

Penn and University of Florida BEST1 License Agreement

On April 10, 2019, Iveric entered into an exclusive patent license agreement (as amended, the "BEST1 License") with Penn and the University of Florida Research Foundation ("UF"), which agreement was assigned to Opus under the terms of the Iveric Agreement. Under the BEST1 License, Opus received exclusive patent rights and non-exclusive know-how and data rights with regard to products to treat diseases associated with mutations in the BEST1 gene. In return for these rights, the Company is obligated to make payments to Penn upon the achievement of certain development, regulatory and commercial milestones, the maximum potential aggregate amount of such payments being $76.4 million. The Company is also obligated to make royalty payments on future net sales of licensed BEST1 products. Until the Company is required to pay royalties under the BEST1 License, the Company must pay a *de minimis* annual license maintenance fee to UF and Penn. The Company must also make payments on any sublicense income, with such percentage depending on the stage of product development. The Company has not recorded any sublicense income during any of the periods presented. In consideration for Penn and UF's consent to the assignment of the BEST1 License to the Company under the Iveric Agreement, the Company will also pay Penn a percentage of each milestone payment that the Company is required to pay to Iveric under the Iveric Agreement. As of September 30, 2026, the Company determined that none of the future obligations under the BEST1 License were probable, and accordingly, no liabilities were recorded related to the BEST1 License.

Penn and UF RHO License Agreement

On June 6, 2018, Iveric entered into an exclusive patent license agreement (the "RHO License") with Penn and UF pursuant to which the Company has exclusive patent rights and non-exclusive know-how and data rights with regard to products to treat rhodopsin-mediated diseases as a result of the Iveric Agreement as defined above. In return for these rights, the Company is obligated to make development, regulatory and commercial milestone payments, the maximum potential aggregate amount of such payments being $93.5 million and royalty payments on future sales of such products. As of September 30, 2026, the Company determined that none of the future obligations under the RHO License were probable, and accordingly, no liabilities were recorded related to the RHO License.

Penn License Assignment and Share Issuance

On July 20, 2026, the Company entered into agreements with Penn to assign and amend certain existing intellectual property license arrangements and extend certain contractual rights and obligations. In connection with these agreements, the Company entered into a stock subscription agreement pursuant to which it agreed to issue 200,000 shares of its common stock with a fair value of $0.6 million to Penn. The common stock was issued on July 23, 2026.

Notes to Condensed Consolidated Financial Statements

Massachusetts Eye and Ear Infirmary License Agreement

On November 9, 2021, Opus entered into a license agreement ("MEEI License") with the Massachusetts Eye and Ear Infirmary ("MEEI"), granting an exclusive worldwide license of MEEI patents for use in the NMNAT1 program for all products and processes including the treatment of retinal disease in humans, and a non-exclusive worldwide license to technological information. In return for these rights, the Company is obligated to make development and regulatory milestone payments, the maximum potential aggregate amount of such payments being $0.4 million and royalty payments on future sales of such products. As of September 30, 2026, the Company determined that none of the future obligations under the MEEI License were probable, and accordingly, no liabilities were recorded related to the MEEI License.

Rexahn CVR Agreement

On November 5, 2020, the Company completed a merger transaction with Rexahn Pharmaceuticals, Inc. ("Rexahn" and such merger transaction, the "Rexahn Merger"). In connection with the Rexahn Merger, the Company, Shareholder Representative Services LLC, as representative of the Rexahn stockholders prior to the Rexahn Merger, and Olde Monmouth Stock Transfer Co., Inc., as the rights agent, entered into the Contingent Value Rights Agreement (the "CVR Agreement").

Pursuant to the terms of the Rexahn Merger and the CVR Agreement, Rexahn stockholders of record as of immediately prior to the effective time of the Rexahn Merger received one contingent value right ("CVR") for each share of Rexahn common stock held.

The CVRs are not transferable, except in certain limited circumstances, will not be certificated or evidenced by any instrument, will not accrue interest and will not be registered with the SEC or listed for trading on any exchange. The CVR Agreement will continue in effect until the later of the end of the CVR Term (as defined in the CVR Agreement) and the payment of all amounts payable thereunder. As of September 30, 2026, no payments subject to the CVR Agreement had been received beyond those previously reported in the second and third quarters of calendar year 2021. In addition, no milestones had been accrued as of September 30, 2026, as there were no potential milestones yet considered probable beyond those previously reported.

Facility and Other Leases

The Company's headquarters and laboratory facilities are located in Durham, North Carolina and are leased on a month-to-month basis. All of the Company's leases qualify for the short-term lease exception under ASC 842, *Leases*.

The rent expense associated with all leases amounted to $0.1 million and $0.3 million during the three and nine months ended September 30, 2026, respectively, and $0.1 million and $0.2 million during the three and nine months ended September 30, 2025, respectively.

Other

In the ordinary course of business, from time to time, the Company may be subject to a broad range of claims and legal proceedings that relate to contractual allegations, patent infringement and other claims. In addition, the Company from time to time may be potentially committed to reimburse third parties for costs incurred associated with business development related transactions upon the achievement of certain milestones. The Company establishes accruals when applicable for matters and commitments for which it believes losses are probable and can be reasonably estimated.

5. Supplemental Balance Sheet Information

Prepaids and other current assets

Prepaids and other current assets consist of the following (in thousands):

Notes to Condensed Consolidated Financial Statements

	As of	
	September 30, 2026	December 31, 2025
Prepaid research and development services	$ 1,459	$ 845
Prepaid insurance and other professional services	496	814
Warrant exercise receivable	1,689	—
Other	605	129
Total	$ 4,249	$ 1,788

Accrued expenses

Accrued expenses consist of the following (in thousands):

	As of	
	September 30, 2026	December 31, 2025
R&D services and supplies	$ 2,790	$ 2,061
Compensation and benefits	2,556	1,992
Professional services	753	420
Other	6	15
Total	$ 6,105	$ 4,488

6. Related Party Transactions

Consulting Agreements with Dr. Pepose

On April 11, 2024, the Company entered into a consulting agreement (the "Pepose Consulting Agreement") with Dr. Jay S. Pepose, a former director of the Company. Pursuant to the Pepose Consulting Agreement, Dr. Pepose was paid a monthly consulting fee and received an award of 32,000 restricted stock units ("RSUs"), as well as stock options to purchase 48,000 shares of the Company's common stock. The RSUs vested in 12 equal monthly installments that began on May 11, 2024 and concluded on April 11, 2025. On November 21, 2024, the Pepose Consulting Agreement was amended to continue through April 11, 2026, and was further amended on April 10, 2026 to continue through May 11, 2026.

Under the Pepose Consulting Agreement, the Company incurred consulting expenses of zero and $0.2 million during the three and nine months ended September 30, 2026, respectively, and $0.1 million and $0.4 million during the three and nine months ended September 30, 2025, respectively.

March 2025 Subscription Agreements with Dr. George Magrath and Cam Gallagher

On March 21, 2025, the Company entered into a subscription agreement with each of Dr. George Magrath, the Company's Chief Executive Officer, and Cam Gallagher, the Chair of the Company's board of directors (the "Board"), in connection with a private offering of the Company's securities (see Note 9 – Financings).

Letter Agreement and Strategic Partnership—FFB

On August 25, 2022, the Company entered into a binding letter of agreement ("2022 Binding Letter Agreement") with Foundation Fighting Blindness ("FFB") and the Jaeb Center for Health Research (the "JCHR") to collaborate on natural history studies involving individuals with retinal dystrophies associated with mutations in multiple genes of interest. Under the terms of the 2022 Binding Letter Agreement, FFB and the JCHR had the sole responsibility and authority to design and conduct the study, with input from the Company. Subject to certain conditions, the 2022 Binding Letter Agreement required that the Company provide FFB with a total of $2.0 million of funding to support the study, such amount being payable in an initial installment of $0.4 million at the time of submission of the final study protocol to the Institutional Review Board of the JCHR and, subject to certain conditions, in four annual installments of $0.4 million on the anniversaries of such submission. On May 27, 2025, the Company entered into a binding letter of agreement ("2025 Letter

Notes to Condensed Consolidated Financial Statements

Agreement") with FFB and the JCHR, which superseded and canceled the 2022 Binding Letter Agreement. As of September 30, 2026, a total of $0.4 million was paid by the Company under the 2022 Binding Letter Agreement and no more payments were due under the 2022 Binding Letter Agreement.

Under the 2025 Letter Agreement, the Company will collaborate with FFB and the JCHR on portions of a study involving individuals with retinal dystrophies associated with mutations in the RDH12 or BEST1 genes (the "Study"). The term of this 2025 Letter Agreement ends on the date that is two months from the Study's completion. FFB and the JCHR, as its designee, shall have the sole responsibility and authority to design and conduct the Study, with input from the Company. Under the 2025 Letter Agreement, the Company is obligated to make two payments to FFB: (1) $0.3 million on or before June 30, 2025 and (2) $0.3 million on or before January 31, 2027 upon receipt of semi-annual reports from FFB outlining the progress being made in the Study, including visit completion status and publication plans, and the ability for the Company to provide ongoing comments and suggestions regarding possible changes to the Study. Such payments shall constitute the sole compensation paid to FFB in return for Company access to research materials and datasets.

The Company paid the initial $0.3 million due on or before June 30, 2025 under the 2025 Letter Agreement which was recorded as research and development expense. As of September 30, 2026, the Company is required to fund one additional installment in the aggregate of $0.3 million upon receipt of future semi-annual reports. A total of zero and $0.2 million of the remaining $0.3 million obligation was recognized in the condensed consolidated financial statements during the three and nine months ended September 30, 2026, respectively.

RDF Agreement

On June 13, 2025, the Company entered into a funding agreement (the "RDF Agreement") with the Foundation Fighting Blindness Retinal Degeneration Fund ("RDF"), whose sole member is FFB, relating to the Company's program to develop gene therapies to treat patients impacted by retinitis pigmentosa caused by pathogenic variants in the Mer proto-oncogene tyrosine kinase (MERTK) gene (the "MERTK Program"). The RDF Agreement provides for nondilutive funding by RDF of up to $2.0 million to support the development of the MERTK Program, $1.0 million of which was disbursed to the Company in June 2025 and up to $1.0 million of which may be disbursed to the Company upon achievement of a specified development milestone subject to RDF's receipt of eligible funds.

Under the RDF Agreement, the Company is subject to certain diligence obligations to develop and commercialize a product under the MERTK Program. If the Company is unable to achieve certain milestones by certain dates, or otherwise fails to meet its diligence obligations, the Company will be obligated to collaborate with RDF to out-license or otherwise make applicable rights available to a third party.

In addition, the Company will pay a milestone payment equal to the total amounts funded by RDF under the RDF Agreement upon the achievement of a regulatory milestone. The Company will also make tiered royalty payments to RDF in low-to-mid single-digit percentages until RDF has received aggregate royalty payments equal to 300% of the amounts funded by RDF under the RDF Agreement. In the event of a change of control of the Company or a sale or exclusive license of the MERTK Program, RDF will have the option to require the Company to buy out RDF's interest under the RDF Agreement for an amount equal to 100% of the funds disbursed to the Company under the RDF Agreement. The RDF Agreement may be terminated by either party for cause, including material breach or bankruptcy, subject to a cure period.

The RDF Agreement was accounted for as debt under ASC 470, *Debt*. ASC 470 requires interest expense to be recorded under an effective interest rate method. Interest expense was $0.1 million and $0.2 million during the three and nine months ended September 30, 2026, respectively, based on an effective rate of interest of 24.2% and was recorded in other income, net in the accompanying condensed consolidated statements of comprehensive loss. There was $0.1 million of interest expense related to the RDF Agreement during the three and nine months ended September 30, 2025. The accreted liability in connection with the RDF Agreement was $1.4 million as of September 30, 2026 and was recorded under the funding agreement, related party line item in the accompanying condensed consolidated balance sheets.

7. Debt

Note Purchase Agreement

On April 2, 2026, the Company and certain of its subsidiaries as guarantors entered into the Note Purchase Agreement with OPCM SA LLC, as Purchaser Agent, and certain Purchasers. Capitalized terms used in this section but not defined herein have the meanings given to such terms in the Note Purchase Agreement.

Notes to Condensed Consolidated Financial Statements

The Note Purchase Agreement provides for, among other things, the issuance of up to $155.0 million of Notes, of which the Purchasers have committed to purchase $105.0 million and the remaining $50.0 million is uncommitted. The issuance and purchase of each tranche of Notes is subject to the satisfaction of customary funding conditions and, in certain cases, achievement of certain pre-determined milestones. An initial tranche of $35.0 million was funded at the initial closing following the effective date of the Note Purchase Agreement, which occurred on April 21, 2026 (the "First Purchase Date") resulting in net proceeds of approximately $34.5 million. A second tranche of $35.0 million will be available at the Company's option until April 2, 2027. A third tranche (the "Third Tranche") of $25.0 million will be funded upon the FDA Application Acceptance Date for OPGx-LCA5 on or prior to March 31, 2028. A fourth tranche of $10.0 million will be available, (a) at the Company's option, if the FDA Approval Date for OPGx-LCA5 has occurred or, (b) at the Purchaser Agent's option, if the Third Tranche has not been funded (whether or not the FDA Application Acceptance Date or FDA Approval Date has occurred), in each case, on or prior to March 31, 2028. An additional $50.0 million of uncommitted financing (separated into two equal tranches of $25.0 million) will be available upon the request of the Company but subject to the approval of each Purchaser in its sole discretion until December 31, 2027.

The Notes mature on April 21, 2033 (the "Maturity Date") and bear interest at a rate per annum equal to Term SOFR for the three-month interest period (subject to a 3.68% floor) plus the Applicable Margin, payable quarterly. Interest payable on the first eight interest payment dates following the effective date of the Note Purchase Agreement shall be payable as follows: 50% of the interest owed for each applicable period shall be paid in kind and the remaining 50% of interest owed for such applicable period shall be paid in cash. Thereafter, 100% of the interest owed for each applicable period shall be paid in cash. The Company shall make a payment of principal on each outstanding Note on the sixth anniversary of the First Purchase Date in an amount equal to half of the aggregate principal amount of such Notes outstanding on such date, with the remaining balance payable on the Maturity Date.

Concurrently with the Note Purchase Agreement, on April 2, 2026, the Company entered into a stock purchase and conversion agreement (the "Purchase and Conversion Agreement") with the Purchasers providing for the issuance of an aggregate of 1,116,070 shares (the "Purchase Shares") of its common stock at a price per share equal to $4.48 (the "Per Share Purchase Price"), for an aggregate purchase price of $5.0 million (see Note 9 – Financings). In connection with the Note Purchase Agreement and Purchase and Conversion Agreement, the Company received aggregate gross proceeds of $40.0 million from the issuance of Notes and Purchase Shares. The Company elected to account for the Notes under the fair value option in accordance with ASC 825 (see Note 3 – Fair Value Measurements). At issuance, the fair value of the Notes was determined to be $35.0 million, and such amount was allocated to the Notes. The residual $5.0 million of proceeds was allocated to the Purchase Shares issued in connection with the Purchase and Conversion Agreement and recorded within stockholders' equity (deficit).

On April 13, 2026, the Company, the Purchaser Agent and the Purchasers entered into a Waiver and Omnibus Amendment to the Note Purchase Agreement and Purchase and Conversion Agreement (the "Waiver and Omnibus Amendment"). The Waiver and Omnibus Amendment revised the Per Share Purchase Price to the lower of $4.48 per share or the 30-day volume-weighted average price of the Company's common stock ending on the issuance date of the Purchase Shares, revised the Notes conversion price to the lower of $6.72 or 150% of the Per Share Purchase Price and modified the timing and closing conditions for issuance of the Purchase Shares. The Purchase Shares were issued on May 26, 2026, for aggregate gross proceeds of $5.0 million.

Pursuant to the Note Purchase Agreement, as amended by the Waiver and Omnibus Amendment, following the Issuance Authorization and prior to the date that is 18 months after the date that is 30 days after the effective date of the Initial Registration Statement, any Purchaser may, at any time and from time to time, subject to the notice and other terms and conditions set forth in the Note Purchase Agreement, elect to convert up to 10% in the aggregate of the principal amount of such Purchaser's Notes then outstanding (the "Conversion Amount") into shares (the "Conversion Shares") of the Company's common stock. The number of Conversion Shares issuable upon such conversion shall be equal to the Conversion Amount divided by the lower of $6.72 and 150% of the Per Share Purchase Price, in each case as amended by the Waiver and Omnibus Amendment. Upon any such conversion, an aggregate principal amount of Notes equal to the Conversion Amount shall be deemed paid and satisfied in full and shall cease to accrue interest. Any interest accrued and unpaid through and including the date of such conversion shall remain outstanding and shall be payable in cash on the next interest payment date (or, if the Company and the Purchasers so elect in writing, shall be convertible into Conversion Shares).

