

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2024

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission File Number: 001-38276

**APELLIS PHARMACEUTICALS, INC.**

(Exact Name of Registrant as Specified in its Charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

100 Fifth Avenue,  
Waltham, MA  
(Address of principal executive offices)

27-1537290  
(I.R.S. Employer  
Identification No.)

02451  
(Zip Code)

Registrant's telephone number, including area code: (617) 977-5700

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	APLS	Nasdaq Global Select Market

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

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

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

Large accelerated filer  Accelerated filer   
Non-accelerated filer  Small reporting company   
Emerging growth company

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

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

As of October 29, 2024, the registrant had 124,393,017 shares of common stock, \$0.0001 par value per share, outstanding.

---

**APELLIS PHARMACEUTICALS, INC.**  
**FORM 10-Q**  
**FOR THE QUARTER ENDED SEPTEMBER 30, 2024**

**TABLE OF CONTENTS**

	<b>Page</b>
<b><u>PART I.</u></b>	
<b><u>FINANCIAL INFORMATION</u></b>	
<u>Item 1.</u>	3
<u>Financial Statements (Unaudited)</u>	3
<u>Condensed Consolidated Balance Sheets as of September 30, 2024 and December 31, 2023</u>	3
<u>Condensed Consolidated Statements of Operations and Comprehensive Loss for the three and nine months ended September 30, 2024 and 2023</u>	4
<u>Condensed Consolidated Statements of Changes in Stockholders' Equity for the three and nine months ended September 30, 2024 and 2023</u>	5
<u>Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2024 and 2023</u>	7
<u>Notes to Unaudited Condensed Consolidated Financial Statements</u>	8
<u>Item 2.</u>	23
<u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	23
<u>Item 3.</u>	37
<u>Quantitative and Qualitative Disclosures About Market Risk</u>	37
<u>Item 4.</u>	37
<u>Controls and Procedures</u>	37
<b><u>PART II.</u></b>	
<b><u>OTHER INFORMATION</u></b>	
<u>Item 1.</u>	39
<u>Legal Proceedings</u>	39
<u>Item 1A.</u>	39
<u>Risk Factors</u>	39
<u>Item 5.</u>	40
<u>Other Information</u>	40
<u>Item 6.</u>	41
<u>Exhibits</u>	41
<u>Signatures</u>	42

## Special Note Regarding Forward-Looking Statements and Industry Data

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, among other things, statements about:

- the ongoing commercialization of EMPAVELI and SYFOVRE;
- our plans with respect to our ongoing and planned clinical trials for our product candidates, whether conducted by us or Swedish Orphan Biovitrum AB (Publ), or Sobi, or by any future collaborators, including the timing of initiation, dosing of patients, enrollment and completion of these trials and of the anticipated results from these trials;
- our sales, marketing and distribution capabilities and strategies, including for the commercialization and manufacturing of EMPAVELI, SYFOVRE and any future products;
- the rate and degree of market acceptance and clinical utility of EMPAVELI, SYFOVRE and any future products for which we receive marketing approval;
- our plans to develop our current and future product candidates for any additional indications;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the potential clinical benefits and attributes of our current and future product candidates we may develop and the inhibition of C3;
- our current and any future collaborations for the development and commercialization of our current and future product candidates;
- the potential benefits of any current or future collaboration, including our collaborations with Sobi and Beam Therapeutics, Inc.;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our ability to identify additional products or product candidates with significant commercial potential;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- developments relating to our competitors and our industry; and
- the impact of new government laws and regulations (including tax).

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, particularly in the “Risk Factors” section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments that we may make or enter into.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed or incorporated by reference as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

This Quarterly Report on Form 10-Q includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. All of the market data used in this Quarterly Report on Form 10-Q involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data. We believe that the information from these industry publications, surveys and studies is reliable. The industry in which we operate is subject to a high

[Table of Contents](#)

degree of uncertainty and risk due to a variety of important factors, including those described in the section titled “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2023 and in this Quarterly Report on Form 10-Q. These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us. The Apellis, EMPAVELI, SYFOVRE and Apellis Assist names and logos are our trademarks, trade names and service marks. The other trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

**Note regarding certain references in this Quarterly Report on Form 10-Q**

Unless otherwise stated or the context indicates otherwise, all references herein to “Apellis,” “Apellis Pharmaceuticals, Inc.,” “we,” “us,” “our,” “our company,” “the Company” and similar references refer to Apellis Pharmaceuticals, Inc. and its wholly owned subsidiaries.

In addition, unless otherwise stated or the context indicates otherwise, all references in this Quarterly Report on Form 10-Q to “EMPAVELI (pegcetacoplan)” and “EMPAVELI” refer to systemic pegcetacoplan in the context of the commercially available product in the United States for the treatment of adults with paroxysmal nocturnal hemoglobinuria, or PNH, and references to Aspaveli refer to pegcetacoplan in the context of the commercially available product in the European Union for the treatment of adults with PNH who are anemic after treatment with a C5 inhibitor for at least three months, in each case, as more fully described herein. Unless otherwise stated or the context indicates otherwise, all references in this Quarterly Report on Form 10-Q to “SYFOVRE (pegcetacoplan injection)” and “SYFOVRE” refer to intravitreal pegcetacoplan in the context of the commercially available product for which we received approval from the United States Food and Drug Administration in February 2023 for the treatment of geographic atrophy secondary to age-related macular degeneration. Unless otherwise stated or the context indicates otherwise, all references herein to “pegcetacoplan” refer to pegcetacoplan in the context of the product candidate for which we are exploring further applications and indications, as more fully described herein. The other trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

**PART I—FINANCIAL INFORMATION**
**Item 1. Financial Statements.**

**APELLIS PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
**(Unaudited)**  
**(Amounts in thousands, except per share amounts)**

	September 30, 2024	December 31, 2023
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 396,864	\$ 351,185
Accounts receivable, net	279,011	206,442
Inventory	121,058	146,362
Prepaid assets	23,809	38,820
Restricted cash	1,373	1,114
Other current assets	11,782	22,408
Total current assets	833,897	766,331
Non-current assets:		
Right-of-use assets	17,060	16,745
Property and equipment, net	3,379	4,345
Long-term inventory	46,080	—
Other assets	1,450	1,309
Total assets	\$ 901,866	\$ 788,730
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 42,730	\$ 37,516
Accrued expenses	139,835	127,806
Current portion of development liability	—	75,830
Current portion of lease liabilities	6,792	6,441
Deferred revenue	1,903	—
Total current liabilities	191,260	247,593
Long-term liabilities:		
Long-term development liability	—	239,817
Long-term credit facility	358,982	—
Convertible senior notes	93,263	93,033
Lease liabilities	11,411	11,454
Other liabilities	9,829	2,312
Total liabilities	664,745	594,209
Commitments and contingencies (Note 13)		
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 10,000 shares authorized, and zero shares issued and outstanding at September 30, 2024 and December 31, 2023	—	—
Common stock, \$0.0001 par value; 200,000 shares authorized at September 30, 2024 and December 31, 2023; 122,069 shares issued and outstanding at September 30, 2024, and 119,556 shares issued and outstanding at December 31, 2023	12	12
Additional paid-in capital	3,239,262	3,035,539
Accumulated other comprehensive loss	(3,140)	(3,542)
Accumulated deficit	(2,999,013)	(2,837,488)
Total stockholders' equity	237,121	194,521
Total liabilities and stockholders' equity	\$ 901,866	\$ 788,730

See accompanying notes to unaudited condensed consolidated financial statements

**APELLIS PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
(Unaudited)  
(Amounts in thousands, except per share amounts)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2024	2023	2024	2023
<b>Revenue:</b>				
Product revenue, net	\$ 176,571	\$ 99,182	\$ 518,782	\$ 227,626
Licensing and other revenue	20,259	11,217	50,057	22,588
Total revenue:	196,830	110,399	568,839	250,214
<b>Operating expenses:</b>				
Cost of sales	33,557	22,410	76,867	38,598
Research and development	88,569	79,421	251,216	285,105
Selling, general and administrative	121,984	145,648	379,571	359,114
Total operating expenses:	244,110	247,479	707,654	682,817
Net operating loss	(47,280)	(137,080)	(138,815)	(432,603)
Loss on extinguishment of development liability	—	—	(1,949)	—
Interest income	2,889	4,989	9,377	16,385
Interest expense	(12,532)	(7,310)	(28,857)	(22,179)
Other income/(expense), net	70	(603)	(405)	(946)
Net loss before taxes	(56,853)	(140,004)	(160,649)	(439,343)
Income tax expense	592	233	876	709
Net loss	\$ (57,445)	\$ (140,237)	\$ (161,525)	\$ (440,052)
<b>Other comprehensive gain/(loss):</b>				
Foreign currency translation	222	(269)	402	(190)
Total other comprehensive income	222	(269)	402	(190)
Comprehensive loss, net of tax	\$ (57,223)	\$ (140,506)	\$ (161,123)	\$ (440,242)
Net loss per common share, basic and diluted	\$ (0.46)	\$ (1.17)	\$ (1.31)	\$ (3.73)
Weighted-average number of common shares used in net loss per common share, basic and diluted	124,234	120,292	123,698	117,827

See accompanying notes to unaudited condensed consolidated financial statements

**APELLIS PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY**  
**(Unaudited)**  
**(Amounts in thousands)**

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
	Outstanding Shares	Amount				
Balance at January 1, 2024	119,556	\$ 12	\$ 3,035,539	\$ (3,542)	\$ (2,837,488)	\$ 194,521
Proceeds from settlement of capped call	—	—	98,763	—	—	98,763
Issuance of common stock upon exercise of stock options	714	—	9,477	—	—	9,477
Vesting of restricted stock units, net of shares withheld for taxes	997	—	(28)	—	—	(28)
Share-based compensation expense	—	—	30,349	—	—	30,349
Net loss	—	—	—	—	(66,423)	(66,423)
Foreign currency translation	—	—	—	17	—	17
Balance at March 31, 2024	121,267	12	3,174,100	(3,525)	(2,903,911)	266,676
Issuance of common stock upon exercise of stock options	233	—	1,962	—	—	1,962
Vesting of restricted stock units, net of shares withheld for taxes	102	—	—	—	—	—
Share-based compensation expense	—	—	29,990	—	—	29,990
Issuance of common stock to employee stock purchase plan	85	—	3,193	—	—	3,193
Net loss	—	—	—	—	(37,657)	(37,657)
Foreign currency translation	—	—	—	163	—	163
Balance at June 30, 2024	121,687	12	3,209,245	(3,362)	(2,941,568)	264,327
Issuance of common stock upon exercise of stock options	112	—	2,556	—	—	2,556
Vesting of restricted stock units, net of shares withheld for taxes	270	—	(14)	—	—	(14)
Share-based compensation expense	—	—	27,475	—	—	27,475
Net loss	—	—	—	—	(57,445)	(57,445)
Foreign currency translation	—	—	—	222	—	222
Balance at September 30, 2024	122,069	\$ 12	\$ 3,239,262	\$ (3,140)	\$ (2,999,013)	\$ 237,121

See accompanying notes to unaudited condensed consolidated financial statements



**APELLIS PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY**  
(Continued from previous page)  
(Unaudited)  
(Amounts in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Outstanding Shares	Amount				
Balance at January 1, 2023	110,772	\$ 11	\$ 2,479,596	\$ (875)	\$ (2,308,860)	\$ 169,872
Issuance of common stock and pre-funded warrants in common stock offering	4,008	1	384,386	—	—	384,387
Issuance of common stock upon exercise of stock options	951	—	17,718	—	—	17,718
Vesting of restricted stock units, net of shares withheld for taxes	448	—	(10,999)	—	—	(10,999)
Share-based compensation expense	—	—	28,823	—	—	28,823
Net loss	—	—	—	—	(177,778)	(177,778)
Foreign currency translation	—	—	—	100	—	100
Balance at March 31, 2023	116,179	12	2,899,524	(775)	(2,486,638)	412,123
Issuance of common stock upon exercise of stock options	1,208	—	22,334	—	—	22,334
Vesting of restricted stock units, net of shares withheld for taxes	119	—	(27)	—	—	(27)
Share-based compensation expense	—	—	29,277	—	—	29,277
Issuance of common stock to employee stock purchase plan	73	—	3,754	—	—	3,754
Net loss	—	—	—	—	(122,037)	(122,037)
Foreign currency translation	—	—	—	(21)	—	(21)
Balance at June 30, 2023	117,579	12	2,954,862	(796)	(2,608,675)	345,403
Issuance of common stock upon exercise of stock options	665	—	5,757	—	—	5,757
Vesting of restricted stock units, net of shares withheld for taxes	115	—	(9)	—	—	(9)
Share-based compensation expense	—	—	21,626	—	—	21,626
Net loss	—	—	—	—	(140,237)	(140,237)
Foreign currency translation	—	—	—	(269)	—	(269)
Balance at September 30, 2023	118,359	12	\$ 2,982,236	\$ (1,065)	\$ (2,748,912)	\$ 232,271

See accompanying notes to unaudited condensed consolidated financial statements

**APELLIS PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**  
(Unaudited)  
(Amounts in thousands)

	For the Nine Months Ended September 30,	
	2024	2023
<b>Operating Activities</b>		
Net loss	\$ (161,525)	\$ (440,052)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation expense	87,814	79,726
Loss on extinguishment of development liability	1,949	—
Loss on disposal of fixed assets	—	152
Depreciation expense	1,350	1,308
Amortization of discounts for credit facility	742	—
Amortization of discounts for convertible notes	230	222
Accretion of discount to development liability	8,936	19,491
Changes in operating assets and liabilities:		
Accounts receivable	(72,569)	(161,542)
Inventory	(20,776)	(12,831)
Prepaid assets	15,022	(7,681)
Other current assets	10,651	5,981
Other assets	(81)	14,811
Right-of-use assets and lease liabilities	(7)	(94)
Accounts payable	(399)	(19,450)
Accrued expenses and other liabilities	19,534	23,099
Deferred revenue	1,903	—
Net cash used in operating activities	<u>(107,226)</u>	<u>(496,860)</u>
<b>Investing Activities</b>		
Purchase of property and equipment	(383)	(678)
Net cash used in investing activities	<u>(383)</u>	<u>(678)</u>
<b>Financing Activities</b>		
Proceeds from credit facility	365,454	—
Payment of issuance cost for credit facility	(1,589)	—
Repayment of development liability	(326,533)	—
Proceeds from settlement of capped call	98,763	—
Proceeds from issuance of common stock and pre-funded warrant offering, net of issuance costs	—	384,387
Payments for development liability	—	(24,500)
Proceeds from exercise of stock options	13,995	45,809
Proceeds from issuance of common stock under employee share purchase plan	3,193	3,754
Payments of employee tax withholding related to equity-based compensation	(42)	(11,038)
Net cash provided by financing activities	<u>153,241</u>	<u>398,412</u>
Effect of exchange rate changes on cash, cash equivalents and restricted cash	306	(449)
Net increase (decrease) in cash, cash equivalents and restricted cash	45,938	(99,575)
Cash, cash equivalents and restricted cash at beginning of period	352,299	553,075
Cash, cash equivalents and restricted cash at end of period	<u>\$ 398,237</u>	<u>\$ 453,500</u>
<b>Reconciliation of cash, cash equivalents and restricted cash to the consolidated balance sheets:</b>		
Cash and cash equivalents	\$ 396,864	\$ 452,414
Restricted cash	1,373	1,086
Total cash, cash equivalents, and restricted cash	<u>\$ 398,237</u>	<u>\$ 453,500</u>
<b>Supplemental Disclosures</b>		
Cash paid for interest	\$ 19,446	\$ 3,286
Cash paid for income taxes	\$ 600	\$ 578
Issuance costs for credit facility incurred but not yet paid	\$ 5,625	\$ —

See accompanying notes to unaudited condensed consolidated financial statements

**APELLIS PHARMACEUTICALS, INC.**  
**NOTES TO UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**  
**(unaudited)**

**1. Nature of Organization and Operations**

Apellis Pharmaceuticals, Inc. (the “Company”) is a commercial-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutic compounds to treat diseases with high unmet needs through the inhibition of the complement system, which is an integral component of the immune system, at the level of C3, the central protein in the complement cascade.