The Company may voluntarily prepay the Notes in full at any time subject to a prepayment premium. Moreover, a prepayment premium may also be payable by the Company upon the occurrence of, (i) at any time prior to the first anniversary of the First Purchase Date, an acceleration of the obligations under the Note Purchase Agreement following an event of default (other than a change of control) or, (ii) at any time prior to the date that is 18 months after the First Purchase Date, a change of control of the Company that is not otherwise consented to by the Purchasers holding a majority

of the outstanding Notes. The Company is required to make mandatory prepayments of the Notes with net cash proceeds from insurance proceeds or condemnation awards, in each case, subject to certain exceptions and reinvestment rights.

The obligations of the Company under the Note Purchase Agreement are guaranteed by certain of its existing subsidiaries and are required to be guaranteed by subsequently acquired or organized subsidiaries, subject to certain exceptions (collectively, the "Guarantors"). The obligations of the Company under the Note Purchase Agreement and of the Guarantors under their respective guaranty are secured, subject to customary permitted liens and other agreed upon exceptions, by (a) a pledge of all of the equity interests of the Company's and the Guarantors' direct subsidiaries, and (b) a perfected security interest in substantially all of the Company's and the Guarantors' tangible and intangible assets. The Note Purchase Agreement contains customary representations and warranties and, among other customary terms, contains events of default which are customary for financings of this type, in certain circumstances subject to specified cure periods. Following an event of default and any cure period, if applicable, the Purchaser Agent will have the right upon notice to terminate any undrawn commitments and may accelerate all amounts outstanding under the Note Purchase Agreement, in addition to other remedies available to it as a secured creditor of the Company.

The Notes contain various embedded features, including conversion, prepayment, acceleration and tranche-related features, and the Company's election of the fair value option allows the Company to account for the Notes at fair value without separately evaluating potential embedded derivatives for bifurcation. Under the fair value option, the financial liability is initially measured at its fair value on the issuance date and subsequently remeasured at estimated fair value on a recurring basis at each reporting date. Changes in the fair value of the Notes, which include accrued interest, if any, are recorded as a component of fair value change in instruments measured at fair value in the condensed consolidated statements of comprehensive loss. The Company has not elected to present interest expense separately from changes in fair value and therefore does not separately present interest expense associated with the Notes. Changes in fair value caused by instrument-specific credit risk are presented separately in other comprehensive income (loss) in the condensed consolidated statements of comprehensive loss and accumulated in accumulated other comprehensive income in the condensed consolidated balance sheets. The portion of total changes in fair value of the Notes attributable to changes in instrument-specific credit risk is determined through specific measurement of periodic changes in the discount rate assumption exclusive of base market changes. Refer to Note 3 – *Fair Value Measurements* for a reconciliation of the change in fair value of the Notes for the period from April 21, 2026 through September 30, 2026.

Under the fair value option, debt issuance costs are expensed as incurred. The Company incurred approximately $1.3 million of transaction costs in connection with the Note Purchase Agreement and Purchase and Conversion Agreement, of which $1.1 million was allocated to the Notes and expensed as incurred, and $0.2 million was allocated to the common stock and recorded as a reduction of additional paid-in capital.

As of September 30, 2026, future principal payments under the Note Purchase Agreement are due as follows (in thousands):

2026	—
2027	—
2028	—
2029	—
2030	—
2031	—
2032	17,826
2033	17,825
Total principal payments	**$ 35,651**

8. Preferred Stock

Series B Preferred Stock

On February 18, 2026, the Company filed a Certificate of Designation of Preferences, Rights and Limitations of the Series B Non-Voting Convertible Preferred Stock with the Secretary of State of the State of Delaware (the "Series B Certificate of Designation") in connection with the February 2026 Private Placement (see Note 9 – Financings). The Series B Certificate of Designation provided for the authorization of 7,374,632 shares of Series B preferred stock, of which 7,374,632 shares of Series B preferred stock were issued upon the closing of the February 2026 Private Placement. On April 20, 2026, the Company held its 2026 Annual Meeting of Stockholders. During the 2026 Annual Meeting, the Company's stockholders

voted to approve an amendment to the Company's restated certificate of incorporation to increase the number of authorized shares of common stock from 125,000,000 to 250,000,000, which permitted the conversion of each share of Series B preferred stock, on a one-for-one basis, into common stock. Subsequently, on April 23, 2026, all shares of Series B preferred stock were converted into 7,374,632 shares of common stock.

Series A Preferred Stock

On October 22, 2024, the Company filed a Certificate of Designation of Preferences, Rights and Limitations of the Series A Non-Voting Convertible Preferred Stock with the Secretary of State of the State of Delaware (the "Series A Certificate of Designation") in connection with its acquisition of legacy Opus Genetics, Inc. in October 2024 (the "Opus Acquisition"). The Series A Certificate of Designation provided for the authorization of 14,146 shares of Series A preferred stock, of which 14,145.374 shares of Series A preferred stock were issued upon close of the Opus Acquisition. On April 30, 2025, the Company held its 2025 Annual Meeting of Stockholders. During the 2025 Annual Meeting, the Company's stockholders voted to approve the conversion of each share of Series A preferred stock into 1,000 shares of common stock. Subsequently, on May 5, 2025, all shares of Series A preferred stock were converted into 14,145,374 shares of common stock.

9. Financings

Purchase and Conversion Agreement

Concurrently with the Note Purchase Agreement, on April 2, 2026, the Company entered into the Purchase and Conversion Agreement with the Purchasers providing for the issuance of an aggregate of 1,116,070 shares of the Company's common stock at a price per share equal to $4.48, for an aggregate purchase price of $5.0 million.

The Purchase and Conversion Agreement also provided the Purchasers with an option to acquire additional Purchase Shares (the "Option Shares") at a price per share equal to $0.0001 (subject to adjustment as set forth in the Purchase and Conversion Agreement, the "Per Share Exercise Price") in the event that prior to October 2, 2026, the Company issued shares of its common stock (or instruments exercisable for or convertible into shares of its common stock) at an effective sale price per share below the Per Share Purchase Price, subject to certain exceptions (a "Dilutive Equity Round"). The number of Option Shares issuable upon exercise of such option will be such that, accounting for the sale and issuance of the Option Shares, the weighted average of the Per Share Purchase Price and Per Share Exercise Price for all Purchase Shares (including Option Shares) issued pursuant to the Purchase and Conversion Agreement, taken together, shall equal the lowest effective sale price per share paid in cash by third party investors to the Company for its common stock issued by the Company in such Dilutive Equity Round.

The Purchase and Conversion Agreement was amended on April 13, 2026 to revise the Per Share Purchase Price to the lower of $4.48 per share or the 30-day volume-weighted average price of the Company's common stock ending on the issuance date of the Purchase Shares and to modify the timing and closing conditions for issuance of the Purchase Shares. The option to acquire Option Shares expired unexercised on October 2, 2026.

The issuance of the Purchase Shares pursuant to the Purchase and Conversion Agreement closed on May 26, 2026, resulting in the issuance of 1,116,070 shares of common stock for aggregate gross proceeds of $5.0 million. The Company evaluated the common stock issued under the Purchase and Conversion Agreement, together with the related forward share commitment and down-round participation right, and concluded that the common stock issuance should be classified in permanent equity. The related forward share commitment and down-round participation right were evaluated as freestanding financial instruments and derivative liabilities; however, the Company determined that the fair value of each was de minimis, and therefore no proceeds were allocated to those instruments. In connection with the Note Purchase Agreement and Purchase and Conversion Agreement, the Company received aggregate gross proceeds of $40.0 million from the issuance of Notes and Purchase Shares. A total of $35.0 million was allocated to the Notes based on the fair value on the First Purchase Date, with the residual $5.0 million of proceeds received under the Purchase and Conversion Agreement being allocated to the Purchase Shares and recorded within stockholders' equity (deficit), net of allocated issuance costs of $0.2 million, with such issuance costs recorded as a reduction of additional paid-in capital.

February 2026 Private Placement

On February 13, 2026, the Company entered into a securities purchase agreement with certain investors for a private placement of 7,374,632 shares of Series B preferred stock, par value $0.0001 per share, at a purchase price of $3.39 per share. The private placement closed on February 18, 2026, resulting in gross proceeds of approximately $25.0 million. As a result of the approval by stockholders of the proposal to increase the Company's authorized shares of common stock at the

2026 Annual Meeting on April 20, 2026 and filing the associated amendment, all of the shares of Series B preferred stock were automatically converted into the same number of shares of common stock (see Note 8 – Preferred Stock).

March 2025 Financings

On March 21, 2025, the Company entered into an underwriting agreement with Craig-Hallum Capital Group, LLC, as the sole underwriter. Pursuant to the underwriting agreement, the Company agreed to issue and sell, in an underwritten public offering (the "March 2025 Offering"), 12,219,736 shares of common stock and warrants to purchase up to 21,052,631 shares of common stock (the "March 2025 Warrants"). Each share of common stock was sold together with one March 2025 Warrant to purchase one share of common stock, at a price to the public of $0.95 per share and related March 2025 Warrant. The Company also issued 8,832,895 pre-funded warrants ("Pre-Funded Warrants") at a price to the public of $0.9499 per Pre-Funded Warrant.

On March 21, 2025, the Company entered into a subscription agreement (the "Subscription Agreement") with each of Dr. George Magrath, the Company's Chief Executive Officer, and Cam Gallagher, the Chair of the Board. Pursuant to the Subscription Agreement, the Company agreed to issue and sell, in a private offering (the "March 2025 Private Placement"), a total of 392,157 shares of common stock to Dr. Magrath and 784,314 shares of common stock to Mr. Gallagher, as well as 392,157 warrants to purchase shares of common stock to Dr. Magrath and 784,314 warrants to purchase shares of common stock to Mr. Gallagher ("March 2025 Private Placement Warrants") at an initial exercise price of $1.15 per share. See Note 6 – Related Party Transactions.

The combined gross proceeds from the March 2025 Offering and the March 2025 Private Placement, which both closed on March 24, 2025 (the "Closing Date"), were approximately $21.5 million, before deducting underwriting discounts and commissions and offering expenses payable by the Company in the amount of $1.8 million.

March 2025 Warrants

The March 2025 Warrants have an initial exercise price equal to $0.95 per share of common stock and are exercisable for five years from the date of issuance. The exercise prices and numbers of shares of common stock issuable upon exercise are subject to appropriate adjustment in the event of stock dividends, stock splits, reorganizations or similar events affecting the common stock and also upon any distributions of assets, including cash, stock or other property to our stockholders. A holder may not exercise the March 2025 Warrant if, after giving effect to such exercise, the holder (together with its affiliates) would beneficially own (as determined in accordance with the terms of the March 2025 Warrants) more than 4.99% (or, at the election of the holder, 9.99%) of the outstanding common stock immediately after giving effect to the exercise. Lastly, certain volatility provisions in the event of a fundamental transaction precluded the March 2025 Warrants from being considered indexed to the Company's own stock, and as such, were classified on the condensed consolidated balance sheets as warrant liabilities.

All outstanding March 2025 Warrants have been called by the Company. The Company announced OPGx-BEST1 Cohort 1 data on September 9, 2026, and on October 22, 2026, following satisfaction of the applicable conditions, the Company delivered call notices to the holders of all outstanding March 2025 Warrants (see Note 16 – Subsequent Events).

The fair value of the March 2025 Warrants as of September 30, 2026 and December 31, 2025 was $11.2 million and $24.6 million, respectively, and was recorded in the warrant liabilities line item in the accompanying condensed consolidated balance sheets. The fair value change during the nine months ended September 30, 2026 and 2025 was an expense of $68.5 million and $5.5 million, respectively. The fair value change during the three months ended September 30, 2026 and 2025 was an expense of $26.7 million and $9.0 million, respectively. The fair value of these instruments was based on a Monte Carlo simulation incorporating a volatility rate of 75.0% and 72.5% as of September 30, 2026 and December 31, 2025, respectively, a risk-free rate of 4.9% and 3.6% as of September 30, 2026 and December 31, 2025, respectively, the market price of the Company's common stock at $4.84 and $2.01 per share as of September 30, 2026 and December 31, 2025, respectively, and other factors over a simulated term of 3.5 years and 4.2 years at September 30, 2026 and December 31, 2025, respectively. As disclosed in Note 3 – Fair Value Measurements, the March 2025 Warrants are measured at fair value using significant unobservable inputs. Following the announcement of OPGx-BEST1 BIRD-1 Cohort 1 data on September 9, 2026, the uncertainty associated with the timing of the applicable data-release milestone was resolved. Accordingly, following satisfaction of the call conditions, changes in the fair value of the remaining March 2025 Warrants are expected to be driven primarily by changes in the market price of the Company's common stock through the earlier of exercise or cancellation of the warrants.

March 2025 Private Placement Warrants

Notes to Condensed Consolidated Financial Statements

The March 2025 Private Placement Warrants had an initial exercise price equal to $1.15 per share of common stock and were exercisable for five years from the date of issuance. The March 2025 Private Placement Warrants were callable by the Company in certain circumstances. As of September 30, 2026, all of the March 2025 Private Placement Warrants have been exercised and none remain outstanding.

All 1,176,471 March 2025 Private Placement Warrants were exercised during the three months ended September 30, 2026. Accordingly, no March 2025 Private Placement Warrants were outstanding, and no related warrant liability was recorded, as of September 30, 2026. Immediately before each exercise, the related warrant liability was remeasured to fair value, with the resulting change in fair value recognized in earnings, and the warrant liability reclassified to additional paid-in capital upon exercise. The fair value change during the nine months ended September 30, 2026 and 2025 was an expense of $3.0 million and $0.3 million, respectively. The fair value change during the three months ended September 30, 2026 and 2025 was an expense of $0.8 million and $0.5 million, respectively. As of December 31, 2025, the fair value of the March 2025 Private Placement Warrants was $1.4 million and was estimated using a Monte Carlo simulation incorporating an expected volatility rate of 72.5%, a risk-free rate of 3.6%, the market price of the Company's common stock of $2.01 per share, and a simulated term of 4.2 years, together with the estimated probability and timing of achieving the applicable data-release milestone and satisfying the related call conditions.

Pre-Funded Warrants

The Pre-Funded Warrants have an exercise price of $0.0001 per share of common stock and are immediately exercisable at any time until exercised in full. The exercise prices and numbers of shares of common stock issuable upon exercise are subject to appropriate adjustment in the event of stock dividends, stock splits, reorganizations or similar events affecting the common stock. A holder may not exercise the Pre-Funded Warrant if, after giving effect to such exercise, the holder (together with its affiliates) would beneficially own (as determined in accordance with the terms of the Pre-Funded Warrants) more than 4.99% (or, at the election of the holder, 9.99%) of the outstanding common stock immediately after giving effect to the exercise. In the event of a fundamental transaction, as defined in the form of Pre-Funded Warrant, the holders of the Pre-Funded Warrants will be entitled to receive upon exercise of the Pre-Funded Warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such fundamental transaction.

The Pre-Funded Warrants were recorded in the accompanying condensed consolidated balance sheets as additional paid-in capital. During the three months ended September 30, 2026, the Company issued 4,157,895 additional pre-funded warrants, with an exercise price of $0.0001 per share and terms substantially consistent with the Pre-Funded Warrants, to holders of March 2025 Warrants that elected to receive pre-funded warrants in lieu of shares of common stock upon exercise. These pre-funded warrants were classified in equity, with the related warrant liability and exercise proceeds recorded in additional paid-in capital.

At-The-Market Programs

On January 13, 2025, the Company filed a prospectus supplement with the SEC establishing an at-the-market equity offering program (the “Prior ATM Program”) for the offer and sale, from time to time, of shares of its common stock having an aggregate offering price of up to $40.0 million. On the same date, the Company entered into a sales agreement with Leerink Partners LLC (“Leerink”) relating to the Prior ATM Program (the “Prior Sales Agreement”). On August 6, 2026, the Company filed a new shelf registration statement on Form S-3 (File No. 333-298100), which included a base prospectus and a sales agreement prospectus relating to a new at-the-market equity offering program (the “New ATM Program”), and entered into a new sales agreement with Leerink and Cantor Fitzgerald & Co. (“Cantor”) (the “New Sales Agreement”). The Form S-3 was declared effective by the SEC on August 14, 2026. Under the New Sales Agreement and related sales agreement prospectus, the Company may offer and sell, from time to time through or to Leerink or Cantor, acting as sales agent or principal, shares of its common stock having an aggregate offering price of up to $125.0 million. The New Sales Agreement replaced the Prior Sales Agreement, and no additional shares may be sold under the Prior ATM Program.

During the three and nine months ended September 30, 2026, the Company sold zero and 1,000,000 shares of common stock, respectively, under the Prior ATM Program for gross proceeds of zero and $2.3 million, respectively, before deducting issuance expenses. Total issuance expenses, including sales agent fees and legal and accounting expenses, were zero and $0.1 million, respectively, for the three and nine month period ended September 30, 2026. The Company did not sell any shares under the New ATM Program during the three and nine months ended September 30, 2026.

During the three and nine months ended September 30, 2025, the Company sold 1,472,703 and 1,825,656 shares of common stock, respectively, under the Prior ATM Program for gross proceeds of $1.7 million and $2.1 million,

respectively, before deducting issuance expenses. Total issuance expenses, including sales agent fees and legal and accounting expenses, were $0.1 million and $0.3 million for the three and nine month periods ended September 30, 2025, respectively.

Registered Direct Offering

On June 4, 2021, the Company entered into a placement agency agreement for a registered direct offering ("RDO") with A.G.P./Alliance Global Partners ("AGP"). Pursuant to the terms of the placement agency agreement, AGP on June 8, 2021 sold an aggregate of 3,076,923 shares of the Company's common stock and warrants to purchase 1,538,461 shares of the Company's common stock (the "RDO Warrants") at an offering price of $4.875 per one share and per one-half of each RDO Warrant. The RDO was made pursuant to the Company's 2021 shelf registration.

The RDO Warrants had an exercise price of $6.09 per share, and were exercisable from the initial issuance date of June 8, 2021 for five years through June 8, 2026. As of September 30, 2026, no RDO Warrants were outstanding and none were exercised prior to their expiration on June 8, 2026.

Subject to limited exceptions, a holder of an RDO Warrant will not have the right to exercise any portion of its RDO Warrants if the holder, together with its affiliates, would beneficially own in excess of 4.99% (or, at the election of a holder prior to the date of issuance, 9.99%) of the number of shares of the Company's common stock outstanding immediately after giving effect to such exercise; provided that upon prior notice to the Company, the holder may increase or decrease the beneficial ownership limitation, provided further that in no event shall the beneficial ownership limitation exceed 9.99%.

Warrant Activity and Summary

	Warrants	Exercise Price Per Warrant	Weighted Average Exercise Price	Weighted Average Term (Years)
Outstanding and exercisable at December 31, 2025	22,904,879	$0.95-$6.09	$ 1.31	3.98
Issued	—	—	—	—
Exercised	(18,507,313)	$0.95-$1.15	$ 0.96	—
Expired	(1,538,461)	$ 6.09	$ 6.09	—
Outstanding and exercisable at September 30, 2026	2,859,105	$ 0.95	$ 0.95	3.47

During the three months ended September 30, 2026, certain warrant holders of March 2025 Private Placement Warrants elected to exercise warrants on a cashless basis in accordance with the terms of the warrant agreements with respect to such March 2025 Private Placement Warrants. As a result, 75,541 of the warrants exercised were surrendered in satisfaction of the aggregate exercise price, and no shares of common stock were issued in respect of those warrants. In addition, during the three months ended September 30, 2026, certain holders of March 2025 Warrants elected to receive 4,157,895 pre-funded warrants, in lieu of shares of common stock, upon the cash exercise of 4,157,895 March 2025 Warrants. Accordingly, of the 18,507,313 warrants exercised during the nine months ended September 30, 2026, 14,273,877 were settled in shares of common stock (as reflected in the condensed consolidated statements of changes in convertible preferred stock and stockholders' equity (deficit)), 4,157,895 were settled in pre-funded warrants, and 75,541 were surrendered in connection with cashless exercises.

The following table summarizes information about warrants outstanding at September 30, 2026:

Exercise Price	Number Outstanding	Weighted Average Remaining Contractual Term (Years)	Number Exercisable at September 30, 2026
$ 0.95	2,859,105	3.47	2,859,105 *
Total	2,859,105		2,859,105

*Liability classified warrants in connection with March 2025 Financings

Notes to Condensed Consolidated Financial Statements

The above tables exclude 20,167,823 pre-funded warrants with a nominal exercise price of $0.0001 per share, consisting of 8,832,895 pre-funded warrants issued in connection with the March 2025 Offering, 7,177,033 pre-funded warrants issued in connection with the November 2025 Registered Direct Offering, and 4,157,895 pre-funded warrants issued during the third quarter of 2026 upon the exercise of March 2025 Warrants with an exercise price of $0.95. All outstanding pre-funded warrants were deemed outstanding common stock for net loss per share purposes (see Note 13 – Net Loss per Share).

10. Stock-based Compensation

Stock-based compensation expense was included in general and administrative and research and development costs as follows in the accompanying condensed consolidated statements of comprehensive loss for the three and nine month periods indicated below (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	$ 577	$ 249	$ 1,374	$ 815
General and administrative	1,013	567	3,087	1,810
Total stock-based compensation	$ 1,590	$ 816	$ 4,461	$ 2,625

Stock Options

During the three and nine months ended September 30, 2026, 310,000 and 1,425,000 stock options were granted, respectively, to directors, officers, and employees. The weighted average grant date fair value of the stock options granted during the three and nine months ended September 30, 2026 was $2.57 and $2.87, respectively.

During the three and nine months ended September 30, 2025, 733,448 and 2,793,277 stock options were granted, respectively, to directors, officers and employees. The weighted average grant date fair value of the stock options granted during the three and nine months ended September 30, 2025 was $0.79 and $0.68, respectively.

Restricted Stock Units

During the three and nine months ended September 30, 2026, the Company granted an aggregate of zero and 3,172,708 RSUs, respectively, to directors, officers, and employees. The weighted average grant date fair value of the RSUs granted during the three and nine months ended September 30, 2026 was zero and $2.65, respectively.

During the three and nine months ended September 30, 2025, the Company granted an aggregate of 200,000 and 1,655,361 RSUs, respectively, to directors, officers, and employees. The weighted average grant date fair value of the RSUs granted during the three and nine months ended September 30, 2025 was $1.33 and $0.99, respectively.

Common Stock Issued for Services

The Company granted zero and 118,465 common shares for services during the three and nine months ended September 30, 2026, respectively, and zero and 84,243 common shares during the three and nine months ended September 30, 2025, respectively. The awards were granted to members of the Board who elected to receive their Board compensation in the form of stock for services. The weighted average fair value of the shares granted was zero and $2.01 per share during the three and nine months ended September 30, 2026, respectively, and zero and $1.26 per share during the three and nine months ended September 30, 2025, respectively. The shares were fully vested upon issuance.

11. Apexian Sublicense Agreement

On January 21, 2020, the Company entered into a sublicense agreement (as amended on June 4, 2020, the “Apexian Sublicense Agreement”) with Apexian, pursuant to which it obtained exclusive worldwide patent and other intellectual property rights that constitute a Ref-1 Inhibitor program relating to therapeutic applications to treat disorders related to ophthalmic and diabetes mellitus conditions. The lead compound in the Ref-1 Inhibitor program was APX3330, which the Company had been developing as an oral tablet therapeutic to treat diabetic retinopathy. In connection with the Apexian

Sublicense Agreement, the Company issued a total of 891,422 shares of its common stock to Apexian and to certain affiliates of Apexian in calendar year 2020.

The Company also agreed to make one-time milestone payments under the Apexian Sublicense Agreement for each of the first ophthalmic indication and the first diabetes mellitus indication for the development and regulatory milestones, and once for each of several sales milestones. These milestone payments include (i) payments for specified developmental and regulatory milestones (including completion of the first Phase 2 trial and the first Phase 3 pivotal trial in the United States, and filing and achieving regulatory approval from the FDA for the first New Drug Application for a compound) totaling up to $11 million in the aggregate and (ii) payments for specified sales milestones of up to $20 million in the aggregate, which net sales milestone payments are payable once, upon the first achievement of such milestone. Lastly, the Company also agreed to make a royalty payment equal to a single-digit percentage of its net sales of products associated with the covered patents under the Apexian Sublicense Agreement. The Apexian Sublicense Agreement remained in effect until its termination on July 10, 2026, as described below.

On March 16, 2026, the Company entered into a termination and IP assignment agreement (the "Termination and IP Assignment Agreement") with Apexian that provided for the termination of the Apexian Sublicense Agreement and the sale and assignment of certain patents, know-how, materials and regulatory assets related to the Company's legacy APX-2009, APX-2014 and APX-3330 programs. The effectiveness of the agreement and the closing of the contemplated transactions were conditioned upon, among other things, the Company's receipt of a non-refundable upfront payment.

On July 10, 2026, the Company received the non-refundable upfront payment, and the transaction closed. Upon closing, the related patents, know-how, materials and regulatory assets associated with the APX-2009, APX-2014 and APX-3330 programs were transferred to Apexian in accordance with the agreement.

The Company recognized the upfront payment in other income, net, in its condensed consolidated statements of comprehensive loss for the three and nine months ended September 30, 2026.

Through the termination of the Apexian Sublicense Agreement on July 10, 2026, none of the milestone or royalty payments under the agreement had been triggered. Following the termination, the Company has no further milestone or royalty payment obligations under the agreement.

12. License and Collaboration Revenue and Other Funding Agreements

Viatris License Agreement

On November 6, 2022, the Company entered into the Viatris License Agreement, pursuant to which it granted Viatris an exclusive, perpetual, sub-licensable license to develop, manufacture, import, export and commercialize (i) PS, for treating (a) reversal of mydriasis, (b) night vision disturbances or dim light vision, and (c) presbyopia, and (ii) PS and low dose pilocarpine for treating presbyopia (together, the "PS Products") worldwide except for certain countries and jurisdictions in Asia (the "Viatris Territory"). The Company retains the exclusive right to develop, manufacture, have manufactured, import, export and commercialize the PS Products outside of the Viatris Territory.

Under the terms of the Viatris License Agreement, the Company, in partnership with Viatris, will develop the PS Products in the United States. Viatris will reimburse the Company for agreed-to budgeted costs related to the development of the PS Products through FDA approval and then share costs above the agreed upon threshold amount. Viatris will be responsible for developing the PS Products in countries and jurisdictions in the Viatris Territory outside of the United States. In addition, in August 2025, Opus and Viatris entered into a side letter to the Viatris License Agreement providing for the sharing of expenses arising in connection with a patent dispute.

Pursuant to the Viatris License Agreement, the Company received a one-time non-refundable cash payment of $35 million in November 2022 for the exclusive, perpetual, sub-licensable license to develop, manufacture, import, export and commercialize the PS Products in the Viatris Territory. In addition, with respect to the PS Products, the Company will be eligible to receive potential additional payments of up to $130 million upon achieving certain specified regulatory or net sales milestones, with the first milestone payment of $10 million already made following approval by the FDA of PS for reversal of mydriasis, which occurred during the third quarter of 2023. On October 16, 2026, the FDA's approval of PS for the treatment of presbyopia in adults triggered a regulatory milestone payment due to the Company (see Note 16 – Subsequent Events). The Company will also receive tiered royalties, starting at low double-digit royalties up to low 20% royalties, based on the aggregate annual net sales of all PS Products in the United States, and will receive low double-digit royalties based on all annual net sales in the Viatris Territory outside of the United States. The royalty payments will

continue on a country-by-country basis from the date of the first commercial sale of the first PS Product in a country of the Viatris Territory until December 31, 2040.

The Viatris License Agreement was accounted for under the provisions of ASC 606. In accordance with the provisions under ASC 606, the Company identified two distinct performance obligations at the effective date: (1) the license to its intellectual property and (2) research and development services.

The Company determined that the licenses transferred represented functional intellectual property. As such, the revenue related to the licenses was recognized at the point in time in which the license/know-how was delivered to Viatris which occurred during the fourth quarter of 2022. The Company determined that revenue related to the initial research and development services that were constrained to the 120-day non-cancellation period were to be recognized over time as the services were rendered based on an estimated percentage of completion input model. The initial research and development services were completed in the first quarter of 2023. Revenue related to the on-going research and development services are based on activities completed during the period.

Recognition of Revenue

Revenue recognized under the Viatris License Agreement during the three and nine months ended September 30, 2026 was $1.1 million and $4.0 million, respectively. Revenue recognized under the Viatris License Agreement during the three and nine months ended September 30, 2025 was $3.1 million and $10.3 million, respectively, related to the output of ongoing research and development services and to a much lesser extent royalty payments. The Company records a provision for credit losses, when appropriate, based on historical experience, current conditions and reasonable and supportable forecasts. The Company has not incurred any bad debt expense to date and no allowance for credit losses has been recorded during the periods presented.

Regulatory Milestones under the Viatris License Agreement

The Company has evaluated the regulatory milestones that may be received in connection with the Viatris License Agreement. There is uncertainty that the events to obtain the remaining regulatory milestones (aside from the approval by the FDA of RYZUMVI) will be achieved given the nature of clinical development and the stage of the development of the PS Products. These remaining regulatory milestones will be constrained until it is probable that a significant revenue reversal will not occur.

Sales Milestone and Royalty Payments

Sales milestones and royalties relate predominantly to a license of intellectual property granted to Viatris and are determined by sales or usage-based thresholds. The sales milestones and royalties are accounted for under the royalty recognition constraint and are accounted for as constrained variable consideration. The Company applies the royalty recognition constraint for each commercial milestone and only recognizes revenues for each once a sale of a licensed product (achievement of each) occurs.

Each of the remaining regulatory and sales milestone performance obligations (aside from the $10 million milestone payment related to the FDA's approval of PS in the third quarter of 2023) were constrained as of September 30, 2026 and no revenue was recognized related to these milestones. On October 16, 2026, the FDA approved the supplemental New Drug Application for phentolamine ophthalmic solution 0.75% for the treatment of presbyopia in adults, which triggered an additional regulatory milestone payment under the Viatris License Agreement. As a result of the approval, the uncertainty associated with this milestone was resolved, and the Company expects to recognize the milestone payment as revenue during the fourth quarter of 2026. See Note 16 – Subsequent Events.

Notes to Condensed Consolidated Financial Statements

A reconciliation of the closing balance of the contract assets and unbilled receivables associated with the Viatris License Agreement is as follows as of September 30, 2026 and 2025 (in thousands):

	Nine Months Ended September 30,	
	2026	2025
Contract assets and unbilled receivables		
Balance as of beginning of nine-month period	$ 1,170	$ 2,209
Revenue recognized	4,049	10,331
Reclassification to Accounts receivable related to costs billed under the Viatris License Agreement	(4,591)	(11,176)
Balance as of end of nine-month period	$ 628	$ 1,364

BioSense License and Assignment

On March 10, 2020, prior to the Rexahn Merger, Rexahn entered into an amendment to its collaboration and license agreement, (as amended, the "BioSense License and Assignment Agreement") with BioSense Global LLC ("BioSense") to advance the development and commercialization of the Rexahn RX-3117 drug compound ("RX-3117") for all human uses in the Republic of Singapore, China, Hong Kong, Macau, and Taiwan (the "BioSense Territory").

Under the BioSense License and Assignment Agreement, the Company is eligible to receive additional milestone payments in an aggregate of up to $84.5 million upon the achievement of development, regulatory and commercial goals and will also be eligible to receive tiered royalties at low double-digit rates on annual net sales in the BioSense Territory. The Company determined that none of the milestone payments under the BioSense License and Assignment Agreement were probable of payment as of September 30, 2026, and as a result, no revenue related to the milestones was recognized, as the achievement of events entitling the Company to any milestone payments were highly susceptible to factors outside of the Company's control. Future sales-based royalties related to the exclusive license to develop RX-3117, if any, will be recognized in the period the underlying sales transaction occurs.

Payments received under the BioSense License and Assignment Agreement will be subject to the CVR Agreement described in Note 4 – Commitments and Contingencies.

Processa License Agreement

On June 16, 2021, the Company entered into a license agreement (the "Processa License Agreement") with Processa Pharmaceuticals, Inc. ("Processa"), pursuant to which the Company agreed to grant Processa an exclusive license to develop, manufacture and commercialize RX-3117 globally, excluding the BioSense Territory.

Pursuant to the agreement, Processa was obligated to make future payments to the Company upon the achievement of certain development, regulatory and commercial milestones. In addition, Processa was obligated to pay the Company mid-single-digit percentage royalties based on annual sales.

On June 27, 2025, Processa notified the Company that it would not be developing RX-3117 and terminated the Processa License Agreement, effective October 25, 2025 (the "Termination Date"). No payments were received under the Processa License Agreement prior to the Termination Date, and no further payments will be received.

SBIR Grant Agreements

In September 2025, the Company received a Small Business Innovation Research ("SBIR") grant through the Department of Health and Human Services of up to $1.7 million to be used on the development of the RHO product. In September 2026, the Company received two additional SBIR grants, one for the development of the RDH12 program of up to $2.1 million and one for the development of the NMNAT1 program of up to $2.0 million (collectively with the September 2025 grant, the "SBIR Grant Agreements"). Direct and allocated indirect costs for development activities under the SBIR Grant Agreements are reimbursed on a draw-down basis as development activities are completed. For the three and nine months ended September 30, 2026, the Company recognized $0.2 million and $0.9 million, respectively, of income related to the SBIR Grant Agreements. For the three and nine months ended September 30, 2025, the Company recognized $0.1 million and $0.3 million, respectively, of income related to the RHO SBIR grant agreement. The amounts are recorded as other income, net in the accompanying condensed consolidated statements of comprehensive loss.