The Company was incorporated in September 2009 under the laws of the State of Delaware. The Company’s principal executive offices are located in Waltham, Massachusetts.

The Company’s operations since inception have been limited to organizing and staffing the Company, acquiring rights to product candidates, business planning, raising capital, developing its product candidates, commercializing EMPAVELI (pegcetacoplan) for the treatment of paroxysmal nocturnal hemoglobinuria (“PNH”) and SYFOVRE (pegcetacoplan injection) for the treatment of geographic atrophy secondary to age-related macular degeneration (“GA”).

The Company is subject to risks common in the biotechnology industry including, but not limited to, raising additional capital, development by its competitors of new technological innovations, its ability to successfully complete preclinical and clinical development of product candidates and receive timely regulatory approval of products, market acceptance of the Company’s products, protection of proprietary technology, healthcare cost containment initiatives, and compliance with governmental regulations, including those of the U.S. Food and Drug Administration (“FDA”).

***Liquidity and Going Concern***

The accompanying unaudited condensed consolidated financial statements have been prepared on the basis of the realization of assets and the satisfaction of liabilities and commitments in the normal course of business. From inception to September 30, 2024, the Company has incurred cash outflows from operations, losses from operations and had an accumulated deficit of \$3.0 billion primarily as a result of expenses incurred through a combination of research and development activities related to the Company’s various product candidates and expenses supporting those activities.

As of November 5, 2024, the date of issuance of these unaudited condensed consolidated financial statements, the Company believes that its cash and cash equivalents of \$396.9 million as of September 30, 2024, combined with cash anticipated to be generated from sales of EMPAVELI and SYFOVRE will be sufficient to fund its operations and capital expenditures for at least the next twelve months.

**2. Basis of Presentation and Summary of Significant Accounting Policies**

***Basis of Presentation***

The accompanying unaudited condensed consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles (“U.S. GAAP”) and following the requirements of the Securities and Exchange Commission (the “SEC”) for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by U.S. GAAP have been condensed or omitted and, accordingly, the consolidated balance sheet as of December 31, 2023 has been derived from audited consolidated financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements. These financial statements have been prepared on the same basis as the Company’s annual financial statements and, in the opinion of management, reflect all adjustments (consisting only of normal recurring adjustments) that are necessary for a fair presentation of the Company’s financial information. The results of operations for the three and nine months ended September 30, 2024 are not necessarily indicative of the results to be expected for the year ending December 31, 2024 or for any other interim period or for any other future year.

The accompanying unaudited condensed consolidated financial statements and related financial information should be read in conjunction with the audited consolidated financial statements and the related notes thereto for the year ended December 31, 2023 included in the Company’s Annual Report on Form 10-K filed with the SEC on February 27, 2024, as amended by Amendment No. 1 thereto filed with the SEC on February 29, 2024 (the “2023 Form 10-K”).

[Table of Contents](#)

***Use of Estimates***

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities as of the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results may differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes, and management must select an amount that falls within that range of reasonable estimates. Estimates are used in the following areas, among others: development liability, accrued expenses, prepaid expenses, convertible debt, reserves for variable consideration, reserves for excess or obsolete inventories, and income taxes.

***Summary of Significant Accounting Policies***

Reference is made to Note 2 Summary of Significant Accounting Policies in our 2023 Form 10-K for a detailed description of significant accounting policies. There have been no significant changes to our accounting policies as disclosed in our 2023 Form 10-K, except as noted below.

**Accounts Receivable**

The Company's accounts receivable primarily arise from product sales. They are generally stated at the invoiced amount and do not bear interest. The accounts receivable from product sales represents receivables due from the Company's SPs or SDs. The Company has had no historical write offs of its accounts receivable as of September 30, 2024 and December 31, 2023, and its payment terms are generally 30-65 days for EMPAVELI and 60-150 days for SYFOVRE. The Company monitors the financial performance and creditworthiness of its customers and provides reserves against trade receivables for expected credit losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. As of September 30, 2024 and December 31, 2023, the credit profiles for the Company's customers were deemed to be in good standing and an allowance for credit losses was not considered necessary.

The Company has an agreement (the "Factoring Agreement") to sell certain trade accounts receivable to a third-party financial institution at a discount to the invoiced amount. Under the Factoring Agreement, the maximum amount of outstanding accounts receivables sold at any time is \$100.0 million. The Company accounts for the transfer of trade accounts receivable under the Factoring Agreement as a sale in accordance with ASC 860, *Transfers and Servicing*, because effective control and risk associated with the transferred accounts receivable is passed to the third-party. Accordingly, the Company derecognizes the sold trade accounts receivable from the consolidated balance sheets. Cash proceeds related to the accounts receivable sold are included in cash from operating activities in the consolidated statements of cash flows. Any discounts or fees incurred in connection with the sales are recorded within "Selling, general and administrative expenses" in the consolidated statements of operations and comprehensive loss. Pursuant to the Factoring Agreement, the Company performs certain collection and administrative functions for the receivable sold. The fair value of these administrative services is not material and therefore, the Company has not recorded any servicing assets or liabilities associated with the Factoring Agreement. See Note 3 for additional information.

**Inventory**

Inventory is recorded at the lower of cost or net realizable value, with cost determined on a first-in, first-out basis. Inventory costs include third-party contract manufacturing, third-party packaging services, and freight. The Company performs an assessment of the recoverability of capitalized inventory during each reporting period, and it writes down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within cost of sales. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required which would be recorded as a cost of sales in the consolidated statements of operations and comprehensive loss.

Inventory not expected to be sold within the Company's normal operating cycle is classified as long-term inventory on the condensed consolidated balance sheet.

Prior to regulatory approval of its product candidates, the Company expenses costs associated with the manufacturing of its product candidates to research and development expense unless the Company is reasonably certain such costs will have future commercial use and net realizable value. When the Company believes regulatory approval and subsequent commercialization of its product candidates is probable, and the Company also expects future economic benefit from the sales of the product candidates to be realized, the Company

[Table of Contents](#)

will then capitalize the costs of production as inventory. Inventory that can be used in either the production of clinical or commercial product is expensed as research and development expense when selected for use in a clinical manufacturing campaign.

Prior to receiving FDA approval for EMPAVELI on May 14, 2021, the Company included in research and development expense the costs associated with the manufacture of EMPAVELI inventory to be sold upon commercialization. As a result, the manufacturing costs related to the EMPAVELI inventory build-up incurred before FDA approval were expensed in a prior period and are, therefore, excluded from the cost of goods sold and inventory. As of September 30, 2024 and December 31, 2023, the remaining pre-FDA approved inventory was \$15.4 million and \$19.4 million, respectively, which primarily consisted of raw materials.

Shipping and handling costs for product shipments are recorded as incurred in cost of sales along with costs associated with manufacturing the product and any inventory write-downs.

### **Recently Issued Accounting Standards**

In December 2023, the Financial Accounting Standards Board (“FASB”) issued an amendment to the accounting guidance on income taxes which requires entities to provide additional information in the rate reconciliation and additional disaggregated disclosures about income taxes paid. This guidance requires public entities to disclose in their rate reconciliation table additional categories of information about federal, state, and foreign income taxes and to provide more details about the reconciling items in some categories if the items meet a quantitative threshold. The guidance is effective for annual periods beginning after December 15, 2024. The Company is currently evaluating this guidance to determine the impact it may have on its consolidated financial statements and disclosures.

In November 2023, the FASB issued an amendment to the accounting guidance on segment reporting. The amendments require disclosure of significant segment expenses and other segment items and requires entities to provide in interim periods all disclosures about a reportable segment's profit or loss and assets that are currently required annually. The amendment also requires disclosure of the title and position of the chief operating decision maker (“CODM”) and an explanation of how the CODM uses the reported measure(s) of segment profit or loss in assessing segment performance and deciding how to allocate resources. The guidance is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024. Retrospective application is required, and early adoption is permitted. The Company is currently evaluating the impact the guidance will have on its consolidated financial statements and disclosures.

### **3. Product Revenues, Accounts Receivable, and Reserves for Product Sales**

The Company received FDA approval for the sale of EMPAVELI in the United States in May 2021 and approval for the sale of SYFOVRE in the United States in February 2023. The Company's product revenues consist of sales of EMPAVELI and SYFOVRE to specialty pharmacies and specialty distributors.

The table reflects product revenue by major source for the following periods (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Products:				
EMPAVELI	\$ 24,611	\$ 23,901	\$ 74,733	\$ 66,643
SYFOVRE	151,960	75,281	444,049	160,983
Total Product revenue, net	<u>\$ 176,571</u>	<u>\$ 99,182</u>	<u>\$ 518,782</u>	<u>\$ 227,626</u>

The Company's accounts receivable balance of \$279.0 million as of September 30, 2024 and \$206.4 million as of December 31, 2023, consisted of EMPAVELI and SYFOVRE product sales receivable and licensing and other revenue receivables from our collaboration with Swedish Orphan Biovitrum AB (“Sobi”). The Company does not have a reserve related to expected credit losses against its receivable balance and expects to collect its accounts receivable in the ordinary course of business.

The Company's product revenue allowance and reserves totaled \$32.9 million and \$16.6 million as of September 30, 2024 and December 31, 2023, respectively. These amounts are included in accrued expenses on the Company's unaudited condensed consolidated balance sheets.

The following table summarizes activity in each of the product revenue allowance and reserve categories for the three and nine months ended September 30, 2024 (in thousands):

[Table of Contents](#)

	Chargebacks, Discounts, and Fees	Government and Other Rebates	Returns	Total
Ending balance at December 31, 2023	\$ 5,674	\$ 8,898	\$ 2,053	\$ 16,625
Provision related to sales in the current year	9,575	13,125	1,355	24,055
Adjustments related to prior period sales	146	(19)	(96)	31
Credits and payments made	(9,724)	(9,906)	(1,859)	(21,489)
Ending balance at March 31, 2024	\$ 5,671	\$ 12,098	\$ 1,453	\$ 19,222
Provision related to sales in the current year	10,025	17,777	1,039	28,841
Adjustments related to prior period sales	(131)	354	(983)	(760)
Credits and payments made	(9,635)	(14,640)	(326)	(24,601)
Ending balance at June 30, 2024	\$ 5,930	\$ 15,589	\$ 1,183	\$ 22,702
Provision related to sales in the current year	11,455	25,364	1,380	38,199
Adjustments related to prior period sales	2,332	420	—	2,752
Credits and payments made	(12,078)	(17,695)	(979)	(30,752)
Ending balance at September 30, 2024	\$ 7,639	\$ 23,678	\$ 1,584	\$ 32,901

The following table summarizes activity in each of the product revenue allowance and reserve categories for the three and nine months ended September 30, 2023 (in thousands):

	Chargebacks, Discounts, and Fees	Government and other rebates	Returns	Total
Ending balance at December 31, 2022	\$ 164	\$ 1,936	\$ 251	\$ 2,351
Provision related to sales in the current year	1,466	2,566	651	4,683
Adjustments related to prior period sales	—	(2)	(249)	(251)
Credits and payments made	(184)	(1,639)	—	(1,823)
Ending balance at March 31, 2023	\$ 1,446	\$ 2,861	\$ 653	\$ 4,960
Provision related to sales in the current year	3,944	5,114	1,588	10,646
Adjustments related to prior period sales	(84)	30	(416)	(470)
Credits and payments made	(1,668)	(2,946)	(141)	(4,755)
Ending balance at June 30, 2023	\$ 3,638	\$ 5,059	\$ 1,684	\$ 10,381
Provision related to sales in the current year	4,698	7,169	1,384	13,251
Adjustments related to prior period sales	39	(964)	(1,685)	(2,610)
Credits and payments made	(4,960)	(5,778)	—	(10,738)
Ending balance at September 30, 2023	\$ 3,415	\$ 5,486	\$ 1,383	\$ 10,284

**Significant customers** - EMPAVELI and SYFOVRE are sold principally through arrangements with specialty pharmacies and specialty distributors, who are the Company's customers. Gross product revenues and product sales receivable from the Company's customers who individually accounted for 10% or more of total gross product revenues and/or 10% or more of total product sales accounts receivable consisted of the following:

	Percent of Total Gross Product Revenues			
	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Customer A	12%	24%	13%	29%
Customer C	17%	16%	18%	14%
Customer D	61%	56%	59%	52%

  

	Percent of Product Sales Receivable	
	As of September 30,	
	2024	2023
Customer A	3%	7%
Customer C	24%	19%
Customer D	61%	68%

Factoring of accounts receivable and associated fees as of September 30, 2024 and December 31, 2023 were as follows (in thousands):

[Table of Contents](#)

	<u>September 30,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
Accounts receivable sold	\$ 56,590	\$ —
Less: factoring fees	(672)	—
Net cash proceeds	<u>\$ 55,918</u>	<u>\$ —</u>

#### 4. Inventory

The Company's inventory consisted of the following as of September 30, 2024 and December 31, 2023 (in thousands):

	<u>September 30,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
Raw materials	\$ 46,639	\$ 32,724
Semi-finished goods	113,708	82,924
Finished goods	6,791	30,714
Total inventory	<u>\$ 167,138</u>	<u>\$ 146,362</u>

The Company's long-term inventory balance consists of raw materials that are not expected to be sold within the Company's normal operating cycle.