Notes to Condensed Consolidated Financial Statements

RDH12 Agreement

On July 22, 2025, the Company, together with its wholly owned subsidiary, OpusTX, LLC (collectively, "Opus"), entered into a funding and license agreement (the "RDH12 Agreement") with Eyes on the Future ("EOTF"), and RDH12 Fund for Sight (the "Fund," and together with EOTF, the "Funding Parties"), charitable organizations, relating to Opus' program to develop gene therapies that treat patients with inherited retinal degeneration associated with mutations in the RDH12 gene (the "RDH12 Program"). The RDH12 Agreement provides for funding by the Funding Parties of up to $1.6 million to support the development of the RDH12 Program. Opus is required to use the funding to conduct development activities in accordance with a mutually agreed development plan.

Under the RDH12 Agreement, Opus is subject to certain diligence obligations to develop a product under the RDH12 Program. If Opus is unable to achieve certain milestones by the specified dates, or if certain other events occur (a "License Trigger Event"), then the Funding Parties may exercise their rights under a non-exclusive, global, royalty-free and fully paid-up license granted by Opus to the Funding Parties to develop products under the RDH12 Program. If the Funding Parties exercise such license rights, then Opus will receive a non-exclusive license under the data and other intellectual property generated by the Funding Parties to develop products under the RDH12 Program, and the right to negotiate an exclusive license to such data and intellectual property to commercialize products under the RDH12 Program. The RDH12 Agreement includes certain restrictions on Opus' ability to out-license rights to the RDH12 Program, and during the term of the RDH12 Agreement, Opus may not grant a third party an exclusive license to develop or commercialize products under the RDH12 Program in the United States without the prior written consent of the Funding Parties.

The term of the RDH12 Agreement continues until the earlier of (a) dosing by Opus of three patients in a Phase 1a/2b clinical trial prior to a License Trigger Event, and (b) the first commercial sale of a product under the RDH12 Program following receipt of regulatory approval in the United States or certain European countries. The RDH12 Agreement will also terminate if an exclusive, global licensee of Opus for the RDH12 Program assumes Opus' obligations under the RDH12 Agreement. The RDH12 Agreement may be terminated by either party for cause, including material breach or bankruptcy, subject to a cure period, or by the Funding Parties for convenience following a License Trigger Event.

Eligible research and development costs under the RDH12 Agreement, as approved by the Funding Parties, are reimbursed on a draw-down basis as development activities are completed by the Company. For the three and nine months ended September 30, 2026, the Company recognized less than $0.1 million and $0.8 million, respectively, of income related to reimbursements under the RDH12 Agreement, which was recorded in other income, net, in the accompanying condensed consolidated statements of comprehensive loss. No income was recognized related to the RDH12 Agreement during the three and nine months ended September 30, 2025.

13. Net Loss per Share

Basic net loss per share of common stock is computed by dividing net loss by the weighted average number of shares of common stock outstanding during the period. For purposes of calculating basic net loss per share, the weighted-average number of common shares outstanding includes the weighted average effect of the pre-funded warrants issued in March 2025, November 2025 and September 2026, the exercise of which requires little or no consideration for the delivery of shares of common stock. Accordingly, the pre-funded warrants are not included in the table below of anti-dilutive securities. Diluted net loss per share is computed similarly to basic net loss per share, except that the denominator is increased to include potential common shares if their effect is dilutive. The Company's outstanding warrants (other than the pre-funded warrants), stock options, RSUs and Conversion Shares are potential common stock equivalents. The treasury stock method is used for warrants, stock options and RSUs, and the if-converted method is used for Conversion Shares. The Series B preferred stock converted into common stock on April 23, 2026; the common shares issued upon conversion are included in the weighted-average common shares outstanding from the conversion date, and the incremental effect of assumed conversion for the pre-conversion period was excluded because the conditions for conversion had not been met. No other incremental common stock equivalents were included because their inclusion would have been anti-dilutive.

The following potential common shares were not considered in the computation of diluted net loss per share as their effect would have been anti-dilutive for the three and nine month periods presented below:

Notes to Condensed Consolidated Financial Statements

	September 30,	
	2026	**2025**
Warrants	2,859,105	29,433,401
Stock options	7,859,320	7,218,064
RSUs	4,026,978	2,409,231
Conversion shares	530,517	—
Total	15,275,920	39,060,696

14. Income Taxes

The effective tax rate for the three and nine months ended September 30, 2026 and 2025 was zero percent. As of September 30, 2026, a full valuation allowance has been established to reduce the Company's net deferred income tax assets. As such, no tax benefit related to the Company's pre-tax loss was recognized for any of the periods presented. The Company's corporate returns are subject to examination for tax years beginning in 2021 for federal income tax purposes and subject to examination in various state jurisdictions beginning in 2020. The Company does not have any reserves for income taxes that represent the Company's potential liability for uncertain tax positions.

15. Deferred Compensation Plan

Effective October 1, 2021, the Company began offering a 401(k) plan ("401K Plan") to its employees. All employees are eligible to participate in the 401K Plan. The Company makes matching contributions equal to 100% on the first 3% of compensation that is deferred as an elective deferral and an additional 50% on the next 2% of compensation. The Company's matching contributions are made on a payroll-by-payroll basis. During the three months ended September 30, 2026 and 2025, the Company contributed $0.1 million and less than $0.1 million to the 401K Plan, respectively. During the nine months ended September 30, 2026 and 2025, the Company contributed $0.3 million and $0.1 million to the 401K Plan, respectively.

16. Subsequent Events

Phentolamine Ophthalmic Solution 0.75% sNDA Approval

On October 16, 2026, the FDA approved the Company's supplemental New Drug Application (sNDA) for phentolamine ophthalmic solution 0.75% for the treatment of presbyopia in adults. Under the Company's global license and collaboration agreement with Viatris, this regulatory approval triggered a milestone payment due to the Company. The Company is also eligible to receive tiered royalties on net sales of PS for the treatment of presbyopia in accordance with the terms of the Viatris License Agreement (see Note 12 – License and Collaboration Revenue and Other Funding Agreements).

March 2025 Warrants Call Notice

On October 22, 2026, the Company delivered call notices to the holders of all 2,859,105 March 2025 Warrants outstanding at September 30, 2026, as disclosed above. The Company exercised its right to call the March 2025 Warrants pursuant to the terms of the warrant agreement following the announcement of OPGx-BEST1 BIRD-1 Cohort 1 data on September 9, 2026 and the satisfaction of the specified stock price and trading volume conditions. Holders may exercise their March 2025 Warrants for cash at the exercise price of $0.95 per share until 6:30 p.m., New York City time, on December 4, 2026. Any March 2025 Warrants that remain unexercised after that time will be cancelled, and the holders will be entitled to receive $0.001 per warrant share. Through the date of issuance of these condensed consolidated financial statements, a total of 2,078,947 of the remaining 2,859,105 March 2025 Warrants had been exercised, resulting in proceeds of $2.0 million.

RDF Agreement Funding

On November 4, 2026, the Company received an additional $0.7 million of funding under the RDF Agreement described in Note 6 – Related Party Transactions, bringing total funding received under the RDF Agreement to $1.7 million.

Opus Genetics, Inc.
Form 10-Q

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

The following discussion of our financial condition and results of operations should be read in conjunction with the unaudited financial statements and notes included in Part I "Financial Information", Item 1 "Financial Statements" of this Quarterly Report on Form 10-Q (this "Report") and the audited financial statements and related footnotes included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Forward-Looking Statements

Certain statements contained in this Report are not statements of historical fact and are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Forward-looking statements give current expectations or forecasts of future events or our future financial or operating performance. Such statements include, but are not limited to, statements concerning our strategic business plans and outlook; the applications, design, potential benefits and market opportunity of our product candidates, including our estimates of patient populations and disease prevalence; our expectations regarding the potential market acceptance and commercial success of our product candidates, if approved; the timing, design, enrollment, progress and results of our ongoing and planned preclinical studies and clinical trials, including the expected timing of patient dosing and data readouts; our planned regulatory submissions and the potential receipt of regulatory approvals or designations; our manufacturing plans; continued drug development and commercialization under our agreement with Viatris, Inc. ("Viatris"); our other license, collaboration, grant and funding arrangements; our expected future revenue, expenses and capital requirements; the sufficiency of our cash and cash equivalents to fund our operations; our anticipated sources and uses of capital. In some cases, you can identify forward-looking statements by the following words: "aim," "anticipate," "believe," "can," "could," "continue," "design," "estimate," "expect," "forecast," "goal," "intend," "may," "might," "ongoing," "plan," "potential," "predict," "project," "seek," "should," "target," "will," "would" or the negative of those terms, and similar expressions that convey uncertainty of future events or outcomes to identify these forward-looking statements, although not all forward-looking statements contain these words.

These forward-looking statements reflect our management's beliefs and views with respect to future events, are based on estimates and assumptions as of the date of this Report and are subject to risks and uncertainties, many of which are beyond our control, that could cause our actual results to differ materially from those in these forward-looking statements, including, without limitation:

- Our gene therapy product candidates are based on a novel technology that is difficult to develop and manufacture, which may result in delays and difficulties in obtaining regulatory approval;
- Our planned clinical trials may face substantial delays, result in failure, or provide inconclusive or adverse results that may not satisfy FDA requirements to further develop our therapeutic products;
- Delays or difficulties associated with patient enrollment in clinical trials may affect our ability to conduct and complete those clinical trials and obtain necessary regulatory approvals;
- Changes in regulatory requirements could result in increased costs or delays in development timelines;
- We depend heavily on the success of our product pipeline; if we fail to find strategic partners or fail to adequately develop or commercialize our pipeline products, our business will be materially harmed;
- Others may discover, develop, or commercialize products similar to those in our pipeline before or more successfully than we do or develop generic variants of our products even while our product patents remain active, thereby reducing our market share and potential revenue from product sales;
- We do not currently have any sales or marketing infrastructure in place and we have limited drug research and discovery capabilities;
- The future commercial success of our products could significantly depend upon several uncertain factors, including market acceptance, third-party reimbursement practices and the success of competitors with similar products;

- Product liability lawsuits against us or our suppliers or manufacturers could cause us to incur substantial liabilities and could limit commercialization of any product candidate that we may develop;
- Failure to comply with health and safety laws and regulations could lead to material fines;
- We have not generated significant revenue from sales of any products and expect to incur losses for the foreseeable future;
- Our future viability is difficult to assess due to our short operating history and our future need for substantial additional capital, access to which could be limited by any adverse developments that affect the financial markets;
- Raising additional capital may cause our stockholders to be diluted, among other adverse effects;
- Instability and operational disruptions at government agencies, such as the FDA, may adversely impact our development and commercialization plans by causing delays and requiring the use of additional, unforeseen resources to obtain regulatory approval for trials or products in our pipeline;
- We operate in a highly regulated industry and face many challenges adapting to sudden changes in legislative reform or the regulatory environment, including due to government shutdowns and disruptions at government agencies, which cause delays, require the use of additional, unforeseen resources, affect our pipeline stability, and could impair our ability to compete in international markets;
- We may not receive regulatory approval to market our developed product candidates within or outside of the U.S.;
- With respect to any of our product candidates that receive marketing approval, we may be subject to substantial penalties if we fail to comply with applicable regulatory requirements;
- Our potential relationships with healthcare providers and third-party payors will be subject to certain healthcare laws and regulations, which could expose us to extensive potential liabilities;
- We rely on third parties for material aspects of our business, such as conducting our nonclinical and clinical trials and supplying and manufacturing bulk drug substances, which exposes us to certain risks;
- We may be unsuccessful in entering into or maintaining licensing arrangements or establishing strategic alliances on favorable terms, which could harm our business;
- Inadequate patent protection for our product candidates may result in our competitors developing similar or identical products or technology, which would adversely affect our ability to successfully commercialize;
- We may be unable to obtain full protection for our intellectual property rights under U.S. or foreign laws;
- We may become involved in lawsuits for a variety of reasons associated with our intellectual property rights, including alleged infringement suits initiated by third parties;
- We are dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy;
- As we grow, we may not be able to operate internationally or adequately develop our sales, marketing, distribution, and other corporate functions, which could disrupt our operations;
- The market price of our common stock is expected to be volatile and if we fail to comply with the continued listing standards of Nasdaq, our common stock may be delisted; and
- Factors out of our control related to our securities, such as securities litigation or actions of activist stockholders, could adversely affect our business and stock price and cause us to incur significant expenses.

We discuss many of these risks in greater detail under Part I, Item 1A “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2025 and below under the heading “Risk Factors,” and in subsequent reports filed with or furnished to the Securities and Exchange Commission (the “SEC”). Moreover, we operate in a very

competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

Any forward-looking statement made by us in this Report speaks only as of the date hereof or as of the date specified herein. We undertake no obligation to publicly update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable laws or regulations.

Overview

Opus Genetics, Inc. (the "Company," "Opus," "we," "us," or "our") is a clinical-stage biopharmaceutical company developing gene therapies to restore vision and prevent blindness in patients with inherited retinal diseases ("IRDs"). We also have a small molecule therapy for other ophthalmic disorders.

Gene Therapy Programs

Our pipeline features a portfolio of seven adeno-associated virus ("AAV") based gene therapies that address mutations in genes that cause different forms of Leber congenital amaurosis ("LCA"), bestrophinopathy, and retinitis pigmentosa.

OPGx-LCA5

OPGx-LCA5 is being developed to address a form of LCA due to biallelic mutations in the LCA5 gene, which encodes the lebercilin protein. LCA5-associated IRD is an early-onset severe inherited retinal dystrophy. Studies in patients with this mutation have reported evidence for the dissociation of retinal architecture and visual function in this disease, suggesting an opportunity for therapeutic intervention through gene augmentation. OPGx-LCA5 uses an adeno-associated virus 8 (AAV8) vector to precisely deliver a functional LCA5 gene to the outer retina via a single subretinal injection. OPGx-LCA5 has been accepted into the FDA's Rare Disease Evidence Principles (RDEP) program, and has also been granted Rare Pediatric Disease, Regenerative Medicine Advanced Therapy (RMAT), and Orphan Drug designations from the FDA. LCA5 affects an estimated 170 patients in the U.S. and 3,240 globally. OPGx-LCA5 is currently being evaluated in an open-label, adaptive Phase 1/2/3 clinical trial.

In September 2025, we reported positive data from six participants. The three pediatric participants treated over three months demonstrated large gains in cone-mediated vision with improvements across multiple measures of visual function. In the three adult participants, responses were observed out to 18 months, underscoring the potential durability of the treatment response. OPGx-LCA5 has been well tolerated with no ocular serious adverse events or dose-limiting toxicities.

In November 2025, we announced the successful completion of a Type B RMAT meeting with the FDA regarding OPGx-LCA5. The meeting provided constructive feedback from the FDA on key elements of the Company's registration strategy, including Chemistry, Manufacturing and Controls (CMC), and the pivotal trial design. The FDA acknowledged the significant unmet medical need for individuals with LCA5-related blindness and reaffirmed its commitment to regulatory flexibility for rare genetic diseases.

In May 2026, OPGx-LCA5 was accepted into the FDA's RDEP program. The FDA introduced this RDEP review process in September 2025 to provide greater speed and predictability in the review of therapies intended to treat ultra-rare diseases with very small patient populations with significant unmet medical need and that are driven by a known genetic defect that is the major driver of the pathophysiology.

Also, in May 2026, six-month pediatric clinical data from the ongoing OPGx-LCA5 Phase 1/2 study was reported at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting. The data demonstrated restoration of cone-mediated vision in pediatric LCA5 patients, with sensitivity improvements reaching normal ranges. In June 2026, data out to 24 months in the adult participants was reported, demonstrating a maintained response in visual acuity improvement.

In July 2026, we announced alignment with the FDA in a Type B RDEP meeting on the design of our registrational Phase 3 clinical trial evaluating OPGx-LCA5 in 8 participants with a six-month run-in period, allowing each participant to serve as their own control, prior to receiving treatment in both eyes.

In August 2026, enrollment in the Phase 3 trial was completed with 8 participants and dosing of OPGx-LCA5 is expected to begin in the fourth quarter of 2026 using clinical drug supply manufactured with the intended commercial processes.

The study's primary efficacy endpoint is a mean improvement of at least seven decibels (dB) in retinal sensitivity across 16 test loci, a clinically meaningful measure of visual function. The study is designed with greater than 90% statistical power to detect a treatment effect of at least seven dB.

Topline data from the Phase 3 trial is expected by the end of 2027. The intent is that data from this study will be used to submit a Biologics License Application (BLA). In addition, OPGx-LCA5 may qualify to receive a Priority Review Voucher if approved, which we believe would represent a potentially significant strategic asset.

OPGx-BEST1

OPGx-BEST1 is being developed for the treatment of IRDs associated with mutations in the BEST1 gene. BEST1 disease, or vitelliform macular dystrophy, is a rare, inherited retinal condition causing macular degeneration by mutations in the BEST1 gene, leading to progressive vision loss and, in some cases, blindness. OPGx-BEST1 uses an AAV2 vector to deliver a functional BEST1 gene designed to target the retinal pigment epithelium (RPE) and restore bestrophin function via a single subretinal injection. In preclinical studies conducted in a naturally occurring canine model of BEST1 disease, OPGx-BEST1 demonstrated restoration of the retinal pigment epithelium-photoreceptor interface using AAV-mediated gene delivery. OPGx-BEST1 is currently being evaluated in an open-label, adaptive Phase 1/2 clinical trial.

New epidemiology research estimates approximately 23,600 symptomatic BEST1 patients in the U.S., including 13,000 diagnosed and 10,600 undiagnosed patients, and approximately 45,400 symptomatic BEST1 patients globally.

In August 2025, we announced FDA clearance of an Investigational New Drug (IND) application to initiate a clinical trial.

In November 2025, we dosed our first (sentinel) participant in an adaptive, open-label, dose-exploring Phase 1/2 trial, known as BIRD-1, to evaluate the safety and efficacy of single-eye subretinal administration of OPGx-BEST1 in adult participants with Best Vitelliform Macular Dystrophy (BVMD) or Autosomal-Recessive Bestrophinopathy (ARB). The trial is designed as a dose escalation trial to evaluate two doses of OPGx-BEST1: 1.5E9 vg/eye (Cohort 1) and 4.5E9 vg/eye (Cohort 2). The trial will also explore biological activity through functional and anatomical endpoints, including changes in visual function and retinal structure.

In December 2025, we announced that the Independent Data Monitoring Committee (IDMC) overseeing the trial completed its pre-specified safety review of the one-month data from the sentinel participant and recommended advancing enrollment and dosing of additional participants in the trial, without modification.

In February 2026, results from the sentinel patient were presented at the Macula Society by the principal investigator, Dr. Mark Pennesi. The data demonstrated that OPGx-BEST1 was well tolerated with no ocular inflammation, no ocular or treatment-related adverse events, and no dose limiting toxicities. Early signals of functional vision improvement were observed, including an equivalent 12-letter gain in Best Corrected Visual Acuity (BCVA) in the treated study eye. In addition, structural improvement in central subfield thickness (CST) was observed with a 23% decrease in the study eye. Resolution of intraretinal fluid was also seen as early as one month in areas with less atrophy.

In May 2026, enrollment in Cohort 1 was completed with five participants in the study, three with BVMD and two with ARB. In the BVMD participants, we completed the added step of using an in vitro platform to confirm that each participant's disease mutation is amenable to gene augmentation.

In July 2026, we provided a program update and timeline for BIRD-1. In Cohort 1, the primary endpoint for evaluation is the safety and tolerability of OPGx-BEST1. In addition to safety measures, Opus will also be assessing a number of structural and functional parameters.

In August 2026, we met with the FDA to discuss OPGx-BEST1 development and potential endpoints for a pivotal clinical trial. We aligned with the FDA on a potential pivotal endpoint based on ≥3 dB microperimetry improvement in ≥5 prespecified loci, in conjunction with a patient-reported outcome in a randomized, controlled trial. BCVA, low-luminance visual acuity (LLVA), and contrast sensitivity (CS) may also be acceptable endpoints. We also aligned with the FDA on Phase 3 and commercial manufacturing requirements, which we expect to complete in early 2027.

In September 2026, we reported positive 3- and 6-month results from the low-dose Cohort 1 of BIRD-1, our ongoing Phase 1/2 clinical trial evaluating OPGx-BEST1 in patients with BEST1-related retinal diseases. Cohort 1 enrolled five participants treated at 1.5×10^9 vg/eye, including three participants with BVMD who had reached three months of follow-up and two with ARB who had reached six months of follow-up. In Cohort 1, OPGx-BEST1 has demonstrated a favorable safety and tolerability profile, with no serious adverse events or dose-limiting toxicities, no intraocular inflammation and no vital-sign or safety-laboratory findings of note. All treatment-related adverse events were mild or moderate in severity. All five participants in Cohort 1 demonstrated clinically meaningful improvement in visual function, measured as one or more of the following: BCVA, LLVA, CS or microperimetry, an advanced eye test that maps how well the central part of the retina sees light. Structural improvements were also observed across four participants. The greatest functional gains were observed in participants with less advanced disease, supporting the potential benefit of treating patients while viable retinal tissue remains.

Based on the safety profile and positive proof-of-concept findings from Cohort 1, we advanced to the higher-dose Cohort 2, evaluating OPGx-BEST1 at 4.5×10^9 vg/eye. Cohort 2 enrolled 11 participants, exceeding its original target of five participants. Dosing in Cohort 2 has been completed, and we expect to report topline data in the first quarter of 2027, including 3 month data for Cohort 2 participants and 6 month data for the three Cohort 1 participants with BVMD. We have also initiated planning for the adaptive Phase 3 trial, with participant dosing expected to begin in the second half of 2027.

OPGx-RDH12

OPGx-RDH12 is being developed for retinal dystrophy caused by mutations in the RDH12 gene, which is a severe, early-onset IRD marked by visual acuity loss in early childhood and rapid progression during adolescence. RDH12 encodes a retinol dehydrogenase enzyme involved in the visual cycle, protecting photoreceptors from toxic metabolite accumulation. RDH12 affects an estimated 2,500 patients in the U.S., 17,500 in the MENA (Middle East/North Africa) region, and 30,900 globally.

We are advancing OPGx-RDH12 to deliver a functional RDH12 gene to photoreceptors using an AAV vector. Preclinical studies in cellular and mouse models have demonstrated restoration of RDH12 activity and functional improvement. In October 2026, the FDA cleared our IND application for OPGx-RDH12, enabling us to advance the program into clinical development. OPGx-RDH12 is expected to enter the clinic in the fourth quarter of 2026 and is partially funded through a partnership with the RDH12 Alliance.

OPGx-MERTK

OPGx-MERTK is being developed for retinal degeneration caused by mutations in the MERTK gene, which plays a critical role in phagocytosis of photoreceptor outer segments by RPE cells. Loss of this function leads to rod-cone dystrophy, progressive vision loss and eventual blindness. MERTK affects an estimated 2,600 patients in the U.S., 14,300 in the MENA region and 21,960 globally.

OPGx-MERTK is designed to restore critical retinal pigment epithelium metabolic functions; therefore, we are advancing OPGx-MERTK using a modern AAV vector design, building on prior preclinical proof of concept and earlier clinical experience, with the aim of improving durability and efficacy. In preclinical studies, OPGx-MERTK provided evidence of retinal preservation of structure/function and preserved outer nuclear layer thickness in a small animal model of retinal degeneration. Preclinical work was funded by the Retinal Degeneration Fund (“RDF”) of the Foundation Fighting Blindness ("FFB"). This program is expected to initiate clinical testing at the Cleveland Clinic Abu Dhabi in the first quarter of 2027. The initial clinical study is being funded through Abu Dhabi’s Healthcare Research and Innovation Fund, in collaboration with the Department of Health – Abu Dhabi (DoH), Cleveland Clinic Abu Dhabi, the Innovative Research Oversight and Support (IROS) division of the M42 group, and the Authority of Social Contribution – Ma’an.

OPGx-RHO

OPGx-RHO is being developed for retinitis pigmentosa caused by autosomal dominant mutations in the rhodopsin protein utilizing a knock-down of the mutant rhodopsin and replacement with wild type rhodopsin protein. RHO affects an estimated 8,800 patients in the U.S., 14,600 in China and 30,200 globally.

We are advancing OPGx-RHO to preserve rod photoreceptors by replacing the mutant RHO gene with a functional copy, addressing the underlying genetic cause of disease. In preclinical studies, OPGx-RHO maintained rod morphology in the retinas in two large animal models. Preclinical work was co-funded by the FFB and the National Institutes of Health. This program is expected to initiate clinical testing globally in the second half of 2027.

Disease Prevalence

Global patient prevalence estimates are from a February 2026 analysis of key markets, including the United States, EU4 (France, Spain, Germany, & Italy), United Kingdom, Middle East/North Africa, and China.

Earlier Stage Programs

Preclinical work is ongoing for other programs, including targeting genetic mutations in CNGB1, NMNAT1 and other undisclosed retinal diseases.

Phentolamine Ophthalmic Solution 0.75% (PS)

Our pipeline also includes Phentolamine Ophthalmic Solution 0.75% (PS), a relatively non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size, administered as an eye drop. It aims to work by uniquely blocking the alpha-1 receptors found on the radial iris dilator muscles, which are activated by the alpha-1 adrenergic receptors. PS is designed to reduce pupil diameter through a sympatholytic mechanism of action that avoids engaging the ciliary muscle, potentially reducing risks such as retinal tears or detachment associated with older parasympathomimetic agents. PS is approved in the United States for two indications, the reversal of pharmacologically-induced mydriasis and, as of October 2026, the treatment of presbyopia in adults, and is being developed for a third indication, decreased vision under mesopic (low) light conditions following keratorefractive surgery.

In November 2022, we entered into a license and collaboration agreement (as amended, the "Viatris License Agreement") with Viatris, Inc. ("Viatris"), pursuant to which we granted Viatris an exclusive license to develop, manufacture, import, export and commercialize (i) PS for treating (a) reversal of pharmacologically-induced mydriasis, (b) decreased vision under mesopic (low) light conditions after keratorefractive surgery, and (c) presbyopia; and (ii) PS and low dose pilocarpine for treating presbyopia (together, the "PS Products") worldwide except for certain countries and jurisdictions in Asia. For more information on the Viatris License Agreement, please refer to Note 12 – License and Collaboration Revenue and Other Funding Agreements included in Part I, Item 1– Financial Statements of this Report.

RYZUMVI® (phentolamine ophthalmic solution) 0.75%: PS was approved by the FDA for the treatment of pharmacologically-induced mydriasis under the brand name RYZUMVI® in September 2023, which triggered a $10 million milestone payment under the Viatris License Agreement. RYZUMVI was commercialized by Viatris in April 2024. In October 2026, the FDA approved the supplemental New Drug Application for RYZUMVI for the treatment of presbyopia in adults, expanding the product's approved indications. The approval triggered a milestone payment due to us under the Viatris License Agreement.

Mesopic, Low-Contrast Conditions: We are conducting our second Phase 3 trial, known as LYNX-3, to treat significant, chronic night driving impairment in keratorefractive patients with reduced mesopic vision. The program is being conducted under a Special Protocol Assessment and has received Fast Track Designation from the FDA. The first Phase 3 trial, LYNX-2, met its primary endpoint of a gain of three lines (or 15 letters) or more of distance vision improvement on a low contrast chart in low light conditions after 15 days of dosing. In the study, 17.3% of participants treated with PS achieved a ≥15-letter Early Treatment Diabetic Retinopathy Study (ETDRS) (≥ 3-line) improvement in Mesopic Low Contrast Distance Visual Acuity (mLCVA) at Day 15, compared to 9.2% in the placebo group ($p<0.05$). We are currently enrolling participants in LYNX-3.

Recent Developments

Note Purchase and Purchase and Conversion Agreements

On April 2, 2026, we entered into a senior secured note purchase agreement (as amended from time to time, the "Note Purchase Agreement") with affiliates of Oberland Capital Management LLC (collectively, "Oberland Capital"). The proceeds of the financing may be used for general corporate purposes, including to accelerate the clinical development, manufacturing, and potential commercialization of our broad IRD gene therapy pipeline.

The Note Purchase Agreement provides us with access to future funding of up to $155.0 million to support our future strategic initiatives and growth, with an initial tranche of $35.0 million of senior secured notes (the "Notes"), which was funded on April 21, 2026 (the "First Purchase Date"), a second $35.0 million tranche available at our option (subject to satisfaction of customary funding conditions) until April 2, 2027, along with additional tranches of up to $35.0 million available to us upon the occurrence of certain milestones and satisfaction of customary funding conditions. The Note Purchase Agreement also provides for up to $50.0 million in additional tranches upon the mutual agreement of the parties. See further details in Note 7 – Debt and the *Liquidity and Capital Resources* section below under *Historical Capital Resources*.

Concurrently with the Note Purchase Agreement, we entered into a stock purchase and conversion agreement (the "Purchase and Conversion Agreement") with the purchasers of the Notes (the "Purchasers"), providing for the issuance of an aggregate of 1,116,070 shares of our common stock (the "Purchase Shares") at a price per share equal to $4.48 (the "Per Share Purchase Price"), for an aggregate purchase price of $5.0 million. See further details in Note 9 – Financings and the *Liquidity and Capital Resources* section below under *Historical Capital Resources*.

The Note Purchase Agreement and Purchase and Conversion Agreement were amended on April 13, 2026. The amendment revised the Per Share Purchase Price to the lower of $4.48 per share or the 30-day volume-weighted average price of the Company's common stock ending on the issuance date of the Purchase Shares and revised the Notes conversion price to the lower of $6.72 or 150% of the Per Share Purchase Price. The issuance of the Purchase Shares closed on May 26, 2026, resulting in the issuance of 1,116,070 shares of our common stock for aggregate gross proceeds of $5.0 million.

February 2026 Private Placement and Conversion of Series B Preferred Stock

On February 13, 2026, we entered into a securities purchase agreement with certain investors for a private placement of 7,374,632 shares of Series B preferred stock at a purchase price of $3.39 per share. The private placement closed on February 18, 2026, resulting in gross proceeds of approximately $25.0 million. The Series B preferred stock was convertible into shares of our common stock on a one-for-one basis, subject to stockholder approval of an increase in our authorized common stock and certain beneficial ownership limitations.

On April 20, 2026, we held our 2026 Annual Meeting. During the 2026 Annual Meeting, our stockholders voted to approve an amendment to our restated certificate of incorporation to increase the number of authorized shares of our common stock from 125 million shares to 250 million shares. Following the vote and filing of the associated amendment to our Certificate of Incorporation with the Delaware Secretary of State, all 7,374,632 outstanding shares of Series B preferred stock were converted on a one-for-one basis into 7,374,632 shares of common stock.

At-The-Market Programs

On August 6, 2026, we entered into a new sales agreement (the "New Sales Agreement") with Leerink Partners LLC ("Leerink") and Cantor Fitzgerald & Co. ("Cantor") in connection with a new at-the-market equity offering program (the "New ATM Program"), under which we may offer and sell shares of our common stock having an aggregate offering price of up to $125.0 million. The New ATM Program became available following the effectiveness of our Form S-3 on August 14, 2026, and replaced our prior at-the-market equity offering program with Leerink (the "Prior ATM Program"). During the nine months ended September 30, 2026, we sold 1,000,000 shares of common stock under the Prior ATM Program (all of which were sold during the three months ended March 31, 2026) for gross proceeds of $2.3 million, before deducting issuance expenses. Total issuance expenses, including sales agent fees and legal and accounting expenses, were $0.1 million for the nine months ended September 30, 2026. We did not sell any shares under the New ATM Program during the three and nine months ended September 30, 2026. See Note 9 – Financings and the Liquidity and Capital Resources section below under Historical Capital Resources.

Strategic Outlook

We intend to advance our current active pipeline and may explore opportunities to out-license from our portfolio or in-license other drug candidates. To date, our primary activities have been conducting research and development activities, performing business and financial planning, recruiting personnel and raising capital. We have one product, RYZUMVI®, approved for sale that is generating royalties based on sales by Viatris, and we do not expect to consistently generate significant revenues, other than license and collaborations revenue, unless and until the FDA or other regulatory authorities approve, and we successfully commercialize, LCA5, BEST1, other internally-developed gene therapy assets or PS for other indications. Until such time, if ever, as we can consistently generate substantial product revenue, we expect to finance our cash needs through a combination of equity, debt and alternative financings as well as through collaborations, strategic alliances and licensing arrangements.

Through September 30, 2026, we have funded our operations primarily through equity financings, the issuance of senior secured notes, and license fee and milestone payments in connection with the Viatris License Agreement.