Inventory amounts written down as a result of excess, obsolete, unmarketability or other reasons are charged to cost of sales. The Company's reserve for excess and obsolete inventory was \$14.1 million and \$9.3 million as of September 30, 2024 and December 31, 2023, respectively.

#### 5. Prepaid and Other Current Assets

Prepaid and other current assets consisted of the following as of September 30, 2024 and December 31, 2023 (in thousands):

	<u>September 30,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
Down payments for inventory	\$ 8,251	\$ 16,296
Prepaid research and development	6,582	13,931
Other prepaid expenses	8,976	8,593
Total prepaid assets	<u>\$ 23,809</u>	<u>\$ 38,820</u>

  

	<u>September 30,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
Royalties receivable	\$ 4,958	\$ 3,054
Receivable from collaboration agreement (1)	—	15,000
Deposits and other current assets	6,824	4,354
Total other current assets	<u>\$ 11,782</u>	<u>\$ 22,408</u>

(1) In January 2024, the Company waived the remaining reimbursement payment of \$15.0 million from Sobi in connection with the decision to discontinue the cold agglutinin disease ("CAD") program.

#### 6. Development Liability

In 2019, the Company entered into a development funding agreement (as amended, the "SFJ agreement") with SFJ Pharmaceuticals Group ("SFJ"), under which SFJ agreed to provide funding to the Company to support the development of pegcetacoplan for the treatment of patients with PNH. Under the SFJ agreement, SFJ paid the Company an aggregate of \$140.0 million between June 2019 and January 2020.

Under the SFJ agreement, the Company granted a security interest to SFJ in all of its assets, excluding intellectual property and license agreements to which it is a party. In connection with the grant of the security interest, the Company agreed to certain affirmative and negative covenants, including restrictions on its ability to pay dividends, incur additional debt or enter into licensing transactions with respect to its intellectual property, other than specified types of licenses.

[Table of Contents](#)

Following regulatory approval for the use of systemic pegcetacoplan as a treatment for PNH by the FDA in May 2021 and by the European Medicines Agency in December 2021, the Company became obligated to pay SFJ an aggregate of \$460.0 million in payments over the period from the time of each regulatory approval until the sixth anniversary of each regulatory approval. The Company paid SFJ a total of \$94.0 million through March 31, 2024.

From December 15, 2021 to the final annual payment due in December 2027, the development liability was accreted from its initial carrying amount to the total payment amount using the effective interest rate method over the remaining life of the SFJ agreement. The difference between the carrying amount and the total payment amount is presented as a discount to the development liability. The accretion is recorded as interest expense in the unaudited condensed consolidated statement of operations.

In May 2024, the Company paid its remaining obligations under the SFJ agreement in full with \$326.5 million of proceeds from the Sixth Street Financing Agreement (as defined below) (see Note 8). Upon such payment, SFJ released its security interest in the Company's assets at that time. The Company concluded that the development liability was extinguished as of the payoff date, and the difference of \$1.9 million between the reacquisition price of \$326.5 million and the net carrying value of the development liability of \$324.6 million was recorded as a loss on the extinguishment of the development liability in the unaudited condensed consolidated statement of operations for the nine month period ended September 30, 2024.

The following table summarizes the development liability (in thousands):

	September 30, 2024	December 31, 2023	Effective Interest Rate
Development liability	\$ —	\$ 366,000	7.91%
Less: Unamortized discount to development liability	—	(50,353)	
Less: Current portion of development liability, net of discount	—	(75,830)	
Total long-term development liability	<u>\$ —</u>	<u>\$ 239,817</u>	

For the three months ended September 30, 2024 the Company did not record interest expense for the accretion of the development liability. For the nine months ended September 30, 2024, interest expense of \$8.9 million was recorded for the accretion of the development liability.

For the three and nine months ended September 30, 2023, interest expense of \$6.4 million and \$19.5 million, respectively, was recorded for the accretion of the development liability.

## 7. Accrued Expenses

Accrued expenses consisted of the following as of September 30, 2024 and December 31, 2023 (in thousands):

	September 30, 2024	December 31, 2023
Accrued research and development	\$ 24,509	\$ 28,318
Accrued royalties	6,676	10,197
Accrued payroll liabilities	34,722	51,781
Accrued goods received not invoiced	19,623	5,902
Product revenue reserves	32,901	16,625
Other	21,404	14,983
Total	<u>\$ 139,835</u>	<u>\$ 127,806</u>

## 8. Long-term Debt

### Convertible Senior Notes

On September 16, 2019, the Company completed a private offering of convertible notes (the "2019 Convertible Notes") with an aggregate principal amount of \$220.0 million issued pursuant to an indenture (the "Indenture") with U.S. Bank National Association, as trustee.



## [Table of Contents](#)

The net proceeds from the sale of the 2019 Convertible Notes were approximately \$212.9 million after deducting the initial purchasers' discounts and commissions of \$6.6 million and offering expenses of \$0.5 million. The Company used \$28.4 million of the net proceeds to pay the cost of the capped call transactions in September 2019 described below.

On May 12, 2020, the Company issued convertible notes (the "2020 Convertible Notes") with an aggregate principal amount of \$300.0 million. The net proceeds from the sale of the 2020 Convertible Notes were approximately \$322.9 million after deducting the purchasers' discounts and commission of \$5.7 million and offering expenses of \$0.3 million. The Company used \$43.1 million of the net proceeds to pay the cost of the additional capped call transactions in May 2020 described below.

The 2019 Convertible Notes and the 2020 Convertible Notes are referred to together as the Convertible Notes. The Convertible Notes are senior unsecured obligations of the Company and bear interest at a rate of 3.5% per year payable semiannually in arrears on March 15 and September 15 of each year, beginning on March 15, 2020. The Convertible Notes will mature on September 15, 2026, unless converted earlier, redeemed or repurchased in accordance with their terms.

The Convertible Notes are convertible into shares of the Company's common stock at an initial conversion rate of 25.3405 shares per \$1,000 principal amount of Convertible Notes (equivalent to an initial conversion price of approximately \$39.4625 per share of common stock). The conversion rate is subject to customary anti-dilution adjustments. In addition, following certain events that occur prior to the maturity date or if the Company delivers a notice of redemption, the Company will increase the conversion rate for a holder who elects to convert its Convertible Notes in connection with such corporate event or a notice of redemption, as the case may be, in certain circumstances as provided in the Indenture.

Prior to March 15, 2026, the Convertible Notes are convertible only under the following circumstances:

- during any calendar quarter, if the last reported sale price of the Company's common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day;
- during the five business day period after any five consecutive trading day period in which the trading price per \$1,000 principal amount of the Convertible Notes for each such trading day was less than 98% of the product of the last reported sale price of the Company's common stock and the conversion rate on each such trading day;
- if the Company calls any or all of the Convertible Notes for redemption, at any time prior to the close of business on the second scheduled trading day immediately preceding the redemption date; or
- upon the occurrence of corporate events specified in the Indenture.

On or after March 15, 2026 until the close of business on the second scheduled trading day immediately preceding the maturity date of the Convertible Notes, holders may convert the Convertible Notes at any time regardless of the foregoing circumstances. Upon conversion of the Convertible Notes, the Company will pay or deliver, as the case may be, cash, shares of the Company's common stock or a combination of cash and shares of common stock, at the Company's election.

On or after September 20, 2023, the Company may redeem for cash all or a portion of the Convertible Notes, at its option, if the last reported sale price of the Company's common stock has been at least 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive), including the trading day immediately preceding the date on which the Company provides a notice of redemption, during any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption. The redemption price will be equal to 100% of the principal amount of the Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. If the Company calls any Convertible Notes for redemption, it will constitute a "make-whole fundamental change" with respect to such Convertible Notes, in which case the conversion rate applicable to the conversion of such Notes, if converted in connection with the redemption, will be increased in certain circumstances. The Company has not called for redemption or redeemed any of the Convertible Notes as of September 30, 2024.

If the Company undergoes a "fundamental change," as defined in the Indenture, prior to maturity, subject to certain conditions, holders may require the Company to repurchase for cash all or any portion of their Convertible Notes at a fundamental change repurchase price equal to 100% of the principal amount of the notes to be repurchased, plus any accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

In January 2021, July 2021 and July 2022, the Company entered into separate, privately negotiated exchange agreements to modify the conversion terms with certain holders of its 2019 Convertible Notes and 2020 Convertible Notes. Under the terms of these exchange agreements, in January 2021, July 2021 and July 2022, the holders exchanged approximately \$126.1 million of 2019 Convertible Notes, \$201.1 million of 2019 Convertible Notes and 2020 Convertible Notes, and \$98.1 million of 2020 Convertible Notes, respectively, in

[Table of Contents](#)

aggregate principal amount held by them for an aggregate of 3,906,869 shares, 5,992,217 shares and 3,027,018 shares, respectively, of common stock issued by the Company. In accordance with FASB ASC Topic 470-20, “*Debt – Debt with Conversion and Other Options*,” (“ASC 470-20”) the Company accounted for the exchange as an induced conversion based on the short period of time the conversion offer was open and the substantive conversion feature offer. The Company accounted for the conversion of the debt as an inducement by expensing the fair value of the shares that were issued in excess of the original terms of the Convertible Notes.

The conditional conversion feature of the Convertible Notes was triggered as of December 31, 2023, and as a result the Convertible Notes were convertible at the option of the holders until March 31, 2024. No Convertible Notes were converted during this period.

The conditional conversion feature of the Convertible Notes was triggered as of March 31, 2024, and as a result the Convertible Notes were convertible at the option of the holders until June 30, 2024. No Convertible Notes were converted during this period.

The conditional conversion feature of the Convertible Notes was not triggered as of June 30, 2024, and as a result the Convertible Notes are not convertible during the quarter ending September 30, 2024.

The conditional conversion feature of the Convertible Notes was not triggered as of September 30, 2024, and as a result the Convertible Notes are not convertible during the quarter ending December 31, 2024.

As of September 30, 2024, the Company held in treasury Convertible Notes in principal amount of \$425.4 million which notes had not been cancelled.

The outstanding balance of the Convertible Notes as of September 30, 2024 and December 31, 2023 consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
<b>Liability</b>		
Principal	\$ 93,897	\$ 93,897
Less: debt discount and issuance costs, net	(634)	(864)
Net carrying amount	\$ 93,263	\$ 93,033

The following table sets forth total interest expense recognized related to the Convertible Notes during the three and nine months ended September 30, 2024 and 2023 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Amortization of debt issuance costs	\$ 78	\$ 75	\$ 230	\$ 222
Contractual interest expense	822	822	2,465	2,465
Total interest expense	\$ 900	\$ 897	\$ 2,695	\$ 2,687

### ***Capped Call Transactions***

On September 11, 2019 and May 6, 2020, concurrently with the pricings of the Convertible Notes, the Company entered into capped call transactions with two counterparties. The capped call transactions are expected generally to reduce the potential dilution to the Company’s common stock upon any conversion of Convertible Notes and/or offset any cash payments the Company is required to make in excess of the principal amount of converted Convertible Notes, as the case may be, in the event that the market price per share of the Company’s common stock, as measured under the terms of the capped call transactions, is greater than the strike price of the capped call transactions, which is initially \$39.4625 (the conversion price of the Convertible Notes) and is subject to anti-dilution adjustments substantially similar to those applicable to the conversion rate of such Convertible Notes. If, however, the market price per share of the Company’s common stock, as measured under the terms of the capped call transactions, exceeds the cap price of the capped call transactions, which is initially \$63.14 per share, there would nevertheless be dilution and/or there would not be an offset of such potential cash payments, in each case, to the extent that such market price exceeds the cap price of the capped call transactions.

On February 27, 2024, the Company unwound a portion of the capped call transactions with the capped call counterparties, which resulted in cash proceeds to the Company of \$98.8 million. The unwind transactions were settled at a volume-weighted average price per share of \$64.11 on March 8, 2024.

As of September 30, 2024, the Company holds remaining capped call transactions in a notional amount corresponding to \$93.9 million principal amount of Convertible Notes.

[Table of Contents](#)**Financing Agreement and Credit Facility**

On May 13, 2024, the Company and certain of its subsidiaries entered into a financing agreement (the “Sixth Street Financing Agreement”) with the lenders party thereto (the “Lenders”), and Sixth Street Lending Partners (“Sixth Street”), as the administrative agent and collateral agent for the Lenders.

The Sixth Street Financing Agreement provides for a senior secured term loan facility of up to \$475.0 million (the “Credit Facility”), consisting of an initial draw of \$375.0 million at closing and a potential additional \$100.0 million draw at the Company’s option upon satisfaction of a \$50.0 million minimum cash requirement and a requirement that the Company’s trailing three-month sales of SYFOVRE is at least \$180.0 million prior to the \$100.0 million draw. The Company can exercise the option for the \$100.0 million draw through September 30, 2025, assuming such requirements are met.

The Credit Facility matures on May 13, 2030 (the “Maturity Date”) and bears interest at (i) in the case of SOFR Loans, an annual rate equal to 3-month Term SOFR (subject to 1.00% floor), plus 5.75%, and (ii) in the case of Base Rate Loans, an annual rate equal to the base rate as defined in the agreement (subject to 2.00% floor), plus 4.75%. Certain additional commitment and undrawn amount fees are also payable in connection with the Credit Facility.

The net proceeds from the initial draw of the Credit Facility were approximately \$358.2 million, net of \$16.8 million of issuance costs. The Company used \$326.5 million of the proceeds from the initial draw of the Credit Facility to buy out its remaining obligations to SFJ. The buyout of the SFJ development liability eliminated \$366.0 million in payments to SFJ between 2024 and 2027, including approximately \$200.0 million payable through 2025 (See Note 6).

The Credit Facility does not provide for scheduled amortization payments during the term. All principal will be due on the Maturity Date. The Company will have the right to prepay loans under the Credit Facility at any time. The Company is required to repay loans under the Credit Facility with proceeds from certain asset sales, condemnation events and extraordinary receipts, subject, in some cases, to reinvestment rights. Repayments are subject to a prepayment premium. Repayments may be made after the first year of the loan and are subject to a prepayment premium up to 3% depending on timing.

All obligations under the Sixth Street Financing Agreement are secured on a first-priority basis, subject to certain exceptions, by security interests in substantially all assets of the Company and certain subsidiaries of the Company, including its intellectual property, and are guaranteed by certain subsidiaries of the Company, including foreign subsidiaries, subject to certain exceptions.

The Sixth Street Financing Agreement contains customary covenants, including, without limitation, a financial covenant to maintain liquidity of at least \$50.0 million if the Company’s market capitalization is below \$3.0 billion, and negative covenants that, subject to certain exceptions, restrict indebtedness, liens, investments (including acquisitions), fundamental changes, asset sales and licensing transactions, dividends, modifications to material agreements, payment of subordinated indebtedness, and other matters customarily restricted in such agreements. Among other permissions, the Company is permitted, on terms and conditions set forth on the Sixth Street Financing Agreement, to enter into a separate asset-based financing arrangement with a third party in an amount of up to \$100.0 million, which amount is increased to \$200.0 million upon certain sales or market capitalization thresholds, and to have outstanding convertible unsecured notes in an amount equal to the greater of \$400.0 million and 10% of the Company’s market capitalization, but not to exceed \$600.0 million. The Company is subject to restrictions on sales and licensing transactions with respect to its core intellectual property, defined to include SYFOVRE, EMPAVELI, and other pegcetacoplan product assets, subject to certain exceptions, including certain transactions related to areas outside the United States and Europe.

The outstanding balance of the Credit Facility as of September 30, 2024 and December 31, 2023 consisted of the following (in thousands):

	<u>September 30,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
<b>Liability</b>		
Principal	\$ 375,000	\$ —
Less: debt discount and issuance costs	(16,018)	—
Net carrying amount	<u>\$ 358,982</u>	<u>\$ —</u>

The following table sets forth total interest expense recognized related to the Credit Facility during the three and nine months ended September 30, 2024 and 2023 (in thousands):

[Table of Contents](#)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Amortization of debt issuance costs	\$ 581	\$ —	\$ 742	\$ —
Contractual interest expense	10,854	—	16,160	—
Total interest expense	\$ 11,435	\$ —	\$ 16,902	\$ —

## 9. Leases

The underlying assets of the Company's leases primarily relate to office space leases, but also include some equipment leases. The Company determines if an arrangement qualifies as a lease at its inception.

As of September 30, 2024 and December 31, 2023, all leases were classified as operating leases. Additional information related to the operating lease assets and liabilities is as follows (in thousands):

	September 30, 2024	December 31, 2023
Right-of-use assets	\$ 17,060	\$ 16,745
Operating lease liabilities	\$ 18,203	\$ 17,895
Weighted average remaining term in years	2.84	2.83
Weighted average discount rate used to measure outstanding lease liabilities	6.30%	7.20%

For the three months ended September 30, 2024 and 2023, the total lease cost for operating lease expense was \$1.7 million and \$1.8 million, respectively. For the nine months ended September 30, 2024 and 2023, the total lease cost for operating lease expense was \$5.6 million and \$5.2 million, respectively.

Supplemental cash flow information related to operating leases for the nine months ended September 30, 2024 and 2023 is as follows (in thousands):

	2024	2023
Operating cash flows from operating leases	\$ 5,992	\$ 6,186

The maturities of the Company's operating lease liabilities as of September 30, 2024 are as follows (in thousands):

2024	\$ 1,966
2025	7,421
2026	6,763
2027	2,064
2028 and thereafter	1,485
Total future minimum lease payments	19,699
Less imputed interest	(1,496)
Total operating lease liabilities	\$ 18,203

## 10. Fair Value Measurements

The Company is required to disclose information on the fair value of financial instruments and inputs that enable an assessment of the fair value. The three levels of the fair value hierarchy prioritize valuation inputs based upon the observable nature of those inputs as follows:

Level 1 – Quoted prices in active markets for identical assets or liabilities;

Level 2 – Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly;

Level 3 – Unobservable inputs that reflect the Company’s own assumptions about the assumptions market participants would use in pricing the asset or liability.

The following table presents the fair value of financial instruments recorded originally at amortized cost or fair value and not remeasured on a recurring basis (in thousands):

		September 30, 2024			
Balance Sheet Classification	Type of Instrument	Level 1	Level 2	Level 3	Total
Financial Assets:					
Cash and cash equivalents:	Money market funds	\$ 202,967	\$ —	\$ —	\$ 202,967
Total Financial Assets		<u>\$ 202,967</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 202,967</u>
		December 31, 2023			
Balance Sheet Classification	Type of Instrument	Level 1	Level 2	Level 3	Total
Financial Assets:					
Cash and cash equivalents	Money market funds	\$ 276,391	\$ —	\$ —	\$ 276,391
Total Financial Assets		<u>\$ 276,391</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 276,391</u>

The Company’s Convertible Notes and development liability are financial instruments that are reported in the financial statements at historical cost. The Convertible Notes are Level 1 within the fair value level hierarchy as of September 30, 2024 and December 31, 2023. The fair value of the Convertible Notes was \$103.6 million as of September 30, 2024 and \$140.8 million as of December 31, 2023. The Convertible Notes accrue a semi-annual coupon at an annual rate of 3.5%, which was included in accrued expenses in the consolidated balance sheets as of September 30, 2024 and December 31, 2023.

The fair value of the development liability was \$306.9 million as of December 31, 2023. The development liability is Level 2 within the fair value hierarchy based on the discounting of fixed cash flows using an observed bond yield for borrowers with similar credit rating. Because the development liability was paid in full in May 2024, no amount is included as of September 30, 2024.

## 11. Income Taxes

For the three and nine months ended September 30, 2024, the Company recorded \$0.6 million and \$0.9 million of income tax expense, respectively, primarily pertaining to state and foreign income taxes.

For the three and nine months ended September 30, 2023, the Company recorded \$0.2 million and \$0.7 million of income tax expense, respectively, primarily pertaining to state and foreign income taxes.

The income tax provision during interim periods is computed by applying an estimated annual effective tax rate to year-to-date pre-tax income, plus adjustments for significant unusual or infrequently occurring items, in accordance with FASB ASC Topic 740-270, *Income Taxes – Interim Reporting*. The income tax provision differs from the U.S. federal statutory rate of 21% primarily due to the effect of valuation allowance against the Company’s net deferred tax assets, which reduces the Company’s net tax benefit.

Deferred tax assets and deferred tax liabilities are determined based on temporary differences between the financial reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some portion or all of the deferred tax assets will not be realized. The Company has recorded a full valuation allowance against its net deferred tax assets as of September 30, 2024 and December 31, 2023.

## [Table of Contents](#)

The Company does not recognize a tax benefit for uncertain tax positions unless it is more likely than not that the position will be sustained upon examination by tax authorities, including resolution of any related appeals or litigation processes, based on the technical merits of the position. The tax benefit that is recorded for these positions is measured at the largest amount of cumulative benefit that has greater than a 50 percent likelihood of being realized upon ultimate settlement. Deferred tax assets that do not meet these recognition criteria are not recorded and the Company recognizes a liability for uncertain tax positions that may result in tax payments. The Company has not recorded any amounts for unrecognized tax benefits as of September 30, 2024 and December 31, 2023. Our policy is to review and update unrecognized tax positions as facts and circumstances change.

## **12. License and Collaboration Agreements**

### ***Sobi License and Collaboration Agreement***

In October 2020, the Company and its subsidiaries, Apellis Switzerland GmbH and APL DEL Holdings, LLC, entered into a Collaboration and License Agreement (the “Sobi collaboration agreement”) with Sobi, concerning the development and commercialization of pegcetacoplan and specified other structurally and functionally similar compstatin analogues or derivatives for use systemically or for local non-ophthalmological administration (collectively referred to as the “Licensed Products”).

Under the Sobi collaboration agreement, the Company granted Sobi an exclusive (subject to certain retained rights of the Company), sublicensable license of certain patent rights and know-how to develop and commercialize Licensed Products in all countries outside of the United States.

The Company retains the right to commercialize Licensed Products in the United States, and, subject to specified limitations, to develop Licensed Products worldwide for commercialization in the United States.

Under the Sobi collaboration agreement, the Company and Sobi agreed to collaborate to develop Licensed Products for certain indications, including PNH, C3G, IC-MPGN and HSCT-TMA (collectively the “Initial Indications”), and any other indications subsequently agreed upon by the parties, for commercialization by or on behalf of the Company in the United States and by or on behalf of Sobi outside of the United States. If the parties do not agree to jointly pursue any development activities for the Licensed Products (whether for an Initial Indication or otherwise), the party proposing to pursue such activities may conduct such activities at its sole expense (with the non-proposing party having the right to obtain rights to the data generated by such development activities by paying a specified percentage of that expense), subject to agreed-upon exceptions that limit each party’s unilateral development rights.

The initial development plan sets forth the initial development activities to be conducted by each of the Company and Sobi, with the Company bearing all costs incurred in conducting the activities set forth in such initial development plan, as well as certain specified additional costs that are not included in the initial development plan that may be incurred by the parties in developing Licensed Products for PNH in the European Union and the United Kingdom. The Company and Sobi formed several governance committees to oversee the development and manufacture, and to review and discuss the commercialization, of Licensed Products.

The Company shall supply Licensed Products to Sobi for development and for commercialization outside of the United States in accordance with a supply agreement to be negotiated by the parties. The Sobi collaboration agreement grants Sobi the right to perform or have performed drug product manufacturing of Licensed Products for development and for commercialization outside the United States and to manufacture or have manufactured drug substance under certain circumstances.

Sobi paid the Company an upfront payment of \$250.0 million in November 2020 and agreed to pay up to an aggregate of \$915.0 million upon the achievement of specified one-time regulatory and commercial milestone events, of which the Company received \$50.0 million in April 2022 for the achievement of a regulatory development milestone in Europe. Sobi also agreed to reimburse the Company for up to \$80.0 million in development costs, of which the Company received a total of \$65.0 million through January 2023 and waived the remaining payment of \$15.0 million in January, 2024. The Company will also be entitled to receive tiered, double-digit royalties (ranging from high teens to high twenties) on sales of Licensed Products outside of the United States, subject to customary deductions and third-party payment obligations, until the latest to occur of: (i) expiration of the last-to-expire of specified licensed patent rights; (ii) expiration of regulatory exclusivity; and (iii) ten (10) years after the first commercial sale of the applicable Licensed Product, in each case on a Licensed Product-by-Licensed Product and country-by-country basis. Under the Sobi collaboration agreement, the Company remains responsible for its license fee obligations (including royalty obligations) to the Trustees of the University of Pennsylvania (“Penn”), as a licensor of the Company.

Under the Sobi collaboration agreement, for the three and nine months ended September 30, 2024, the Company recognized \$5.0 million and \$13.9 million, respectively, of royalty revenue. For the three and nine months ended September 30, 2023, the Company recognized \$2.7 million and \$6.8 million, respectively, of royalty revenue. For the three and nine months ended September 30, 2024 and 2023, the Company did not recognize any contra-research and development expense in the unaudited condensed consolidated statement of operations related to the \$80.0 million reimbursement commitment from Sobi. Since contract

[Table of Contents](#)

inception, the Company has recognized \$65.0 million in contra-research and development expenses and waived the remaining \$15.0 million in connection with the decision to discontinue the CAD program.

As of December 31, 2023, the Company recorded \$15.0 million in current assets, which represented the receivable for contra-research and development expenses incurred but not yet reimbursed from Sobi. In January 2024, the Company waived the remaining reimbursement payment of \$15.0 million in connection with the decision to discontinue the CAD program.

***University of Pennsylvania License Agreement***

The Company is a party to a license agreement with Penn for an exclusive, worldwide license to specified patent rights. The Company is required to pay annual maintenance fees of \$0.1 million until the first sale of a licensed product. The Company is also required to make milestone payments aggregating up to \$3.2 million based upon the achievement of specified development and regulatory milestones and up to \$5.0 million based upon the achievement of specified annual sales milestones with respect to each licensed product, and to pay low single-digit royalties based on net sales of each licensed product, subject to a step-down upon patent expiry, with minimum quarterly royalty thresholds. In addition, the Company is obligated to pay a specified portion of income it receives from sublicensees.

In April 2023, the Company paid \$2.3 million for the achievement of a regulatory milestone as a result of the FDA approval of SYFOVRE in February 2023. In 2023, the Company incurred \$5.0 million as a result of the achievement of sales milestones for SYFOVRE of which the Company paid \$2.0 million in October 2023 and the remaining \$3.0 million in January 2024.

For the three and nine months ended September 30, 2024, the Company incurred royalty expense of \$4.9 million and \$14.4 million on sales of SYFOVRE.

For the three and nine months ended September 30, 2023, the Company has incurred royalty expense of \$2.4 million and \$5.2 million on sales of SYFOVRE.

In addition, the Company is also party to a license agreement with Penn for an exclusive, worldwide license to specified patent rights for the development and commercialization of products in fields of use, as defined therein. The Company is required to make milestone payments aggregating up to \$1.7 million, based upon the achievement of development and regulatory approval milestones, and up to \$2.5 million, based upon the achievement of annual sales milestones with respect to each of the first two licensed products. The license agreement also requires the Company to pay low single digit royalties based on net sales of each licensed product, subject to a step-down upon patent expiry, with minimum quarterly royalty thresholds. In addition, the Company is obligated to pay a specified portion of income it receives from sublicensees.

In January 2021, the Company paid \$25.0 million for a sublicense fee owed to Penn related to the Sobi collaboration agreement and another licensing transaction. In August 2021, the Company paid \$1.0 million to Penn upon the achievement of a development milestone, net of a credit for the annual license maintenance payment. In June 2022, the Company paid an additional \$5.0 million to Penn upon the achievement of a development milestone. In January 2023, the Company paid \$1.0 million to Penn upon the achievement of a sales milestone for EMPAVELI in 2022. In January 2024, the Company paid \$0.5 million for a sublicense fee owed to Penn related to Sobi obtaining regulatory approval in Japan. Additionally, in January 2024, the Company paid \$1.5 million as a result of the achievement of a sales milestone for EMPAVELI and Aspaveli.

For the three and nine months ended September 30, 2024, the Company incurred royalty expense of \$1.8 million and \$5.0 million on sales of EMPAVELI and Aspaveli.

For the three and nine months ended September 30, 2023, the Company has incurred royalty expense of \$1.3 million and \$3.4 million on sales of EMPAVELI and Aspaveli.