Our net loss was $46.5 million and $120.1 million for the three and nine months ended September 30, 2026, as compared to a net loss of $17.5 million and $33.1 million for the three and nine months ended September 30, 2025. As of September 30, 2026, we had an accumulated deficit of $308.7 million. We anticipate that our expenses will continue to increase as we:

- continue clinical trials for LCA5, BEST1, PS and for any other product candidate in our future pipeline;
- continue nonclinical studies for our pipeline of gene therapies;
- develop additional product candidates that we identify, in-license or acquire;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- contract to manufacture our product candidates;
- maintain, expand and protect our intellectual property portfolio;
- hire additional staff, including clinical, scientific, operational and financial personnel, to execute our business plan;
- add operational, financial and management information systems and personnel to support our product development and potential future commercialization efforts;
- continue to operate as a public company; and
- establish on our own or with partners, a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval.

Our net loss will likely continue to fluctuate significantly from quarter to quarter and year to year, depending on fair value adjustments related to the Notes, the timing of our nonclinical studies, clinical trials, expenditures on other research and development activities (and reimbursement thereof), and from potential milestone payments received from and revenue earned under the Viatris License Agreement or any other license and collaboration agreements that we enter into.

Financial Operations Overview

License and collaborations revenue

License and collaborations revenue to date was derived from a one-time, non-refundable payment related to a license transfer, an additional milestone payment and reimbursement of expenses earned under the Viatris License Agreement, and to a much lesser degree, from license agreements with BioSense Global LLC (“BioSense”) and Processa Pharmaceuticals, Inc. (“Processa”). We anticipate that we will recognize revenue as we earn reimbursement for research and development services in connection with the Viatris License Agreement, up to a cap of $50.0 million, and we may earn additional revenues from potential milestone and royalty payments from the agreements with Viatris or from other license

agreements entered into in the future; however, the attainment of milestones or level of sales required to earn significant royalty payments is highly uncertain for the reasons explained below. Until further notice, we will report earned RYZUMVI® royalties as a component of license and collaboration revenue in the condensed consolidated statements of comprehensive loss.

To date, outside of the license and collaborations revenue referenced above, we do not expect to generate significant revenue unless or until RYZUMVI® sales become material, or regulatory approval is obtained, and commercialization begins for LCA5, BEST1, other internally-developed assets or PS for additional indications. In October 2026, the FDA approved PS for the treatment of presbyopia in adults, which triggered a milestone payment due to us under the Viatris License Agreement and expands the indications on which royalties may be earned. If we fail to complete the development of LCA5, BEST1, PS, or any other product candidate we may pursue or fail to obtain regulatory approval, our ability to generate significant revenue will be compromised.

Operating expenses

Our operating expenses are classified into two categories: research and development and general and administrative.

Research and development expenses

To date, our research and development expenses have related primarily to the clinical stage development of our IRD programs, including LCA5 and BEST1, and PS. Research and development expenses consist of costs incurred in performing research and development activities, including compensation, benefits and stock-based compensation costs for research and development employees and costs for consultants, costs associated with nonclinical studies and clinical trials, regulatory activities, manufacturing activities to support clinical activities, license fees, nonlegal patent costs, fees paid to external service providers that conduct certain research and development, and an allocation of overhead expenses.

Pursuant to the Viatris License Agreement, our research and development expenses related to the development of PS to date have been fully reimbursed by Viatris.

We expect that LCA5, BEST1, PS and other internally-developed assets will have higher development costs during the later stages of clinical development, as compared to costs incurred during their earlier stages of development, primarily due to the increased size and duration of the later-stage clinical trials and associated nonclinical studies. We expect our research and development expenses to increase over the next several years. However, it is difficult for us to determine with certainty the duration, costs and timing to complete our current or future nonclinical programs and clinical trials of LCA5, BEST1, PS and other internally-developed assets.

General and administrative expenses

General and administrative expenses consist primarily of personnel-related costs, including salaries, benefits and stock-based compensation costs, for personnel in functions not directly associated with research and development activities. Other significant costs include insurance coverage for directors and officers and other property and liability exposures, legal fees relating to intellectual property and corporate matters, business development costs, professional fees for accounting and tax services, other services provided by business consultants and legal settlements.

Fair value change in instruments measured at fair value

The fair value change in instruments measured at fair value consists of the fair value changes associated with the March 2025 Warrants, March 2025 Private Placement Warrants, and the Notes from the Note Purchase Agreement described further below in the *Liquidity and Capital Resources* section under *Historical Capital Resources*.

Financing costs

Financing costs consist of issuance costs attributed to our March 2025 Warrants and March 2025 Private Placement Warrants as well as in connection with the April 2, 2026 Note Purchase Agreement described further in Note 7 – Debt and below in the *Liquidity and Capital Resources* section under *Historical Capital Resources*.

Other income, net

Other income, net includes interest earned from cash and cash equivalent investments, reimbursements in connection with grants and other sources when they occur, and non-cash interest expense under the Company's RDF Agreement, which is accounted for as debt under ASC 470, *Debt*.

Provision for income taxes

Provision for income taxes consists of federal and state income taxes in the United States, as well as deferred income taxes and changes in related valuation allowance reflecting the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Currently, a full valuation allowance has been provided on the net deferred tax assets as of September 30, 2026 and December 31, 2025 given the uncertainty of future taxable income and other related factors impacting the realizability of our remaining net deferred tax assets.

Results of Operations

Comparison of Three Months Ended September 30, 2026 and 2025

The following table summarizes our operating results for the periods indicated (in thousands):

	Three Months Ended September 30,		
	2026	**2025**	**Change**
License and collaborations revenue	$ 1,137	$ 3,079	$ (1,942)
Operating expenses:			
Research and development	14,494	6,409	8,085
General and administrative	5,890	4,981	909
Total operating expenses	20,384	11,390	8,994
Loss from operations	(19,247)	(8,311)	(10,936)
Fair value change in instruments measured at fair value	(28,610)	(9,525)	(19,085)
Other income, net	1,362	382	980
Loss before income taxes	(46,495)	(17,454)	(29,041)
Benefit (provision) for income taxes	—	—	—
Net loss	$ (46,495)	$ (17,454)	$ (29,041)

License and collaborations revenue

License and collaborations revenue was $1.1 million and $3.1 million for the three months ended September 30, 2026 and 2025, respectively. Revenue during both quarterly periods was derived from the Viatris License Agreement, primarily for the reimbursement of research and development services, and to a much lesser extent, earned royalties from sales of RYZUMVI, indicated for the treatment of pharmacologically-induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents by our commercial partner. The $1.9 million decrease in license and collaborations revenue during the three month period ended September 30, 2026 compared to the corresponding prior year period was due to a decrease in PS research and development services.

Research and development

The following table illustrates the components of our research and development expenses for the periods presented (in thousands):

	Three Months Ended September 30,		
	2026	2025	Change
External costs:			
IRD Programs	$ 10,582	$ 2,575	$ 8,007
Phentolamine Ophthalmic Solution 0.75% (“PS”)	586	2,421	(1,835)
APX 3330	6	203	(197)
Unallocated	3	102	(99)
Total external cost	11,177	5,301	5,876
Internal costs:			
Employee related expenses	3,006	1,016	1,990
Facilities, supplies and other	311	92	219
Total internal costs	3,317	1,108	2,209
Total research and development expense	$ 14,494	$ 6,409	$ 8,085

A greater percentage of our research and development expense incurred has been allocated to IRD programs for the three months ended September 30, 2026 as compared to the three months ended September 30, 2025 as we continue to focus on developing our broad IRD gene therapy pipeline. Conversely, a lesser percentage of research and development expense incurred has been allocated to PS for the three months ended September 30, 2026 as compared to the three months ended September 30, 2025.

Research and development expenses for the three months ended September 30, 2026 were $14.5 million compared to $6.4 million for the three months ended September 30, 2025. The $8.1 million increase was primarily attributable to higher manufacturing and clinical costs associated with the Company's IRD programs of $5.5 million and $1.7 million, respectively, as development progresses, partially offset by lower clinical costs associated with the PS related programs of $1.7 million due to the timing of clinical trial activities. Additional period-over-period changes included an increase of $0.9 million in discovery costs related to IRD programs and an increase of $1.9 million in employee compensation costs, partially offset by a decrease of $0.2 million in miscellaneous research and development costs. Research and development expenses included $0.6 million and $0.2 million in stock-based compensation expense for the three months ended September 30, 2026 and 2025, respectively.

General and administrative

General and administrative expenses for the three months ended September 30, 2026 were $5.9 million compared to $5.0 million for the three months ended September 30, 2025. The increase of $0.9 million was primarily attributable to higher employee compensation costs on a period-over-period basis. General and administrative expenses included $1.0 million and $0.6 million in stock-based compensation expense during the three months ended September 30, 2026 and 2025, respectively.

Fair value change in instruments measured at fair value

The fair value change in instruments measured at fair value was attributed to the March 2025 Warrants and March 2025 Private Placement Warrants, and to a lesser extent, the Notes remeasured at fair value on a recurring basis. The $28.6 million expense for the three months ended September 30, 2026 consisted of $27.5 million of expense from the warrant fair value remeasurement and $1.1 million of expense from the fair value remeasurement of the Notes. The expense of $9.5 million for the three months ended September 30, 2025 is due exclusively to the warrant fair value remeasurement. The fair value changes are attributed to the fluctuations in our common stock fair value and underlying changes in volatility, expected term and interest rates, as well as discount rate for the Notes.

Other income, net

During the three months ended September 30, 2026, we had other income, net of $1.4 million, which primarily consisted of $0.8 million of interest income earned on our cash and cash equivalents and $0.7 million of other income, including amounts related to the Apexian Termination and IP Assignment Agreement and grant income, partially offset by non-cash interest expense in the amount of $0.1 million.

During the three months ended September 30, 2025, we had other income, net of $0.4 million, which primarily consisted of $0.3 million of interest income earned on our cash and cash equivalents and grant income in the amount of $0.1 million.

Comparison of Nine Months Ended September 30, 2026 and 2025

The following table summarizes our operating results for the periods indicated (in thousands):

	For the Nine Months Ended		
	2026	**2025**	**Change**
License and collaborations revenue	$ 4,049	$ 10,331	$ (6,282)
Operating expenses:			
Research and development	36,278	20,384	15,894
General and administrative	17,862	17,093	769
Total operating expenses	54,140	37,477	16,663
Loss from operations	(50,091)	(27,146)	(22,945)
Fair value change in instruments measured at fair value	(72,800)	(5,803)	(66,997)
Financing costs	(1,113)	(1,337)	224
Other income, net	3,876	1,218	2,658
Loss before income taxes	(120,128)	(33,068)	(87,060)
Benefit (provision) for income taxes	—	—	—
Net loss	$ (120,128)	$ (33,068)	$ (87,060)

License and collaborations revenue

License and collaborations revenue was $4.0 million and $10.3 million for the nine months ended September 30, 2026 and 2025, respectively. Revenue during the nine month periods was derived from the Viatris License Agreement, primarily for the reimbursement of research and development services, and to a much lesser extent, earned royalties from sales of RYZUMVI, indicated for the treatment of pharmacologically-induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents by our commercial partner. The $6.3 million decrease in license and collaborations revenue during the nine month period ended September 30, 2026 compared to the corresponding prior year period was due to a decrease in PS research and development services.

Research and development

The following table illustrates the components of our research and development expenses for the periods presented (in thousands):

	Nine Months Ended September 30,		
	2026	2025	Change
External costs:			
IRD Programs	$ 25,425	$ 6,624	$ 18,801
Phentolamine Ophthalmic Solution 0.75% (“PS”)	3,201	8,914	(5,713)
APX 3330	24	325	(301)
Unallocated	34	340	(306)
Total external cost	28,684	16,203	12,481
Internal costs:			
Employee related expenses	6,855	3,880	2,975
Facilities, supplies and other	739	301	438
Total internal costs	7,594	4,181	3,413
Total research and development expense	$ 36,278	$ 20,384	$ 15,894

A greater percentage of our research and development expense incurred has been allocated to IRD programs for the nine months ended September 30, 2026 as compared to the nine months ended September 30, 2025 as we continue to focus on developing our broad IRD gene therapy pipeline. Conversely, a lesser percentage of research and development expense incurred has been allocated to PS for the nine months ended September 30, 2026 as compared to the nine months ended September 30, 2025.

Research and development expenses for the nine months ended September 30, 2026 were $36.3 million compared to $20.4 million for the nine months ended September 30, 2025. The $15.9 million increase was primarily attributable to higher manufacturing and clinical costs associated with the Company's IRD programs of $13.3 million and $3.1 million, respectively, as development progresses, partially offset by lower clinical costs associated with the PS related programs of $5.5 million as clinical trials near completion. Additional period-over-period changes included an increase of $2.5 million in discovery costs related to IRD programs and an increase of $2.7 million in employee compensation costs, partially offset by a decrease of $0.2 million in miscellaneous research and development costs. Research and development expenses included $1.4 million and $0.8 million in stock-based compensation expense for the nine months ended September 30, 2026 and 2025, respectively.

General and administrative

General and administrative expenses for the nine months ended September 30, 2026 were $17.9 million compared to $17.1 million for the nine months ended September 30, 2025. The increase of $0.8 million was primarily attributable to higher employee compensation related costs of $2.6 million, partially offset by lower legal and public company related costs of approximately $1.8 million. General and administrative expenses included $3.1 million and $1.8 million in stock-based compensation expense during the nine months ended September 30, 2026 and 2025, respectively.

Fair value change in instruments measured at fair value

The fair value change in instruments measured at fair value was attributed to the March 2025 Warrants and March 2025 Private Placement Warrants, and to a lesser extent, the Notes remeasured at fair value on a recurring basis. The expense of $72.8 million for the nine months ended September 30, 2026 is due to expense of $71.5 million from the warrant fair value remeasurement, and expense of $1.3 million from the fair value remeasurement of the Notes. The expense of $5.8 million for the nine months ended September 30, 2025 is due exclusively to the warrant fair value remeasurement. The fair value changes are attributed to the fluctuations in our common stock fair value and underlying changes in volatility, expected term and interest rates, as well as discount rate for the Notes.

Financing costs

Financing costs of $1.1 million for the nine months ended September 30, 2026 consisted of issuance costs attributed to the April 2, 2026 Note Purchase Agreement described further in Note 7 – Debt and below in the *Liquidity and Capital Resources* section under *Historical Capital Resources*.

Financing costs of $1.3 million for the nine months ended September 30, 2025 consisted of issuance costs attributed to the March 2025 Warrants and March 2025 Private Placement Warrants.

Other income, net

During the nine months ended September 30, 2026, we had other income, net of $3.9 million, which primarily consisted of $1.9 million of interest income earned on our cash and cash equivalents and $2.2 million of other income, including amounts related to the Apexian Termination and IP Assignment Agreement and grant income, partially offset by non-cash interest expense in the amount of $0.2 million.

During the nine months ended September 30, 2025, we had other income, net of $1.2 million, which primarily consisted of $1.0 million of interest income earned on our cash and cash equivalents and grant income in the amount of $0.3 million, partially offset by non-cash interest expense in the amount of $0.1 million.

Liquidity and Capital Resources

Capital Resources

As of September 30, 2026, our principal sources of liquidity consisted of cash and cash equivalents of $89.0 million. We believe our current available cash and cash equivalents will be sufficient to fund our operations for at least the next 12 months from the date of issuance of these financial statements. As of September 30, 2026, our cash and cash equivalents were invested primarily in cash deposits and cash equivalent investments at four large financial institutions.

Historical Capital Resources

Our primary sources of cash to fund our operations have been equity offerings totaling $166.8 million, including proceeds from the exercise of March 2025 Warrants and March 2025 Private Placement Warrants; $35.0 million of Notes issued on the First Purchase Date under the Note Purchase Agreement with Oberland Capital, as described further below; and $8.5 million from the issuance of convertible notes. In addition, we received a one-time non-refundable cash payment of $35.0 million during the fourth quarter of 2022, a $10.0 million milestone payment during the fourth quarter of 2023, and have received reimbursement for costs related to development since the fourth quarter of 2022 totaling $42.1 million through September 30, 2026, all in connection with the Viatris License Agreement. Lastly, we have received funding in the amount of $3.4 million from the RDF Agreement and various research and development grants.

April 2026 Note Purchase Agreement

On April 2, 2026, we entered into the senior secured Note Purchase Agreement with Oberland Capital. The proceeds of the financing may be used for general corporate purposes, including to accelerate the clinical development, manufacturing, and potential commercialization of our broad IRD gene therapy pipeline. Capitalized terms used in this section but not defined herein have the meanings given to such terms in the Note Purchase Agreement.

The Note Purchase Agreement with Oberland Capital provides us with access to future funding of up to $155.0 million to support our future strategic initiatives and growth, with an initial tranche of $35.0 million, which was funded on April 21, 2026, the First Purchase Date, a second $35.0 million tranche available at our option (subject to satisfaction of customary funding conditions) until April 2, 2027, along with additional tranches of up to $35.0 million available to us upon the occurrence of certain milestones and satisfaction of customary funding conditions. The Note Purchase Agreement also provides for up to $50.0 million in additional tranches upon the mutual agreement of the parties.