**13. Commitments and Contingencies**

The Company has certain non-cancelable purchase obligations related to the manufacturing of drug substance and drug product. The Company has agreed to purchase from Bachem Americas, Inc. a significant portion of its requirements for the pegcetacoplan drug substance. Under a commercial supply agreement with NOF Corporation ("NOF"), the Company has agreed to purchase activated polyethylene glycol derivative, or PEG, which is a component of pegcetacoplan. In September 2024, the Company terminated the minimum purchase obligation with NOF for 2025. Under these agreements, as of September 30, 2024, the Company is obligated to pay

## [Table of Contents](#)

up to an aggregate of \$56.1 million to these vendors. As a result of this termination, the Company incurred an expense of \$6.4 million, which is included in Other Liabilities on the consolidated balance sheet.

In addition, the Company has other non-cancelable purchase agreements as of September 30, 2024, under which it is obligated to pay up to an aggregate of \$17.5 million to vendors.

The Company is a party to a master lease agreement under which the Company leases vehicles with initial terms of 36 months from the date of delivery. If the Company were unable to take delivery of a previously ordered vehicle, the Company may incur nominal fees.

*Indemnifications*—In the ordinary course of business, the Company enters into agreements that may include indemnification provisions. Pursuant to such agreements, the Company may indemnify, hold harmless and defend indemnified parties for losses suffered or incurred by the indemnified party. Some of the provisions will limit losses to those arising from third-party actions. In some cases, the indemnification will continue after the termination of the agreement. The maximum potential amount of future payments the Company could be required to make under these provisions is not determinable. The Company has not incurred any cost to defend lawsuits or settle claims related to these indemnification provisions.

*Legal*—During the normal course of business, the Company may be a party to legal claims that may not be covered by insurance.

On August 2, 2023, Judith M. Soderberg filed a putative class action in the United States District Court for the District of Delaware against the Company and certain current and former executive officers of the Company (the “Complaint”). The Complaint alleges, among other things, that the defendants violated Sections 10(b) and/or 20(a) of the Exchange Act and Rule 10b-5 promulgated thereunder by misrepresenting and/or omitting certain material facts related to the design of SYFOVRE’s clinical trials and the risks associated with SYFOVRE’s commercial adoption. The Complaint seeks, among other relief, compensatory damages and equitable relief in favor of the alleged class against all defendants, including interest, and reasonable costs and expenses incurred by plaintiffs, including attorneys’ and expert fees.

On October 2, 2023, the defendants moved to transfer the action to the United States District Court for the District of Massachusetts.

On October 23, 2023, the Court appointed Ray Peleckas and Michigan Laborers’ Pension Fund together as Co-Lead Plaintiffs and assigned the action the caption In Apellis Pharmaceuticals, Inc. Securities Litigation, Case 1:23-cv-00834-MN. The Co-Lead Plaintiffs filed an amended complaint on February 8, 2024 (the “Amended Complaint”). The Amended Complaint is brought on behalf of a class of all persons and entities who purchased or otherwise acquired Apellis common stock between January 28, 2021 and July 28, 2023, inclusive, names the Company and Cedric Francois, our chief executive officer, as defendants, and makes similar allegations, asserts the same claims and seeks the same relief as the Complaint. On May 17, 2024, the United States District Court for the District of Delaware approved the motion to transfer to the United States District Court for the District of Massachusetts. The defendants moved to dismiss the Complaint on June 12, 2024, and the Court has scheduled oral argument on this motion for November 14, 2024.

The Company’s businesses may also be subject at any time to other commercial disputes, product liability claims, personal injury claims, third-party subpoenas or various other lawsuits arising in the ordinary course of business, including intellectual property infringement, employment or investor matters, and the Company expects that this will continue to be the case in the future.

For example, in August 2024, an individual filed a civil action against the Company in the United States District Court in the Northern District of Texas, alleging personal injury claims in connection with the use of SYFOVRE. We moved to dismiss this civil action in September 2024. The Court has not yet ruled on this motion to dismiss, as of the date of issuance of these unaudited condensed consolidated financial statements.

The outcome of the matters described above cannot be predicted with certainty and therefore any loss is neither probable nor reasonably estimable. However, the Company intends to vigorously defend against these matters.

## **14. Net Loss per Share**

Basic and diluted net loss per share is calculated based upon the weighted average number of shares of common stock outstanding during the period. Shares of the Company’s common stock underlying pre-funded warrants are included in the weighted average number of shares of common stock used to calculate basic and diluted net loss per share attributable to common stockholders as they are exercisable at any time for nominal cash. Since the Company was in a loss position for all periods presented, basic net loss per common share is the same as diluted net loss per common share for all periods presented as the inclusion of all potential common shares



[Table of Contents](#)

would have been anti-dilutive. Convertible notes and potential common shares presented below were excluded from the calculation of diluted net loss per share, prior to the use of the treasury stock method, as their effect is anti-dilutive (in thousands):

	As of September 30,	
	2024	2023
Convertible notes	2,379	2,379
Common stock options	8,063	9,738
Restricted stock units	4,017	4,449
Total	14,459	16,566

## **Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations.**

*The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and our audited financial statements and related notes for the year ended December 31, 2023 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 27, 2024, as amended by Amendment No. 1 thereto filed with the Securities and Exchange Commission on February 29, 2024, which we refer to as the 2023 Annual Report on Form 10-K.*

*This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.*

*We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. Please also refer to those factors described in “Part I, Item 1A. Risk Factors” of our 2023 Annual Report on Form 10-K for important factors that we believe could cause actual results to differ materially from those in our forward-looking statements.*

### **Overview**

We are a commercial-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutic compounds to treat diseases with high unmet needs through the inhibition of the complement system, which is an integral component of the immune system, at the level of C3, the central protein in the complement cascade. We believe that this approach can result in broad inhibition of the principal pathways of the complement system and has the potential to effectively control diseases with high unmet need and that are driven by excessive complement activation.

In February 2023, the U.S. Food and Drug Administration, or the FDA, approved SYFOVRE® (pegcetacoplan injection), the first approved treatment for geographic atrophy secondary to age-related macular degeneration, or GA. In June 2024, we announced additional results from the GALE long-term extension study which demonstrated that SYFOVRE injection preserved visual function in patients with GA at 36 months. We believe SYFOVRE has the potential to be a best-in-class treatment for patients with GA, a disease that affects approximately 1.5 million people in the United States and five million people worldwide. We launched SYFOVRE in the United States in March 2023. For the three and nine months ended September 30, 2024, we generated \$152.0 million and \$444.0 million, respectively, in U.S. net product revenue from sales of SYFOVRE.

We have pending marketing applications for SYFOVRE for the treatment of GA with regulatory authorities in various jurisdictions, including the United Kingdom and Switzerland, which are each under review by the applicable regulatory authorities. In September 2024, the Committee for Medicinal Products for Human Use, or CHMP, adopted a negative opinion following the re-examination of the marketing authorization application, or MAA, with the European Medicines Agency, or EMA. We have exclusive, worldwide commercialization rights for intravitreal pegcetacoplan.

In May 2021, the FDA approved EMPAVELI (systemic pegcetacoplan), the first targeted C3 therapy, for the treatment of paroxysmal nocturnal hemoglobinuria, or PNH, for use in adults who are either treatment-naïve or who are switching from C5 inhibitors eculizumab or ravulizumab. For the three and nine months ended September 30, 2024, we generated \$24.6 million and \$74.7 million, respectively, in U.S. net product revenue from sales of EMPAVELI and received \$5.0 million and \$13.9 million, respectively, in royalties from our collaboration partner, Swedish Orphan Biovitrum AB (Publ), or Sobi, which has exclusive ex-U.S. commercialization rights for systemic pegcetacoplan outside of the United States. We have commercialization rights for systemic pegcetacoplan in the United States.

We believe that EMPAVELI has the potential to be a best-in-class treatment for additional high unmet need areas. We are leading the development of systemic pegcetacoplan in C3 glomerulopathy, or C3G, and primary immune complex membranoproliferative glomerulonephritis, or primary IC-MPGN, in nephrology under our collaboration with Sobi. In August 2024, we reported top-line data from the Phase 3 VALIANT trial investigating systemic pegcetacoplan in adolescent and adult patients with naive and post-transplant recurrence IC-MPGN and C3G and provided further details at the annual meeting of the American Society of Nephrology in October 2024. The VALIANT study demonstrated a 68% reduction in proteinuria in C3G and IC-MPGN patients compared to placebo ( $p < 0.0001$ ), favorable results across key secondary endpoints, including stabilized estimated glomerular filtration rate, or eGFR, a key measure of kidney function, and a reduction in C3G staining intensity, and favorable safety and tolerability results, consistent with pegcetacoplan’s established profile. All patients who have already completed the VALIANT study have been enrolled into the VALE

## [Table of Contents](#)

long-term extension study. We intend to submit a supplemental new drug application to the FDA in early 2025, and Sobi plans to submit an MAA to the EMA in 2025.

Sobi is leading the development of systemic pegcetacoplan for hematopoietic stem cell transplantation-associated thrombotic microangiopathy, or HSCT-TMA, in hematology.

We are developing additional product candidates with other routes of administration. These candidates include APL-3007, a small interfering RNA, or siRNA, which is in a Phase 1 clinical trial in healthy volunteers, as well as an oral complement inhibitor in preclinical development. Furthermore, we are collaborating with Beam Therapeutics, Inc., or Beam, on up to six research programs focused on C3 and other complement targets in the eye, liver and brain, using Beam's proprietary base editing technology to discover new treatments for complement-driven diseases.

Since our commencement of operations in May 2010, we have devoted substantially all of our resources to developing our proprietary technology, developing product candidates, undertaking preclinical studies and conducting clinical trials for pegcetacoplan, building our intellectual property portfolio, organizing and staffing our company, business planning, raising capital, preparing for and executing the commercial launch of our products and providing general and administrative support for these operations.

To date, we have financed our operations primarily through approximately \$2.6 billion in net proceeds from public and private offerings of our common stock and convertible securities, \$392.0 million in payments and royalties from Sobi pursuant to our collaboration agreement, \$532.5 million under various credit arrangements, including with Sixth Street Lending Partners, or Sixth Street, and SFJ Pharmaceuticals Group, or SFJ, and \$98.8 million relating to the unwinding of certain capped call transactions in March 2024, as well as from the proceeds of our operations. To date, we have exchanged \$425.4 million and converted \$0.7 million of aggregate principal amount of our Convertible Notes for shares of our common stock. Our non-dilutive financing activities in the nine months ended September 30, 2024, which include the Sixth Street Financing Agreement (as defined below), the repayment of our remaining obligations to SFJ and the partial unwinding of our capped call transactions, increased the amount of cash available to us through 2025 by approximately \$270.0 million, with the potential for us to access additional short-term liquidity through a second draw of \$100.0 million under the Credit Facility (as defined below).

We have incurred significant annual net operating losses in every year since our inception, and we expect to continue to incur net operating losses for at least this year. Our net losses were \$57.4 million and \$140.2 million for the three months ended September 30, 2024 and 2023, respectively. As of September 30, 2024, we had an accumulated deficit of \$.3.0 billion.

Our operating results may fluctuate significantly from quarter to quarter and year to year. We anticipate that we will continue to incur significant commercialization expenses related to sales, marketing, medical affairs, manufacturing, distribution and other commercial infrastructure associated with the commercialization of EMPAVELI for the treatment of PNH and other indications and the commercialization of SYFOVRE for the treatment of GA. In addition, we expect to continue to incur these expenses if and as we continue to develop and conduct our ongoing and planned clinical trials of pegcetacoplan and our other product candidates; initiate and continue research and preclinical and clinical development efforts for any future product candidates; seek to identify and develop additional product candidates for complement-dependent diseases; seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any; establish sales, marketing, distribution and other commercial infrastructure to commercialize any additional products for which we may obtain marketing approval; require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization; maintain, expand and protect our intellectual property portfolio; hire and retain additional personnel, such as clinical, quality control, regulatory and scientific personnel; add operational, financial and management information systems and personnel, including personnel to support our product development and add equipment and physical infrastructure to support our research and development programs and commercialization.

### ***Financing Agreement and Credit Facility***

On May 13, 2024, we entered into a financing agreement, or the Sixth Street Financing Agreement, with certain of our material subsidiaries as guarantors party thereto, the lenders party thereto, or the Lenders, and Sixth Street Lending Partners, as the administrative agent and collateral agent for the Lenders.

The Sixth Street Financing Agreement provides for a senior secured term loan facility of up to \$475.0 million, or the Credit Facility, consisting of an initial draw of \$375.0 million at closing and a potential additional \$100.0 million draw at our option upon satisfaction of a \$50.0 million minimum cash requirement and a requirement that our trailing three-month sales of SYFOVRE is at least \$180.0 million prior to the \$100.0 million draw. We can exercise the option for the additional \$100.0 million draw through September 30, 2025, assuming such requirements are met.

The Credit Facility matures on May 13, 2030 (the "Maturity Date") and bears interest at (i) in the case of SOFR Loans, an annual rate equal to 3-month Term SOFR (subject to 1.00% floor), plus 5.75%, and (ii) in the case of Base Rate Loans, an annual rate equal to the

## [Table of Contents](#)

base rate as defined in the agreement (subject to 2.00% floor), plus 4.75%. Certain additional commitment and undrawn amount fees are also payable in connection with the Credit Facility.

The net proceeds from the initial draw of the Credit Facility were approximately \$358.2 million, net of \$16.8 million of issuance costs. We used the majority of the proceeds of the \$375.0 million draw at closing to buy out our remaining obligations to SFJ, in the amount of approximately \$326.5 million.

The Credit Facility does not provide for scheduled amortization payments during the term. All principal will be due on the Maturity Date. We have the right to prepay loans under the Credit Facility at any time. We are required to repay loans under the Credit Facility with proceeds from certain asset sales, condemnation events and extraordinary receipts, subject, in some cases, to reinvestment rights. Repayments are subject to a prepayment premium. Repayments may be made after the first year of the loan and are subject to a prepayment premium up to 3% depending on timing.

All obligations under the Sixth Street Financing Agreement are secured on a first-priority basis, subject to certain exceptions, by security interests in substantially all of our assets and assets of our material subsidiaries, including our intellectual property, and are guaranteed by our material subsidiaries, including foreign subsidiaries, subject to certain exceptions.