The Notes mature on April 21, 2033 (the “Maturity Date”) and bear interest at a rate per annum equal to Term SOFR for the three-month interest period (subject to a 3.68% floor) plus the Applicable Margin, payable quarterly. Interest payable on the first eight interest payment dates following the effective date of the Note Purchase Agreement shall be payable as follows: 50% of the interest owed for each applicable period shall be paid in kind and the remaining 50% of interest owed for such applicable period shall be paid in cash. Thereafter, 100% of the interest owed for each applicable

period shall be paid in cash. We shall make a payment of principal on each outstanding Note on the sixth anniversary of the First Purchase Date in an amount equal to half of the aggregate principal amount of such Notes outstanding on such date, with the remaining balance payable on the Maturity Date.

Pursuant to the Note Purchase Agreement, as amended by the Waiver and Omnibus Amendment, following the Issuance Authorization and prior to the date that is 18 months after the date that is 30 days after the effective date of the Initial Registration Statement, any Purchaser may, at any time and from time to time, subject to the notice and other terms and conditions set forth in the Note Purchase Agreement, elect to convert up to 10% in the aggregate of the principal amount of such Purchaser's Notes then outstanding (the "Conversion Amount") into shares (the "Conversion Shares") of the Company's common stock. The number of Conversion Shares issuable upon such conversion shall be equal to the Conversion Amount divided by the lower of $6.72 and 150% of the Per Share Purchase Price, in each case as amended by the Waiver and Omnibus Amendment. Upon any such conversion, an aggregate principal amount of Notes equal to the Conversion Amount shall be deemed paid and satisfied in full and shall cease to accrue interest. Any interest accrued and unpaid through and including the date of such conversion shall remain outstanding and shall be payable in cash on the next interest payment date (or, if we and the Purchasers so elect in writing, shall be convertible into Conversion Shares).

We may voluntarily prepay the Notes in full at any time subject to a prepayment premium. Moreover, a prepayment premium may also be payable by us upon the occurrence of, (i) at any time prior to the first anniversary of the First Purchase Date, an acceleration of the obligations under the Note Purchase Agreement following an event of default (other than a change of control) or, (ii) at any time prior to the date that is 18 months after the First Purchase Date, a change of control of the Company that is not otherwise consented to by the Purchasers holding a majority of the outstanding Notes. We are required to make mandatory prepayments of the Notes with net cash proceeds from insurance proceeds or condemnation awards, in each case, subject to certain exceptions and reinvestment rights.

Purchase and Conversion Agreement

Concurrently with the Note Purchase Agreement, on April 2, 2026, we entered into the Purchase and Conversion Agreement with the Purchasers providing for the issuance of an aggregate of 1,116,070 shares of our common stock at a price per share equal to $4.48, for an aggregate purchase price of $5.0 million.

The Purchase and Conversion Agreement also provided the Purchasers with an option to acquire additional Purchase Shares (the "Option Shares") at a price per share equal to $0.0001 (subject to adjustment as set forth in the Purchase and Conversion Agreement, the "Per Share Exercise Price") in the event that prior to October 2, 2026, we issued shares of our common stock (or instruments exercisable for or convertible into shares of our common stock) at an effective sale price per share below the Per Share Purchase Price, subject to certain exceptions (a "Dilutive Equity Round"). The number of Option Shares issuable upon exercise of such option will be such that, accounting for the sale and issuance of the Option Shares, the weighted average of the Per Share Purchase Price and Per Share Exercise Price for all Purchase Shares (including Option Shares) issued pursuant to the Purchase and Conversion Agreement, taken together, would equal the lowest effective sale price per share paid in cash by third party investors to us for our common stock issued by us in such Dilutive Equity Round. The option to acquire Option Shares expired unexercised on October 2, 2026.

The Purchase and Conversion Agreement was amended on April 13, 2026 to revise the Per Share Purchase Price to the lower of $4.48 per share or the 30-day volume-weighted average price of our common stock ending on the issuance date of the Purchase Shares and to modify the timing and closing conditions for issuance of the Purchase Shares. The issuance of the Purchase Shares contemplated by the Purchase and Conversion Agreement closed on May 26, 2026, resulting in the issuance of 1,116,070 shares of common stock for aggregate gross proceeds of $5.0 million.

February 2026 Private Placement

On February 13, 2026, we entered into a securities purchase agreement with certain investors for a private placement of an aggregate of 7,374,632 shares of Series B preferred stock at a purchase price of $3.39 per share. The private placement closed on February 18, 2026, resulting in gross proceeds of approximately $25.0 million. As a result of the approval by stockholders of the proposal to increase our authorized shares of common stock at the 2026 Annual Meeting, all 7,374,632 outstanding shares of Series B preferred stock were converted on a one-for-one basis into 7,374,632 shares of common stock.

November 2025 Registered Direct Offering

On November 5, 2025, we entered into a securities purchase agreement to sell securities in a registered direct offering for gross proceeds of approximately $23.0 million, before deducting offering expenses. The financing was led by Perceptive Advisors and Balyasny Asset Management, with participation by new and existing institutional investors, including Nantahala Capital.

In the offering, we sold an aggregate of 3,827,751 shares of common stock at a price of $2.09 per share and, in lieu of common stock to certain investors, pre-funded warrants to purchase up to an aggregate of 7,177,033 shares of common stock at a purchase price of $2.0899 per pre-funded warrant. Each pre-funded warrant has an exercise price of $0.0001 per share of common stock, will be immediately exercisable subject to certain conditions set forth in each pre-funded warrant, and will not expire. The offering closed on November 10, 2025.

August 2025 Private Placement

On August 25, 2025, we entered into subscription agreements pursuant to which we agreed to issue and sell to certain investors an aggregate of 3,138,338 shares of our common stock (the "August 2025 Private Placement"). The aggregate gross proceeds from the August 2025 Private Placement were approximately $3.5 million. The August 2025 Private Placement closed on August 25, 2025.

The August 2025 Private Placement was led by Cam Gallagher, Chair of our board of directors, with an investment of $1.0 million, along with participation by Sean Ainsworth, the lead independent director of the Board, and other investors.

March 2025 Financings

On March 21, 2025, we entered into an underwriting agreement with Craig-Hallum Capital Group, LLC, as the sole underwriter. Pursuant to the underwriting agreement, we agreed to issue and sell, in an underwritten public offering (the "March 2025 Offering"), 12,219,736 shares of common stock and warrants to purchase up to 21,052,631 shares of common stock (the "March 2025 Warrants"). Each share of common stock was sold together with one March 2025 Warrant to purchase one share of common stock, at a price to the public of $0.95 per share and related March 2025 Warrant. We also issued 8,832,895 pre-funded warrants ("Pre-Funded Warrants") at a price to the public of $0.9499 per Pre-Funded Warrant.

Also on March 21, 2025, we entered into a subscription agreement (the "Subscription Agreement") with each of Dr. George Magrath, the Company's Chief Executive Officer, and Cam Gallagher, the chairman of our Board. Pursuant to the Subscription Agreement, the Company agreed to issue and sell, in a private offering (the "March 2025 Private Placement"), a total of 392,157 shares of common stock to Dr. Magrath and 784,314 shares of common stock to Mr. Gallagher, as well as 392,157 warrants to purchase shares of common stock to Dr. Magrath and 784,314 warrants to purchase shares of common stock to Mr. Gallagher ("March 2025 Private Placement Warrants") at an initial exercise price of $1.15.

The combined gross proceeds from the March 2025 Offering and the March 2025 Private Placement, which both closed on March 24, 2025, were approximately $21.5 million, before deducting underwriting discounts and commissions and offering expenses payable by us. All of the March 2025 Private Placement Warrants were exercised as of September 30, 2026.

March 2025 Warrants

The March 2025 Warrants have an initial exercise price equal to $0.95 per share of common stock and are exercisable for five years from the date of issuance. The exercise prices and numbers of shares of common stock issuable upon exercise are subject to appropriate adjustment in the event of stock dividends, stock splits, reorganizations or similar events affecting the common stock and also upon any distributions of assets, including cash, stock or other property to our stockholders. A holder may not exercise the March 2025 Warrant if, after giving effect to such exercise, the holder (together with its affiliates) would beneficially own (as determined in accordance with the terms of the March 2025 Warrants) more than 4.99% (or, at the election of the holder, 9.99%) of the outstanding common stock immediately after giving effect to the exercise.

All outstanding March 2025 Warrants have been called by the Company. We announced OPGx-BEST1 Cohort 1 data on September 9, 2026 and, in October 2026, following satisfaction of the applicable conditions, we delivered call notices to the holders of all 2,859,105 March 2025 Warrants outstanding at September 30, 2026. Holders may exercise their March 2025 Warrants for cash at the exercise price of $0.95 per share until 6:30 p.m. Eastern Time on December 4, 2026, after which any unexercised March 2025 Warrants will be cancelled in exchange for $0.001 per warrant share.

In the event of a fundamental transaction, as defined in the Form of Warrant, the holders of the March 2025 Warrants will be entitled to receive upon exercise the kind and amount of securities, cash or other property that the holders would have received had they exercised immediately prior to such fundamental transaction. Additionally, as more fully described in the Form of Warrant, in the event of certain fundamental transactions, the holders of the March 2025 Warrants will be entitled to receive consideration in an amount equal to a defined Black Scholes calculation of the remaining unexercised portion of the March 2025 Warrants on the date of consummation of such fundamental transaction.

March 2025 Private Placement Warrants

The March 2025 Private Placement Warrants had an initial exercise price equal to $1.15 per share of common stock and were exercisable for five years from the date of issuance. As of September 30, 2026, all of the March 2025 Private Placement Warrants have been exercised and none remain outstanding.

Pre-Funded Warrants

The Pre-Funded Warrants have an exercise price of $0.0001 per share of common stock and are immediately exercisable and are exercisable at any time until exercised in full. The exercise prices and numbers of shares of common stock issuable upon exercise are subject to appropriate adjustment in the event of stock dividends, stock splits, reorganizations or similar events affecting the common stock. A holder may not exercise the Pre-Funded Warrant if, after giving effect to such exercise, the holder (together with its affiliates) would beneficially own (as determined in accordance with the terms of the Pre-Funded Warrants) more than 4.99% (or, at the election of the holder, 9.99%) of the outstanding common stock immediately after giving effect to the exercise. In the event of a fundamental transaction, as defined in the form of Pre-Funded Warrant, the holders of the Pre-Funded Warrants will be entitled to receive upon exercise of the Pre-Funded Warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such fundamental transaction. During the three months ended September 30, 2026, we issued 4,157,895 additional pre-funded warrants, with an exercise price of $0.0001 per share, to holders of March 2025 Warrants that elected to receive pre-funded warrants in lieu of shares of common stock upon exercise (see Note 9 – Financings).

At-The-Market Programs

Since 2021, we have maintained a succession of three at-the-market equity offering programs: the JonesTrading ATM Program under a sales agreement with JonesTrading Institutional Services LLC (“JonesTrading”); the Prior ATM Program under the Prior Sales Agreement with Leerink; and the New ATM Program under the New Sales Agreement with Leerink and Cantor (collectively, the “ATM Programs”).

On March 11, 2021, we entered into a sales agreement with JonesTrading, which permitted us to offer and sell, from time to time at our sole discretion, to or through JonesTrading, acting as agent and/or principal, shares of our common stock having an aggregate offering price of up to $40.0 million (the "JonesTrading ATM Program"). On January 13, 2025, we terminated the JonesTrading ATM Program. No shares were sold under the JonesTrading ATM Program during the three and nine months ended September 30, 2026 or 2025.

On January 13, 2025, we entered into the Prior Sales Agreement with Leerink, which permitted us to offer and sell, from time to time at our sole discretion, to or through Leerink, acting as agent and/or principal, shares of our common stock having an aggregate offering price of up to $40.0 million under the Prior ATM Program. During the three and nine months ended September 30, 2025, we sold 1,472,703 and 1,825,656 shares of common stock, respectively, under the Prior ATM Program for gross proceeds of $1.7 million and $2.1 million, respectively, before deducting issuance expenses. Total issuance expenses, including sales agent fees and legal and accounting expenses, were $0.1 million and $0.3 million for the three and nine month periods ended September 30, 2025, respectively. During the nine months ended September 30, 2026, we sold 1,000,000 shares of common stock under the Prior ATM Program (all of which were sold during the three months ended March 31, 2026) for gross proceeds of $2.3 million, before deducting issuance expenses. Total issuance expenses, including sales agent fees and legal and accounting expenses, were $0.1 million for the nine months ended September 30, 2026.

On August 6, 2026, we entered into the New Sales Agreement with Leerink and Cantor, which permits us to offer and sell, from time to time at our sole discretion, to or through Leerink or Cantor, acting as agent and/or principal, shares of our common stock having an aggregate offering price of up to $125.0 million. The New ATM Program became available upon the effectiveness of our registration statement on Form S-3 on August 14, 2026. No shares were sold under the New ATM Program through September 30, 2026, and $125.0 million remained available under the New ATM Program as of that date. The New Sales Agreement replaced the Prior Sales Agreement, and no additional shares may be sold under the Prior ATM Program.

As of September 30, 2026, we had sold an aggregate of 10,573,250 shares of common stock under the ATM Programs since their inception, resulting in gross proceeds of $31.0 million and total issuance costs of $1.5 million.

Registered Direct Offering

On June 4, 2021, we entered into a placement agency agreement for a registered direct offering ("RDO") with A.G.P./Alliance Global Partners ("AGP"). Pursuant to the terms of the placement agency agreement, AGP on June 8, 2021 sold an aggregate of 3,076,923 shares of our common stock and warrants to purchase 1,538,461 shares of our common stock (the "RDO Warrants") at an offering price of $4.875 per one share and per one-half of each RDO Warrant. The RDO was made pursuant to the Company's 2021 shelf registration.

The RDO Warrants had an exercise price of $6.09 per share, and were exercisable from the initial issuance date of June 8, 2021 for five years through June 8, 2026. As of September 30, 2026, no RDO Warrants were outstanding and none were exercised prior to their expiration on June 8, 2026.

Subject to limited exceptions, a holder of an RDO Warrant will not have the right to exercise any portion of its RDO Warrants if the holder, together with its affiliates, would beneficially own in excess of 4.99% (or, at the election of a holder prior to the date of issuance, 9.99%) of the number of shares of the Company's common stock outstanding immediately after giving effect to such exercise; provided that upon prior notice to the Company, the holder may increase or decrease the beneficial ownership limitation, provided further that in no event shall the beneficial ownership limitation exceed 9.99%.

Cash Flows

The following table summarizes our cash flows for the periods indicated (in thousands):

	For the Nine Months Ended September 30,	
	2026	**2025**
Net cash used in operating activities	$ (38,713)	$ (25,420)
Net cash used in investing activities	(189)	—
Net cash provided by financing activities	82,885	25,914
Net increase in cash, cash equivalents and restricted cash	$ 43,983	$ 494

Cash Flow from Operating Activities

For the nine months ended September 30, 2026, cash used in operating activities of $38.7 million was attributable to a net loss of $120.1 million, partially offset by non-cash expense of $78.6 million, primarily driven by the change in fair value attributable to the warrant liabilities and Notes resulting in a non-cash expense of $72.1 million, non-cash stock-based compensation expense of $4.5 million, and the reclassification of $1.1 million of debt issuance costs associated with the Note Purchase Agreement to financing activities. The net change in our operating assets and liabilities provided cash of $2.8 million and resulted primarily from an increase in accrued expenses of $1.6 million driven by an increase in accrued employee compensation, and a decrease in accounts receivable of $1.0 million due to a lower receivable balance from Viatris in connection with the Viatris License Agreement.

For the nine months ended September 30, 2025, cash used in operating activities of $25.4 million was attributable to a net loss of $33.1 million, adjusted by a reclassification to financing activities related to the March 2025 financings and by non-cash expenses of approximately $9.9 million in the aggregate, and by a net use of cash of $2.2 million from

changes in operating assets and liabilities. The non-cash expenses consisted principally of a fair value change in warrant and other derivative liabilities expense of $5.8 million, and stock-based compensation of $2.6 million. The reclassification to financing activities for issuance costs attributed to our liability classified warrants was $1.3 million. The change in operating assets and liabilities was primarily attributable to a net decrease in our aggregate accounts payable and accrued expenses and by an increase in our prepaid expenses and other assets, offset in part by a decrease in our accounts receivable and contract assets. All of the changes were attributed to fluctuations in our operating expenses and collections under the normal course of business.

Cash Flow from Investing Activities

For the nine months ended September 30, 2026, cash used in investing activities of $0.2 million was attributable to the purchase of research equipment to be used in our research and development efforts.

For the nine months ended September 30, 2025, there were no sources or uses of cash from investing activities.