The Sixth Street Financing Agreement contains customary covenants, including, without limitation, a financial covenant to maintain liquidity of at least \$50.0 million if our market capitalization is below \$3.0 billion, and negative covenants that, subject to certain exceptions, restrict indebtedness, liens, investments (including acquisitions), fundamental changes, asset sales and licensing transactions, dividends, modifications to material agreements, payment of subordinated indebtedness, and other matters customarily restricted in such agreements. Among other permissions, we are permitted, on terms and conditions set forth on the Sixth Street Financing Agreement, to enter into a separate asset-based financing arrangement with a third party in an amount of up to \$100.0 million, which amount is increased to \$200.0 million upon certain sales or market capitalization thresholds, and to have outstanding convertible unsecured notes in an amount equal to the greater of \$400.0 million and 10% of our market capitalization, but not to exceed \$600.0 million. We are subject to restrictions on sales and licensing transactions with respect to our core intellectual property, defined to include SYFOVRE, EMPAVELL, and other pegcetacoplan product assets, subject to certain exceptions, including certain transactions related to areas outside the United States and Europe.

The Sixth Street Financing Agreement also contains certain events of default after which loans under the Credit Facility may be due and payable immediately, including payment defaults, material inaccuracy of representations and warranties, covenant defaults, bankruptcy and insolvency proceedings, cross-defaults to certain other agreements, judgments against us and our subsidiaries, and change of control.

### ***SFJ Agreement***

In 2019, we entered into a development funding agreement (as amended, the “SFJ agreement”) with SFJ Pharmaceuticals Group (“SFJ”), under which SFJ agreed to provide funding to us to support the development of pegcetacoplan for the treatment of patients with PNH. Under the SFJ agreement, SFJ paid us an aggregate of \$140.0 million between June 2019 and January 2020.

Following regulatory approval for the use of systemic pegcetacoplan as a treatment for PNH by the FDA in May 2021 and by the EMA in December 2021, we became obligated to pay SFJ an aggregate of \$460.0 million in payments between 2021 and 2027. We paid SFJ an aggregate of \$94.0 million through March 31, 2024.

On May 13, 2024, we used proceeds from the Sixth Street Financing Agreement to buy out our remaining obligations owed to SFJ, in the amount of approximately \$326.5 million. The buyout of the SFJ development liability eliminated the remaining \$366.0 million in payments to SFJ, including a total of approximately \$200.0 million payable in 2024 and 2025.

### ***Convertible Notes***

On September 16, 2019, we completed a private offering of convertible notes, or the 2019 Convertible Notes, with an aggregate principal amount of \$220.0 million issued pursuant to an indenture, or the Indenture, with U.S. Bank National Association, as trustee.

The net proceeds from the sale of the 2019 Convertible Notes were approximately \$212.9 million after deducting the initial purchasers’ discounts and commissions of \$6.6 million and offering expenses of \$0.5 million. We used \$28.4 million of the net proceeds from the sale of the 2019 Convertible Notes to pay the cost of the capped call transactions in September 2019 described below.

On May 12, 2020, we issued convertible notes, or the 2020 Convertible Notes, with an aggregate principal amount of \$300.0 million. The net proceeds from the sale of the 2020 Convertible Notes were approximately \$322.9 million after deducting the purchasers’ discounts and commission of \$5.7 million and offering expenses of \$0.3 million. We used \$43.1 million of the net proceeds from the sale to pay the cost of the additional capped call transactions in May 2020 described below.

## [Table of Contents](#)

The 2019 Convertible Notes and the 2020 Convertible Notes are referred to together as the Convertible Notes. The Convertible Notes are our senior unsecured obligations and bear interest at a rate of 3.5% per year payable semiannually in arrears on March 15 and September 15 of each year, beginning on March 15, 2020. The Convertible Notes will mature on September 15, 2026, unless converted earlier, redeemed or repurchased in accordance with their terms.

The Convertible Notes are convertible into shares of our common stock at an initial conversion rate of 25.3405 shares per \$1,000 principal amount of notes (equivalent to an initial conversion price of approximately \$39.4625 per share of common stock). The conversion rate is subject to customary anti-dilution adjustments. In addition, following certain events that occur prior to the maturity date or if we deliver a notice of redemption, we will increase the conversion rate for a holder who elects to convert its Convertible Notes in connection with such corporate event or a notice of redemption, as the case may be, in certain circumstances as provided in the Indenture.

Prior to March 15, 2026, the Convertible Notes are convertible only under the following circumstances:

- during any calendar quarter, if the last reported sale price of our common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day;
- during the five business day period after any five consecutive trading day period in which the trading price per \$1,000 principal amount of the Convertible Notes for each such trading day was less than 98% of the product of the last reported sale price of our common stock and the conversion rate on each such trading day;
- if we call any or all of the Convertible Notes for redemption, at any time prior to the close of business on the second scheduled trading day immediately preceding the redemption date; or upon the occurrence of corporate events specified in the Indenture.

On, or after, March 15, 2026 until the close of business on the second scheduled trading day immediately preceding the maturity date of the Convertible Notes, holders may convert the Convertible Notes at any time regardless of the foregoing circumstances. Upon conversion of the Convertible Notes, we will pay or deliver, as the case may be, cash, shares of our common stock or a combination of cash and shares of common stock, at our election.

As of September 20, 2023, we may redeem for cash all or a portion of the Convertible Notes, at our option, if the last reported sale price of our common stock has been at least 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive), including the trading day immediately preceding the date on which we provide a notice of redemption, during any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which we provide notice of redemption. The redemption price will be equal to 100% of the principal amount of the Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. If we call any Convertible Notes for redemption, it will constitute a “make-whole fundamental change” with respect to such Convertible Notes, in which case the conversion rate applicable to the conversion of such Notes, if converted in connection with the redemption, will be increased in certain circumstances. We have not called for redemption any of the Convertible Notes as of September 30, 2024.

If we undergo a “fundamental change,” as defined in the Indenture, prior to maturity, subject to certain conditions, holders may require us to repurchase for cash all or any portion of their Convertible Notes at a fundamental change repurchase price equal to 100% of the principal amount of the notes to be repurchased, plus any accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

In January 2021, July 2021 and July 2022, we entered into separate, privately negotiated exchange agreements to modify the conversion terms with certain holders of our Convertible Notes. Under the terms of these exchange agreements, in January 2021, July 2021 and July 2022, the holders exchanged approximately \$126.1 million of 2019 Convertible Notes, \$201.1 million of 2019 Convertible Notes and 2020 Convertible Notes, and \$98.1 million of 2020 Convertible Notes, respectively, in aggregate principal amount held by them for an aggregate of 3,906,869 shares, 5,992,217 shares and 3,027,018 shares, respectively, of common stock we issued. In accordance with FASB ASC Topic 470-20, “Debt – Debt with Conversion and Other Options,” or ASC 470-20, we accounted for the exchange as an induced conversion based on the short period of time the conversion offer was open and the substantive conversion feature offer. We accounted for the conversion of the debt as an inducement by expensing the fair value of the shares that were issued in excess of the original terms of the Convertible Notes.

The conditional conversion feature of the Convertible Notes was triggered as of December 31, 2023, and as a result the Convertible Notes were convertible at the option of the holders until March 31, 2024. No Convertible Notes were converted during this period.

## [Table of Contents](#)

The conditional conversion feature of the Convertible Notes was triggered as of March 31, 2024, and as a result the Convertible Notes were convertible at the option of the holders until June 30, 2024. No Convertible Notes were converted during this period.

The conditional conversion feature of the Convertible Notes was not triggered as of June 30, 2024, and as a result the Convertible Notes are not convertible during the quarter ending September 30, 2024.

The conditional conversion feature of the Convertible Notes was not triggered as of September 30, 2024, and as a result the Convertible Notes are not convertible during the quarter ending December 31, 2024.

As of September 30, 2024, we held in treasury Convertible Notes in principal amount of \$425.4 million, which notes had not been cancelled.

### ***Collaboration Agreement with Sobi***

On October 27, 2020, we entered into the Sobi collaboration agreement, concerning the development and commercialization of pegcetacoplan and specified other structurally and functionally similar compstatin analogues or derivatives for use systemically or for local non-ophthalmological administration, collectively referred to as the licensed products. We granted Sobi an exclusive (subject to certain rights retained by us), sublicensable license of certain patent rights and know-how to develop and commercialize licensed products in all countries outside of the United States. We retained the right to commercialize licensed products in the United States, and, subject to specified limitations, to develop licensed products worldwide for commercialization in the United States. Under the Sobi collaboration agreement, Sobi made an upfront payment of \$250.0 million in November 2020, and agreed to pay up to an aggregate of \$915.0 million upon the achievement of specified one-time regulatory and commercial milestone events, including a \$50.0 million milestone payable following the first regulatory and reimbursement approval of systemic pegcetacoplan in any major European country, and to reimburse us for up to \$80.0 million in development costs.

In January 2021 we received a \$25.0 million development reimbursement payment from Sobi and in January 2022 and 2023, we received a \$20.0 million development reimbursement payment from Sobi. In January 2024, we waived the remaining reimbursement payment of \$15.0 million in connection with the decision to discontinue the cold agglutinin disease program.

The European Commission approved systemic Aspaveli (pegcetacoplan) for the treatment of adults with PNH in December 2021. In March 2022, we earned a \$50.0 million payment from Sobi related to the first regulatory and reimbursement milestone in Europe, which we received in April 2022. We are also entitled to receive tiered, double-digit royalties (ranging from high teens to high twenties) on sales of licensed products outside of the United States, subject to customary deductions and third-party payment obligations, until the latest to occur of: (i) expiration of the last-to-expire of specified licensed patent rights; (ii) expiration of regulatory exclusivity; and (iii) ten (10) years after the first commercial sale of the applicable licensed product, in each case on a licensed product-by-licensed product and country-by-country basis. We remain responsible for our license fee obligations (including royalty obligations) to the University of Pennsylvania.

### **Financial Operations Overview**

#### ***Revenue***

Our revenues consist of product sales of EMPAVELI and SYFOVRE, and revenues derived from our collaboration agreement with Sobi.

Revenue is recognized when, or as, we satisfy a performance obligation by transferring a promised good or service to a customer. An asset is transferred when, or as, the customer obtains control of that asset. For performance obligations that are satisfied over time, we recognize revenue using an input or output measure of progress that best depicts the satisfaction of the relevant performance obligation.

#### ***Product Revenues***

Product revenue is derived from our sales of our commercial products, EMPAVELI and SYFOVRE, in the United States.

#### ***Licensing and Other Revenue***

Licensing and other revenue is derived from our collaboration agreement with Sobi concerning the development and commercialization of pegcetacoplan and specified other compstatin analogues or derivatives for use systemically or for local non-ophthalmic administration.

### ***Cost of Sales***

Cost of sales consists primarily of costs associated with the manufacturing of EMPAVELI and SYFOVRE, royalties owed to our licensor, costs associated with supply provided under the Sobi collaboration agreement, costs of products provided under our patient assistance programs, and certain period costs.

### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our product candidates, which include:

- employee-related expenses including salaries, bonuses, benefits and share-based compensation expense related to individuals performing research and development activities;
- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct clinical trials and research and development activities on our behalf, and contract manufacturing organizations that manufacture quantities of drug supplies for both our preclinical studies and clinical trials;
- the cost of consultants, including share-based compensation expense; and
- various other expenses incident to the management of our preclinical studies and clinical trials.

Research and development costs are expensed as incurred. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed. We have not provided program costs since inception because historically we have not tracked or recorded our research and development expenses on a program-by-program basis.

The successful development of our product candidates in clinical development is highly uncertain. Accordingly, at this time, we cannot reasonably estimate the nature, timing and costs of the efforts that will be necessary to complete the remainder of the clinical development of these product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from pegcetacoplan in other jurisdictions and indications or any other potential product candidates. This is due to the numerous risks and uncertainties associated with developing therapeutics, including the uncertainties of:

- establishing an appropriate safety profile in preclinical studies;
- successful enrollment in, and completion of clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- launching commercial sales of the products, if and when approved, whether alone or in collaboration with others; and
- an acceptable safety profile of the products following approval.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase for the foreseeable future as our product candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

### ***Selling, General and Administrative Expenses***

Selling, general and administrative expenses consist primarily of costs associated with the commercialization of approved products and general and administrative costs to support operations, including salaries, bonuses, benefits and share-based compensation. Selling expenses include product marketing, sales operations costs, and other costs incurred to support our sales efforts. General and administrative expenses include corporate support functions such as executive management, finance and accounting, business

[Table of Contents](#)

development, legal, human resources, information technology, and associated external costs to support those functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters, and fees for accounting and consulting services.

We anticipate that our selling, general and administrative expenses will increase in the future to support continued commercial activities for our approved products, potential commercialization of our product candidates and costs of operating as a public company.

### Critical Accounting Estimates

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reported periods. On an ongoing basis, we evaluate our estimates and judgments, including those related to product revenue, licensing revenue, costs of research collaboration arrangements, inventory, accrued research and development expenses, convertible notes, capped call transactions and development liability, which we described in our 2023 Annual Report on Form 10-K. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our critical accounting policies are described in Note 2 of Part I, Item 1 of this Quarterly Report on Form 10-Q and in Part I, Item 7, “Critical Accounting Estimates” in our 2023 Annual Report on Form 10-K. There have been no changes to our critical accounting estimates since our 2023 Annual Report on Form 10-K, except as noted in Note 2.

### Results of Operations

*Three Months Ended September 30, 2024 and 2023 (in thousands, except percentages)*

	For the Three Months Ended September 30,		Change \$	Change %
	2024	2023		
Revenue:				
Product revenue, net	\$ 176,571	\$ 99,182	\$ 77,389	78%
Licensing and other revenue	20,259	11,217	9,042	81%
Total revenue:	196,830	110,399	86,431	78%
Operating expenses:				
Cost of sales	33,557	22,410	11,147	50%
Research and development	88,569	79,421	9,148	12%
Selling, general and administrative	121,984	145,648	(23,664)	(16%)
Total operating expenses:	244,110	247,479	(3,369)	(1%)
Net operating loss	(47,280)	(137,080)	89,800	(66%)
Interest income	2,889	4,989	(2,100)	(42%)
Interest expense	(12,532)	(7,310)	(5,222)	71%
Other income/(expense), net	70	(603)	673	(112%)
Net loss before taxes	(56,853)	(140,004)	83,151	(59%)
Income tax expense	592	233	359	154%
Net loss	\$ (57,445)	\$ (140,237)	\$ 82,792	(59%)

#### *Product Revenue, Net*

Our product revenue, net is derived from EMPAVELI sales in the United States which was launched in May 2021 and SYFOVRE sales in the United States which was launched in March 2023. We recognized \$176.6 million and \$99.2 million of net product revenue for the three months ended September 30, 2024 and 2023, respectively. The net product revenue of \$176.6 million for the nine months ended September 30, 2024, consists of \$24.6 million in net product revenue from sales of EMPAVELI and \$152.0 million in net product revenue from sales of SYFOVRE. The net product revenue of \$99.2 million for the nine months ended September 30, 2023, consists of



[Table of Contents](#)

\$23.9 million in net product revenue from sales of EMPAVELI and \$75.3 million in net product revenue from sales of SYFOVRE. The increase in product revenue was primarily driven by an increase in volume of SYFOVRE sales in the second year of launch.

*Licensing and Other Revenue*

Licensing and other revenue of \$20.3 million for the three months ended September 30, 2024 consisted of \$15.3 million in revenue from product supplied to Sobi and \$5.0 million in royalty revenue from Sobi. Licensing and other revenue of \$11.2 million for the three months ended September 30, 2023 consisted of \$3.5 million in revenue from product supplied to Sobi, \$2.7 million in royalty revenue from Sobi and \$5.0 million from collaboration milestones from Sobi.

*Cost of Sales*

Cost of sales was \$33.6 million for the three months ended September 30, 2024 and \$22.4 million for the three months ended September 30, 2023. The increase in cost of sales was primarily driven by a \$9.0 million increase in expenses incurred related to excess, obsolete or scrapped inventory and a \$6.4 million expense incurred in connection with the termination of the minimum purchase obligation of PEG in September 2024, which were partially offset by a \$3.9 million decrease in royalty expense as sales-based milestones incurred in the prior year did not recur in the current period.

In addition, prior to receiving FDA approval for EMPAVELI and SYFOVRE, the costs associated with the manufacturing of EMPAVELI and SYFOVRE inventory were expensed as incurred as research and development expense. This did not materially impact cost of sales for the three months ended September 30, 2024 and 2023. As of September 30, 2024, the remaining pre-FDA approved inventory was \$15.4 million, which primarily consisted of raw materials.

*Research and Development Expenses*

The following table summarizes our research and development expenses incurred during the three months ended September 30, 2024 and 2023 (in thousands, except percentages):

(In thousands)	For the Three Months Ended September 30,		Change \$	Change %
	2024	2023		
<b>Program-specific external costs:</b>				
PNH	\$ 3,584	\$ 2,240	\$ 1,344	60 %
IC-MPGN & C3G	7,715	8,445	(730)	(9 %)
ALS	154	1,671	(1,517)	(91 %)
CAD	1,736	1,112	624	56 %
H SCT-TMA	989	417	572	137 %
GA	14,610	10,083	4,527	45 %
Other development and discovery programs	16,836	12,105	4,731	39 %
<b>Total program-specific costs</b>	<b>45,624</b>	<b>36,073</b>	<b>9,551</b>	<b>26 %</b>
<b>Unallocated external costs</b>				
Non-program specific external costs	10,875	8,137	2,738	34 %
<b>Total unallocated external costs</b>	<b>10,875</b>	<b>8,137</b>	<b>2,738</b>	<b>34 %</b>
<b>Unallocated internal costs</b>				
Compensation and related personnel costs	30,886	34,046	(3,160)	(9 %)
Other expenses	1,184	1,165	19	2 %
<b>Total unallocated internal costs</b>	<b>32,070</b>	<b>35,211</b>	<b>(3,141)</b>	<b>(9 %)</b>
<b>Total research and development costs</b>	<b>\$ 88,569</b>	<b>\$ 79,421</b>	<b>\$ 9,148</b>	<b>12 %</b>

Research and development expenses increased by \$9.1 million to \$88.6 million for the three months ended September 30, 2024 from \$79.4 million for the three months ended September 30, 2023, an increase of 12%. The increase in research and development expenses was primarily attributable to a \$9.6 million increase in program specific external costs, a \$2.7 million increase in non-program specific external costs and a \$3.2 million decrease in compensation and related personnel costs.

The increase in our program-specific external costs of \$9.6 million was driven by an increase of \$4.5 million in GA costs and an increase of \$4.7 million in other development and discovery programs costs. The increases were partially offset by a decrease of \$0.8 million in IC-MPGN and C3G costs and a decrease of \$1.5 million in ALS due to the discontinuation of the Phase 2 MERIDIAN study in 2024.

## [Table of Contents](#)

The decrease in compensation and related personnel costs of \$3.2 million was driven by a \$3.8 million decrease in salaries and benefits due to lower headcount compared to the prior year and reclassification of certain employee costs to selling, general and administrative expenses, which was partially offset by a \$0.6 million increase in stock compensation expense associated with the grant of stock options and restricted stock units to employees.

### *Selling, General and Administrative Expenses*

Selling, general and administrative expenses decreased by \$23.6 million to \$122.0 million for the three months ended September 30, 2024, from \$145.6 million for the three months ended September 30, 2023, a decrease of 16%. The decrease was primarily attributable to a decrease in personnel related costs of \$2.1 million, a decrease in general commercial activities of \$20.1 million, lower office costs of \$1.8 million and a \$0.5 million reduction in professional and consulting fees. The decrease was partially offset by an increase of \$0.2 million in travel and insurance expense and factoring fees of \$0.7 million. The decrease in personnel related costs of \$2.1 million was primarily attributable to a decrease in salaries expenses of \$7.1 million, and a decrease of \$0.3 million in recruiting expenses, partially offset by higher stock compensation expenses of \$5.3 million associated with the grant of stock options and restricted stock units to employees.

### *Loss on extinguishment of development liability*

We paid our remaining obligations under the SFJ agreement in full in May 2024. We concluded that the development liability was extinguished as of the payoff date. The difference of \$1.9 million between the reacquisition price of \$326.5 million and the net carrying value of the development liability of \$324.6 million was recorded as a loss on the extinguishment of the development liability as of September 30, 2024.

### *Interest Income*

Interest income was \$2.9 million for the three months ended September 30, 2024, a decrease of \$2.1 million, compared to \$5.0 million for the three months ended September 30, 2023. The decrease in interest income was primarily attributable to decreased investments during the three months ended September 30, 2024.

### *Interest Expense*

Interest expense was \$12.5 million for the three months ended September 30, 2024, an increase of \$5.2 million, compared to \$7.3 million for the three months ended September 30, 2023. The increase is primarily due to the interest incurred under the Credit Facility and was partially offset by a decrease in the balance of the development liability.

### *Other (Expense)/Income, Net*

Other expense was \$70.0 thousand, for the three months ended September 30, 2024, as compared to other income of \$0.6 million for the three months ended September 30, 2023.

### *Income Tax Expense*

Income tax expense was \$0.6 million for the three months ended September 30, 2024, an increase of \$0.4 million, compared to \$0.2 million for the three months ended September 30, 2023. The increase primarily pertained to an increase in foreign and state taxes.

[Table of Contents](#)

*Nine Months Ended September 30, 2024 and 2023 (in thousands, except percentages)*

	For the Nine Months Ended September 30,		Change \$	Change %
	2024	2023		
<b>Revenue:</b>				
Product revenue, net	\$ 518,782	\$ 227,626	\$ 291,156	128 %
Licensing and other revenue	50,057	22,588	27,469	122 %
Total revenue:	568,839	250,214	318,625	127 %
<b>Operating expenses:</b>				
Cost of sales	76,867	38,598	38,269	99 %
Research and development	251,216	285,105	(33,889)	(12 %)
Selling, general and administrative	379,571	359,114	20,457	6 %
Total operating expenses:	707,654	682,817	24,837	4 %
Net operating loss	(138,815)	(432,603)	293,788	(68 %)
Loss on extinguishment of development liability	(1,949)	—	(1,949)	100 %
Interest income	9,377	16,385	(7,008)	(43 %)
Interest expense	(28,857)	(22,179)	(6,678)	30 %
Other (expense)/ income, net	(405)	(946)	541	(57 %)
Net loss before taxes	\$ (160,649)	\$ (439,343)	\$ 278,694	(63 %)
Income tax expense	876	709	167	24 %
Net loss	\$ (161,525)	\$ (440,052)	\$ 278,527	(63 %)

*Product Revenue, Net*

Our product revenue, net is derived from EMPAVELI sales in the United States, which was launched in May 2021 and SYFOVRE sales in the United States which was launched in March 2023. We recognized \$518.8 million and \$227.6 million of net product revenue for the nine months ended September 30, 2024 and 2023, respectively. The net product revenue of \$518.8 million for the nine months ended September 30, 2024, consisted of \$74.7 million in net product revenue from sales of EMPAVELI and \$444.0 million in net product revenue from sales of SYFOVRE. The net product revenue of \$227.6 million for three months ended September 30, 2023, consisted of \$66.7 million in net product revenue from sales of EMPAVELI and \$161.0 million in net product revenue from sales of SYFOVRE. The increase in product revenue was primarily driven by an increase in volume of SYFOVRE sales in the second year of launch.

*Licensing and Other Revenue*

Licensing and other revenue of \$50.1 million for the nine months ended September 30, 2024 consisted of \$36.2 million in revenue for product supplied to Sobi and \$13.9 million in royalty revenue from Sobi. Licensing and other revenue of \$22.6 million during the nine months ended September 30, 2023 included \$10.8 million in revenue for product supplied to Sobi, \$6.8 million in royalty revenue from Sobi and \$5.0 million from collaboration milestones from Sobi.

*Cost of Sales*

Cost of sales was \$76.9 million for the nine months ended September 30, 2024 and \$38.6 million for the nine months ended September 30, 2023. The increase in cost of sales was primarily driven by a \$1.6 million increase due to higher volume from commercial sales and product provided under our patient assistance programs, a \$8.4 million increase due to higher volume of product supplied to Sobi, a \$4.0 million increase in royalty expense, a \$17.9 million increase in expenses incurred related to excess, obsolete or scrapped inventory, and a \$6.4 million expense incurred in connection with the termination of the minimum purchase obligation of PEG in September 2024.

In addition, prior to receiving FDA approval for EMPAVELI and SYFOVRE, the costs associated with the manufacturing of EMPAVELI and SYFOVRE inventory were expensed as incurred as research and development expense. This did not materially impact cost of sales for the nine months ended September 30, 2024 and 2023. As of September 30, 2024, the remaining pre-FDA approved inventory was \$15.4 million, which primarily consisted of raw materials.

[Table of Contents](#)

*Research and Development Expenses*

The following table summarizes our research and development expenses incurred during the nine months ended September 30, 2024 and 2023 (in thousands, except percentages):

(In thousands)	For the Nine Months Ended September 30,		Change \$	Change %
	2024	2023		
<b>Program-specific external costs:</b>				
PNH	\$ 13,412	\$ 13,289	\$ 123	1 %
IC-MPGN & C3G	26,431	24,227	2,204	9 %
ALS	832	8,508	(7,676)	(90 %)
CAD	19,348	4,821	14,527	301 %
HSCT-TMA	2,024	2,229	(205)	(9 %)
GA	35,801	40,041	(4,240)	(11 %)
Other development and discovery programs	39,840	40,963	(1,123)	(3 %)
<b>Total program-specific costs</b>	<b>137,688</b>	<b>134,078</b>	<b>3,610</b>	<b>3 %</b>
<b>Unallocated external costs</b>				
Non-program specific external costs	12,967	15,211	(2,244)	(15 %)
<b>Total unallocated external costs</b>	<b>12,967</b>	<b>15,211</b>	<b>(2,244)</b>	<b>(15 %)</b>
<b>Unallocated internal costs</b>				
Compensation and related personnel costs	96,720	131,647	(34,927)	(27 %)
Other expenses	3,841	4,169	(328)	(8 %)
<b>Total unallocated internal costs</b>	<b>100,561</b>	<b>135,816</b>	<b>(35,255)</b>	<b>(26 %)</b>
<b>Total research and development costs</b>	<b>\$ 251,216</b>	<b>\$ 285,105</b>	<b>\$ (33,889)</b>	<b>(12 %)</b>

Research and development expenses decreased by \$33.9 million to \$251.2 million for the nine months ended September 30, 2024 from \$285.1 million for the nine months ended September 30, 2023, a decrease of 12%. The decrease in research and development expenses was primarily attributable to a \$34.9 million decrease in compensation and related personnel costs and \$2.2 million decrease in non-program specific external costs, which was partially offset by a \$3.6 million increase in program specific external costs.

The increase in our program-specific external costs of \$3.6 million was driven by an increase of \$2.2 million related to IC-MPGN and C3G costs, and an increase of \$15.0 million as a result of a one-time expense related to the discontinuation of the CAD program. These increases were partially offset by a \$4.2 million decrease in GA costs which largely reflects the impact of the approval of SYFOVRE in February 2023, a \$7.7 million decrease in ALS costs due to the discontinuation of the Phase 2 MERIDIAN study, and a \$1.1 million decrease in other development and discovery program costs.

The decrease in compensation and related personnel costs of \$34.9 million was driven by a \$32.2 million decrease in salaries and benefits due to lower headcount compared to the prior year and reclassification of certain employee costs to selling, general and administrative expenses and a \$2.7 million decrease in stock compensation expense associated with the grant of stock options and restricted stock units to employees.

*Selling, General and Administrative Expenses*

Selling, general and administrative expenses increased by \$20.5 million to \$379.6 million for the nine months ended September 30, 2024, from \$359.1 million for the nine months ended September 30, 2023, an increase of 6%. The increase was primarily attributable to an increase in personnel related costs of \$7.1 million, an increase in general commercial activities of \$11.5 million, higher office costs of \$3.1 million, \$0.7 million in factoring fees and an increase of \$1.1 million in travel expenses, which were partially offset by a decrease of 2.5 million in professional and consulting fees and a decrease of \$0.4 million in insurance expenses. The increase in personnel related costs of \$7.1 million consisted of a \$10.8 million increase related to stock compensation expense associated with the grant of stock options and restricted stock units to employees, partially offset by a decrease of \$0.9 million in recruiting expenses and a \$2.8 million decrease in salaries and benefits.

*Loss on extinguishment of development liability*

We paid our remaining obligations under the SFJ agreement in full in May 2024. We concluded that the development liability was extinguished as of the payoff date. The difference of \$1.9 million between the reacquisition price of \$326.5 million and the net carrying value of the development liability of \$324.6 million was recorded as a loss on the extinguishment of the development liability as of September 30, 2024.

## [Table of Contents](#)

### *Interest Income*

Interest income was \$9.4 million for the nine months ended September 30, 2024, a decrease of \$7.0 million compared to \$16.4 million for the nine months ended September 30, 2023. The decrease in interest income was primarily attributable to decreased investments during the nine months ended September 30, 2024.

### *Interest Expense*

Interest expense was \$28.9 million for the nine months ended September 30, 2024, an increase of \$6.7 million compared to \$22.2 million for the nine months ended September 30, 2023. The increase is primarily due to the interest incurred under the Credit Facility and was partially offset by a decrease in the balance of the development liability.