Cash Flow from Financing Activities

Net cash provided by financing activities during the nine months ended September 30, 2026 was $82.9 million that consisted principally of gross proceeds received from the Note Purchase Agreement of $35.0 million, gross proceeds received from the Purchase and Conversion Agreement of $5.0 million, gross proceeds received from the issuance of Series B preferred stock in the February 2026 private placement of $25.0 million, gross proceeds from the Prior ATM Program of approximately $2.3 million, proceeds from the exercise of warrants of $15.7 million, and proceeds from the exercise of stock options totaling $1.6 million. These financing activities were offset by issuance costs of $1.1 million related to the Note Purchase Agreement and issuance costs of $0.5 million related to equity instruments.

Net cash provided by financing activities during the nine months ended September 30, 2025 was $25.9 million that consisted principally of gross proceeds received from the March 2025 Offering and March 2025 Private Placement of $21.5 million in the aggregate, the August 2025 Private Placement in the amount of $3.5 million, and from gross proceeds received from the Prior ATM Program of $2.1 million. These financing activities were offset by issuance costs of $2.2 million in the aggregate. Lastly, we received funding of $1.0 million in connection with the RDF Agreement.

Liquidity and Capital Resource Requirements

As of September 30, 2026, we had cash and cash equivalents of $89.0 million. Our primary sources of cash to fund our operations have been equity offerings totaling $166.8 million, including proceeds from the exercise of March 2025 Warrants and March 2025 Private Placement Warrants; $35.0 million of Notes issued under the Note Purchase Agreement with Oberland Capital, as described above; and $8.5 million from the issuance of convertible notes. License and collaborations revenue inception to date was derived from a one-time non-refundable payment of $35.0 million, a milestone payment of $10.0 million, and we have received reimbursement for costs related to development since the fourth quarter of 2022 totaling $42.1 million through September 30, 2026, all in connection with the Viatris License Agreement. We anticipate that we will recognize revenue as we earn reimbursement for research and development services in connection with the Viatris License Agreement and we may earn additional revenues from future potential milestone and royalty payments from the agreement with Viatris, or from other license agreements entered into in the future; however, the attainment of milestones or level of sales required to earn royalty payments is highly uncertain.

To date, outside of the license and collaborations revenue referenced above, we do not expect to generate significant revenue unless or until RYZUMVI sales become material, or regulatory approval is obtained and commercialization begins for LCA5, BEST1, other internally-developed assets or PS for additional indications. If we fail to complete the development of LCA5, BEST1, other internally-developed assets, PS or any other product candidate we may pursue in the future in a timely manner or fail to obtain regulatory approval for any of such product candidates, our ability to generate significant revenue would be compromised.

The April 2026 Note Purchase Agreement provides for, among other things, the issuance of up to $155.0 million of senior secured notes, of which the Purchasers have committed, subject to the satisfaction of customary funding conditions and, in certain cases, achievement of certain milestones, to purchase $105.0 million (the first tranche of $35.0 million has been purchased), and the remaining $50.0 million is uncommitted.

The April 2026 Purchase and Conversion Agreement, as amended, provided for the issuance of an aggregate of 1,116,070 shares of our common stock at a price per share equal to the lower of $4.48 or the 30-day volume weighted

average price of our common stock ending on the issuance date of the Purchase Shares. The issuance closed on May 26, 2026, resulting in the issuance of 1,116,070 shares of our common stock for aggregate gross proceeds of $5.0 million.

Under the New ATM Program, we may offer and sell, from time to time, through or to Leerink or Cantor, acting as sales agent or principal, shares of our common stock having an aggregate offering price of up to $125.0 million. We did not sell any shares under the New ATM Program during the three and nine months ended September 30, 2026. As of September 30, 2026, we had sold an aggregate of 10,573,250 shares of our common stock under the ATM Programs since their inception, resulting in gross proceeds of $31.0 million and total issuance costs of $1.5 million.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation, warrants or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through future collaborations, strategic alliances or licensing arrangements with pharmaceutical partners, we may have to relinquish valuable rights to our technologies, future revenue streams or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or through collaborations, strategic alliances or licensing arrangements when needed, we may be required to delay, limit, reduce or terminate our product development, future commercialization efforts, or grant rights to develop and market our product candidates that we would otherwise prefer to develop and market ourselves.

Future Capital Requirements

Pursuant to the Viatris License Agreement, our budgeted research and development expenses related to the development of PS are fully reimbursed by Viatris. The development of LCA5, BEST1 and other internally-developed assets is subject to numerous uncertainties, and we have based these estimates on assumptions that may prove to be substantially different than what we currently anticipate and could result in cash resources being used sooner than what we currently expect. Additionally, the process of advancing early-stage product candidates and testing product candidates in clinical trials is costly, and the timing of progress in these clinical trials is uncertain. Our ability to successfully transition to profitability will be dependent upon achieving a level of product sales adequate to support our cost structure. We cannot give any assurance that we will ever be profitable or generate positive cash flow from operating activities.

Contractual Obligations and Commitments

Except for the Note Purchase Agreement described in Note 7 – Debt and the other matters discussed in Note 4 – Commitments and Contingencies, our contractual obligations have not materially changed from those disclosed in our Annual Report on Form 10-K for the year ended December 31, 2025, filed with the SEC on March 12, 2026.

Other Commitments

In the course of normal operations, we enter into cancelable purchase commitments from time to time with our suppliers for various key research, clinical and manufacturing services. The purchase commitments covered by these arrangements are subject to change based on our research and development efforts.

Other Funding Requirements

As noted above, certain of our cash requirements relate to the funding of our ongoing research and development of our gene therapy product candidates, inclusive of any potential milestone and royalty obligations under our intellectual property licenses. See “Part I, Item 1— Business— Pipeline— Sales and Marketing—Manufacturing—Review and Approval of Drugs and Biologics in the United States— Review and Approval of Drug Products in the European Union” in our Annual Report on Form 10-K for the year ended December 31, 2025, filed with the SEC on March 12, 2026, for a discussion of design, development, pre-clinical and clinical activities that we may conduct in the future, including expected cash expenditures required for some of those activities, to the extent we are able to estimate such costs.

Our other cash requirements within the next 12 months include accounts payable, accrued expenses, purchase commitments, quarterly cash interest obligations associated with the Note Purchase Agreement and other current liabilities. Our other cash requirements greater than 12 months from various contractual obligations and commitments may include future principal repayments and additional obligations under the Note Purchase Agreement, operating leases and contractual agreements with third-party service providers for clinical research, product development, manufacturing,

commercialization, supplies, payroll, equipment maintenance, and audits for periods into calendar year 2027. Refer to Note 4 – Commitments and Contingencies in Part I, Item 1 – "Financial Statements" of this Report for further detail of our license agreements with regard to the timing of expected future payments.

We expect to satisfy our short-term and long-term obligations through cash on hand, from future equity and debt financings, and from reimbursement payments, potential milestone and royalty payments under the Viatris License Agreement and any future collaborations and license agreements, until we generate an adequate level of revenue from commercial sales to cover expenses, if ever.

Critical Accounting Policies and Estimates

Our financial statements are prepared in accordance with U.S. generally accepted accounting principles (GAAP). These accounting principles require us to make estimates and judgments that can affect the reported amounts of assets and liabilities as of the date of the financial statements as well as the reported amounts of revenue and expense during the periods presented. We believe that the estimates and judgments upon which we rely are reasonably based upon information available to us at the time that we make these estimates and judgments. To the extent that there are material differences between these estimates and actual results, our financial results will be affected. The accounting policies that reflect our more significant estimates and judgments and which we believe are the most critical to aid in fully understanding and evaluating our reported financial results are described below.

Our significant accounting policies are discussed in Note 1 — Company Description and Summary of Significant Accounting Policies, included in "Part I, Item 1 – Financial Statements" of this Report. We believe that the following accounting policies and estimates are the most critical to aid in fully understanding and evaluating our reported financial results. These estimates require our most difficult, subjective, or complex judgments because they relate to matters that are inherently uncertain. We have reviewed these critical accounting policies and estimates and related disclosures with the Audit Committee of our Board.

License and Collaborations Revenue

We account for license and collaborations revenue in accordance with the provisions of the Financial Accounting Standards Board Accounting Standards Codification 606, *Revenue from Contracts with Customers*. The guidance provides a unified model to determine how revenue is recognized. We have entered into license and collaboration agreements which have revenue recognition implications. We recognize license and collaborations revenue by first allocating the transaction price of a contract to each performance obligation under the contract based on its standalone selling price. The standalone selling price of each performance obligation is based on its fair value utilizing a discounted cash flow approach, taking into consideration assumptions, including projected worldwide net profit for each of the respective programs based on probability assessments, projections based on internal forecasts, industry data, and information from other guideline companies within the same industry and other relevant factors. We do not expect to have in the future significant variable consideration adjustments related to our existing license and collaborations revenue recognized. For discussion about the determination of license and collaborations revenue, see Note 12 — License and Collaboration Revenue and Other Funding Agreements included in Part I, Item 1 – "Financial Statements" of this Report.

Accrued research and development expense

As part of the process of preparing our condensed consolidated financial statements, we are required to estimate certain accrued research and development expenses. This process involves estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. We make estimates of our accrued expenses as of each balance sheet date in our condensed consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of the estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include those related to fees paid to:

- vendors in connection with discovery and preclinical development activities;
- Contract research organizations ("CROs") in connection with clinical trials, preclinical studies and testing; and
- Contract manufacturing organizations ("CMOs") in connection with the process development and scale up activities and the production of materials.

We base the expense recorded related to contract research and manufacturing on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CROs and CMOs that conduct services and

supply materials. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. While the majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met, some require advance payments. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. Payments we make for research and development services prior to the services being rendered are recorded as prepaid assets in our condensed consolidated balance sheets and are expensed as the services are provided.

Warrant Liabilities

We issued warrants to purchase equity securities in connection with the March 2025 financings that are recorded in the warrant liabilities line item in the accompanying condensed consolidated balance sheets. We account for these warrants as liabilities at fair value when the valuation inputs are not fixed and determinable. We adjust the liabilities for changes in fair value until the earlier of the exercise or expiration of the warrants. Any future change in fair value of the warrant liabilities, when outstanding, is recognized in the condensed consolidated statements of comprehensive loss under the fair value change in instruments measured at fair value line item. For further discussion about the determination of the warrant liabilities, see Note 9 – Financings included in “Part I, Item 1 – Financial Statements" of this Report.

Fair Value of Notes

The fair value of the Notes pursuant to the Note Purchase Agreement represents the present value of estimated future payments, including interest, principal, and Repayment Amount (each as defined in the Note Purchase Agreement). The fair value measurement is based on significant Level 3 unobservable inputs such as the probability and timing of the Repayment Amount and the discount rate. We determined the fair value of the Notes utilizing a discounted cash flow model of estimated future payments including interest, principal, and Repayment Amount utilizing a discount rate calculated as the term matched risk-free rate plus credit spread. Significant increases or decreases in any of these inputs in isolation could result in a significantly lower or higher fair value measurement.

While the assumptions used to calculate and account for the fair value of the Notes represent management’s best estimates, these estimates involve inherent uncertainties and the application of management’s judgment. As a result, if revisions are made to our underlying assumptions and estimates, the fair value of the Notes and consequently the change in fair value of the Notes could vary significantly from period to period.

Income Tax Assets and Liabilities

A full valuation allowance has been provided on our net deferred tax assets given the uncertainty of future taxable income and other related factors impacting the realizability of our remaining net deferred tax assets. For additional information, see Note 12 — Income Taxes included in “Part II, Item 8 – Financial Statements and Supplementary Data” in our Annual Report filed on Form 10-K for the year ended December 31, 2025, and see Note 14 — Income Taxes included in “Part I, Item 1 – Financial Statements” of this Report.

Recent Accounting Pronouncements

Refer to Note 1 — “Company Description and Summary of Significant Accounting Policies” of our condensed consolidated financial statements included in “Part I, Item 1 – Financial Statements” in this Report for a discussion of recently issued accounting pronouncements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Not applicable for smaller reporting companies.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information we are required to disclose in our Exchange Act reports is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

We designed and evaluated our disclosure controls and procedures recognizing that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance and not absolute assurance of achieving the desired control objectives. Also, the design of a control system must reflect the fact that there are resource constraints and that the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud, if any, have been detected. These inherent limitations include the realities that judgments in decision-making can be faulty and that breakdowns can occur because of simple error or mistake. The design of any system of controls is based, in part, upon certain assumptions about the likelihood of future events and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions.

Under the supervision of and with the participation of our management, including our principal executive officer and principal financial officer, we evaluated the effectiveness of our disclosure controls and procedures, as such term is defined in Rules 13a-15(e) and 15d-15(e) promulgated under the Exchange Act as of September 30, 2026. Based on this evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective as of September 30, 2026.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rule 13a-15(f) under the Exchange Act) during the quarter ended September 30, 2026 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any legal proceedings that, in the opinion of our management, are likely to materially affect our business or financial results. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors

There have been no material changes in our risk factors previously disclosed in Part I, Item 1A "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2025. You should carefully consider the risks and uncertainties described therein.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

On July 23, 2026, we issued 200,000 shares of our common stock to the Trustees of the University of Pennsylvania ("Penn") under a stock subscription agreement entered into on July 20, 2026 in connection with agreements with Penn to assign and amend certain existing intellectual property license arrangements and extend certain contractual rights and obligations. The shares were issued as consideration under those agreements rather than for cash, had an aggregate fair value of approximately $0.6 million, and were issued without any underwriter or underwriting discounts or commissions. The shares were issued in reliance on the exemption from registration provided by Section 4(a)(2) of the Securities Act of 1933, as amended, as a transaction not involving a public offering.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable to our Company.

Item 5. Other Information

Trading Plans

On September 10, 2026, Dr. George Magrath, the Company's Chief Executive Officer, terminated the Rule 10b5-1 trading plan previously adopted on June 5, 2026.

On September 22, 2026, Dr. Sally Tucker, the Company's Chief Medical Officer, terminated the Rule 10b5-1 trading plan previously adopted on May 1, 2026 and adopted a new Rule 10b5-1 trading plan (as defined in Rule 10b5-1(c) under the Securities Exchange Act of 1934) providing for the potential sale of up to 191,316 shares of the Company's common stock. Pursuant to the new trading plan, sales of shares may commence on or after October 23, 2026, and are scheduled to terminate on or prior to May 31, 2028, subject to earlier termination in accordance with its terms.

Item 6. Exhibits

EXHIBIT NUMBER	DESCRIPTION OF DOCUMENT
3.1	Restated Certificate of Incorporation of Ocuphire Pharma, Inc., dated as of June 12, 2024 (incorporated by reference to Exhibit 3.1 to the Registrant's Quarterly Report on Form 10-Q, filed on August 13, 2024).
3.2	Certificate of Amendment to the Restated Certificate of Incorporation of the Company, effective as of October 23, 2024 (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 22, 2024).
3.3	Certificate of Amendment to the Restated Certificate of Incorporation of the Company, filed on April 20, 2026 (incorporated by reference to Exhibit 3.2 to the Quarterly Report on Form 10-Q filed on May 12, 2026).
3.4	Amended and Restated Bylaws, dated as of March 19, 2025 (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on March 20, 2025).
31.1**	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2**	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document.
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)

* Documents are furnished and not filed.

** Indicates exhibits that are being filed herewith.

Opus Genetics, Inc.
Form 10-Q

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.

Dated: November 10, 2026

Opus Genetics, Inc.

By: /s/ George Magrath

George Magrath
Chief Executive Officer and Director
(Principal Executive Officer)

By: /s/ Robert Gagnon

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

Exhibit 31.1

CERTIFICATION PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a), AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES OXLEY ACT OF 2002

I, George Magrath, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended September 30, 2026 of Opus Genetics, 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 consolidated 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 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 10, 2026

/s/ George Magrath

Name: George Magrath

Title: Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a), AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES OXLEY ACT OF 2002

I, Robert Gagnon, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarter ended September 30, 2026 of Opus Genetics, 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 consolidated 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 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 10, 2026

/s/ Robert Gagnon

Name: Robert Gagnon
Title: Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002 (Subsections (a) and (b) of Section 1350, Chapter 63 of Title 18, United States Code)

In connection with the Quarterly Report on Form 10-Q for the quarter ended September 30, 2026 (the "Report") of Opus Genetics, Inc., a Delaware corporation (the "Company") as filed with the Securities and Exchange Commission, George Magrath, as Chief Executive Officer of the Company, and Robert Gagnon, as Chief Financial Officer of the Company, each hereby certifies, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to the best of his knowledge and belief:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)); and

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

Date: November 10, 2026

/s/ George Magrath

George Magrath

Chief Executive Officer

(*Principal Executive Officer*)

/s/ Robert Gagnon

Robert Gagnon

Chief Financial Officer

(*Principal Financial Officer and Principal Accounting Officer*)