### *Other (Expense)/Income, Net*

Other expense was \$0.4 million for the nine months ended September 30, 2024, an increase of \$0.5 million compared to \$0.9 million for the nine months ended September 30, 2023. The increase was primarily due to foreign currency revaluation losses.

### *Income Tax Expense*

Income tax expense was \$0.9 million for the nine months ended September 30, 2024, an increase of 0.2 million compared to \$0.7 million for the nine months ended September 30, 2023. The increase primarily pertained to an increase in foreign and state taxes.

## **Liquidity and Capital Resources**

### *Sources of Liquidity*

To date, we have financed our operations primarily through approximately \$2.6 billion in net proceeds from public and private offerings of our common stock and convertible securities, \$397.0 million in payments and royalties from Sobi pursuant to our collaboration agreement, \$532.5 million under various credit arrangements, including with Sixth Street and SFJ, and \$98.8 million relating to the unwinding of the capped call transactions in March 2024, as well as from the proceeds of our operations.

In May 2024, we entered into the Sixth Street Financing Agreement, which provides for the Credit Facility, consisting of an initial draw of \$375.0 million at closing and a potential additional \$100.0 million draw at our option upon satisfaction of a \$50.0 million minimum cash requirement and a requirement that our trailing three-month sales of SYFOVRE is at least \$180.0 million prior to the \$100.0 million draw. The Credit Facility matures on May 13, 2030 and bears interest at an annual rate equal to the 3-month Secured Overnight Financing Rate (SOFR) + 5.75% (subject to 1.00% floor). Certain additional commitment and undrawn amount fees are also payable in connection with the Credit Facility. We used the majority of the proceeds of the \$375.0 million draw at closing to buy out our remaining obligations owed to SFJ, in the amount of approximately \$326.5 million.

We are permitted under the Sixth Street Financing Agreement to enter into a separate asset-based financing arrangement with a third party in an amount of up to \$100.0 million, which amount is increased to \$200.0 million upon certain sales or market capitalization thresholds, and to have outstanding convertible unsecured notes in an amount equal to the greater of \$400.0 million and 10% of our market capitalization, but not to exceed \$600.0 million.

In August 2024, the Company entered into the Factoring Agreement with Citi to sell certain accounts receivable to Citi at a discount to the face value of the accounts receivable. Under the Factoring Agreement, the maximum amount of outstanding accounts receivables sold at any time is \$100.0 million.

In November 2023, we entered into a sales agreement, or the sales agreement, with Cowen and Company, LLC, or Cowen, as agent, pursuant to which we may offer and sell shares of our common stock having an aggregate offering from of up to \$300.0 million from time to time. Any sales made under the sales agreement will be made at market prices by any method that is deemed to be an “at the market offering” as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933. Any sales under the sales agreement will be made pursuant to our registration statement on Form S-3, which became effective on February 22, 2023. We agreed to pay Cowen compensation of up to 3.0% of the gross proceeds of the sale of shares made under the sales agreement. We did not make any sales under the sales agreement during the nine months ended September 30, 2024.

In February 2023, we issued and sold 4,007,936 shares of our common stock and, in lieu of common stock to investors who so chose, pre-funded warrants to purchase 2,380,956 shares of our common stock in a follow-on offering, including 833,333 shares sold pursuant to the underwriters’ exercise in full of their option to purchase additional shares of common stock. The price to the public of the shares of common stock was \$63.00 per share and the price to the public of the pre-funded warrants was \$62.9999 per pre-funded warrant. The

[Table of Contents](#)

pre-funded warrants have an exercise price equal to \$0.0001 per share and do not expire. The pre-funded warrants were accounted for as equity instruments. We received total net proceeds of \$384.4 million, after deducting underwriting discounts and commissions of \$18.8 million and offering cost of \$0.3 million.

In February 2024, we entered into agreements with the capped call counterparties to unwind a portion of the capped call transactions. The unwind agreements applied to the portion of the capped call transactions in a notional amount corresponding to the \$426.1 million principal amount of Convertible Notes that we held in treasury as of December 31, 2023 or have been previously converted. The unwind transactions were settled at volume-weighted average price per share of \$64.11, which resulted in cash proceeds to us of \$98.8 million. As of September 30, 2024, the remaining capped call transactions had a notional amount corresponding to \$93.9 million principal amount of Convertible Notes.

### Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2024 and 2023 (in thousands):

	For the Nine Months Ended September 30,	
	2024	2023
Net cash used in operating activities	\$ (107,226)	\$ (496,860)
Net cash used in investing activities	(383)	(678)
Net cash provided by financing activities	153,241	398,412
Effect of exchange rate changes on cash, cash equivalents and restricted cash	306	(449)
Net increase (decrease) in cash, cash equivalents and restricted cash	\$ 45,938	\$ (99,575)

#### Net Cash Used in Operating Activities

Net cash used in operating activities was \$107.2 million for the nine months ended September 30, 2024 and consisted primarily of a net loss of \$161.5 million adjusted for \$101.0 million of non-cash items, including share-based compensation expense of \$87.8 million, depreciation expense of \$1.4 million, loss on extinguishment of development liability of \$1.9 million and accretion of discount to the development liability of \$8.9 million. Further, it included a net increase in operating assets and liabilities of \$46.7 million, which was driven by an increase in accounts receivable of \$72.6 million, an increase in inventory of \$20.8 million, a decrease in prepaid assets of \$15.2 million, a decrease in other current assets of \$10.7 million, a decrease in accounts payable of \$0.4 million, an increase in accrued expenses of \$19.5 million, and an increase in deferred revenue of \$1.9 million. The increase in accounts receivable was reduced by the transfer of \$56.6 million of accounts receivable during the three months ended September 30, 2024 to CITIBANK N.A. ("Citi") under the factoring agreement entered into on August 5, 2024.

Net cash used in operating activities was \$496.9 million for nine months ended September 30, 2023 and consisted primarily of a net loss of \$440.1 million adjusted for \$100.8 million of non-cash items, including share-based compensation expense of \$79.7 million, depreciation expense of \$1.3 million, accretion of discount to the development liability of \$19.5 million. Further, it includes a net increase in operating assets of \$161.3 million, which included an increase in accounts receivable of \$161.5 million, a decrease in accounts payable of \$19.5 million and an increase in accrued expenses of \$23.1 million.

#### Net Cash Used in Investing Activities

Net cash used in investing activities during the nine months ended September 30, 2024 was \$0.4 million due primarily to purchases of fixed assets.

Net cash used in investing activities during the nine months ended September 30, 2023 was \$0.7 million due primarily to purchases of fixed assets.

#### Net Cash Provided by Financing Activities

Net cash provided by financing activities was \$153.2 million during the nine months ended September 30, 2024 and consisted primarily of net proceeds from the initial draw of the Credit Facility of \$363.9 million, the settlement of capped call unwind transactions of \$98.8 million, \$14.0 million of proceeds from the exercise of stock options, of \$3.2 million proceeds from the issuance of our common stock under the employee stock purchase plan partially offset by repayment of \$326.5 million for the development liability.

## [Table of Contents](#)

Net cash provided by financing activities was \$398.4 million during the nine months ended September 30, 2023 and consisted primarily of proceeds from the follow-on common stock and pre-funded warrant offering in March 2023 of \$384.4 million, \$45.9 million proceeds upon the exercise of stock options and \$3.7 million proceeds from the issuance of our common stock under the employee stock purchase plan partially offset by payments of \$24.5 million for the development liability as well as the payments of employee tax withholding related to equity-based compensation of \$11.1 million.

### ***Funding Requirements***

We expect to continue incur expenses to support our ongoing commercial activities related to product manufacturing, marketing, sales and distribution of EMPAVELI for PNH and SYFOVRE for GA. In addition, we expect to continue to incur expenses as we prioritize the ongoing development of systemic pegcetacoplan and focus our research initiatives on high potential opportunities.

Together with the cash that we anticipate will be generated from sales of EMPAVELI and SYFOVRE, we expect that our current cash and cash equivalents will be sufficient to fund our projected operating expenses and capital expenditure requirements for at least the next 12 months, as well as our anticipated longer-term cash requirements and obligations. Our expectations regarding our short-term and long-term funding requirements are based on assumptions that may prove to be wrong, and we may need additional capital resources to fund our operating plans and capital expenditure requirements.

We are devoting substantial resources to the commercial infrastructure for SYFOVRE for GA. We are also devoting substantial resources to the development of our product candidates. Because of the numerous risks and uncertainties associated with the commercialization of EMPAVELI and SYFOVRE and development of other product candidates, and because the extent to which we may enter into collaborations with third parties for any of these activities is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with the research, development and commercialization. Our future funding requirements and long-term capital requirements will depend on many factors, including:

- our ability to continue to successfully commercialize and sell EMPAVELI and SYFOVRE in the United States;
- the cost of and our ability to obtain regulatory approvals of SYFOVRE outside of the United States and continue to build a commercial infrastructure for SYFOVRE for GA in the United States and worldwide;
- the cost of and our ability to effectively establish and maintain, the commercial infrastructure and manufacturing capabilities required to support the continued commercialization of EMPAVELI, SYFOVRE and any other products for which we receive marketing approval including product sales, medical affairs, marketing, manufacturing and distribution;
- the scope, progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for systemic pegcetacoplan, SYFOVRE and our other product candidates;
- our ability to maintain a productive collaborative relationship with Sobi with respect to systemic pegcetacoplan, including our ability to achieve milestone payments under our agreement with Sobi;
- our ability to identify additional collaborators for any of our product candidates and the terms and timing of any collaboration agreement that we may establish for the development and any commercialization of such product candidates;
- the number and characteristics of future product candidates that we pursue and their development requirements;
- the outcome, timing and costs of clinical trials and of seeking regulatory approvals of pegcetacoplan in other jurisdictions and indications and other product candidates we may pursue;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of pegcetacoplan in other jurisdictions and indications and our other product candidates;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- the effect of competing technological and market developments;
- the effect of public health crises, including pandemics and epidemics, on the healthcare system and the economy generally and on our clinical trials and other operations specifically;

## [Table of Contents](#)

- our ability to obtain adequate reimbursement for EMPAVELI and SYFOVRE in the United States or any other product we commercialize; and
- the costs of operating as a public company.

If our cash and cash equivalents, and cash generated from sales of EMPAVELI and SYFOVRE are not sufficient to fund our planned expenditures, we will need to finance our cash needs through external sources of funds, which may include equity offerings, debt financings, collaborations, strategic alliances or licensing arrangements. We currently do not have any committed external source of funds.

If we are unable to generate sufficient funds from sales of EMPAVELI and SYFOVRE, or raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

### ***Contractual Obligations***

The disclosure of our contractual obligations and commitments is set forth under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources—Contractual Obligations” in our 2023 Annual Report on Form 10-K. See Note 13 Commitments and Contingencies in the Notes to Unaudited Condensed Consolidated Financial Statements in Part I, Item I of this Form 10-Q for a discussion of obligations and commitments.

### **Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

We are exposed to market risk related to changes in interest rates. As of September 30, 2024, we had cash and cash equivalents of \$396.9 million, consisting primarily of money market funds and U.S. Government obligations. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio. We have the ability to hold our marketable securities until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

### **Item 4. Controls and Procedures.**

#### **Limitations on Effectiveness of Controls and Procedures**

The term “disclosure controls and procedures,” as defined in Rules 13a-15(f) and 15d-15(e) under the Exchange Act of 1934 as amended, or the Exchange Act, refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Our disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to our management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

#### **Evaluation of Disclosure Controls and Procedures**

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures (as defined in Rules



[Table of Contents](#)

13a-15(e) and 15d-15(e) under the Exchange Act). Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of September 30, 2024.

**Changes in Internal Control Over Financial Reporting**

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2024 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## PART II—OTHER INFORMATION

### Item 1. Legal Proceedings

See Note 13 Commitments and Contingencies in the Notes to Unaudited Condensed Consolidated Financial Statements in Part I, Item I of this Form 10-Q.

### Item 1A. Risk Factors.

In addition to the other information set forth in this Quarterly Report on Form 10-Q, you should carefully consider the factors discussed in Part I, “Item 1A. Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2023 and in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2024, which could materially affect our business, financial condition or future results. The risk factors disclosure in our Annual Report on Form 10-K for the year ended December 31, 2023 and in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2024 are qualified by the information that is described in this Quarterly Report on Form 10-Q. The risks described below and in our Annual Report on Form 10-K for the year ended December 31, 2023 and in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2024 are not our only risks. Additional risks and uncertainties not currently known to us or that we currently deem to be immaterial also may materially adversely affect our business, financial condition or future results.

**Item 5. Other Information.**

None of our directors or officers adopted or terminated a Rule 10b5-1 trading arrangement or a “non-Rule 10b5-1 trading arrangement” (as defined in Item 408(c) of Regulation S-K) during the third quarter of 2024.

[Table of Contents](#)

**Item 6. Exhibits.**

<b>Exhibit Number</b>	<b>Description</b>
31.1*	<a href="#">Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>
31.2*	<a href="#">Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>
32.1*	<a href="#">Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</a>
32.2*	<a href="#">Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</a>
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL 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)

\* Filed herewith.

**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.

Apellis Pharmaceuticals, Inc.

Date: November 5, 2024

By: /s/ Cedric Francois  
Cedric Francois  
President and Chief Executive Officer  
(principal executive officer)

Date: November 5, 2024

By: /s/ Timothy Sullivan  
Timothy Sullivan  
Chief Financial Officer and Treasurer  
(principal financial officer)

**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO EXCHANGE ACT RULES 13a-14(a) AND 15d-14(a), AS ADOPTED PURSUANT TO SECTION 302 OF SARBANES-OXLEY ACT OF 2002**

I, Cedric Francois, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Apellis Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer 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 5, 2024

By: /s/ Cedric Francois  
Cedric Francois  
Chief Executive Officer

**CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A), AS ADOPTED PURSUANT TO SECTION 302 OF SARBANES-OXLEY ACT OF 2002**

I, Timothy Sullivan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Apellis Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) (Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer 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 5, 2024

By: /s/ Timothy Sullivan

Timothy Sullivan  
Chief Financial Officer and Treasurer

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

In connection with the Quarterly Report of Apellis Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending September 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Cedric Francois, President and Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 5, 2024

By: /s/ Cedric Francois

Cedric Francois

President and Chief Executive Officer

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

---



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

In connection with the Quarterly Report of Apellis Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending September 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Timothy Sullivan, Chief Financial Officer and Treasurer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 5, 2024

By: /s/ Timothy Sullivan

Timothy Sullivan

Chief Financial Officer and Treasurer

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

---

